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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-K

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

For the fiscal year ended December 31, 2022

☐ **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 001-36576

Marinus Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**20-0198082**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

5 Radnor Corporate Center, Suite 500
100 Matsonford Road
Radnor, PA 19087
(Address of principal executive offices including zip code)

(484) 801-4670
(Registrant's telephone number, including area code)

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

Title of Each Class	**Trading Symbol(s)**	**Name of Each Exchange on Which Registered**
Common Stock, par value $0.001 per share	MRNS	Nasdaq Global Market

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

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

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Exchange 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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

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

Emerging growth company ☐

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

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

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

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

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

The aggregate market value of the registrant's common stock (the only common equity of the registrant) held by non-affiliates of the registrant on the last business day of the registrant's most recent completed second fiscal quarter (June 30, 2022) was $179,039,833, based on the closing price reported on the Nasdaq Global Market on June 30, 2022.

The total number of shares of the registrant's common stock, par value $0.001 per share, outstanding as of March 3, 2023 was 49,665,117.

Documents Incorporated by Reference

Certain portions of the registrant's Definitive Proxy Statement for its 2023 Annual Meeting of the Stockholders, which is expected to be filed with the U.S. Securities and Exchange Commission within 120 days after the end of the registrant's fiscal year ended December 31, 2022, are incorporated by reference into Part III, Items 10-14 of this Annual Report on Form 10-K.

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Cautionary Note Regarding Forward-Looking Statements.

This Annual Report on Form 10-K contains forward-looking statements, within the meaning of the U.S. Private Securities Litigation Reform Act of 1995, that involve substantial risks and uncertainties. In some cases, you can identify forward-looking statements by the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," "will," or "would," and or the negative of these terms, or other comparable terminology intended to identify statements about the future. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Annual Report on Form 10-K, we caution you that these statements are based on a combination of facts and factors currently known by us and our expectations of the future, about which we cannot be certain.

The forward-looking statements in this Annual Report on Form 10-K include, among other things, statements about:

- our plans to successfully commercialize ganaxolone in Cyclin-dependent Kinase-like 5 Deficiency Disorder (CDD) in the U.S.;
- our plans to meet our post-approval commitments to the U.S. Food and Drug Administration (FDA) for ganaxolone;
- our plans to achieve regulatory approval for ganaxolone in the European Union (EU), and the expected timing thereof;
- our ability to develop ganaxolone for additional indications, including Refractory Status Epilepticus (RSE), Established Status Epilepticus (ESE), Tuberous Sclerosis Complex (TSC) and Lennox Gastaut Syndrome (LGS);
- the status, timing and results of preclinical studies and clinical trials;
- the design of and enrollment in clinical trials, availability of data from ongoing clinical trials, expectations for regulatory approvals, or the attainment of clinical trial results that will be supportive of regulatory approvals;
- the potential benefits of ganaxolone, including in indications other than CDD;
- the timing of seeking marketing approval of ganaxolone in specific additional indications;
- our ability to maintain marketing approval for ganaxolone in CDD and obtain regulatory approval for ganaxolone in other indications;
- the possibility that we expand the targeted indication footprint and explore new potential formulations of ganaxolone;
- our estimates of expenses and future revenue and profitability;
- our estimates regarding our capital requirements and our needs for additional financing;
- our estimates of the size of the potential markets for ganaxolone;
- our expectations regarding our collaborations with Orion Corporation (Orion), Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia) and NovaMedica LLC (NovaMedica), including the expected amounts and

timings of milestone, royalty and other payments, including research and development reimbursement if applicable, pursuant thereto;

- our ability to attract collaborators with acceptable development, regulatory and commercial expertise;
- the benefits and contractual requirements derived from corporate collaborations, license agreements, and other collaborative or acquisition efforts, including those relating to the development and commercialization of ganaxolone;
- sources of revenue, including expected future sales of ganaxolone in CDD, revenue contributions from our contract (BARDA Contract) with the Biomedical Advanced Research and Development Authority (BARDA), corporate collaborations, license agreements, and other collaborative efforts for the development and commercialization of ganaxolone for CDD and in other indications being developed for ganaxolone;
- our eligibility to receive funding under the remaining debt tranche available under the Credit Agreement with Oaktree;
- our ability to create an effective sales and marketing infrastructure where we elect to market and sell ganaxolone directly;
- the timing and amount of reimbursement for ganaxolone;
- the success of other competing therapies that may become available;
- the manufacturing capacity and supply for ganaxolone;
- the possibility that third parties, such as Ovid Therapeutics, Inc. (Ovid), may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business;
- the possibility that we expand and diversify our product pipeline through acquisitions of additional drug candidates that fit our business strategy;
- our belief that our existing cash and cash equivalents will be sufficient to fund our operating expenses and capital expenditure requirements, as well as maintain the minimum cash balance required under our debt facility, into the second half of 2024;
- our ability to maintain and protect our intellectual property rights;
- our results of operations, financial condition, liquidity, prospects, and growth strategies;
- our ability to, among other actions, secure additional financing or strategic transactions and continue as a going concern;
- the extent to which our business may be adversely impacted by the effects of the COVID-19 coronavirus pandemic or by other pandemics, epidemics or outbreaks;
- the enforceability of the exclusive forum provisions in our fourth amended and restated certificate of incorporation; and
- the industry in which we operate and trends which may affect the industry or us.

Forward-looking statements appear primarily in the sections of this Annual Report on Form 10-K entitled “Item 1 – Business,” “Item 1A “Risk Factors,” “Item 7 – Management’s Discussion and Analysis of Financial Condition and Results of Operations,” “Item 7A – Quantitative and Qualitative Disclosures About Market Risk,” and “Item 8 – Financial Statements and Supplementary Data.” Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame or at all. We undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

You should read this Annual Report on Form 10-K and the documents that we reference in this Annual Report on Form 10-K and have filed as exhibits to this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

Risk Factors Summary

The following summarizes the principal factors that make an investment in us speculative or risky, all of which are more fully described in "Item 1A – Risk Factors" of this Annual Report on Form 10-K. This summary should be read in conjunction with the Risk Factors section and should not be relied upon as an exhaustive summary of the material risks facing our business.

Risks Related to our Financial Position and Need for Additional Capital

- We may never achieve or sustain profitability, which could depress the market price of our common stock, and could cause you to lose all or a part of your investment.
- We will require additional capital to fund our operations and if we fail to obtain necessary financing, we may be unable to successfully commercialize ZTALMY or complete the development and commercialization, if approved, of ganaxolone in the other indications we are developing.
- Our failure to comply with the covenants or other terms of the Credit Agreement or Revenue Interest Financing Agreement, including as a result of events beyond our control, could result in a default under these agreements that could materially and adversely affect the ongoing viability of our business.
- If we are unable to satisfy certain conditions in our Credit Agreement, we will be unable to draw down the remaining amount of the term loan facility.
- Our Credit Agreement and Revenue Interest Financing Agreement contains restrictions that limit our flexibility in operating our business.
- Raising additional capital could dilute our stockholders, restrict our operations or require us to relinquish rights to ganaxolone or any other future product candidates.
- We intend to expend our limited resources to pursue ganaxolone and may fail to capitalize on other technologies or any other future product candidates that may be more profitable or for which there may be a greater likelihood of success.

Risks Related to the Commercialization of ZTALMY and Other Future Product Candidates

- ZTALMY is our first commercial product and we have a limited history of commercializing drugs, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.
- Even though we have obtained regulatory approval for ZTALMY in the U.S., we will still face extensive FDA regulatory requirements and may face regulatory difficulties.
- Our commercial success depends upon attaining significant market access and acceptance of ZTALMY among physicians, patients, government and private payers and others in the medical community and attaining sufficient reimbursement for ganaxolone.
- We operate in a competitive market, which may result in others discovering, developing or commercializing products before or more successfully than we do.
- If we are unable to differentiate ZTALMY from current and future products or existing methods of treatments, our ability to successfully commercialize ZTALMY would be adversely affected.
- ZTALMY is our first commercial product. If our sales and marketing capabilities to market and sell ganaxolone are not effective, we may be unable to generate meaningful revenue.
- While ZTALMY has received favorable reimbursement determinations to date from third party payers for its approved indication, adverse changes in reimbursement or failure to obtain favorable reimbursement for future indications, if approved, could harm our business.
- If the market opportunities for ZTALMY in CDD and other indications for which we obtain regulatory approval, if any, are smaller than we believe they are, our results of operations may be adversely affected and our business may suffer.
- If the FDA or other applicable regulatory authorities approve generic or other products that compete with any of our products or product candidates, it could reduce our sales of those products or product candidates.

Risks Related to Clinical Development and Regulatory Approval of our Product Candidates

- Failure to obtain regulatory approval in international jurisdictions would prevent ganaxolone from being marketed in these jurisdictions.
- Our future success is dependent on the successful clinical development, regulatory approval and commercialization of ganaxolone, which is being studied in several indications and will require significant capital resources and years of additional clinical development effort.

- We are conducting clinical development activities for ganaxolone across multiple indications, and such clinical development activities may not produce favorable results, which could adversely impact our ability to achieve regulatory approval for ganaxolone in such indications.
- Ganaxolone may cause undesirable side effects, or have other properties, such as abuse potential, that could delay or prevent its regulatory approval or result in significant negative consequences following any marketing approval.
- The therapeutic efficacy and safety of ganaxolone in indications other than CDD have not been established by regulatory authorities, and we may not be able to successfully develop and commercialize ganaxolone in the other indications under clinical development in the future.
- We may not be able to obtain or maintain orphan drug exclusivity for ganaxolone across all indications and markets, which could limit the potential profitability of ganaxolone.
- ZTALMY is regulated as a controlled substance, which is subject to significant regulation by the Drug Enforcement Administration (DEA) and other regulatory agencies.

Risks Related to Our Dependence on Third Parties

- We rely on third parties to conduct our preclinical studies and clinical trials. If these third parties do not successfully carry out their duties in compliance with contractual terms and/or regulatory requirements or meet expected timelines, our development plans may be adversely affected and we may not be able to obtain regulatory approval for ganaxolone in indications other than CDD.
- We have multiple ganaxolone drug products in development, and until such products are approved by regulatory authorities, there remains the risk that the drug product quality requirements may not support continued clinical investigation and result in delays or termination of such clinical studies, and product approvals.
- Our experience manufacturing ganaxolone has historically consisted of supplying the needs of our preclinical studies and clinical trials. We have limited experience manufacturing ganaxolone on a commercial scale and do not operate our own manufacturing facility. We are dependent on third-party manufacturers for the manufacture of ganaxolone drug substance and drug products as well as on third parties for our supply chain, and if we experience problems with any such third parties, the manufacturing and supply of ganaxolone could be delayed.
- We have entered into and may enter into additional collaboration or out-license agreements with third parties for the development or commercialization of ganaxolone in jurisdictions outside of the U.S. (OUS). If these collaborations or out-licenses are not successful, we may not be able to capitalize on the market potential of ganaxolone.
- Government funding for certain aspects of our programs adds uncertainty to our research efforts with respect to those programs and may impose requirements that increase the costs of commercialization and production of certain product candidates developed under those government-funded programs.
- We may elect to enter into license or collaboration agreements to partner ganaxolone in territories currently retained by us. Our dependence on such relationships may adversely affect our business.

Risks Related to Regulatory Compliance

- Currently enacted and future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain marketing approval of and commercialize ganaxolone and affect the prices we may obtain.

Risks Related to Intellectual Property

- If we are unable to protect our intellectual property rights or if our intellectual property rights are inadequate for our technology and product candidates, our competitive position could be harmed.
- Third parties, such as Ovid Therapeutics, Inc., may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.
- We may not be able to protect our intellectual property rights throughout the world.
- Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time.
- We rely on government funding for certain aspects of our research and development activities and we may develop intellectual property through such activities and therefore may be subject to federal regulations such as “march-in” rights, certain reporting requirements and a preference for U.S. based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.

Risks Related to our Business Operations

- The COVID-19 pandemic could continue to adversely affect our business and our ability to conduct and complete clinical trials.

PART I

Unless the context requires otherwise, any references in this Annual Report on Form 10-K to "we," "us," "our," the "Company" or "Marinus" refers to Marinus Pharmaceuticals, Inc. and its wholly-owned subsidiary. Unless otherwise indicated, all share and per share amounts in this Annual Report on Form 10-K reflect, as applicable, the occurrence of a 1-for-4 reverse split of our common stock that occurred on September 23, 2020.

Item 1. Business.

Overview

Our Company

We are a commercial-stage pharmaceutical company dedicated to the development of innovative therapeutics for the treatment of seizure disorders, including rare genetic epilepsies and status epilepticus. On March 18, 2022, the U.S. Food and Drug Administration (FDA) approved our new drug application (NDA) for the use of ZTALMY (ganaxolone) oral suspension for the treatment of seizures associated with Cyclin-dependent Kinase-like 5 Deficiency Disorder (CDD) in patients 2 years of age and older. In June 2022, the U.S. Drug Enforcement Administration (DEA) published an interim final rule in the Federal Register placing ganaxolone and its salts in schedule V (CV) of the Controlled Substances Act (CSA), which rule became final on December 9, 2022. ZTALMY, our first FDA approved product, became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022. We plan to develop ganaxolone for the treatment of other rare genetic epilepsies, including Tuberous Sclerosis Complex (TSC), and for the treatment of status epilepticus (SE). We are developing ganaxolone in formulations for two different routes of administration: intravenous (IV) and oral. The different formulations are intended to maximize potential therapeutic applications of ganaxolone for adult and pediatric patient populations, in both acute and chronic care, and for both in-patient and self-administered settings. While the precise mechanism by which ganaxolone exerts its therapeutic effects in the treatment of seizures associated with CDD is unknown, its anticonvulsant effects are thought to result from positive allosteric modulation of the gamma-aminobutyric acid type A ($GABA_A$) receptor in the central nervous system (CNS). Ganaxolone is a synthetic analog of allopregnanolone, an endogenous neurosteroid. Ganaxolone acts at both synaptic and extrasynaptic $GABA_A$ receptors, a target known for its anti-seizure, antidepressant and anxiolytic potential.

Our Strategy

Our mission is to maximize the value of ganaxolone as a best-in-class therapy for rare seizure and neurological disorders through development of multiple formulations for oral and IV administration. The key elements of our strategy include the following:

- ***Pursuing orphan, genetic epilepsy indications for ganaxolone***. Within epilepsy, there are several disorders where the symptoms have been linked to deficits in GABAergic signaling. Based on our clinical data, we believe that increasing activation of GABA receptors and associated processes (GABAergic tone) with ganaxolone could provide therapeutic benefits and that treatments for these small populations have the potential for more efficient paths through clinical development, regulatory approval and commercialization. In addition to CDD and TSC, we may in the future develop ganaxolone in one or more additional indications for rare epilepsies. We may also seek to in-license complementary products to leverage our development and commercial investment.
- ***Pursuing hospital-based orphan indications for ganaxolone.*** We believe that hospitalized SE patients who do not respond to available first- and second-line treatments are significantly underserved with severely limited treatment options. Additionally, SE is associated with significant morbidity and mortality. Due to its activity at extrasynaptic $GABA_A$ receptors, ganaxolone may provide a therapeutic benefit for patients whose SE is refractory to currently available first- and second-line treatments. To that end, and based on our Phase 2 trial results, we are conducting the Randomized Therapy In Status Epilepticus trial (RAISE trial), a pivotal Phase 3 trial in refractory status epilepticus (RSE) patients. We are also conducting

the Researching Established Status Epileptics Treatment Trial, or the RESET trial, a Phase 2 trial in established status epileptics (ESE), and may in the future study similar and other hospital-based indications that could benefit from ganaxolone's mechanism of action.

- ***Reformulation and prodrug compounds.*** We intend to further develop the current oral formulation of ganaxolone through second-generation reformulation and prodrug compounds. A reformulation or prodrug of ganaxolone that increases bioavailability and improves the pharmacokinetics (PK) profile may create substantial indication expansion opportunities and have the potential to enhance efficacy by better achieving desired ganaxolone blood levels, improve the safety profile through a more consistent PK profile, reduce dosing frequency, generate new IP, and improve costs of goods through lower active pharmaceutical ingredient (API) requirements. Top-line data from a Phase 1 trial with healthy volunteers utilizing the first candidate for a second-generation formulation of ganaxolone were announced in the second quarter of 2022, including PK characteristics that may allow for twice-daily dosing. We believe that the data support further clinical development of this formulation of ganaxolone. An additional Phase 1 cohort assessing the PK of a second-generation oral formulation candidate has been completed which assessed higher doses of ganaxolone than in the initial phase 1 cohorts. A multiple ascending dose study will be initiated in the second quarter of 2023. This study will also incorporate food effect assessments**.** We plan to pursue the development of Lennox Gastaut Syndrome (LGS) as a lead indication for a reformulated oral form of ganaxolone, with a Phase 2 trial targeted to begin in the fourth quarter of 2023, and seek additional indications in epilepsy and potentially other therapeutic areas as activities progress.
- ***Building on our product pipeline.*** We may expand and diversify our product pipeline through acquisition of additional drug candidates that fit our business strategy.

COVID-19

The continued global spread of COVID-19 has impacted our clinical operations and timelines. For example, our RAISE trial is conducted in hospitals, primarily intensive care units in academic medical centers, which have experienced high rates of COVID-19 admissions. Several of these sites participating in the RAISE trial have experienced COVID-related difficulties, including staff turnover and the need to devote significant resources to patients with COVID-19, which has resulted in site initiation and enrollment delays for the RAISE trial. Given these COVID-19-related challenges and the interruption in drug supply in mid-2022, we previously adjusted our expectation for our top-line data readout for the RAISE trial to the second half of 2023. In May 2022, we resumed screening and recruitment for the RAISE trial. Several of the sites participating in the RAISE trial continue to encounter COVID-related setbacks, including staff turnover and the need to devote significant resources for patients with COVID-19. In addition, our ganaxolone clinical trials in the outpatient setting may be negatively impacted if patients and their caregivers do not want to participate while the COVID-19 pandemic persists. The duration and severity of the pandemic and its long-term impact on our business are uncertain at this time.

Our Products and Product Candidates

ZTALMY® (ganaxolone) oral suspension CV

ZTALMY is an oral suspension given three times per day that we have developed for the treatment of CDD. ZTALMY was approved by the FDA in March 2022 for the treatment of seizures associated with CDD in patients 2 years of age and older. In June 2022, the DEA published an interim final rule in the Federal Register placing ganaxolone and its salts in schedule V of the CSA, which rule became final on December 9, 2022. ZTALMY, our first FDA approved product, became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022. We recorded net U.S. product revenue related to ZTALMY of $2.9 million for the year ended December 31, 2022.

CDD is a serious and rare genetic disorder that is caused by a mutation of the CDKL5 gene, located on the X chromosome. CDD is a severely debilitating and potentially fatal genetic condition, which occurs with an estimated frequency of 1:40,000 live births in the U.S. It predominantly affects females and is characterized by early onset, difficult to control seizures and severe neurodevelopmental impairment. The CDKL5 gene encodes proteins essential for

normal brain function. Most children affected by CDD have neurodevelopmental deficits such as difficulty walking, talking and taking care of themselves. Many also suffer from scoliosis, gastrointestinal dysfunction or sleep disorders. Genetic testing is available to determine if a patient has a mutation in the CDKL5 gene.

In June 2017, we were granted FDA orphan drug designation for ganaxolone for the treatment of CDD. The designation provides the drug developer with a seven-year period of U.S. marketing exclusivity, as well as tax credits for clinical research costs, the ability to apply for annual grant funding, clinical research trial design assistance and waiver of Prescription Drug User Fee Act filing fees. Additionally, in November 2019, the European Medical Agency's (EMA) Committee for Orphan Medicinal Products (COMP) granted orphan drug designation for ganaxolone for the treatment of CDD. In July 2020, the FDA granted Rare Pediatric Disease Designation (RPD Designation) for ganaxolone for the treatment of CDD. The FDA grants RPD Designation for diseases that affect fewer than 200,000 people in the U.S. and in which the serious or life-threatening manifestations occur primarily in individuals 18 years of age and younger. The approval of ZTALMY in CDD is based on data from the Phase 3 Marigold double-blind placebo-controlled trial, in which 101 patients were randomized and treated with ZTALMY. Patients showed a median 30.7% reduction in 28-day major motor seizure frequency, compared to a median 6.9% reduction for those receiving placebo, achieving the trial's primary endpoint (p=0.0036). In the Marigold open label extension study, patients treated with ZTALMY for at least 12 months (n=48) experienced a median 49.6% reduction in major motor seizure frequency. On October 13, 2022, we presented two posters at the Child Neurology Society Meeting from our Phase 3 Marigold clinical trial of ZTALMY, including open label extension data showing continued seizure reduction over a two-year period. In the Marigold open-label extension, patients on ganaxolone at 2 years (n=50) showed a median 48.2% reduction in major motor seizure frequency suggesting that ganaxolone may provide sustained efficacy for the treatment of seizures associated with CDD. The discontinuation rate was about 30% during the first year of the open label phase but declined to about 10% during the second year. These data in total suggest that patients who remain on treatment long-term may demonstrate continued reductions in seizure frequency. In the clinical development program, ZTALMY demonstrated efficacy, safety and tolerability with the most common adverse reactions (AEs) (incidence >5% and at least twice the rate of placebo) in the ZTALMY group being somnolence, pyrexia, salivary hypersecretion, and seasonal allergy.

We own families of patents and pending patent applications that claim certain formulations of ganaxolone and cover certain therapeutic uses of ganaxolone, including for treating CDD. The 20-year terms for patents, and applications that issue as patents, in these families run from 2026 through 2042, absent any available patent term adjustments or extensions. We have also licensed from Ovid certain patents that claim certain therapeutic uses of ganaxolone for the treatment of CDD. The licensed patents include a granted U.S. patent, and pending applications in the U.S. and Europe. The 20-year term for these licensed patents and applications that issue as patents will run through 2037, absent any available patent term adjustments.

Priority Review Voucher. As a result of the RPD Designation for ganaxolone for the treatment of CDD, the FDA awarded us a Rare Pediatric Disease Priority Review Voucher (PRV) on March 18, 2022 in connection with the approval of the use of ZTALMY in CDD. On July 13, 2022, we entered into an asset purchase agreement (PRV Asset Purchase Agreement) with Novo Nordisk Inc., pursuant to which we agreed to sell the PRV to Novo Nordisk, Inc. for $110.0 million, payable in cash, upon the closing of the transaction. In August 2022, the transaction closed and we received $110.0 million from Novo Nordisk, Inc.

Commercial Strategy. Since ZTALMY was approved by FDA, we have been focused on the implementation and execution of an integrated launch plan to make ZTALMY available to CDD patients through a specialty pharmacy. Key launch strategies have included and continue to include: (1) establishing our supply chain network and quality management system to assure product is available to patients; (2) driving clinical awareness of ZTALMY as the first and only FDA approved product indicated specifically for seizures associated with CDD; (3) deploying our field sales force to target physicians who treat this rare pediatric patient population; (4) engaging commercial and government payers with the objective of obtaining insurance coverage; and (5) developing our internal capabilities (such as Finance, Human Resources, Information Technology, Data Analytics and Compliance) to support our first launch as a commercial company.

Marketing Strategy. Our marketing strategy is to reinforce that seizures are central to the constellation of CDD symptoms, establish ZTALMY as central to the comprehensive management of seizures associated with CDD, and

ensure that patients have seamless access to ZTALMY from prescription through fulfillment. Our marketing campaign for ZTALMY is active, and our integrated commercial launch activities initiated in the third quarter of 2022.

Sales Strategy. Our commercial sales force includes 16 regional account managers experienced in rare disease. Our field force is targeting identified key accounts and centers of excellence for CDD. Based on our market research, we estimate the addressable patient population for ZTALMY in CDD in the U.S. is approximately 2,000 patients. For the year ended December 31, 2022, we received over 90 CDD prescription enrollment forms, of which more than 70 were for new commercial patients not previously treated with ZTALMY. As this is the first product approved by the FDA specifically for seizures associated with CDD and the International Classification of Diseases, Tenth Revision (ICD10) code for CDD was established in 2020, there is limited data available for this specific market. We have strengthened both our market access and field force teams, and both payer and customer engagement are underway.

Market Access. We have established a cross-functional payer and reimbursement account team with the objective of obtaining and maintaining reimbursement (coverage) of ZTALMY. We are focusing our efforts on reimbursement from commercial payers where pharmacy benefit managers (PBMs) control the majority of commercial pharmacy-benefit lives and government payers, primarily Medicaid for the target population for CDD. We expect approximately 60% of the CDD patient population will access coverage through both Fee-for-Service and Managed Medicaid, with the remaining 40% accessing commercial coverage, with the top PBMs having significant influence. Of the CDD patient enrollment forms received in the year ended December 31, 2022, over 75% received favorable reimbursement coverage and therapy in the year. The prescribing and fulfillment process for ZTALMY is managed through ZTALMY One™, a comprehensive patient support program. Enrollment in the program offers various support and information to help caregivers and patients prescribed ZTALMY access their ZTALMY prescription and assist in determining eligibility for and access to co-pay support or free drug programs.

Specialty Pharmacy. We are utilizing Orsini Pharmaceutical Services, LLC (Orsini), a specialty pharmacy, to provide services for patients, including patient enrollment, benefit verification and investigation, prior authorization support, patient education and drug counseling, dispensing of product and shipment coordination. We recorded our first sales of ZTALMY to Orsini in the third quarter of 2022.

Infrastructure. We continue to enhance our internal capabilities and processes to support a commercial stage company. We have implemented a healthcare compliance program to guide our compliance with rules and regulations regarding pharmaceutical sales.

Manufacture of Commercial Supply. We have executed commercial supply agreements for ganaxolone API with our current manufacturer and also with our current supplier for finished bulk drug product. Additionally, we have executed a master supply agreement with a second API supplier in the U.S. to undertake certain process development activities and subsequently to provide commercial supplies of API and/or API intermediates.

Regulated as a Controlled Substance. On June 1, 2022, the DEA published an interim final rule in the Federal Register placing ganaxolone and its salts in schedule V of the CSA, which rule became final December 9, 2022. Under the CSA, drugs are classified into five (5) distinct categories or schedules depending upon the drug's acceptable medical use and the drug's abuse or dependency potential. Schedule V is defined by the DEA as drugs with lower potential for abuse than schedule IV and consist of preparations containing limited quantities of certain narcotics. ZTALMY became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022. As a controlled substance, ganaxolone is subject to the applicable CSA requirements such as registration, security, recordkeeping and reporting, storage manufacturing, distribution, importation and other requirements.

Post Marketing Requirements. In connection with FDA approval of ZTALMY for CDD, we have several post marketing commitments. The Phase 1 renal impairment study commitment was completed and submitted to the FDA in May 2022. The Phase 1 hepatic impairment study and the thorough QTc study were completed and submitted to the FDA in December 2022. The remaining post-marketing requirements include: 2-year carcinogenicity studies of ganaxolone and the major human unconjugated plasma metabolite, M2, in rats; a 26-week carcinogenicity of ganaxolone in transgenic mice; a juvenile animal toxicity study of the major human unconjugated plasma metabolite, M2, in rats;

extractable/leachable study results on the container closure system; a CNS distribution study of the M47 metabolite in rats; and in vitro studies to assess the drug interaction potential of M47 metabolite. We expect to be able to complete these remaining required FDA studies within the requested FDA timeframe.

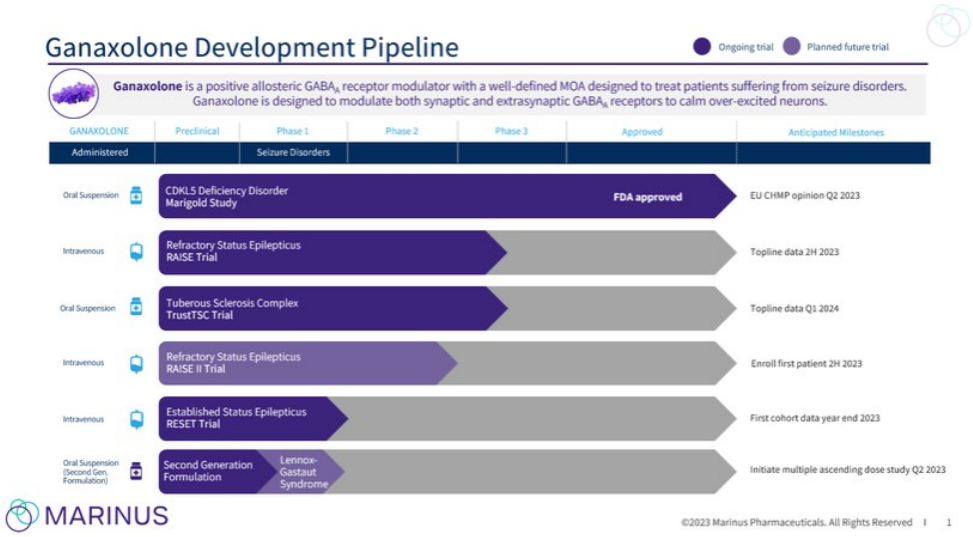
Marketing Authorization Application

In August 2021, the Committee for Medicinal Products for Human Use (CHMP) of the EMA granted our request for accelerated assessment of ganaxolone for the treatment of seizures associated with CDD. The marketing authorization application (MAA) for ganaxolone was submitted to the EMA on October 11, 2021 and on October 28, 2021 we received formal notification from the EMA that the CDD MAA was validated. With this validation, the EMA began its formal review of the MAA under the centralized procedure. In February 2022, the MAA was converted to a standard review timeline.

Further, the CHMP granted an extension to our Day 120 clock stop, and as a result, we submitted our Day 120 responses to the EMA on November 22, 2022. We received the Day 180 report, including a List of Outstanding Issues (LoOI) from the EMA on January 26, 2023. The LoOI contains a number of outstanding major objections and other concerns, including a major objection related to the choice of our regulatory starting material (RSM). The CHMP has indicated that the proposed RSM is not acceptable and should be redefined further upstream. We may not be able to timely address all of the objections to the CHMP's satisfaction. An initial 30-day clock stop extension was requested and granted to allow for time to respond to the issues raised by the CHMP. The CHMP is expected to present its opinion on the MAA in the second quarter of 2023. If outstanding issues remain unresolved, we or the CHMP may request an oral explanation, which may or may not be granted. Further delays in the review and approval process could occur if we are not able to timely or adequately respond to the CHMP's objections and concerns. If we were to ultimately receive a negative opinion on the MAA, we would have the opportunity to request a re-examination by the CHMP.

Our Pipeline

We are developing ganaxolone in indications where there is a mechanistic rationale for ganaxolone to provide a benefit, including the following indications:



Status Epilepticus (SE)

SE is a life-threatening condition characterized by continuous, prolonged seizures or rapidly recurring seizures without intervening recovery of consciousness. If SE is not treated urgently, permanent neuronal damage may occur, which contributes to high rates of morbidity and mortality. Patients with SE who do not respond to first-line benzodiazepine treatment are classified as having ESE and those who then progress to and then fail at least one second-line antiepileptic drug (AED) are classified as having RSE. In RSE, synaptic $GABA_A$ receptors are internalized into the neuron, resulting in decreased responsiveness to drugs such as benzodiazepines. RSE unresponsiveness to one or more second-line AEDs requires treatment with IV anesthesia to terminate seizures and prevent neuronal injury and other complications. The IV anesthetic is increased to a level that induces deep coma and is maintained at that rate for 24 hours or more. SE that recurs following an attempted wean of IV anesthesia is classified as super refractory status epilepticus (SRSE). In April 2016, we were granted FDA orphan drug designation for the IV formulation of ganaxolone for the treatment of SE, which includes RSE.

In January 2021, we enrolled the first patient in the Phase 3 pivotal RAISE trial. The RAISE trial is a randomized, double-blind, placebo-controlled clinical trial in patients with RSE. We expect approximately 80 trial sites in hospitals, primarily across the U.S. and Canada, to participate. The RAISE trial is designed to enroll approximately 124 patients, who will be randomized to receive ganaxolone or placebo added to standard of care. With this number of patients, the trial is designed to provide over 90% power to detect a 30% efficacy difference between ganaxolone and placebo.

The co-primary endpoints for the RAISE trial are (1) proportion of patients with RSE who experience seizure cessation within 30 minutes of treatment initiation without other medications for SE treatment, and (2) proportion of patients with no progression to IV anesthesia for 36 hours following initiation of the study drug. In June 2022, we announced that we amended the protocol for the RAISE trial to expand eligibility criteria to support recruitment. We broadened the inclusion criteria to permit patients previously treated with up to 18 hours of high-dose IV anesthesia to qualify for the trial, rather than excluding patients treated with anesthetics at high doses for any duration. We believe this will facilitate the enrollment of patients transferred to the ICU from other hospitals or the emergency room, who may already have received high doses of anesthetic medication for less than 18 hours. We reached alignment with the FDA on the protocol amendment, including a proposal for a potential interim analysis when two-thirds of the patients (approximately 82) have completed the trial.

Several academic medical centers and intensive care units participating in the RAISE trial have experienced COVID-related difficulties, including staff turnover and the need to devote significant resources to patients with COVID-19, which has resulted in site initiation and enrollment delays. Additionally, in February 2022, we temporarily paused the RAISE trial after routine monitoring of stability batches of clinical supply material indicated that it became necessary to reduce the shelf life to less than the anticipated 24 months to meet product stability testing specifications. We notified the FDA of this issue and our plans to proactively pause the trial, and we subsequently provided additional information to the FDA to support resuming trial activities. In May 2022, we announced that the trial had resumed utilizing new batches of the current IV formulation of ganaxolone, and we implemented a reduced shelf life of 12 months. In agreement with the FDA, ganaxolone clinical supplies will be stored under refrigerated conditions for the entire duration of clinical use. We manufactured the IV ganaxolone formulation with a new buffer and are targeting a shelf life of at least 24 months. The FDA agreed that in principle a buffer change in the ganaxolone IV formulation is acceptable.

We are working closely with key investigators and site coordinators to support enrollment efficiencies at existing RAISE trial sites and are also increasing the number of U.S. centers participating in the trial. Additionally, we plan to expand the trial to sites in Canada and Australia. Consistent with the prior announcement, we expect our top-line data readout for the RAISE trial to be available in the second half of 2023.

Planning continues for a separate Phase 3 RSE trial to support an MAA in Europe (RAISE II trial). We gained alignment on the trial design at a meeting with the EMA in the first quarter of 2021. Due to the delay in clinical trial supply mentioned for the RAISE trial, the RAISE II trial initiation is planned for the second half of 2023. RAISE II will be a double blind, placebo-controlled pivotal registration trial expected to enroll 70 patients who have failed first-line

benzodiazepine treatment and at least one second-line AED. Patients will receive either ganaxolone or placebo, administered in combination with a standard-of-care second-line AED. The simultaneous administration of a standard-of-care AED with the trial medication is expected to provide data complementary to that from the RAISE trial. There are two additional key differences between the RAISE and RAISE II trials. First, rather than specifying progression to IV anesthesia as a treatment failure, under the RAISE II protocol any escalation of care will constitute a treatment failure. This could be IV anesthesia or another second-line IV AED. Second, the primary analysis for the RAISE II trial will be a responder analysis, with response defined as SE cessation within 30 minutes and no escalation of care within 36 hours, rather than the co-primary endpoints in the RAISE trial, which require statistical significance to be achieved independently on both the 30-minute and 36-hour outcomes.

The FDA has indicated alignment on the overall trial design for a third SE trial, the RESET trial, a Phase 2 trial evaluating ganaxolone in the treatment of ESE, for which enrollment in the U.S. is expected to commence in the first half of 2023. The RESET trial will enroll patients with convulsive SE presenting to emergency departments, and will be conducted under Exception from Informed Consent (EFIC) guidelines. The RESET trial will consist of two phases: an initial open-label, dose optimization phase and a subsequent double-blind placebo-controlled phase. In the open-label portion of the trial, sequential cohorts will receive IV ganaxolone for varying durations and at different doses. The dosing for each cohort will depend on the treatment effect and tolerability seen in the previous one, with the expected optimal dose and duration of ganaxolone incorporated in the double-blind phase of the trial to follow. We expect that the double-blind placebo-controlled phase will enroll approximately 80 ESE patients randomized equally to IV ganaxolone or placebo added to a standard-of-care AED. The primary efficacy endpoint will be the absence of electrographic (rapid EEG) evidence of SE or recurrence of generalized convulsions at 1 hour after the initiation of treatment. We are targeting data from the first dose-finding cohort of the RESET trial by the end of 2023.

In September 2021, the U.S. Patent and Trademark Office (USPTO) granted us a patent on a method of treating SE, including dosing regimens. This issued patent expires in 2040. That patent is a member of a patent family we own that includes pending patent applications that claim certain therapeutic regimens for the treatment of SE, including RSE, using intravenous ganaxolone. On July 26, 2022, the USPTO issued a patent to Ovid with claims that encompass our product candidate for the treatment of SE. Ovid may file a lawsuit against us alleging infringement of its patents and/or we may challenge the validity of Ovid's patents with the USPTO or through the courts. Any such proceeding, regardless of its outcome, would likely result in the expenditure of significant financial resources and the diversion of management's time and resources. In addition, any such proceeding may cause negative publicity, adversely impact patients, and we may be prohibited from marketing or selling ganaxolone for SE, RSE and ESE during such proceedings or if we are not successful in such proceedings. If Ovid does decide to bring an infringement lawsuit, we do not expect that it will be filed before a commercial launch of ganaxolone for SE, RSE or ESE based upon the "safe harbor" provisions of the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman Act). We may need to acquire or obtain a license to the Ovid patents to market or sell ganaxolone for SE, RSE and ESE, which may not be available on commercially acceptable terms or at all. If we are not able to acquire the Ovid patents or negotiate a license on acceptable terms, and if our product is determined to infringe Ovid's patents and the patents are determined to be valid, then we may be forced to pay Ovid royalties, damages and costs, or we may be prevented from commercializing ganaxolone for SE, RSE and ESE altogether, which would have a material adverse impact on our business.

Tuberous Sclerosis Complex (TSC)

TSC is a rare genetic disorder that affects many organs by causing, typically non-malignant, tumors in the brain, skin, kidney, heart, eyes, and lungs. The condition is caused by inherited mutations in either the *TSC1* or *TSC2* gene. It occurs with a frequency of approximately 1:6,000 live births, with a mutation being found in 85% of patients. While the disease phenotype can be extremely variable, epilepsy occurs with a frequency of up to 85%. TSC is a leading cause of genetic epilepsy, often manifesting in the first year of life as either focal seizures or infantile spasms. There are currently few disease-specific treatments approved for seizures in TSC. Orphan drug designation for ganaxolone for the treatment in TSC was granted by the FDA in August 2021 and by the EMA in October 2021.

In August 2021, we announced top-line data from our open-label Phase 2 trial (CALM trial) evaluating the safety and efficacy of adjunctive oral ganaxolone in 23 patients with seizures associated with TSC. The CALM trial enrolled 23 patients ages 2 to 32 who entered a four-week baseline period followed by a 12-week treatment period, during which they received up to 600 mg of ganaxolone (oral liquid suspension) three times a day. Patients who met

eligibility criteria were able to continue ganaxolone treatment during a 24-week extension. The primary endpoint was the percent change in 28-day TSC-associated seizure frequency during the 12-week treatment period relative to the four-week baseline period. Secondary outcome measures included the percentage of patients experiencing a greater than or equal to 50% reduction in 28-day TSC-associated seizure frequency through the end of the 12-week treatment period compared to the 4-week baseline period.

The primary endpoint showed a median 16.6% reduction in 28-day in the frequency of TSC-associated seizures relative to the four-week baseline period. A secondary endpoint showed that the proportion of patients that achieved at least a 50% seizure reduction was 30.4%. During the trial, patients with focal seizures (n=19) showed a median 25.2% reduction in focal seizure frequency. Ganaxolone was generally well-tolerated with somnolence reported as the most common AE. In addition, one serious adverse event (SAE) of worsening seizures occurred, which was assessed by the investigator as treatment-related. Four patients discontinued the trial due to AEs. Additionally, the data from the trial suggested that in patients on concomitant Epidiolex, early elevation of ganaxolone blood levels occurred and appeared to be linked to greater somnolence. The interpretation of these findings is limited by the small sample size and open-label design of the trial. A formal Phase 1 drug-drug interaction trial was completed, demonstrating a lack of significant interaction between ganaxolone and Epidiolex. Additionally, the titration schedule for all subjects in the Phase 3 TSC trial has been adjusted to maximize tolerability.

In response to our request for an End of Phase 2 meeting with the FDA regarding a proposed Phase 3 TSC trial, the FDA provided written responses to our questions in lieu of a meeting. We believe the written responses show overall alignment on the clinical development plan in TSC. We believe that, based on the FDA's written responses, and with FDA approval of CDD, a single trial could serve as necessary support for regulatory approval for TSC in the U.S. In response to our request for Protocol Assistance, which is a special form of scientific advice available for developers of designated orphan medicines for rare diseases, the EMA provided written feedback in December 2021 in lieu of a meeting. We believe the written responses from the EMA, like those from the FDA, show overall alignment on the clinical development plan in TSC. After commencing site initiations in the first quarter of 2022 and dosing the first patient in the second quarter of 2022, we are actively enrolling patients in the U.S., Spain, Germany and the United Kingdom for this global Phase 3 randomized, double blind, placebo-controlled trial (TrustTSC trial) of adjunctive ganaxolone in approximately 160 TSC patients. We expect to expand the trial to include up to 90 sites, including several TSC centers of excellence, predominantly in the U.S., Western Europe, Canada and Israel. The primary endpoint for the TrustTSC trial is percent change in 28-day frequency of TSC-associated seizures. We plan to announce top-line data from the TrustTSC trial in the first quarter of 2024.

Second-Generation Formulation, Prodrug Development and Lennox-Gastaut Syndrome (LGS)

Top-line data from a Phase 1 trial with healthy volunteers utilizing the first candidate for a second-generation formulation of ganaxolone were announced in the second quarter of 2022, including PK characteristics that may allow for twice-daily dosing. We believe that the data support further clinical development of this formulation of ganaxolone. An additional Phase 1 cohort of assessing the PK of the second-generation oral formulation candidate has been completed, which assessed higher doses of ganaxolone than in the initial phase 1 cohorts. A multiple ascending dose study will be initiated in the second quarter of 2023. This study will also incorporate food effect assessments**.**

The development of ganaxolone prodrug compounds continues to advance, with lead oral and IV candidates selected, and Phase 1 data targeted for 2024.

We plan to pursue the development of ganaxolone for LGS, a severe form of epilepsy that typically begins between one and eight years of age. Affected children have neurodevelopmental impairments and intractable seizures, including focal, atonic, tonic and atypical absence seizures. Given the overlap in seizure types and etiologies with other disorders where ganaxolone has potential to reduce seizures, such as CDD and TSC, we believe that LGS represents a promising opportunity for ganaxolone development. We are planning to utilize a second-generation formulation of ganaxolone for the LGS development program, with a Phase 2 trial targeted to begin in the fourth quarter of 2023.

Orphan Designations

The FDA has granted orphan drug designation to ganaxolone for the treatment of Infantile Spasms, SE, CDD, TSC, PCDH19-RE and Fragile X Syndrome. Orphan drug designation is granted by the FDA Office of Orphan Products Development to novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. The designation provides the drug developer with a seven-year period of U.S. marketing exclusivity, as well as tax credits for clinical research costs, the ability to apply for annual grant funding, clinical research trial design assistance and waiver of Prescription Drug User Fee Act filing fees.

Ganaxolone Mechanism of Action

Ganaxolone is a methylated analog of the endogenous neurosteroid, allopregnanolone. Allopregnanolone exhibits potent anxiolytic, antidepressant and anti-seizure activity. Unlike allopregnanolone, ganaxolone cannot be back-converted to active intermediates possessing steroid hormone activity.

Both ganaxolone and allopregnanolone bind to $GABA_A$ receptors that, when activated, permit flow of chloride ions into the neuron. This change in concentration of chloride ions results in hyperpolarization and is the basis for the inhibitory effect of GABA. Classic $GABA_A$ active drugs bind only at receptors located on the synapse between neurons. However, both allopregnanolone and ganaxolone also bind to extrasynaptic $GABA_A$ receptors. Synaptic $GABA_A$ receptors respond quickly to inhibit neurotransmission (phasic inhibition), while extrasynaptic $GABA_A$ receptors provide a constant baseline level of inhibition (tonic inhibition).

Activity at extrasynaptic $GABA_A$ receptors may be of particular importance for the prolonged seizures in SE, during which synaptic, but not extrasynaptic, receptors become internalized into the neuron and are unavailable for binding of GABAergic drugs.

Safety Overview

Oral Safety

More than 2,300 individuals have received oral formulations of ganaxolone for durations from one day to more than two years at doses of 50 to 2,000 mg/day. Ganaxolone was administered in Phase 2 clinical trials to pediatric patients at doses up to 1,800 mg/day and to adult patients at doses up to 1,875 mg/day. The majority of AEs were non-serious and resolved upon discontinuation of therapy. The most common side effects with oral ganaxolone relate to sedation or somnolence. In the oral ganaxolone safety database there are no trends of medically important changes in blood chemistry, vital signs, liver function, renal function or cardiovascular parameters in adult or pediatric populations.

In the pivotal Phase 3 clinical trial (Marigold Trial), which evaluated the use of oral ganaxolone in children and young adults with CDD, ganaxolone was generally well tolerated with a safety profile consistent with previous clinical trials. The most common adverse reactions (incidence of at least 5% and at least twice the rate of placebo) were somnolence, pyrexia, salivary hypersecretion, and seasonal allergy. Somnolence and sedation appeared early during treatment and were generally dose related. Antiepileptic drugs, including ZTALMY, increase the risk of suicidal thoughts or behavior. In addition, as with most antiepileptic drugs, ganaxolone should be withdrawn gradually to minimize the risk of increased seizure frequency and SE.

There were no deaths reported in the double-blind phase of the Marigold Trial. Three deaths globally have occurred during the open label extension phase of the trial, two of which were assessed by the investigators as unrelated to trial treatment. The third death was assessed by the investigator as probably related to trial medication. Given the severity of CDD and its medical complications, serious adverse events or deaths may occur which, in the absence of a control group, make determination of relatedness to treatment difficult.

In the Phase 2 TSC trial, ganaxolone was generally well tolerated with somnolence reported as the most common AE, consistent with previous studies. Concomitant Epidiolex appeared to be linked to greater somnolence. A

formal Phase 1 drug-drug interaction trial was completed, demonstrating a lack of significant interaction between ganaxolone and Epidiolex. Additionally, the titration schedule for all subjects in the Phase 3 TSC trial has been adjusted to maximize tolerability.

IV Safety

In 2016, we completed a Phase 1 dose-escalation trial with IV ganaxolone that enrolled 36 patients, designed to determine the PK, pharmacodynamics (PD), and safety of IV ganaxolone administered as an ascending bolus dose (Stage 1) or continuous infusion (Stage 2). Thirty-six healthy volunteers were enrolled.

Every dose regimen of IV ganaxolone, either bolus or continuous infusion, was generally safe and well-tolerated and rapidly reached targeted dose levels. Six treatment-emergent AEs were reported, all of which were mild in severity and resolved without intervention. Only headache was considered possibly related to trial treatment. No patient discontinued due to an AE and no SAEs were reported. IV ganaxolone plasma concentrations were generally proportional to the administered dose. In addition, the continuous infusion of IV ganaxolone achieved the targeted exposure levels associated with anticonvulsant activity.

In 2019, we announced positive top-line results in our open-label, dose-finding Phase 2 clinical trial evaluating IV ganaxolone in patients with RSE. In the trial, ganaxolone had an acceptable safety and tolerability profile for the RSE patient population in all dose groups. There were 10 SAEs; eight were considered not related to treatment and two were considered treatment-related (TRSAEs). The TRSAEs were severe sedation in two patients that led to early ganaxolone discontinuation: one in the medium dose group on day three and one in the target dose group on day one. There were 50 AEs, thirteen of which were considered treatment-related (TRAEs) reported in seven patients. The most commonly reported TRAEs were somnolence, mild hypotension and sedation.

Preclinical Pharmacology and Toxicology

We have completed preclinical safety pharmacology and toxicology testing, including reproductive toxicology. Animal pharmacokinetic and *in vitro* studies show that ganaxolone is metabolized primarily by the Cytochrome P450, family 3, subfamily A (CYP3A) family of liver enzymes, a common route of drug metabolism. All *in vitro* studies have shown that ganaxolone has low potential for interaction with other drugs at several multiples of observed human ganaxolone levels. Neither ganaxolone nor its metabolites have a ketone ring at the 3-position, a requirement for hormonal activity. In binding and functional activity studies, ganaxolone has no appreciable affinity for estrogen or progesterone receptors. We found no evidence of changes in blood, liver, kidney or the gastrointestinal systems indicating functional or anatomical adverse effects associated with either single- or multiple-dose treatment with ganaxolone in preclinical safety pharmacology studies, nor have we seen evidence of any end organ toxicity from human clinical trials. We have not detected potential for ganaxolone to cause cellular mutations or carcinogenicity in trials to date.

Ganaxolone is metabolized extensively in animals and humans. During the development of CDD, one major metabolite (M2) was present in plasma of humans that was not found in plasma of rats or dogs. The chemical structure of M2 has been identified. An activity assay, dose range finding study in rats and an in vivo micronucleus with comet analysis for the detection of genotoxicity have been conducted and the results submitted to the FDA. Results from additional preclinical studies are required to the FDA as post-marketing requirement(s). These include: 2-year carcinogenicity studies of ganaxolone and the major human unconjugated plasma metabolite, M2, in rats; a 26-week carcinogenicity of ganaxolone in transgenic mice; a juvenile animal toxicity study of the major human unconjugated plasma metabolite, M2, in rats; a CNS distribution study of the M47 metabolite in rats; and in vitro studies to assess the drug interaction potential of M47 metabolite. Additional post-marketing requirements include: phase 1 renal and hepatic impairment studies and a thorough QTc study; and extractable/leachable study results on the container closure system. The Phase 1 renal impairment study commitment was completed and submitted to the FDA in May 2022. The Phase 1 hepatic impairment study and the thorough QTc study were completed and submitted to the FDA in December 2022. We plan to complete the required FDA studies within the required FDA timeframe. However, there is a risk that the studies could take longer than expected to complete or the studies may have adverse findings which may require additional

investments and have the potential to materially impact our marketing of Ztalmy. In the EU, if additional studies are needed, these are usually required before or during MAA review.

In reproductive toxicology studies, ganaxolone did not cause malformations of the embryo or fetus in rats or mice and did not significantly affect the development of offspring. No changes in sperm parameters were found. We believe these findings are important, as many currently marketed AEDs have shown developmental toxicities in animal studies, including fetal death and skeletal abnormalities. Valproate, carbamazepine, phenytoin, topiramate and other AEDs have been linked to birth defects in humans (e.g., head and facial malformations and lowered birth weight). These findings have resulted in labeling for these drugs indicating evidence of human fetal risk.

Intellectual Property

The proprietary nature of and protection for ZTALMY, other indications being developed for ganaxolone and any other future product candidates, discovery programs and know-how are important to our business. We have sought patent protection in the U.S. and internationally for synthetic methods for making ganaxolone, ganaxolone nanoparticles, which are used in certain oral solid, oral liquid, and IV dose formulations, other injectable and oral ganaxolone formulations, and methods of treatment using ganaxolone. Our policy is to pursue, maintain and defend patent rights whether developed internally or licensed from third parties and to protect the technology, inventions and improvements that are commercially important to the development of our business. The patents and patent applications owned by us comprise approximately 17 different patent families, filed in various jurisdictions around the world.

Nanoparticle Ganaxolone Formulations. We own approximately four patent families directed to nanoparticle formulations of ganaxolone and complexing agents that deliver consistent exposure and improved stability of ganaxolone, and certain uses of the formulations. One of the patent families includes eight issued U.S. patents with claims directed to certain solid and liquid ganaxolone formulations and certain methods for the making and use thereof. Corresponding foreign patents have been granted in Australia, Canada, China, Europe, Hong Kong, India, Israel, Japan, Mexico, South Africa, New Zealand, Singapore and South Korea. The 20-year term for patents in this family runs through 2026, absent any available patent term adjustments or extensions. We have filed for a patent term extension of a granted U.S. patent in this patent family that covers ZTALMY. Our patent term extension application requests an extension of five (5) years, which is the maximum extension available under the Hatch-Waxman Act. If the full extension is granted, this U.S. patent would be extended to November 28, 2031. The application for patent term extension is pending at the USPTO. We have not out-licensed any rights to practice these patents in any of these territories. Pursuant to our agreement with Domain Russia Investments Limited (DRI), we assigned to DRI patent rights, which rights were subsequently assigned to NovaMedica LLC (NovaMedica), along with the right to develop and commercialize ganaxolone in Russia and certain other member countries of the Eurasian Patent Organization.

We have approximately four patent families consisting of three pending U.S. provisional applications directed to ganaxolone analogs and/or additional formulations of ganaxolone, and an international application filed under the Patent Cooperation Treaty (PCT) that is directed to additional formulations of ganaxolone. The 20-year term for patents based on this international application will run through 2042, absent any available patent term adjustments.

Process for Manufacturing Ganaxolone. Our patent portfolio contains patents issued in Australia, Brazil, Canada, China, Europe, Hong Kong, India, Israel Japan, Mexico, New Zealand, South Korea, and the U.S. covering our synthetic process for manufacturing ganaxolone. The 20-year term for patents in this family runs through 2030, absent any available patent term adjustments or extensions. The European patent has been validated in France, Germany, Ireland, Italy, Spain, and Switzerland.

Intravenous Ganaxolone Formulations. We own approximately four patent families directed to our IV ganaxolone formulations that we are developing for the treatment of SE and certain other disorders. One of the patent families includes pending applications in Australia, Canada, China, Europe, Israel, India, Japan, South Africa, and the U.S. that claim certain injectable ganaxolone formulations containing sulfobutyl ether-beta-cyclodextrin and certain methods of use of the formulations, including for the treatment of SE. The 20-year term for this patent family runs through to 2036, absent any available patent term adjustments or extensions. A second patent family currently includes one issued U.S. patent with claims directed to certain therapeutic regimens for the treatment of SE using IV ganaxolone,

and an international application filed under the PCT that is directed to certain therapeutic regimens for the treatment of SE using IV ganaxolone. The 20-year term for patents based on this international application will run through 2040, absent any available patent term adjustments. We intend to file national phase applications in various foreign jurisdictions based on this PCT application before applicable deadlines. A third patent family currently consists of a pending international application filed under the PCT that is directed to certain therapeutic uses of IV ganaxolone. We intend to file national phase applications in various foreign jurisdictions based on the PCT application before applicable deadlines. The 20-year term for patents based on this international application will run through 2041, absent any applicable available patent term adjustments. A fourth patent family currently consists of a pending international application filed under the PCT directed to certain therapeutic uses for SE. We intend to file national phase applications in various foreign jurisdictions based on this PCT application before applicable deadlines. The 20-year term for patents based on this international application will run through 2042, absent any available patent term adjustments.

Additional Therapeutic Uses. We own approximately four patent families directed to certain therapeutic uses of ganaxolone, including for treating genetic epilepsy disorders, such as CDD and PCDH19-Related Epilepsy (PCDH19-RE), and TSC. One of the patent families includes pending applications filed in Australia, Canada, China, Eurasia, Europe, Japan, Korea, Malaysia, New Zealand, Singapore, and the U.S. that claim certain methods of treating epileptic disorders. The 20-year term for patents in this family runs through 2038, absent any available patent term adjustments or extensions. A second patent family currently includes one pending international patent application filed under the PCT that claims certain methods of treating TSC. We intend to file national phase applications in various foreign jurisdictions based on this PCT application before applicable deadlines. The 20-year term for patents based on this family run through 2040, absent any available patent term adjustments or extensions. A third family currently comprises one pending international patent application filed under this PCT directed to certain therapeutic regimens for certain disorders using ganaxolone. We intend to file national phase applications in various foreign jurisdictions based on this PCT application before applicable deadlines. The 20-year term for patents based on this family run through 2041, absent any available patent term adjustments or extensions. A fourth patent family currently consists of a pending international patent application filed under this PCT directed to certain therapeutic regimens for certain disorders using ganaxolone. We intend to file national phase applications in various foreign jurisdictions based on this PCT application before applicable deadlines. The 20-year term for patents based on this family run through 2042, absent any available patent term adjustments or extensions.

We have also licensed from Ovid certain patents that are directed to certain therapeutic uses of ganaxolone for the treatment of CDD. The licensed patent family includes a granted U.S. patent and a pending application in Europe. The 20-year term for patents based on this international application will run through 2037, absent any available patent term adjustments.

In addition to patents, we rely upon unpatented trade secrets, know-how and continuing technological innovation to develop and maintain a competitive position. We seek to protect our proprietary information, in part, through confidentiality agreements with our employees, collaborators, contractors and consultants, and invention assignment agreements with our employees and some of our collaborators. The confidentiality agreements are designed to protect our proprietary information and, in the case of agreements or clauses requiring invention assignment, to grant us ownership of technologies that are developed through a relationship with a third party.

General Considerations

As with other biotechnology and pharmaceutical companies, our ability to maintain and solidify a proprietary position for our ganaxolone synthesis and formulations will depend upon our success in obtaining effective patent claims and enforcing those claims once granted. Our commercial success will depend in part upon not infringing upon the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent could require us to alter our development or commercial strategies, obtain licenses, or cease certain activities. The biotechnology and pharmaceutical industries are characterized by extensive litigation regarding patents and other intellectual property rights.

As discussed in the Government Regulation section below, the term of a patent that covers an FDA-approved drug may be eligible for patent term extension, which provides patent term restoration as compensation for the patent

term lost during the FDA regulatory review process. The Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman Act) permits a patent term extension of up to five years beyond the expiration of the patent. The length of the patent term extension is related to the length of time the drug is under regulatory review. Patent extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug may be extended. Similar provisions are available in Europe and other foreign jurisdictions to extend the term of a patent that covers an approved drug. In the future, if and when our pharmaceutical products receive approval in the U.S. or other countries, we expect to apply for patent term extensions, where available, on patents covering those products in the respective jurisdictions.

Many pharmaceutical companies, biotechnology companies and academic institutions are competing with us in the field of neuropsychiatric disorders and filing patent applications potentially relevant to our business. Even if a particular third-party patent is identified as possibly being relevant to our product candidates or technology, we may conclude upon a thorough analysis, that we do not infringe upon the patent or that the patent is invalid. If the third-party patent owner disagrees with our conclusion and we continue with the business activity in question, we may be subject to patent litigation. Alternatively, we might decide to initiate litigation in an attempt to have a court declare the third-party patent invalid or non-infringed by our activity. In either scenario, patent litigation typically is costly and time-consuming, and the outcome can be favorable or unfavorable.

Licenses and Collaborations

Orion

On July 30, 2021, we entered into a collaboration agreement (Orion Collaboration Agreement) with Orion Corporation (Orion), whereby Orion received exclusive rights to commercialize the oral and IV dose formulations of ganaxolone in the European Economic Area, United Kingdom and Switzerland in multiple seizure disorders, including CDD, TSC and RSE. Under the agreement, we received a €25 million ($29.6 million) upfront fee and are eligible to receive up to an additional €97 million in R&D reimbursement and cash milestone payments based on specific clinical and commercial achievements, as well as tiered royalty payments based on net sales ranging from the low double digits to the high teens for the oral programs and the low double-digits to the low twenties for the IV programs.

Tenacia

On November 16, 2022, we entered into a collaboration and supply agreement with Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia), whereby Tenacia received exclusive rights to develop and commercialize certain oral and IV formulations of ganaxolone in Mainland China, Hong Kong, Macau and Taiwan for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions, initially for the treatment of CDD, TSC and SE. In connection with the agreement, we received an upfront cash payment of $10 million in December 2022 and are eligible to receive up to an additional $256 million upon the achievement of certain development, regulatory and sales-based milestones. Tenacia has further agreed to pay us tiered royalty payments based on annual net sales ranging from the low double digits to the mid-teens for each of the oral formulation, IV formulation and selected product formulation of licensed products.

Other Distribution Agreements

We have entered into an agreement for commercialization of ganaxolone in other territories with NovaMedica whereby NovaMedica has the right to market and sell ganaxolone in Armenia, Azerbaijan, Belarus, Georgia, Kazakhstan, Kyrgyzstan, Moldova, Russia, Tajikistan, Turkmenistan, Ukraine and Uzbekistan. We continue to assess opportunities in other markets to further expand the distribution and commercialization of ganaxolone globally.

CyDex

In March 2017, we entered into a License Agreement and a Supply Agreement with CyDex Pharmaceuticals, Inc. (CyDex). Under the terms of the License Agreement, CyDex granted us an exclusive license to use CyDex's sulfobutylether beta-cyclodextrin, or Captisol®, drug formulation system and related intellectual property in connection

with the development and commercialization of ganaxolone in any and all therapeutic uses in humans, with some exceptions.

As consideration for this license, we paid an upfront fee and are required to make additional payments in the future upon achievement of various specified clinical and regulatory milestones. We will also be required to pay royalties to CyDex on sales of ganaxolone, if successfully developed, in the low-to-mid single digits based on levels of annual net sales. As of March 24, 2022, we have achieved one milestone under the License Agreement, which occurred and was paid in the first quarter of 2021. Certain patents relating to Captisol®, including some that were licensed to us by CyDex, have expired, while other patents that are licensed to us remain in force.

Under the terms of the Supply Agreement, we are required to purchase all of our requirements for Captisol with respect to ganaxolone from CyDex, and CyDex is required to supply us with Captisol for such purposes, subject to certain limitations.

Purdue Neuroscience Company (Purdue)

In September 2004, we entered into a license agreement with Purdue, which was amended and restated in May 2008 (Purdue License Agreement), that granted us exclusive rights to certain know-how and technology relating to ganaxolone, excluding the field of treatment of unpleasant sensory or emotional experience associated with actual or potential tissue damage or described in terms of such damage. The agreement contains a right by us to sublicense, subject to prior written approval by Purdue, and we have sublicensed our licensed rights to NovaMedica for the Covered Territory. We are obligated to pay royalties as a percentage in the range of high single digits up to 10% of net product sales for direct licensed products, such as ganaxolone. The obligation to pay royalties expires, on a country-by-country basis, ten years from the first commercial sale of a licensed product in each country. Upon commercialization, we estimate the in-licensed technology would result in us paying royalties to Purdue in the low single digits as a percentage of sales. Other payment obligations may be triggered if we successfully partner our product candidates with third parties. In addition, the agreement also requires that we pay Purdue a percentage in the mid-single digits of the non-royalty consideration that we receive from a sublicensee and a percentage in the twenties of milestone payments received from sublicensees for indications other than seizure disorders and vascular migraine headaches not associated with mood disorders. Under the license agreement, we are committed to use commercially reasonable efforts to develop and commercialize at least one licensed product.

On July 14, 2022, we announced that we had entered into a definitive agreement to sell our PRV for $110 million. Thereafter, we received a letter dated August 1, 2022 from Purdue in which Purdue claimed that it was owed $5.5 million by us from the sale of the PRV pursuant to the Purdue License Agreement. Our position communicated to Purdue is that we do not owe Purdue any of the proceeds from the sale of the PRV. Purdue maintains that they are owed the $5.5 million. No associated payment has been made and we continue to discuss this matter with Purdue.

Ovid License

In March 2022, we entered into an exclusive patent license agreement (License Agreement) with Ovid Therapeutics Inc. (Ovid). Under the License Agreement, we have an exclusive, non-transferable (except as provided in the License Agreement), royalty-bearing, sublicensable license under certain of Ovid's patent(s) and patent applications to develop, make, have made, commercialize, promote, distribute, sell, offer for sale and import, ganaxolone, including any analogues or derivatives, including its salts, and pharmaceutical formulations of the foregoing (Licensed Products), in the U.S., the member states of the EU, Iceland, Lichtenstein, Norway, the United Kingdom, and Switzerland (Territory) for the treatment of CDD in humans (Field). Under the License Agreement, we have the sole right and responsibility for, and control over, all development, manufacturing, and commercialization activities, including all regulatory activities, with respect to the Licensed Products in the Field in the Territory. In addition, all regulatory approvals and related filings with respect to the Licensed Products in the Field in the Territory will be in the name of, and be owned solely by, us. We were required, at Ovid's option exercisable in accordance with the License Agreement, to (i) pay to Ovid the sum of $1.5 million in cash; or (ii) issue to Ovid 123,255 shares of our common stock, which option to obtain shares of our common stock was exercisable within the five-business day period following the filing of

our Annual Report on Form 10-K for the year ended December 31, 2021 on March 24, 2022. On March 29, 2022, we issued 123,255 shares of our common stock to Ovid, per Ovid's option in accordance with the License Agreement. As such, we recorded $1.2 million of IP license fee expenses related to the Ovid License Agreement in the year ended December 31, 2022. The License Agreement provides for payment of royalties by us to Ovid in the low single digits on net sales by us, our affiliates and sublicensees, of Licensed Products in the Field in the Territory. Such royalties are subject to reduction in the event of generic competition in accordance with the License Agreement. We may terminate the License Agreement at any time without cause on thirty days' prior written notice. Either party may terminate the License Agreement for the other party's material breach or insolvency subject to certain cure periods. Also, Ovid has the right to terminate the License Agreement if there has not been a first commercial sale of any Licensed Products in the Field in the Territory on or before June 30, 2025. In the event of termination, all licenses granted under the License Agreement will terminate.

Competition

The pharmaceutical industry is highly competitive and subject to rapid and significant technological change. While we believe that our development experience and scientific knowledge provide us with competitive advantages, we face competition from both large and small pharmaceutical and biotechnology companies, specifically from companies that treat rare seizure disorders.

There are a variety of available therapies marketed for rare seizure disorders. In many cases, these products are administered in combination to enhance efficacy or to reduce side effects. Some of these drugs are branded and subject to patent protection, some are in clinical development and not yet approved, and others are available on a generic basis. Many of these approved drugs are well established therapies or products and are widely accepted by physicians, patients and third-party payers. Insurers and other third-party payers may also encourage the use of generic products. More established companies have a competitive advantage over us due to their greater size, cash flows, established commercial infrastructure, clinician relationships and institutional experience. Compared to us, many of our competitors have significantly greater financial, technical and human resources with longer histories of marketed products.

Our competitors may also develop drugs that are safer, more effective, more widely used and less costly than ours, and may also be more successful than us in manufacturing and marketing their products. These appreciable advantages could render ganaxolone obsolete or non-competitive before we can recover the expenses of ganaxolone's development and commercialization.

We primarily compete with pharmaceutical and biotechnology companies that are developing clinical-stage therapies or marketing drugs to treat indications that we are targeting.

SE

SE patients generally are treated with benzodiazepines as first-line treatment. When benzodiazepines are not effective the patients are in ESE and are treated with various second-line IV AEDs, such as levetiracetam, fosphenytoin, lacosamide, or valproate. In 2019, a multicenter, randomized clinical trial (Established Status Epilepticus Treatment Trial; ESETT) was conducted by a group of academic investigators and was designed to evaluate the effectiveness of second-line IV AEDs in ESE. In this trial, the efficacy of levetiracetam, fosphenytoin, or valproate was evaluated in convulsive ESE patients. It was reported that levetiracetam, fosphenytoin, and valproate were effective at stopping SE in 47%, 45%, and 46% of the patients, respectively. When second-line AEDs are not effective, RSE patients are generally placed in a medically-induced coma under IV anesthesia in an attempt to stop the seizures and prevent further damage to the brain and death. Patients on third-line IV anesthesia are at higher risk for anesthesia-associated morbidities, such as infection, and have 2.9 times greater mortality rate. In addition, patients on IV anesthetics for SE treatment have increased lengths of stays in the hospital and ICU resulting in increased healthcare utilization. To our knowledge, there are no treatments indicated for RSE. To our knowledge, there is only one other company currently developing a clinical-stage product for the treatment of SE and RSE. Bio-Pharm Solutions Co., a private South Korea-based company, is currently in Phase 2 trials for intravenously administered treatment for SE and RSE.

CDD and TSC

There are no drugs other than our product ZTALMY approved specifically for the treatment of seizures associated with CDD, and two drugs approved for the treatment of seizures associated with TSC: Novartis Pharmaceuticals Corp.'s Afinitor DISPERZ® (everolimus tablets for oral suspension) and Jazz Pharmaceuticals, Inc.'s EPIDIOLEX® (cannabidiol). CDD and TSC patients are typically prescribed drugs approved for epileptic seizures, which often fail to control seizures in these patient populations. To our knowledge, there is only one other company with a drug in active clinical development for the treatment of CDD (UCB S.A's, FINTEPLA® Fenfluramine Hydrochloride). To our knowledge, there are currently two products in development for the treatment of seizures associated with TSC: Noema Pharma AG's Basimglurant (NOE-101) in a Phase 2b trial and Ovid's OV329 in a Phase 1A trial.

LGS

There are currently three FDA-approved branded drugs for the treatment of seizures in LGS. These include Assertio Holdings Inc.'s SYMPAZAN® (clobazam) oral film CIV for patients 2+ years old, Jazz Pharmaceutical's EPIDIOLEX® (cannabidiol) for patients 1+ years old, and UCB S.A's, FINTEPLA® (Fenfluramine Hydrochloride) for patients 2+ years old. Patients with LGS experience life-long epilepsy and exhibit multiple seizure types that are often refractory to treatment. To our knowledge, there are currently four products in clinical-stage development for the treatment of LGS: Takeda Pharmaceutical Company Limited's Soticlestat in Phase 3 trials, SK Life Science, Inc's Carisbamate in Phase 3 trials, Eisai Inc.'s FYCOMPA® (perampanel) in Phase 3 trials, and Epygenix Therapeutics Inc.'s EPX-100 (clemizole hydrochloride) entering pivotal Phase 2/3 trials.

Manufacturing

Manufacturing of drugs and product candidates, including ganaxolone, must comply with FDA current good manufacturing practice (cGMP) regulations. Ganaxolone is a synthetic small molecule made through a series of organic chemistry steps starting with commercially available organic chemical raw materials. We conduct manufacturing activities under individual purchase orders with independent contract manufacturing organizations (CMOs) to supply our clinical trials. We have executed commercial supply agreements for ganaxolone API with our current manufacturer and also with our current supplier for finished bulk drug product. Additionally, we have executed a master supply agreement with a second API supplier in the U.S. to undertake certain process development activities and subsequently to provide commercial supplies of API and/or API intermediates. We have an internal quality program and have qualified and signed quality agreements with our major CMOs. We conduct periodic quality audits of their facilities. We believe that our existing suppliers of ganaxolone's API and finished product will be capable of providing sufficient quantities of each to meet our clinical trial supply needs. We also believe our current API manufacturer will be able to meet our currently forecasted commercial needs for ZTALMY for at least the next three years. CMOs may be used in the future for clinical supplies and commercial manufacturing.

Ganaxolone Formulations

The therapeutic possibilities of ganaxolone have been understood for some time; however, because ganaxolone is a high-dose water insoluble compound, developing a formulation that could provide consistent drug exposure and could be manufactured at a commercially feasible cost had proven challenging. We believe our patented nanoparticulate formulation and novel manufacturing process for ganaxolone can successfully address the cost of manufacturing and pharmacokinetic challenges that previously encumbered the clinical and commercial feasibility of ganaxolone.

Ganaxolone is currently formulated for oral and IV administration. In addition, we are evaluating various formulation approaches to improve ganaxolone's oral drug properties.

Commercial Operations

In connection with the commercialization of ZTAMLY, our first FDA approved product, we have built a commercial operations infrastructure, including, marketing infrastructure, market access capabilities, and sales field

force to reach high prescribing neurologists, critical care, epilepsy specialists and other target physician populations in the U.S. ZTALMY is regulated by the DEA as a controlled substance under the CSA as a schedule V drug. ZTALMY became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022. We believe a focused sales and marketing organization could be leveraged to market ganaxolone across multiple epilepsy indications. We believe that there could also be significant market opportunities for ganaxolone in epilepsy and other neurological and psychiatric conditions outside of the U.S. In order to capitalize on such opportunities, we have entered into collaborations and plan to seek additional collaborations with pharmaceutical companies that have greater reach and resources by virtue of their size and experience in the field.

Government Regulation

As a pharmaceutical company that operates in the U.S., we are subject to extensive regulation by the FDA and other federal, state, and local regulatory agencies. The Federal Food, Drug, and Cosmetic Act (FDC Act) and its implementing regulations set forth, among other things, requirements for the research, testing, development, manufacture, quality control, safety, effectiveness, approval, packaging, labeling, storage, record keeping, reporting, distribution, import, export, advertising and promotion of our products. Although the discussion below focuses on regulation in the U.S., we anticipate seeking approval for, and marketing of, our product candidates in other countries. Generally, our activities in other countries will be subject to regulation that is similar in nature and scope as that imposed in the U.S., although there can be important differences. In addition, some significant aspects of regulation in the European Union (EU) are addressed in a centralized way through the European Medicines Agency (EMA), but country-specific regulation also remains in many essential respects. The process of obtaining regulatory marketing approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations will require the expenditure of substantial time and financial resources in order to be successful.

United States Government Regulation

The FDA is the main agency that regulates pharmaceuticals in the U.S., and its regulatory authority is based in the FDC Act. Pharmaceutical products are also subject to other federal, state and local statutes. A failure to comply with applicable requirements during the product development, approval, or post-approval periods may lead to administrative or judicial sanctions. These sanctions could include the imposition by the FDA or an institutional review board (IRB) of a hold on clinical trials, refusal to approve pending marketing applications or supplements, withdrawal of approval, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties or criminal prosecution.

The steps required before a new drug may be marketed in the U.S. generally include:

- completion of preclinical laboratory tests and animal studies in compliance with the FDA's good laboratory practice (GLP) regulations, as applicable, including pharmacology and formulation studies to develop detailed information relating to the product's chemistry, manufacturing and controls;
- submission to the FDA of an Investigational New Drug application (IND) to support human clinical trials;
- approval by an IRB at each clinical site before each trial may be initiated;
- performance of adequate and well-controlled clinical trials in accordance with federal regulations, including requirements for good clinical practices (GCP) to establish the safety and efficacy of the investigational product candidate for each targeted indication;
- submission of a new drug application (NDA) to the FDA;
- satisfactory completion of an FDA Advisory Committee review, if applicable;
- satisfactory completion of an FDA inspection of clinical trial sites to ensure compliance with GCP, if applicable;

- satisfactory completion of an FDA inspection of the manufacturing facilities at which the investigational product candidate is produced to assess compliance with cGMP, and to assure that the facilities, methods and controls are adequate; and

- FDA review and approval of the NDA.

The preclinical and clinical testing and approval process requires substantial time, effort, and financial resources, and we cannot be certain that any approvals for our product candidates will be granted on a timely basis, if at all.

Clinical Trials

An IND is a request for authorization from the FDA to administer an investigational product candidate to humans. A 30-day waiting period after the initial submission of an IND is required prior to the commencement of clinical testing in humans. If the FDA has not raised concerns or questions about the proposed clinical testing and placed the IND on clinical hold within this 30-day period, the clinical trial proposed in the IND may initiate. If an IND has been placed on clinical hold, the sponsor must resolve the FDA's outstanding concerns or questions before clinical trials can begin.

Clinical trials involve the administration of the investigational product candidate to subjects under the supervision of qualified investigators in accordance with GCP, which are requirements meant to protect the rights and health of subjects and to assure the quality, reliability and integrity of data collected in clinical trials. Clinical trials are conducted under protocols that detail, among other things, the subject inclusion and exclusion criteria, the dosing regimen, the parameters to be used in monitoring safety, and the efficacy criteria to be evaluated. Each protocol involving testing on U.S. subjects and subsequent protocol amendments must be submitted to the FDA as part of the IND. The informed written consent of each participating subject is required, and an IRB at each site where the trial is conducted must approve the trial. The IRB must monitor the trial until completed. There are also requirements governing the registration of ongoing clinical trials and the reporting of clinical trial results to public registries.

The clinical investigation of an investigational product candidate is generally divided into three phases. Although the phases are usually conducted sequentially, they may overlap or be combined. The three phases of an investigation are as follows:

- *Phase 1.* Phase 1 includes the initial introduction of an investigational product candidate into humans. Phase 1 trials generally are conducted in healthy volunteers but in some cases are conducted in patients with the target disease or condition. These trials are designed to evaluate the safety, metabolism, PKs and pharmacologic actions of the investigational product candidate in humans, the side effects associated with increasing doses, and if possible, to gain early evidence on effectiveness. During Phase 1 trials, sufficient information about the investigational product candidate's PKs and pharmacological effects may be obtained to permit the design of Phase 2 trials. The total number of participants included in Phase 1 trials varies but is generally in the range of 20 to 80.

- *Phase 2.* Phase 2 includes the controlled clinical trials conducted in patients with the target disease or condition, to determine dosage tolerance and optimal dosage, to identify possible adverse side effects and safety risks associated with the product candidate, and to obtain initial evidence of the effectiveness of the investigational product candidate for a particular indication. Phase 2 trials are typically well-controlled, closely monitored, and conducted in a limited subject population, usually involving no more than several hundred participants.

- *Phase 3.* Phase 3 trials are controlled clinical trials conducted in an expanded subject population at geographically dispersed clinical trial sites. They are performed after preliminary evidence suggesting effectiveness of the investigational product candidate has been obtained, and are intended to further evaluate dosage, clinical effectiveness and safety, to establish the overall benefit-risk relationship of the product candidate, and to provide an adequate basis for drug approval. Phase 3 trials usually involve several hundred to several thousand participants. In most cases, the FDA requires two adequate and well

controlled Phase 3 trials to demonstrate the efficacy and safety of the drug; however, the FDA may find a single Phase 2 or Phase 3 trial with other confirmatory evidence to be sufficient in rare instances, particularly in an area of significant unmet medical need and if the trial design provides a well-controlled and reliable assessment of clinical benefit.

- *Phase 4*. In some cases, the FDA may condition approval of an NDA for a product candidate on the sponsor's agreement to conduct additional clinical trials after approval. In other cases, a sponsor may voluntarily conduct additional clinical trials after approval to gain more information about the product. Such post-approval trials are typically referred to as Phase 4 clinical trials.

Clinical trials may not be completed successfully within a specified period of time, if at all. The decision to terminate development of an investigational product candidate may be made by either a health authority, such as the FDA, or IRB/ethics committees, or by a company for various reasons. The FDA may order the temporary, or permanent, discontinuation of a clinical trial, which is referred to as a clinical hold, at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. In some cases, clinical trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data monitoring committee or data safety monitoring board. Such a group provides recommendations to the sponsor for whether or not a trial may move forward at designated check points, based on limited access to data from the ongoing trial. The suspension or termination of development can occur during any phase of clinical trials if it is determined that the participants or subjects are being exposed to an unacceptable health risk. In addition, there are requirements for the registration of ongoing clinical trials of product candidates on public registries and the disclosure of certain clinical trial results and other trial information after completion.

A sponsor may be able to request a special protocol assessment (SPA) the purpose of which is to reach agreement with the FDA on the design and size of certain clinical trials or animal studies that will adequately address scientific and/or regulatory requirements that could support marketing approval. A sponsor may make a specific request for an SPA and provide information regarding the design and size of the proposed clinical trial. An SPA request must be made before the proposed trial begins, and all open issues must be resolved before the trial begins. If a written agreement is reached, it will be documented and made part of the regulatory record. The agreement will be binding on the FDA and may not be changed by the sponsor or the FDA after the trial begins except with the written agreement of the sponsor and the FDA or if the FDA determines that a substantial scientific issue essential to determining the safety or efficacy of the product candidate was identified after the testing began. An SPA is not binding if new circumstances arise, and there is no guarantee that a trial will ultimately be adequate to support an approval even if the trial is subject to an SPA.

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, detailed investigational product candidate information is submitted to the FDA in the form of an NDA to request marketing approval for the product in specified indications.

New Drug Applications

In order to obtain approval to market a drug in the U.S., a marketing application must be submitted to the FDA that provides data establishing the safety and effectiveness of the product candidate for the proposed indication. The application includes all relevant data available from pertinent preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational product candidate to the satisfaction of the FDA.

In most cases, the NDA must be accompanied by a substantial user fee; there may be some instances in which the user fee is waived. The FDA will initially review the NDA for completeness before it accepts the NDA for filing. The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. After the NDA

submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of NDAs. Applications for standard review product candidates are reviewed within ten months of FDA's acceptance for filing. An accelerated six-month review can be given to applications that meet certain criteria. The FDA can extend the review period by three months, or potentially longer, to consider certain late-submitted information or information intended to clarify information provided in the initial submission. 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. FDA Advisory Committee meetings are often held for New Chemical Entities (NCEs), novel indications, or for applications that otherwise present scientific, technical, or policy questions on which the agency believes it would benefit from the perspectives of outside experts. An advisory committee meeting includes a panel of independent experts, including clinicians and other scientific experts, who review, evaluate and make 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.

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. In addition, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. After the FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, the FDA may require a risk evaluation and mitigation strategy (REMS) to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use (ETASU). ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory requirements is not maintained or problems are identified following initial marketing.

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

Compounds that have a potential for dependence and abuse are scheduled as controlled substances under the Controlled Substances Act and similar state and foreign laws. In the U.S., for new chemical entities under development for therapeutic use, FDA and HHS complete an analysis and recommendation about whether a drug should be scheduled as a controlled substance, and the Drug Enforcement Administration (DEA) takes the analysis and recommendation into account in the scheduling process. In the case of a new drug approved by the FDA, if scheduling is warranted then the DEA issues an interim final rule controlling the drug 90 days after receipt of the FDA/HHS analysis and recommendation or notice of FDA approval of the drug, whichever is later. Drugs that are scheduled as controlled substances are subject to stringent regulatory requirements, including requirements for registering manufacturing and distribution facilities, security controls and employee screening, recordkeeping, reporting, product labeling and packaging, import and export. There are five federal schedules for controlled substances, known as Schedule I, II, III, IV and V. The regulatory requirements that apply to a drug vary depending on the particular controlled substance schedule into which a drug is placed, based on consideration of a number of factors, including its potential for dependence and

abuse. Schedules I and II contain the most stringent restrictions and requirements, and Schedule V the least. For all controlled substances, there are potential criminal and civil penalties that apply for the failure to meet applicable legal requirements, and healthcare professionals must have a DEA license in order to handle, prescribe, or dispense controlled substances.

Breakthrough Therapy Designation

In the U.S., the FDA may grant breakthrough therapy designation to a drug candidate if preliminary clinical evidence indicates that the therapy may offer substantial improvement on a clinically significant endpoint over existing options for patients with a serious condition. Features of breakthrough therapy designation include intensive guidance to ensure that the design of clinical trials are as efficient as practicable, increased involvement of senior managers and experienced review staff and where appropriate, a cross-disciplinary project lead assigned to the FDA review team, and rolling review of the NDA. Breakthrough designation can be requested with the IND or ideally no later than the end-of-Phase 2 meeting.

Fast Track Designation

Fast Track is a designation by the FDA of an investigational drug that is intended to treat a serious condition and for which nonclinical or clinical data demonstrate the potential to address an unmet medical need. The request for fast track designation can be initiated with the IND or ideally no later than the pre-NDA/BLA meeting. Features of fast track designation include more frequent meetings and interactions with FDA to expedite development and review, including to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval, and a rolling review of the NDA/BLA.

Priority Review

Based on results of the Phase 3 clinical trials submitted in an NDA, upon the request of an applicant, a priority review designation may be granted to a product by the FDA, which sets the target date for FDA action on the application at six months from FDA acceptance for filing. Priority review may be granted where a product is intended to treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness of the treatment, diagnosis or prevention of the serious condition. If criteria are not met for priority review, the standard FDA review period is ten months from FDA acceptance for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Post-Approval Regulation

After regulatory approval of a drug is obtained, a sponsor is required to comply with several post-approval requirements. For example, as a condition of approval of an NDA, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product's safety and effectiveness after commercialization. In addition, as a holder of an approved NDA, a sponsor is required to report adverse reactions and production problems to the FDA, provide updated safety and efficacy information, submit annual reports and comply with advertising and promotional labeling requirements. Additionally, any distribution of prescription drug products and pharmaceutical samples must comply with the U.S. Prescription Drug Marketing Act and the Drug Supply Chain Security Act.

Manufacturing must continue to conform to cGMP after approval, and the FDA periodically inspects manufacturing facilities to assess compliance with cGMP. Compliance with cGMP includes adhering to requirements relating to organization and training of personnel, buildings and facilities, equipment, control of components and drug product containers and closures, production and process controls, quality control and quality assurance, packaging and labeling controls, holding and distribution, laboratory controls, and records and reports. 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. FDA regulations also require investigation and correction of any deviations from cGMP and impose manufacturing documentation requirements. Accordingly, sponsors must continue to expend time, money and effort to maintain quality control and compliance with cGMP. We rely, and expect to continue to rely, on

third parties for the production of clinical and commercial quantities of ganaxolone. The FDA also conducts regular, periodic visits to re-inspect equipment, facilities, and processes following the initial approval of a product. Future FDA and state inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct. In addition, discovery of previously unknown problems with a product or the failure to comply with applicable requirements may lead to the FDA taking enforcement actions or seeking sanctions, including fines, issuance of warning letters, civil penalties, injunctions, suspension of manufacturing operations, operating restrictions, withdrawal of FDA approval, seizure or recall of products, and criminal prosecution. Although we periodically monitor the FDA compliance of our third-party manufacturers, we cannot be certain that our present or future third-party manufacturers will consistently comply with cGMP and other applicable FDA regulatory requirements.

The FDA and other federal regulatory agencies closely regulate the marketing and promotion of drugs through, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities, and promotional activities involving the Internet. A product cannot be commercially promoted before it is approved. After approval, product promotion can include only those claims relating to safety and effectiveness that are consistent with the labeling approved by the FDA. Healthcare providers are permitted to prescribe drugs for "off-label" uses—that is, uses not approved by the FDA and therefore not described in the drug's labeling—because the FDA does not regulate the practice of medicine. However, FDA regulations impose stringent restrictions on manufacturers' communications regarding off-label uses. In general, a manufacturer may not promote a drug for off-label use, but may engage in non-promotional, balanced communication regarding off-label use under specified conditions. Failure to comply with applicable FDA requirements and restrictions in this area may subject a company to adverse publicity and enforcement action by the FDA, the U.S. Department of Justice (DOJ) or the Office of the Inspector General of the U.S. Department of Health and Human Services (HHS OIG), as well as state authorities. Enforcement action could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products.

NDA holders must comply with other regulatory requirements, including submitting annual reports, reporting information about adverse drug experiences, and maintaining certain records.

Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings, contraindications, or limitations of use, 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.

The Hatch-Waxman Amendments to the FDC Act

Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product or a method of using the product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be referenced by potential generic competitors in support of approval of an abbreviated new drug application (ANDA) or 505(b)(2) application. An ANDA provides for marketing of a drug product that has the same active ingredients, generally in the same strengths and dosage form, as a referenced listed drug (RLD) and has been shown through PK testing to be bioequivalent to the RLD. Other than the requirement for bioequivalence testing, ANDA applicants are generally not required to conduct, or submit results of, preclinical studies or clinical trials to prove the safety or effectiveness of their drug product. 505(b)(2) applications provide for marketing of a drug product that may have the same active ingredients as the reference drug and contains full safety and effectiveness data, but at least some of this information comes from studies not conducted by or for the applicant and to which the applicant does not have a right of reference. Drugs approved through an ANDA are commonly referred to as "generic equivalents" and can often be substituted by pharmacists under prescriptions written for the RLD, depending on applicable state laws.

The ANDA or 505(b)(2) applicant is required to certify to the FDA concerning any patents listed for the reference product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA or 505(b)(2) applicant may also elect to submit a statement certifying that its proposed ANDA label does not contain (or carves out) any language regarding a patented method of use or use covered by regulatory exclusivity. If the applicant does not challenge the listed patents by filing a certification that the listed patent is invalid or will not be infringed by the new product, the ANDA or 505(b)(2) application will not be approved until all the listed patents claiming the referenced product have expired.

A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA or 505(b)(2) applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA or 505(b)(2) application has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA or 505(b)(2) application until the earliest of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA or 505(b)(2) applicant. The ANDA or 505(b)(2) application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the reference product has expired.

Marketing Exclusivity

Upon NDA approval of a new chemical entity, which is a drug that contains no active moiety that has been approved by the FDA in any other NDA, that drug receives five years of marketing exclusivity during which the FDA cannot approve any ANDA seeking approval of a generic version of that drug. Certain changes to a drug, such as the addition of a new indication to the package insert, are associated with a three-year period of exclusivity during which the FDA cannot approve an ANDA for a generic drug that includes the change. An ANDA may be submitted one year before five-year marketing exclusivity expires if a Paragraph IV certification is filed. In this case, the 30-month stay, if applicable, runs from the end of the five-year marketing exclusivity period.

In the European Economic Area (EEA), which is comprised of twenty-seven Member States of the EU plus Norway, Iceland, and Liechtenstein, medicinal products can only be commercialized after a related Marketing Authorization (MA) has been granted. MA for medicinal products can be obtained through several different procedures. These procedures include a centralized, mutual recognition procedure, decentralized procedure, or national procedure (if marketing authorization is sought for a single EU Member State). The centralized procedure allows a company to submit a single application to the European Medicines Agency (EMA). If a related positive opinion is provided by the EMA, the European Commission will grant a centralized marketing authorization that is valid in all twenty-seven EU Member States and three of the four European Free Trade Association countries (Norway, Iceland, and Liechtenstein) all of whom are part of the EEA.

The centralized procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products, and medicinal products containing a new active substance indicated for the treatment of HIV, AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions and viral diseases. The centralized procedure is optional for products containing a new active substance that is not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or for which a grant of centralized marketing authorization is in the interest of patients at EU level within the EU.

In the EU, a medicinal product containing a new active substance, which has never been approved in a medicinal product in the EU before, as well as in certain other circumstances, is entitled to eight years of data exclusivity and ten years of market exclusivity following a grant of MA. During the first eight years, no generic company may refer to the data used by the innovator to obtain a marketing authorization. After eight years, generics may reference the innovator data, but generic medicinal products may only be placed on the market after a total of ten years. Approval of a new indication will not result in a separate additional period of regulatory data protection and market exclusivity. If,

however, during the first eight years after initial marketing authorization, a new indication is approved which is considered by the competent authorities to be of significant clinical benefit in comparison to existing therapies, this would result in one additional year of market exclusivity, in addition to the initial eight plus two years. Such significant clinical benefit would generally have to be supported by comparative clinical trials.

Patent Term Extension

After NDA approval, owners of relevant drug patents may apply for up to a five year patent extension. The allowable patent term extension is calculated as half of the drug's testing phase—the time between an effective IND and NDA submission—and all of the review phase—the time between NDA submission and approval, up to a maximum of five years. The time can be shortened if the FDA determines that the applicant did not pursue approval with due diligence. The total patent term after the extension may not exceed 14 years.

Many other countries also provide for patent term extensions or similar extensions of patent protection for pharmaceutical products. For example, in Japan, it may be possible to extend the patent term for up to five years and in the EU, it may be possible to obtain a supplementary protection certificate that would effectively extend patent protection for up to five years.

In the EU, if pediatric studies are conducted in accordance with a pediatric investigation plan that was previously agreed upon with the EMA, it may be possible to obtain an extension of a supplementary protection certificate of up to six months. This pediatric extension would not be available if the product is an orphan medicinal product. The extension would also not be available if one additional year of market exclusivity was granted for a new pediatric indication on the basis of the results of pediatric studies conducted in compliance with an agreed pediatric investigation plan.

Orphan Drug Designation

The FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the U.S., or, if the disease or condition affects more than 200,000 individuals in the U.S., there is no reasonable expectation that the cost of developing and making the drug would be recovered from sales in the U.S. In the EU, the EMA's Committee for Orphan Medicinal Products assesses applications for orphan designations after which the European Commission may grant orphan drug designation. In the EU, orphan designation is granted if it is established that a medicinal product is intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU. In addition, designation is granted for products intended for the diagnosis, prevention or treatment of a life- threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the EU would be sufficient to justify the necessary investment in developing the drug. In order to obtain orphan designation in the EU, it must be established that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in the EU or if such method exists, that the medicinal product will be of significant benefit to those affected by the condition.

In the U.S., orphan drug designation may confer eligibility for financial incentives, such as opportunities for grant funding towards clinical trial costs, tax credits for certain research and user fee waivers. In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to seven years of market exclusivity. The FDA has interpreted the statutory provisions for orphan drug exclusivity to mean that the FDA may not approve any other application for the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition.

In the EU, orphan drug designation may be granted to drugs that can be used to treat life-threatening diseases or chronically debilitating conditions with an incidence of no more than five in 10,000 people or, for economic reasons, would be unlikely to be developed without incentives. Orphan drug designation also entitles an applicant for MA to financial incentives such as reduction of fees or fee waivers, and protocol assistance, a type of scientific advice specific

for designated orphan medicinal products. Following a grant of MA, the product is entitled to ten years of exclusivity if the product continues to be designated as an orphan medical product upon grant of the marketing authorization. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is not sufficiently profitable to justify maintenance of market exclusivity. During the orphan exclusivity period, the competent authorities in the EU may not accept a marketing authorization application for a similar medicinal product for the same therapeutic indication. However, marketing authorization may be granted to a similar medicinal product (i.e., a medicinal product with an identical active substance, or an active substance with the same principal molecular structural features and which acts via the same mechanism) with the same orphan indication during the ten-year period with the consent of the marketing authorization holder for the original orphan medicinal product or if the manufacturer of the original orphan medicinal product is unable to supply sufficient quantities. Marketing authorization may also be granted to a similar medicinal product with the same orphan indication if this latter product is demonstrated to be safer, more effective or otherwise clinically superior to the original orphan medicinal product.

In the EU, if pediatric studies are conducted in accordance with a pediatric investigation plan that was previously agreed upon with the European Medicines Agency, it may be possible to obtain an extension of orphan market exclusivity of two years, resulting in a total orphan market exclusivity period of twelve years.

Orphan drug designation must be requested before submission of an application for marketing approval. Orphan drug designation does not change the scientific/medical standards for approval or the quality of evidence necessary to support approval, or shorten the duration of the regulatory review and approval process.

Controlled Substances

The federal Controlled Substances Act of 1970 (CSA) and its implementing regulations establish a “closed system” of regulations for controlled substances. The CSA imposes registration, security, recordkeeping and reporting, storage, manufacturing, distribution, importation and other requirements under the oversight of the DEA. The DEA is the federal agency responsible for regulating controlled substances, and requires those individuals or entities that manufacture, import, export, distribute, research, or dispense controlled substances to comply with the regulatory requirements in order to prevent the diversion of controlled substances to illicit channels of commerce.

The DEA categorizes controlled substances into one of five schedules — Schedule I, II, III, IV or V — with varying qualifications for listing in each schedule. Schedule I substances by definition have a high potential for abuse, have no currently accepted medical use in treatment in the U.S. and lack accepted safety for use under medical supervision. Pharmaceutical products having a currently accepted medical use that are otherwise approved for marketing may be listed as Schedule II, III, IV or V substances, with Schedule II substances presenting the highest potential for abuse and physical or psychological dependence, and Schedule V substances presenting the lowest relative potential for abuse and dependence.

Facilities that manufacture, distribute, import or export any controlled substance must register annually with the DEA. The DEA registration is specific to the particular location, activity(ies) and controlled substance schedule(s). For example, separate registrations are required for importation and manufacturing activities, and each registration authorizes which schedules of controlled substances the registrant may handle. However, certain coincident activities are permitted without obtaining a separate DEA registration, such as distribution of controlled substances by the manufacturer that produces them.

The DEA inspects all manufacturing facilities to review security, recordkeeping, reporting and handling prior to issuing a controlled substance registration. The specific security requirements vary by the type of business activity and the schedule and quantity of controlled substances handled. The most stringent requirements apply to manufacturers of Schedule I and Schedule II substances. Required security measures commonly include background checks on employees and physical control of controlled substances through storage in approved vaults, safes and cages, and thorough use of alarm systems and surveillance cameras. Registrants must also report any controlled substance thefts or significant losses, and must comply with CSA and DEA regulatory requirements to destroy or dispose of controlled substances.

The states also maintain separate controlled substance laws and regulations, including licensing, recordkeeping, security, distribution, and dispensing requirements. State authorities, including Boards of Pharmacy, regulate use of controlled substances in each state. Failure to maintain compliance with applicable requirements, particularly as manifested in the loss or diversion of controlled substances, can result in enforcement action that could have a material adverse effect on our business, operations and financial condition. The DEA may seek civil penalties, refuse to renew necessary registrations, or initiate proceedings to revoke those registrations. In certain circumstances, violations could lead to criminal prosecution.

The Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act (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 U.S. to comply with accounting provisions requiring such companies 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.

European and Other International Government Regulation

In addition to regulations in the U.S., we are subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. 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. Some countries outside of the U.S. have a similar process that requires the submission of a request for a clinical trial authorization (CTA) much like the IND prior to the commencement of human clinical trials. In the EU, for example, a request for a CTA must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the CTA request is approved in accordance with a country's requirements, clinical trial development may proceed. The conduct of a clinical trial in the EU must comply with regulatory requirements based on the Clinical Trials Directive 2001/20/EC (Clinical Trials Directive), the details of which may vary per EU Member State. On January 31, 2022, the EU Clinical Trials Regulation (EU) No 536/2014 (Clinical Trials Regulation) came into effect. The Clinical Trials Regulation applies to clinical trials in all countries of the European Economic Area (EEA, i.e. the EU Member States plus Iceland, Norway and Liechtenstein). The Clinical Trials Regulation allows investigators to start and conduct a clinical trial in accordance with the Clinical Trials Directive during a transitional period of one year after the application date (i.e. January 31, 2022). Clinical trials that were authorized under the current Clinical Trials Directive before January 31, 2023 can continue to be conducted under the Clinical Trials Directive until January 31, 2025. An application to transition ongoing trials from the current Clinical Trials Directive to the new Clinical Trials Regulation will need to be submitted and authorized in time before the end of the transitional period. The new Clinical Trials Regulation is intended to simplify and streamline the approval of clinical trials in the EEA. The main characteristics of the regulation include: (i) a streamlined application procedure through a single entry point known as the "EU portal"; (ii) a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors; and (iii) a harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts.

In addition, when conducting a clinical trial in the EU, the processing of personal data, including pseudonymized data, must comply with the EU General Data Protection Regulation (GDPR). The GDPR imposes strict obligations on the processing of personal data, including relating to the transfer of personal data to third countries such as the US. The competent authorities of the EU Member States may impose significant financial penalties in the event of violation of the GDPR.

To obtain regulatory approval to commercialize a new drug under EU regulatory systems, we must submit a marketing authorization application (MAA). MAAs can be submitted to the EMA through a centralized procedure, resulting in one marketing authorization valid throughout the EU (27 EU Member States as well as in Iceland, Liechtenstein and Norway). The centralized procedure is mandatory for certain products, such as orphan medicinal products or products with a new active substance for certain therapeutic indications and is optional for certain other

products, such as products that contain a new active substance that has not previously been approved in a medicinal product in the EU. Alternative MAA routes in the EU are the decentralized procedure in which it is possible to request marketing authorization in a selection of various EU Member States, the national procedure in which a marketing authorization is requested for one EU Member State only or the mutual recognition procedure in which marketing authorization in one or more EU Member States is requested on the basis of a prior marketing authorization in another EU Member State.

For other countries outside of the EU, such as countries in Eastern Europe, Russia, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. Internationally, clinical trials are generally required to be conducted in accordance with GCP, applicable regulatory requirements of each jurisdiction and the medical ethics principles that have their origin in the Declaration of Helsinki.

Small Medium Enterprise (SME) designation

In the EU, small medium enterprise designation (SME) can be granted to non-subsidiary, independent firms which employ fewer than 250 employees to promote innovation and the development of new medicinal products by smaller companies. The criteria for designation are dependent on staff headcount, either turnover or balance sheet total and the ownership structure, including any partnership or linkage. Benefits of SME designation include direct assistance on regulatory aspects of the pharmaceutical legislation, help navigating the array of services available, fee exemptions and reductions for pre- and post-authorization regulatory procedures, assistance with translations of product information into all official EU languages, guidance on clinical data publication and a free redaction tool license, liaison with academic investigators in pediatric-medicine research through the European Network of Pediatric Research at the EMA and workshops and training sessions. In 2021, we renewed our SME designation in the EU.

Compliance

During all phases of development (pre- and post-marketing), failure to comply with applicable regulatory requirements may result in administrative or judicial sanctions. These sanctions could include the FDA's imposition of a clinical hold on trials, refusal to approve pending applications, withdrawal of an approval, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, product detention or refusal to permit the import or export of products, injunctions, fines, civil penalties or criminal prosecution. Any agency or judicial enforcement action could have a material adverse effect on us.

Accelerated Review (EU)

Under the Centralized Procedure in the EU, the maximum timeframe for the evaluation of a MAA is 210 days (excluding "clock stops," when additional written or oral information is to be provided by the applicant in response to questions asked by the Committee for Medicinal Products for Human Use (CHMP)). Accelerated evaluation might be granted by the CHMP in exceptional cases, such as when a medicinal product is expected to be of a major public health interest, which should be justified on a case-by-case basis. In this circumstance, the EMA ensures that the opinion of the CHMP is given within 150 days upon submission of an MAA.

Healthcare Reform

The Patient Protection and Affordable Care Act, as amended (Affordable Care Act), has substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacts the pharmaceutical industry. The Affordable Care Act has impacted pre-existing government healthcare programs and resulted in the development of new programs. For example, the Affordable Care Act provides for Medicare payments for performance initiatives and improvements to Medicare physician quality reporting system and feedback program.

Among the Affordable Care Act's provisions of importance to the pharmaceutical industry are the following:

- an annual, nondeductible fee on any covered entity engaged in manufacturing or importing certain branded prescription drugs and biological products, apportioned among such entities in accordance with their respective market share in certain government healthcare programs;

- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, retroactive to January 1, 2010, to 23.1% and 13.0% of the average manufacturer price (AMP), for most branded and generic drugs, respectively;

- expansion of healthcare fraud and abuse laws, including the False Claims Act (FCA) and the Anti-Kickback Statute (AKS), new government investigative powers, and enhanced penalties for noncompliance;

- a new prescription drug benefit for Medicare recipients (Medicare Part D), coverage gap discount program, in which manufacturers must agree to offer 70.0% (as of January 1, 2019) 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 (the Inflation Reduction Act of 2022 (IRA) replaces the coverage gap discount program with a new manufacturer discount program beginning in 2025);

- extension of manufacturers' Medicaid rebate liability to covered outpatient 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 beginning in 2014 and by adding new mandatory eligibility categories for individuals with income at or below 133.0% of the Federal Poverty Level, thereby potentially increasing manufacturers' Medicaid rebate liability;

- expansion of the types of entities eligible for participation in and discounts under the Public Health Service 340B drug pricing program;

- new requirements to report annually specified financial arrangements with physicians and teaching hospitals, as defined in the Affordable Care Act and its implementing regulations, including reporting any "payments or transfers of value" made or distributed to physicians and teaching hospitals, and reporting any ownership and investment interests held by physicians and their immediate family members and applicable group purchasing organizations during the preceding calendar year, with data collection to be required beginning August 1, 2013 and reporting to the Centers for Medicare & Medicaid Services (CMS), to be required by March 31, 2014, and by the 90th day of each subsequent calendar year;

- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians;

- 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

- a mandatory nondeductible payment for employers with 50 or more full-time employees (or equivalents) who fail to provide certain minimum health insurance coverage for such employees and their dependents, beginning in 2016.

Certain provisions of the Affordable Care Act have been subject to judicial challenges as well as efforts to modify them or to alter their interpretation and implementation. For example, the Tax Cuts and Jobs Act (Tax Act), enacted on December 22, 2017, eliminated the tax-based shared responsibility payment for individuals who fail to

maintain minimum essential coverage under section 5000A of the Internal Revenue Code of 1986, commonly referred to as the "individual mandate," effective January 1, 2019. Additional legislative changes, regulatory changes, and judicial challenges related to the Affordable Care Act remain possible. It is unclear how the Affordable Care Act and its implementation, as well as efforts to modify the Affordable Care Act, its implementing regulations, or portions thereof, will affect our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, the Budget Control Act of 2011, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee did not achieve its targeted deficit reduction, which triggered the legislation's automatic reductions to several government programs. These reductions include aggregate reductions to Medicare payments to healthcare providers of, on average, 2.0% per fiscal year, starting in 2013 and continuing through 2031. Sequestration is currently set at 2% and will increase to 2.25% for the first half of fiscal year 2030, to 3% for the second half of fiscal year 2030, and to 4% for the remainder of the sequestration period that lasts through the first six months of fiscal year 2031. Additionally, the American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several categories of healthcare providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

Further, on August 16, 2022, President Biden signed into law the IRA which, among other things, establishes a Medicare Part B inflation rebate scheme, under which, generally speaking, manufacturers will owe rebates if the average sales price of a Part B drug increases faster than the pace of inflation. Failure to timely pay a Part B inflation rebate is subject to a civil monetary penalty. The IRA also establishes a Medicare Part D inflation rebate scheme, under which generally speaking manufacturers will owe rebates if the average manufacturer price of a Part D drug increases faster than the pace of inflation. Failure to timely pay a Part D inflation rebate is subject to a civil monetary penalty. The IRA also creates a drug price negotiation program under which the prices for Medicare units of certain high Medicare spend drugs and biologics without generic or biosimilar competition will be capped by reference to, among other things, a specified non-federal average manufacturer price, starting in 2026. Failure to comply with requirements under the drug price negotiation program is subject to an excise tax and/or a civil monetary penalty. The IRA further makes changes to the Medicare Part D benefit, including a limit on annual out-of-pocket costs, and a change in manufacturer liability under the program which could negatively affect the profitability of our product candidates. Failure to pay a discount under this new program will be subject to a civil monetary penalty. Congress continues to examine various policy proposals that may result in pressure on the prices of prescription drugs in the government health benefit programs. The IRA or other legislative changes could impact the market conditions for our product candidates.

We anticipate that the Affordable Care Act and other healthcare reform measures, including those enacted in the future, 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 payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products. In addition, it is possible that there will be further state and federal healthcare reform measures adopted in the future, any of which could limit the amounts that state and federal governments will pay for healthcare products and services, which could result in reduced demand for our products or additional pricing pressure.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we obtain regulatory approval. In the U.S. and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of reimbursement from third-party payers. Third-party payers include government health administrative authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payer will provide coverage for a drug product may be separate from the process for setting the price or reimbursement rate that the payer will pay for the drug product. Third-party payers may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drugs for a particular indication. Third-party payers are increasingly challenging the price and

examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain FDA approvals. Ganaxolone may not be considered by payers to be medically necessary or cost-effective for particular diseases or conditions. A payer's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

In addition, Medicare Part D and further legislation may limit payments for pharmaceuticals such as the product candidates that we are developing. While government payment pursuant to Medicare Part D for some of the costs of prescription drugs may increase demand for any products for which we receive marketing approval, to obtain payments under this program, we would be required to sell products to Medicare recipients through prescription drug plans operating pursuant to this legislation. These plans typically negotiate discounted prices for our products. Federal, state and local governments in the U.S. continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs. The IRA has made a number of changes to the Part D program, set to take effect in 2024 and 2025.

Different pricing and reimbursement schemes exist in other countries. In the EU, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national healthcare 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 upon. 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 product 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 healthcare costs in general, particularly prescription drugs, has become more intense. As a result, increasingly high barriers are being erected to the entry of new products. The EU provides options for its Member States to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. We may face competition for ganaxolone from lower-priced products in foreign countries that have placed price controls on pharmaceutical 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 any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payers fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on managed care in the U.S. has increased and 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 one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Other Healthcare Laws and Compliance Requirements

The federal Anti-Kickback Statute (AKS) prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration (i.e., anything of value), directly or indirectly, in cash or in kind, to induce or in return either for the referral of an individual for, or for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting certain common business arrangements and activities from prosecution or regulatory sanctions, the exceptions and safe harbors are drawn narrowly and practices that involve remuneration intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not fit squarely within an exception or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from AKS liability. Moreover, there are no safe harbors for many common practices, such as educational and research grants,

charitable donations, product support and patient assistance programs. The regulatory safe harbors also are subject to regulatory revision and interpretation by a number of government agencies. Liability under the AKS may be established without proving actual knowledge of the statute or specific intent to violate it. In addition, the government may assert that a claim including items or services resulting from a violation of the AKS constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act (discussed below). Violations of the AKS are punishable by imprisonment, criminal fines, damages, civil monetary penalties, and exclusion from participation in federal healthcare programs.

The federal civil False Claims Act (FCA) prohibits any person from, among other things, knowingly presenting, or causing to be presented, a false or fraudulent claim for payment of government funds, or knowingly making, using, or causing to be made or used a false record or statement material to an obligation to pay money to the government, or knowingly concealing or knowingly and improperly avoiding, decreasing, or concealing an obligation to pay money to the federal government. Actions under the FCA may be brought by the Attorney General or as a *qui tam* action by a private individual in the name of the government. Such private individuals may share in amounts paid by the entity to the government in recovery or settlement. Recently, several 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. Other companies have been prosecuted for causing false claims to be submitted because of the companies' marketing of the product for unapproved, and thus non-reimbursable, uses. FCA liability is potentially significant in the healthcare industry because the statute provides for treble damages and significant mandatory penalties per false or fraudulent claim or statement for violations, as well as exclusion from participation in federal healthcare programs. Pharmaceutical and other healthcare companies also are subject to other federal false claims laws, including, among others, federal criminal healthcare fraud and false statement statutes that extend to non-government health benefit programs.

The Health Insurance Portability and Accountability Act of 1996 and its implementing regulations (collectively, HIPAA) imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payers, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement or representation, or making or using any false writing or document knowing the same to contain any materially false, fictitious, or fraudulent statement or entry, in connection with the delivery of or payment for healthcare benefits, items or services.

The federal Physician Payments Sunshine Act, implemented as the Open Payments Program, requires certain manufacturers of drugs, devices, biologics 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 and other transfers of value to physicians (defined to include doctors, dentists, optometrists, podiatrists, and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. As of 2022, applicable manufacturers are required to report information regarding payments and transfers of value provided to physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, and certified nurse-midwives.

Also, many states have analogous fraud and abuse statutes or regulations, such as state anti-kickback and false claims laws, that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. Other state laws require posting of information relating to clinical trials and their outcomes. Some states restrict the ability of manufacturers to offer co-pay support to patients for certain prescription drugs. Other states require identification or licensing of sales representatives.

In addition, we may be subject to data privacy and security regulations by both the federal government and the states in which we conduct our business. HIPAA imposes requirements relating to the privacy, security and transmission of individually identifiable health information. HIPAA imposes privacy and security obligations on covered entity health care providers, health plans, and health care clearinghouses, as well as their "business associates"—certain persons or

entities that create, receive, maintain, or transmit protected health information in connection with providing a specified service or performing a function on behalf of a covered entity. We may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA and other privacy and data security and consumer protection laws. Although we are not directly subject to HIPAA, other than potentially with respect to providing certain employee benefits, we could potentially be subject to criminal penalties if we, our affiliates, or our agents knowingly receive individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA, and subject to other civil and/or criminal penalties if we obtain, use, or disclose information in a manner not permitted by other privacy and data security and consumer protection laws. In addition, numerous other federal and state laws and regulations govern privacy and security, including state data breach notification laws, state health information and/or genetic privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the Federal Trade Commission Act, and the California Consumer Privacy Act (CCPA)), many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. Compliance with these laws is difficult, constantly evolving, time consuming, and requires a flexible privacy framework and substantial resources. Compliance efforts will likely be an increasing and substantial cost in the future. Federal regulators, state attorneys general, and plaintiffs' attorneys have been and will likely continue to be active in this space.

In California, the CCPA establishes certain requirements for data use and sharing transparency and creates new data privacy rights for California consumers. The CCPA and its implementing regulations have already been amended multiple times since their enactment, including by the California Privacy Rights Act, or CPRA. The CPRA introduced significant amendments to the CCPA and established and funded a dedicated California privacy regulator, the California Privacy Protection Agency, or CPPA. The amendments introduced by the CPRA went into effect on January 1, 2023, and new implementing regulations are expected to be introduced by the CPPA. Failure to comply with the CCPA may result in, among other things, significant civil penalties and injunctive relief, or statutory or actual damages. In addition, California residents have the right to bring a private right of action in connection with certain types of incidents. These claims may result in significant liability and damages. These laws and regulations are evolving and subject to interpretation, and may impose limitations on our activities or otherwise adversely affect our business. Similarly, there are a number of legislative proposals in the EU, the United States, at both the federal and state level, as well as other jurisdictions that could impose new obligations or limitations in areas affecting our business. For example, other states, including Virginia, Colorado, Utah, and Connecticut have enacted privacy laws similar to the CCPA that impose new obligations or limitations in areas affecting our business and we continue to assess the impact of these state legislation, on our business as additional information and guidance becomes available. In addition, some countries are considering or have passed legislation implementing data protection requirements or requiring local storage and processing of data or similar requirements that could increase the cost and complexity of delivering our services and research activities. These laws and regulations, as well as any associated claims, inquiries, or investigations or any other government actions may lead to unfavorable outcomes including increased compliance costs, delays or impediments in the development of new products, negative publicity, increased operating costs, diversion of management time and attention, and remedies that harm our business, including fines or demands or orders that we modify or cease existing business practices.

The EU, EEA countries and other jurisdictions have adopted data protection laws and regulations, which impose significant compliance obligations. The GDPR is directly applicable in each EEA country. The GDPR imposes strict restrictions and obligations on companies' ability to collect, analyze and transfer personal data or otherwise process personal data, especially if they process sensitive personal data (such as data concerning patient health), including significant fines for non-compliance with the GDPR. Implementation of the GDPR has influenced other jurisdictions to either amend or propose legislation to amend their existing data privacy and cybersecurity laws to resemble the requirements of GDPR.

With regard to transfer of personal data, the GDPR restricts the ability of companies to transfer personal data from the EEA to the U.S. and other countries, which may adversely affect our ability to transfer personal data or otherwise may cause us to incur significant costs to come into compliance with applicable data transfer impact assessments and implementation of legal data transfer mechanisms. One mechanism previously relied upon by U.S. companies for such transfers was the EU-U.S. Privacy Shield Framework, or Privacy Shield. However, in July 2020, the European Court of Justice ruled the Privacy Shield to be an invalid data transfer mechanism and confirmed that the European Commission's Standard Contractual Clauses, or the Model Clauses, remain valid and in June 2021, the

European Commission published updated versions of the Model Clauses, which must be incorporated into new and existing agreements within prescribed timeframes in order to continue to lawfully transfer personal data outside of the EU. As a result, companies may no longer rely on the Privacy Shield as a basis on which to transfer personal data from the EU to the U.S. U.S.-based companies are permitted to rely on other authorized means and procedures to transfer personal data provided by the GDPR. The Model Clauses may also come under increased scrutiny as a result of the European Court of Justice's judgement in July 2020, though they remain the most common authorized procedure to transfer personal data out of the EU. On December, 13 2022, the European Commission adopted a draft adequacy decision for the EU-U.S. Data Privacy Framework. This draft decision follows the signature of a US Executive Order by President Biden on October, 7 2022, along with the regulations issued by the US Attorney General Merrick Garland. These two instruments implemented into U.S. law the agreement in principle announced by President von der Leyen and President Biden in March 2022. The draft adequacy decision, which reflects the assessment by the European Commission of the U.S. legal framework has now been published and transmitted to the European Data Protection Board, or EDPB, for its opinion. The draft decision concludes that the United States ensures an adequate level of protection for personal data transferred from the EU to U.S. companies. The draft adequacy decision text will also have to be approved by a committee composed of representatives of the EU Member States and the European Parliament can exercise its right of scrutiny. After this process, the European Commission is then expected to adopt the final adequacy decision, which will allow data to flow freely from the EU to the U.S. After one year from the notification date of the adequacy decision to the Member States and subsequently at least every four years, the European Commission will carry out a new evaluation and could conclude that an adequate level of protection is no longer ensured and decide to suspend, amend or repeal the adequacy decision, or limit its scope.

In addition to the foregoing requirements, we have certain price reporting obligations to the Medicaid Drug Rebate Program. Under the Medicaid Drug Rebate Program, we are required to pay a rebate to each state Medicaid program for our covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state Medicaid program as a condition of having federal funds made available to states for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data that we report on a monthly and quarterly basis to CMS, the federal agency that administers the Medicare and Medicaid Drug Rebate Programs. These data include the average manufacturer price and, in the case of innovator products, the best price for each drug which, in general, represents the lowest price available from the manufacturer to any entity in the U.S. in any pricing structure, calculated to include all sales and associated rebates, discounts and other price concessions, subject to certain exclusions. The Affordable Care Act made significant changes to the Medicaid Drug Rebate Program, and CMS issued a final regulation, which became effective on April 1, 2016, to implement the changes to the Medicaid Drug Rebate Program under the Affordable Care Act. On December 21, 2020, CMS issued a final regulation that (i) modified existing Medicaid Drug Rebate Program regulations to permit reporting multiple best price figures with regard to value-based purchasing arrangements and (ii) provided definitions for "line extension," "new formulation," and related terms with the practical effect of expanding the scope of drugs considered to be line extensions, with such changes taking effect in 2022. Our failure to comply with the aforementioned price reporting and rebate payment obligations, as well as pharmacy benefit manager (PBM) "accumulator" programs, could negatively impact our financial results. In addition, statutory and regulatory changes or other agency action regarding the Medicaid Drug Rebate Program could negatively affect our financial results or expand our rebate liability.

Federal law requires that any company that participates in the Medicaid Drug Rebate Program also participate in the 340B program in order for federal funds to be available for the manufacturer's drugs under Medicaid and Medicare Part B. The 340B program, which is administered by the Health Resources and Services Administration (HRSA) requires participating manufacturers to agree to charge statutorily defined covered entities no more than the 340B "ceiling price" for the manufacturer's covered outpatient drugs. These 340B covered entities include a variety of community health clinics and other entities that receive health services grants from the Public Health Service, as well as hospitals that serve a disproportionate share of low-income patients. The Affordable Care Act expanded the list of covered entities to include certain free-standing cancer hospitals, critical access hospitals, rural referral centers and sole community hospitals, but exempts "orphan drugs" from the ceiling price requirements for these covered entities. The 340B ceiling price is calculated using a statutory formula based on the average manufacturer price and rebate amount for the covered outpatient drug as calculated under the Medicaid Drug Rebate Program, and in general, products subject to Medicaid price reporting and rebate liability are also subject to the 340B ceiling price calculation and discount requirement. Any additional future changes to the definition of average manufacturer price and the Medicaid rebate

amount under the Affordable Care Act or other legislation or regulation could affect our 340B ceiling price calculations and negatively impact our results of operations if we successfully commercialize one or more products for which we receive regulatory approval.

HRSA issued a final regulation, effective January 1, 2019, regarding the calculation of the 340B ceiling price and the imposition of civil monetary penalties on manufacturers that knowingly and intentionally overcharge covered entities. HRSA also has implemented a ceiling price reporting requirement, pursuant to which manufacturers must report the 340B ceiling prices for their covered outpatient drugs to HRSA on a quarterly basis. HRSA then publishes those prices to 340B covered entities. Moreover, under a final regulation effective January 13, 2021, HRSA newly established an administrative dispute resolution (ADR) process for claims by covered entities that a manufacturer has engaged in overcharging, and by manufacturers that a covered entity violated the prohibitions against diversion or duplicate discounts. Such claims are to be resolved through an ADR panel of government officials rendering a decision that could be appealed only in federal court. An ADR proceeding could subject a manufacturer to onerous procedural requirements and could result in additional liability. In addition, changes to legislation, regulations, or guidance could modify 340B program compliance or expand discount liability.

Federal law also requires that a company report average sales price information each quarter to CMS for certain categories of drugs that are payable under the Medicare Part B program. Manufacturers calculate the average sales price based on a statutorily defined formula as well as regulations and interpretations of the statute by CMS. CMS uses these submissions to determine payment rates for drugs under Medicare Part B. Beginning in 2023, manufacturers must pay refunds to Medicare for single source drugs or biologics, or biosimilar biological products, reimbursed under Medicare Part B and packaged in single-dose containers or single-use packages, for units of discarded drug reimbursed by Medicare Part B in excess of 10% of total allowed charges under Medicare Part B for that drug (or a percentage established for drugs with unique circumstances). Manufacturers that fail to pay refunds could be subject to civil monetary penalties of 125% of the refund amount. The IRA establishes a Medicare Part B inflation rebate scheme, under which, generally speaking, manufacturers will owe rebates if the average sales price of a Part B drug increases faster than the pace of inflation. Failure to timely pay a Part B inflation rebate is subject to a civil monetary penalty.

In addition, manufacturers are required to provide to CMS a 70% discount on brand name prescription drugs utilized by Medicare Part D beneficiaries when those beneficiaries are in the coverage gap phase of the Part D benefit design. The IRA replaces the coverage gap discount program with a new manufacturer discount program beginning in 2025. Under either program, civil monetary penalties could be applied if a manufacturer fails to provide these discounts in the amount of 125% of the discount that was due. Moreover, the IRA also establishes a Medicare Part D inflation rebate scheme, under which, generally speaking, manufacturers will owe rebates if the average manufacturer price of a Part D drug increases faster than the pace of inflation. Failure to timely pay a Part D inflation rebate is subject to a civil monetary penalty.

In order to be eligible to have our products that we successfully commercialize paid for with federal funds under the Medicaid and Medicare Part B programs and purchased by certain federal agencies and grantees, we also would have to participate in the U.S. Department of Veterans Affairs (VA), Federal Supply Schedule (FSS), pricing program. As part of this program, we would be obligated to make our products available for procurement on an FSS contract under which we must comply with standard government terms and conditions and charge a price that is no higher than the statutory Federal Ceiling Price (FCP) to four federal agencies (VA, U.S. Department of Defense (DOD), Public Health Service, and U.S. Coast Guard).

The FCP is based on the Non-Federal Average Manufacturer Price (Non-FAMP), which we would be required to calculate and report to the VA on a quarterly and annual basis. Pursuant to applicable law, knowing provision of false information in connection with a Non-FAMP filing can subject a manufacturer to significant civil monetary penalties for each item of false information. The FSS pricing and contracting obligations also contain extensive disclosure and certification requirements. For additional information regarding obligations under federal health care programs, refer to the risk factor entitled "*We participate in the Medicaid Drug Rebate Program and if we fail to comply with our reporting and payment obligations under that program or other governmental pricing programs that we participate in, we could be subject to additional reimbursement requirements, penalties, sanctions and fines, which could have a material*

adverse effect on our business, financial condition, results of operations and growth prospects" in this Annual Report on Form 10-K.

In the U.S. our activities are potentially subject to additional regulation by various federal, state and local authorities in addition to the FDA, including CMS, other divisions of HHS (for example, the OIG), the DOJ and individual U.S. Attorney offices within the DOJ, and state and local governments.

Because of the breadth of these laws and the narrowness of available statutory and regulatory exemptions, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the federal and state laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including criminal and significant civil monetary penalties, damages, fines, imprisonment, exclusion from participation in government programs, injunctions, recall or seizure of products, total or partial suspension of production, denial or withdrawal of pre-marketing product approvals, or refusal to allow us to enter into supply contracts, including government contracts, 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. To the extent that any of our products are sold in a foreign country, we may be subject to similar foreign laws and regulations, which may include, for instance, applicable post-marketing requirements, including safety surveillance, anti-fraud and abuse laws, and implementation of corporate compliance programs and reporting of payments or transfers of value to healthcare professionals.

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 some states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state. Several states have enacted legislation requiring pharmaceutical companies to, among other things, establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing specified physician prescribing data to pharmaceutical companies for use in sales and marketing, and to prohibit other specified sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

Human Capital

We are committed to a work environment that is welcoming, inclusive and encouraging. To achieve our plans and goals, it is imperative that we attract and retain top talent. In order to do so, we aim to have a safe and encouraging workplace, with opportunities for our employees to grow and develop professionally, supported by strong compensation, benefits, and other incentives. In addition to competitive base salaries, we offer full-time employees a cash target bonus, a comprehensive benefits package and equity compensation.

As of December 31, 2022, we had 151 full-time employees and two part-time employees. In addition to our employees, we contract with third parties for the conduct of certain clinical development, manufacturing, accounting and administrative activities. We anticipate increasing the number of our employees as we continue to grow as a commercial organization and increase our research and development. We have no collective bargaining agreements with our employees, and none are represented by labor unions.

Throughout the COVID-19 pandemic, most of our employees have been working remotely. We implemented a number of significant safety measures based on current guidelines recommended by the Centers for Disease Control for employees who choose to work at our facilities.

Corporate Information

We were incorporated in Delaware in August 2003. Our principal executive offices are located at 5 Radnor Corporate Center, Suite 500, 100 Matsonford Rd, Radnor, Pennsylvania 19087 and our telephone number is (484) 801-4670. Our website address is *www.marinuspharma.com*. The inclusion of our website address is, in each case, intended to be an inactive textual reference only and not an active hyperlink to our website. The information contained in, or that

can be accessed through, our website is not part of this Annual Report on Form 10-K. We make available free of charge on our website, Form 10-Ks, Form 10-Qs, Form 8-Ks and amendments to those reports as soon as reasonably practicable after filing with or furnishing to the Securities and Exchange Commission (SEC).

Item 1A. Risk Factors

Our business is subject to substantial risks and uncertainties. The occurrence of any of the following risks and uncertainties, either alone or taken together, could materially and adversely affect our business, financial condition, results of operations or prospects. In these circumstances, the market price of our common stock could decline and you may lose all or part of your investment. The risks and uncertainties described below are not the only ones we face. Risks and uncertainties of general applicability and additional risks and uncertainties not currently known to us or that we currently deem to be immaterial may also materially and adversely affect our business, financial condition, results of operations or prospects.

Risks Related to our Financial Position and Need for Additional Capital

We have generated limited revenue to date from product sales. We may never achieve or sustain profitability, which could depress the market price of our common stock, and could cause you to lose all or a part of your investment.

To date, we have generated limited revenue from sales of ZTALMY in CDD in the U.S. and no sales from any other geographic markets or any other indications being developed for ganaxolone. The extent to which we can generate revenue from product sales and achieve profitability will depend upon our ability to successfully commercialize ZTALMY in CDD and to achieve successful clinical development of ganaxolone in the other indications we are developing or other product candidates that we may develop, in-license or acquire in the future. Our ability to generate revenue from product sales of ZTALMY, and from other indications we are developing for ganaxolone or any other future product candidates also depends on a number of additional factors, including our ability to:

- successfully complete pre-clinical and clinical development activities, including enrollment of clinical trial participants, completion of the necessary pre-clinical studies and clinical trials and attainment of study and trial results that will support regulatory approvals;
- complete and submit NDAs to the FDA, MAAs with the EMA and other marketing authorization filings with regulatory agencies in other countries, and obtain regulatory approval for indications for which there is a commercial market;
- make or have made commercial quantities of our products at acceptable cost levels;
- develop a commercial organization capable of manufacturing, selling, marketing and distributing any products we intend to sell ourselves in the markets in which we choose to commercialize on our own;
- find suitable partners to help us market, sell and distribute our approved products in other markets;
- obtain adequate pricing, coverage and reimbursement from third parties, including government and private payers;
- launch and commercialize ZTALMY in other indications being developed for ganaxolone and any other future product candidates for which we obtain regulatory approval;
- obtain market acceptance of ZTALMY in other indications being developed for ganaxolone and any other future product candidates as viable treatment options;
- address any competing technological and market developments;

- implement additional internal systems and infrastructure, as needed;
- identify and validate new product candidates;
- negotiate favorable terms in any collaboration, licensing or other commercial arrangements into which we may enter;
- resolve potential intellectual property disputes with third parties;
- maintain, protect and expand our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- attract, hire and retain qualified personnel.

In addition, because of the numerous risks and uncertainties associated with product development, including that ganaxolone may not advance through development or achieve the endpoints of applicable preclinical studies and clinical trials for ganaxolone in the other indications we are developing, we are unable to predict the timing or amount of increased expenses, or if or when we will be able to achieve or maintain profitability. Our expenses could increase beyond expectations if we are required by the FDA or other regulatory agencies, domestic or foreign, to perform preclinical studies and clinical trials or other studies in addition to those that we currently anticipate. Even if we are able to complete the development and regulatory process for ZTALMY or ganaxolone in the other indications we are developing, we anticipate incurring significant costs associated with commercializing ZTALMY, any other indications for ganaxolone or other product candidates.

Even if we are able to generate substantial revenue from the sale of ZTALMY, other indications being developed for ganaxolone or any future commercial products, we may not become profitable and will need to obtain additional funding to continue operations. If we fail to become profitable or are unable to sustain profitability on a continuing basis, and we are not successful in obtaining additional funding, then we may be unable to continue our operations at planned levels, or at all, which would likely materially and adversely affect our business and the market price of our common stock.

We will require additional capital to fund our operations and if we fail to obtain necessary financing, we may be unable to commercialize ZTALMY or complete the development and commercialization, if approved, of ganaxolone in the other indications we are developing.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to commercialize ZTALMY in CDD in the U.S. and to advance the clinical and regulatory development of ganaxolone in the other indications we are developing and, if approved, commercialize ganaxolone in those indications. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our commercialization efforts or our research and development programs.

We believe that our existing cash and cash equivalents as of December 31, 2022 will be sufficient to fund our operating expenses and capital expenditure requirements, as well as maintain the minimum cash balance required under our debt facility, into the second half of 2024. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the results of our preclinical studies and clinical trials;
- the development, formulation and commercialization activities related to ganaxolone, including ZTALMY;
- the scope, progress, results and costs of researching and developing ganaxolone, including ZTALMY, or any other future product candidates, and conducting preclinical studies and clinical trials;

- the timing of, and the costs involved in, obtaining regulatory approvals for ganaxolone, including ZTALMY, or any other future product candidates;
- the cost of commercialization activities for ZTALMY in CDD in the US, including marketing, sales and distribution costs;
- the cost of commercialization activities for ZTALMY, ganaxolone in any other indications, or any other future product candidates, are approved for sale, including marketing, sales and distribution costs;
- the cost of manufacturing and formulating ganaxolone, or any other future product candidates, to internal and regulatory standards for use in preclinical studies, clinical trials and, if approved, commercial sale;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;
- our ability to receive funding under the BARDA Contract;
- our expectations regarding the amount and timing of milestone and royalty payments owed to us pursuant to our exclusive license agreement with Orion for the commercialization of ganaxolone in Europe and our exclusive license agreement with Tenacia for the commercialization of ganaxolone in Mainland China, Hong Kong, Macau and Taiwan;
- our expectations regarding the amount and timing of milestone and royalty payments owed by us pursuant to our revenue interest financing agreement with Sagard Healthcare Royalty Partners, LP (Sagard);
- any product liability, infringement or other lawsuits related to ZTALMY or other indications being developed for ganaxolone and, if approved, products;
- capital needed to attract and retain skilled personnel;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation; and
- the timing, receipt and amount of sales of, or royalties on, ZTALMY in CDD and on future approved products, if any.

If we are unable to expand our operations or otherwise capitalize on our business opportunities due to a lack of capital, our ability to become profitable will be compromised. Failure to progress our product development or commercialization of ganaxolone as anticipated will have a negative effect on our business, future prospects and ability to obtain further financing on acceptable terms, if at all, and the value of the enterprise, which could require us to, among other things:

- significantly delay, scale back or discontinue the development or commercialization of ganaxolone or one or more of our other research and development initiatives;
- seek collaborators for one or more of our current or future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available;
- sell or license on unfavorable terms our rights to ganaxolone or one of our future product candidates that we otherwise would seek to develop or commercialize ourselves; or
- seek bankruptcy protection.

Our failure to comply with the covenants or other terms of the Credit Agreement, including as a result of events beyond our control, could result in a default under the Credit Agreement or Revenue Interest Financing Agreement that could materially and adversely affect the ongoing viability of our business.

On May 11, 2021 (Credit Agreement Closing Date), we entered into a Credit Agreement and Guaranty (as amended by that certain letter agreement on May 17, 2021, that certain letter agreement on May 23, 2022 and that certain Limited Consent and First Amendment to Credit Agreement on October 28, 2022, the Credit Agreement) with Oaktree Fund Administration, LLC, as administrative agent (Oaktree) and the lenders party thereto (collectively, the Lenders) that provides for a five-year senior secured term loan facility in an aggregate original principal amount of up to $100.0 million, consisting of (i) tranche A-1 term loans in an aggregate principal amount of $15.0 million advanced on the Credit Agreement Closing Date; (ii) tranche A-2 term loans in an aggregate principal amount of $30.0 million advanced on September 27, 2021; (iii) tranche B term loans in an aggregate principal amount of $30.0 million advanced on March 30, 2022; and (iv) tranche C term loans in an aggregate principal amount of $25.0 million (collectively, the Term Loans). Our ability to draw each tranche of the Term Loans is subject to the satisfaction of certain conditions applicable to each tranche as specified in the Credit Agreement. The Term Loans bear interest at a fixed per annum rate (subject to increase during an event of default) of 11.50% and are scheduled to mature on the fifth anniversary of the Credit Agreement Closing Date (Maturity Date). In addition, at the time of funding of any tranche of the Term Loans, we are required to pay an upfront fee of 2.0% of the aggregate principal amount being funded. We are required to make quarterly interest payments until the Maturity Date. We are also required to make principal payments, which are payable in quarterly installments beginning on the last day of the first quarter ending after the third anniversary of the Credit Agreement Closing Date, in an amount equal to 5.0% of the aggregate amount of the Term Loans outstanding on the date of the first such quarterly principal payment and continuing until the Maturity Date, on which date all outstanding Term Loans and other amounts owed under the Credit Agreement will be required to be paid in full. The Term Loans will be guaranteed by certain of our future subsidiaries. Our obligations under the Credit Agreement and the guarantee of such obligations are secured, subject to customary permitted liens and other agreed upon exceptions and subject to an intercreditor agreement with Sagard Healthcare Royalty Partners, LP (Sagard), by a pledge of substantially all of our assets and will be secured by a pledge of substantially all of the assets of the future guarantors. The Credit Agreement contains various covenants that limit our ability to engage in specified types of transactions without Oaktree's prior consent, as well as a financial covenant that requires us to maintain at all times cash and cash equivalents in certain deposit accounts in an amount at least equal to (i) from the funding date of the tranche A-2 term loans until the funding date of the tranche B term loans, $20.0 million, and (ii) from the funding of the tranche B term loans until the Maturity Date, $15.0 million.

Oaktree may elect to accelerate the repayment of all unpaid principal of the Term Loans, accrued interest and other amounts owed under the Credit Agreement upon consummation of a specified change of control transaction or the occurrence of certain events of default (as specified in the Credit Agreement), including, among other things:

- our default in a payment obligation under the Credit Agreement;
- our breach of the restrictive covenants or other terms of the Credit Agreement;
- our breach of reporting obligations;
- our failure to properly maintain the collateral;
- certain regulatory actions that cause an ongoing delay in commercialization of ganaxolone and which could reasonably be expected to result in a material adverse effect;
- a recall of ganaxolone that could reasonably be expected to result in a material adverse effect;
- an injunction against the sale or manufacture of ganaxolone for more than 45 days that could, after the termination of such 45-day period, reasonably be expected to result in a material adverse effect; and

- certain specified insolvency and bankruptcy-related events.

Subject to any applicable cure period set forth in the Credit Agreement, all amounts outstanding with respect to the Term Loans (principal and accrued interest), as well as any applicable prepayment premiums, interest "make-whole" payments or exit fees, would become due and payable (i) immediately, in the case of a payment or bankruptcy event of default or (ii) in the case of any other event of default, upon the request of Lenders holding at least a majority of the outstanding Term Loans and Term Loan commitments, at a default interest rate of 13.50%. Our assets or cash flow may not be sufficient to fully repay our obligations under the Term Loans if the obligations thereunder are accelerated upon any events of default. The duration and magnitude of any negative impact from the COVID-19 pandemic on ganaxolone commercialization, development or net revenues could also affect our ability to meet the requirements to draw on one or more of the Term Loan tranches and to remain in compliance with our liquidity financial covenant. Further, if we are unable to repay, refinance or restructure our obligations under the Term Loans, Oaktree on behalf of the Lenders could proceed to protect and enforce their rights under the Credit Agreement and other loan documents by exercising such remedies (including foreclosure on the assets securing our obligations under the Credit Agreement and the other loan documents) as are available to Oaktree and the Lenders and in respect thereof under applicable law, either by suit in equity or by action at law, or both, whether for specific performance of any covenant or other agreement contained in the Credit Agreement or other loan documents or in aid of the exercise of any power granted in the Credit Agreement or other loan documents. The foregoing would materially and adversely affect the ongoing viability of our business.

On October 28, 2022 (RIFA Closing Date), we entered into a revenue interest financing agreement (Revenue Interest Financing Agreement) with Sagard pursuant to which Sagard agreed to pay us $32.5 million (Investment Amount) to provide funding for our development and commercialization of ganaxolone and related pharmaceutical products, including the commercial launch of ZTALMY, and for working capital and general administrative purposes.

In exchange for the Investment Amount, we have agreed to make quarterly payments to Sagard (the Payments) as follows: (i) for each calendar quarter from and after the RIFA Closing Date through and including the quarter ended June 30, 2026, an amount equal to 7.5% of (a) our net sales of ZTALMY and all other pharmaceutical products that contain ganaxolone (Net Sales), in each case with any dosage form, dosing regimen, or strength, or any improvements related thereto (collectively, the Included Products); and (b) payments received by us in connection with the manufacture, development and sale of Included Products in the U.S., including in connection with any out-licensing of U.S. rights to any Included Product (Other Included Payments, and together with Net Sales, Product Revenue), and (ii) for each calendar quarter following the calendar quarter ended June 30, 2026, an amount equal to (x) 15.0% of the first $100.0 million in annual Product Revenues of the Included Products and (y) 7.5% of annual Product Revenues of the Included Products in excess of $100.0 million.

The Payments are subject to a hard cap equal to 190% ($61.8 million) of the Investment Amount (Hard Cap). Sagard's right to receive payments will terminate when Sagard has received payments in respect of the Included Products, including any additional payments described below, equal to the Hard Cap. Further, we have the right to make voluntary prepayments to Sagard, and such payments will be credited against the Hard Cap.

If Sagard has not received aggregate payments equaling at least 100% of the Investment Amount by December 31, 2027 or at least 190% of the Investment Amount by December 31, 2032 (each, a Minimum Amount), then we will be obligated to make a cash payment to Sagard in an amount sufficient to gross up Sagard up to the applicable Minimum Amount within a specified period of time after each reference date.

The obligations under the Revenue Interest Financing Agreement, including the Payments, will be guaranteed by certain of our future subsidiaries (Subsidiaries) that are required to become a party thereto as guarantors (Guarantors). Our obligations under the Revenue Interest Financing Agreement and the guarantee of such obligations are secured, subject to customary permitted liens and other agreed upon exceptions and subject to an intercreditor agreement with Oaktree as administrative agent for the lenders under the Credit Agreement, by a pledge of substantially all of ours and the Guarantors' assets that relate to, or are used or held for use for, the development, manufacture, use and/or commercialization of ZTALMY and all other pharmaceutical products that contain ganaxolone in the U.S., including the Product Revenue, pursuant to the terms of the Security Agreement dated as of the RIFA Closing Date by and among us, the Guarantors from time to time party thereto, and Sagard (Security Agreement).

At any time, we have the right, but not the obligation (Call Option), to repurchase all, but not less than all, of Sagard's interest in the Payments at a repurchase price (Put/Call Price) equal to: (a) on or before the third anniversary of the RIFA Closing Date, 160% of the Investment Amount; (b) after the third anniversary but on or prior to the fourth anniversary of the RIFA Closing Date, 180% of the Investment Amount; and (c) after the fourth anniversary of the RIFA Closing Date, 190% of the Investment Amount, in each case, less the aggregate of all of our payments in respect of the Payments made to Sagard prior to such date.

The Revenue Interest Financing Agreement contains certain restrictions on ours and our Subsidiaries' abilities, among other things, to incur additional debt, grant or permit additional liens, make investments and acquisitions, dispose of assets, pay dividends and distributions and enter into affiliate transactions, in each case, subject to certain exceptions. In addition, the Revenue Interest Financing Agreement contains a financial covenant that requires us to maintain at all times cash and cash equivalents in certain deposit accounts in an amount at least equal to (i) from the RIFA Closing Date until the repayment of the loans under the Credit Agreement, $15.0 million and (ii) thereafter, $10.0 million.

In addition, the Revenue Interest Financing Agreement provides that if certain events occur, including certain bankruptcy events, a change of control, non-payment of Payments, divestiture of rights to commercialize Included Products in the U.S., divestiture of certain assets related to the Included Products (subject to customary carve-outs), and (subject to applicable cure periods) non-compliance with the covenants in the Revenue Interest Financing Agreement, Sagard has the right, but not the obligation, to require us to repurchase all, but not less than all, of Sagard's interest in the Payments at the Put/Call Price. Our assets or cash flow may not be sufficient to fully repurchase all of Sagard's interest in the Payments if such obligation is triggered upon any events of default. The duration and magnitude of any negative impact from the COVID-19 pandemic on ganaxolone commercialization, development or net revenues could also affect our ability to remain in compliance with our liquidity financial covenant. Further, if we are unable to repay, refinance or restructure our obligations under the Revenue Interest Financing Agreement, Sagard could proceed to protect and enforce its rights under the Revenue Interest Financing Agreement and other transaction documents by exercising such remedies (including foreclosure on the assets securing our obligations under the Revenue Interest Financing Agreement and the other transaction documents) as are available to Sagard and in respect thereof under applicable law, either by suit in equity or by action at law, or both, whether for specific performance of any covenant or other agreement contained in the Revenue Interest Financing Agreement or other transaction documents or in aid of the exercise of any power granted in the Revenue Interest Financing Agreement or other transaction documents. The foregoing would materially and adversely affect the ongoing viability of our business.

If we are unable to satisfy certain conditions in our Credit Agreement, we will be unable to draw down the remaining amount of the term loan facility.

For our Credit Agreement, we must satisfy certain conditions to be eligible to draw down the tranche C term loans of $25.0 million. The tranche C term loans of $25.0 million may be drawn by us on or before December 31, 2023, provided that we satisfy certain conditions described in the Credit Agreement, including (i) the completion of one or more financings, including through the issuance of common stock, convertible debt, subordinated debt, a synthetic royalty or a sublicense in which we receive gross proceeds in an aggregate amount of at least $40.0 million and net proceeds in an aggregate amount of at least $36.0 million (Qualified Financing Condition) and (ii) either our current Phase 3 RAISE trial or a Phase 3 trial in tuberous sclerosis complex (TSC) achieving statistical significance (p value < 0.05) across all primary endpoints and ganaxolone being generally well tolerated, with a safety profile generally consistent with previous clinical trials. We satisfied the Qualified Financing Condition in connection with our November 2022 underwritten public offering, however, if we are unable to satisfy the remaining condition, we would not be able to draw down the remaining tranche of loans and may not be able to obtain alternative financing on commercially reasonable terms or at all.

Our Credit Agreement and Revenue Interest Financing Agreement contain restrictions that limit our flexibility in operating our business.

The Credit Agreement and the Revenue Interest Financing Agreement contain various covenants that limit our ability to engage in specified types of transactions without the prior consent of Oaktree and the Lenders holding a

majority of the Term Loan commitments and/or Sagard, as applicable,. These covenants limit our ability to, among other things:

- sell, transfer, lease or dispose of our assets;
- create, incur or assume additional indebtedness;
- encumber or permit liens on certain of our assets;
- make restricted payments, including paying dividends on, repurchasing or making distributions with respect to our common stock;
- make specified investments (including acquisitions, loans and advances);
- consolidate, merge, sell or otherwise dispose of all or substantially all of our assets;
- enter into certain transactions with our affiliates;
- grant certain license rights related to our products, technology and other intellectual property rights;
- in the case of the Credit Agreement, permit our cash and cash equivalents held in certain deposit accounts to at any time be less than (i) from the funding of the tranche A-2 term loans until the funding of the tranche B term loans, $20.0 million and (ii) from the funding date of the tranche B term loans until the Maturity Date, $15.0 million; and
- in the case of the Revenue Interest Financing Agreement, permit our cash and cash equivalents held in certain deposit accounts to be less than (i) from the RIFA Closing Date until the repayment of the loans under the Credit Agreement, $15.0 million and (ii) thereafter, $10.0 million.

The covenants in our Credit Agreement, Revenue Interest Financing Agreement and related security agreements may limit our ability to take certain actions that may be in our long-term best interests. In the event that we breach one or more covenants, Oaktree and/or Sagard may choose to declare an event of default and (i) in the case of the Credit Agreement, require that we immediately repay all amounts outstanding under the Credit Agreement, plus penalties and interest, terminate the Lenders' commitments to fund any undrawn Term Loan tranches and foreclose on the collateral granted to them to secure the obligations under the Credit Agreement and the other loan documents and/or (ii) in the case of the Revenue Interest Financing Agreement, require that we repurchase all, but not less than all, of Sagard's interest in the Payments and foreclose on the collateral granted to them to secure the obligations under the Revenue Interest Financing Agreement and the other transaction documents. Such repayment could have a material adverse effect on our business, operating results and financial condition.

Raising additional capital could dilute our stockholders, restrict our operations or require us to relinquish rights to ganaxolone or any other future product candidates.

Until we can generate substantial revenue from product sales, if ever, we expect to seek additional capital through a combination of private and public equity offerings, debt financings, government funding, collaborations, licensing arrangements and other commercial transactions and funding opportunities. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of stockholders. Debt financing or other commercial transactions, if available, may involve agreements that include liens or restrictive covenants limiting our ability to take important actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, licensing arrangements or other commercial with third parties, we may have to relinquish valuable rights to ganaxolone or any other future product candidates in particular countries, or grant licenses on terms that are not favorable to us. If we are unable to raise additional funds

when needed, we may be required to delay, limit, reduce or terminate our product development or commercialization efforts or grant rights to develop and market ganaxolone or any other future product candidates that we would otherwise prefer to develop and market ourselves.

We intend to expend our limited resources to pursue ganaxolone and may fail to capitalize on other technologies or any other future product candidates that may be more profitable or for which there may be a greater likelihood of success.

Because we have limited financial and managerial resources, we are focusing on commercializing ZTALMY and on research programs relating to ganaxolone, which concentrates the risk of product failure in the event ganaxolone proves to be ineffective or inadequate for clinical development or commercialization. As a result, we may forego or delay pursuit of opportunities for other technologies or product candidates that later could prove to have greater commercial potential. We may be unable to capitalize on viable commercial products or profitable market opportunities as a result of our resource allocation decisions. Our spending on proprietary research and development programs relating to ganaxolone may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for ganaxolone, we may relinquish valuable rights to ganaxolone through collaboration, licensing or other commercial arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to ganaxolone.

We have a limited operating history, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Our operations to date have been limited to conducting preclinical and clinical development and regulatory activities for ganaxolone as well as early commercialization of ZTALMY for CDD in the US. In addition, we have not yet demonstrated an ability to successfully overcome many of the risks and uncertainties frequently encountered by companies in new and rapidly evolving fields, particularly in the pharmaceutical area. Commercial sales from ZTALMY in CDD, an ultra rare disease, are not expected to be sufficient to fund our clinical development of new indications for ganaxolone, including RSE and TSC. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a longer history of successfully developing and commercializing pharmaceutical products. Further, our budgeted expense levels are based in part on our expectations concerning the costs of commercialization of ZTALMY and on our research, preclinical development and clinical trials, which depend on the success of such activities, and our ability to effectively and efficiently conduct such research, preclinical development, clinical trials and our expectations related to our efforts to achieve FDA or foreign regulatory approval with respect to ganaxolone for additional indications. Our limited operating history and clinical trial experience make these costs difficult to forecast accurately. We may be unable to adjust our operations in a timely manner to compensate for any unexpected increase in costs. Further, our manufacturing costs and operating expenses may increase significantly as we expand our operations and our commercial activities. Accordingly, a significant increase in costs could have an immediate and material adverse effect on our business, results of operations and financial condition.

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

As of December 31, 2022, we had U.S. net operating loss, or NOL, carryforwards of approximately $233.5 million for U.S. federal income tax and approximately $204.6 million for state income tax purposes available to offset future taxable income and U.S. federal and state research and development tax credits of approximately $30.2 million, prior to consideration of annual limitations that may be imposed under Section 382 of the Internal Revenue Code of 1986, as amended, or Section 382. Our U.S. NOL carryforwards begin to expire in 2029 if not utilized.

The NOL carry forwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. NOL and tax credit carry forwards are subject to an annual limitation due to certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, as well as similar state tax provisions. This limits the amount of NOLs that we can utilize annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of our company immediately prior to an ownership change. Subsequent ownership changes may further affect the limitation in future years. In addition, U.S. tax laws limit

the time during which these carry forwards may be applied against future taxes, therefore, we may not be able to take full advantage of these carry forwards for federal income tax purposes.

We may experience additional ownership changes in the future as a result of subsequent shifts in our stock ownership, including through completed or contemplated financings, some of which may be outside of our control. If we determine that a future ownership change has occurred and our ability to use our historical net operating loss and tax credit carryforwards is materially limited, it would harm our future operating results by effectively increasing our future tax obligations. Furthermore, the losses could expire before we generate sufficient income to utilize them.

Risks Related to the Commercialization of ZTALMY and Other Future Product Candidates

ZTALMY is our first commercial product and we have a limited history of commercializing drugs, which may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Our operations to date have been largely focused on raising capital and developing ganaxolone in several indications, including undertaking preclinical studies and conducting clinical trials. We have only recently received FDA approval of ZTALMY, and as such we have not yet demonstrated our ability to successfully supply ZTALMY for ongoing commercial sale or conduct sales, marketing and distribution activities necessary for successful product commercialization. Consequently, any predictions about our future success or viability may not be as accurate as they could be if we had a longer history of successfully developing and commercializing drugs.

Even though we have obtained regulatory approval for ZTALMY in the U.S., we will still face extensive FDA regulatory requirements and may face regulatory difficulties.

Even though we have obtained regulatory approval in the U.S. for ZTALMY, the FDA and state regulatory authorities (and, if we obtain foreign regulatory approvals, comparable foreign regulatory authorities) may still impose significant restrictions on the indicated uses or marketing of ZTALMY, or impose ongoing requirements for potential costly post-approval studies or post-marketing surveillance. For example, as part of its approval of ZTALMY for the treatment of CDD, the FDA requires several post-marketing commitments. The Phase 1 renal impairment study commitment was completed and submitted to the FDA in May 2022. The Phase 1 hepatic impairment study and the thorough QTc study were completed and submitted to the FDA in December 2022. The remaining post-marketing commitments include the following studies:

- 2-year carcinogenicity studies of ganaxolone and the major human unconjugated plasma metabolite, M2 in rats.
- a 26-week carcinogenicity study of ganaxolone in transgenic mice.
- a juvenile animal toxicity study of the major human unconjugated plasma metabolite, M2, in rats.
- extractable/leachable study results on the container closure system.
- a CNS distribution study with M47 metabolite, in rats.
- in vitro studies to assess the drug interaction potential of M47 metabolite.

These additional studies will likely require us to undergo a costly and time-consuming development process. If we do not meet our obligations, the FDA may issue a non-compliance letter and may also consider ZTALMY to be misbranded and subject to potential enforcement action. There is a risk that the studies could take longer than expected to complete or the studies may have adverse findings which may require additional investments and have the potential to materially impact our marketing of Ztalmy.

We are also subject to ongoing FDA requirements governing the manufacture, quality control, further development, labeling, packaging, storage, distribution, safety surveillance, patient registry, import, export, advertising,

promotion, recordkeeping and reporting of safety and other post-market information. The safety profile of ganaxolone will continue to be closely monitored by the FDA and comparable foreign regulatory authorities after approval. If new safety information becomes available after approval of ganaxolone, the FDA or comparable foreign regulatory authorities may require labeling changes or establishment of a REMS or similar strategy, impose significant restrictions on ganaxolone's indicated uses or marketing, or impose ongoing requirements for potentially costly post-approval trials or post-market surveillance. We will also be subject to continued compliance with current good manufacturing practices (cGMP) and good clinical practices (GCP) requirements for any clinical trials that we conduct post-approval.

In addition, manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP and other regulations. If we or a regulatory authority discover previously unknown problems with a product, such as AEs of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory authority may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. If we, ganaxolone or the manufacturing facilities for ganaxolone fail to comply with applicable regulatory requirements, a regulatory authority may, among other things:

- issue warning letters or untitled letters;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications filed by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of products, or require us to initiate a product recall.

The occurrence of any event or penalty described above may inhibit or preclude our ability to commercialize ganaxolone and generate revenue.

The FDA's and other regulatory authorities' policies may change, and additional government regulations may be enacted. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the U.S. or abroad. 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 for ganaxolone 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.

Advertising and promotion of pharmaceutical products, including ZTALMY, is heavily scrutinized by, among others, the FDA, the U.S. Department of Justice (DOJ), the Office of the Inspector General of the U.S. Department of Health and Human Services (HHS OIG), state attorneys general, members of Congress and the public. The FDA and other federal regulatory agencies closely regulate the marketing and promotion of drugs through, among other things, standards and regulations for direct-to-consumer advertising, advertising and promotion to healthcare professionals, communications regarding unapproved uses, industry-sponsored scientific and educational activities, and promotional

activities involving the Internet. A product cannot be promoted before it is approved. After approval, product promotion can include only those claims relating to safety and effectiveness that are consistent with the labeling approved by the FDA. Healthcare providers are permitted to prescribe drugs for "off-label" uses — that is, uses not approved by the FDA and not described in the product's labeling — because the FDA does not regulate the practice of medicine. However, FDA regulations impose restrictions on manufacturers' communications regarding off-label uses. Broadly speaking, a manufacturer may not promote a drug for off-label use, but under certain conditions may engage in non-promotional, balanced, scientific communication regarding off-label use. Failure to comply with applicable FDA requirements and restrictions in this area may subject a company to adverse publicity and enforcement action, including enforcement letters, inquiries and investigations, and civil and criminal sanctions by the FDA or other government agencies. In addition, advertising and promotion of ganaxolone, if approved outside of the U.S., will be heavily scrutinized by comparable foreign regulatory authorities.

In the U.S., promoting ganaxolone for unapproved indications can also subject us to false claims litigation under federal and state statutes, and other litigation and/or investigation, which can lead to civil and criminal penalties and fines and agreements that materially restrict the manner in which we promote or distribute our drug products. These false claims statutes include the False Claims Act (FCA), which allows any individual to bring a lawsuit against a pharmaceutical company on behalf of the federal government alleging submission of false or fraudulent claims, or causing to present such false or fraudulent claims, for payment by a federal program such as Medicare or Medicaid. If the government prevails in the lawsuit, the individual will share in any fines or settlement funds. If we do not lawfully promote our approved products, we may become subject to such litigation and/or investigation and, if we are not successful in defending against such actions, those actions could adversely affect our business prospects, financial condition and results of operations.

In the European Union (EU), strict requirements and restrictions regarding advertising and promotion apply, the details of which may vary per EU Member States. Violation of those rules could subject us to litigation, investigations and/or civil and criminal penalties, which could adversely affect our business, prospects, financial condition and results of operations.

Our commercial success depends upon attaining significant market access and acceptance of ZTALMY among physicians, patients, government and private payers and others in the medical community and attaining sufficient reimbursement for ganaxolone.

Even though ZTALMY received FDA approval for CDD, it may not gain market acceptance among physicians, patients, government and private payers, or others in the medical community. Market acceptance of ZTALMY (and of any potential future products we commercialize) depends on a number of factors, including:

- clinically and commercially viable product profile as supported by clinical trials;
- efficacy and safety of ganaxolone, or ganaxolone administered with other drugs, each as demonstrated in clinical trials and post-marketing experience;
- clinical indications for which ganaxolone is approved;
- acceptance by physicians and patients of ganaxolone as a safe and effective treatment;
- potential and perceived advantages of ganaxolone over alternative treatments;
- safety of ganaxolone seen in a broader patient group, including its use outside the approved indications should physicians choose to prescribe for such uses;
- prevalence and severity of any side effects and drug interactions with other medications, including other antiseizure medications;

- product labeling or product insert requirements of the FDA or comparable foreign regulatory authorities;
- restrictions in distribution and use due to controlled substance laws and regulations;
- timing of market introduction of ganaxolone as well as competitive products;
- cost of treatment in relation to alternative treatments;
- availability of coverage and adequate reimbursement and pricing by government and private payers;
- ability to manufacture commercial quantities of ZTALMY (or any future products) at a reasonable cost and with sufficient speed to meet commercial demand;
- ability to obtain and maintain appropriate state licenses in the states in which we intend to sell ZTALMY (or any future products);
- ability to successfully defend any challenges to our intellectual property relating to ZTALMY (or any future products);
- relative convenience and ease of administration;
- effectiveness of our sales and marketing strategy and efforts and effective use of promotional resources;
- adequate commercial investment; and
- stability and continuity of product supply chains.

If ZTALMY fails to achieve market acceptance among physicians, patients, government or private payers or others in the medical community, or the products or product candidates that are being administered with ganaxolone cause AEs, as the case may be, we may not be able to generate significant revenues, which would compromise our ability to become profitable. Many of these matters are beyond our control and are subject to other risks described elsewhere in this "Risk Factors" section. Accordingly, we cannot assure you that we will be able to successfully commercialize or generate revenue from ZTALMY (or any future products).

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to ganaxolone both for the treatment of CDD and for other indications, and will face competition with respect to any other product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing ganaxolone. Some of these competitive products and therapies are based on scientific approaches that are the same as, or similar to, our approach, and others are based on entirely different approaches. For example, there are several companies developing product candidates that target the same $GABA_A$ neuroreceptor that we are targeting or that are testing product candidates in the same indications that we are testing. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Ganaxolone is presently being developed as an antiepileptic therapeutic. There are a variety of marketed therapies available for these patients.

Specifically, there are more than 25 approved AEDs available in the U.S. and worldwide, including the generic products levetiracetam, lamotrigine, carbamazepine, oxcarbazepine, valproic acid and topiramate. Recent market entrants include branded products developed by UCB (including acquisition of Zogenix), Eisai, Jazz Pharmaceuticals (via acquisition of GW Pharmaceuticals), SK Biopharmaceuticals and Sunovion Pharmaceuticals. In addition, there are several drugs in clinical development for the treatment of pediatric orphan epilepsy indications, including compounds being developed by Jazz Pharmaceuticals (via acquisition of GW Pharmaceuticals), UCB (including acquisition of Zogenix) and Takeda.

Many of the approved drugs are well established therapies or products and are widely accepted by physicians, patients and third-party payers. Insurers and other third-party payers may also encourage the use of generic products. These factors may make it difficult for us to achieve market acceptance at desired levels or in a timely manner to ensure viability of our business.

More established companies may have a competitive advantage over us due to their greater size, cash flows and institutional experience. Compared to us, many of our competitors may have significantly greater financial, technical and human resources.

As a result of these factors, our competitors may obtain regulatory approval of their products before we are able to, which may limit our ability to develop or commercialize ganaxolone. Our competitors may also develop products that are safer, more effective, more widely used and cheaper than ours, and may also be more successful than us in manufacturing and marketing their products. These appreciable advantages could render ganaxolone obsolete or non-competitive before we can recover the expenses of ganaxolone's development and commercialization.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific, management and commercial personnel, establishing clinical trial sites and subject registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

If we are unable to differentiate ZTALMY from current and future products or existing methods of treatments, our ability to successfully commercialize ZTALMY would be adversely affected.

We initially intend to commercialize ZTALMY for the treatment of CDD and seek FDA approval for ganaxolone with respect to additional indications. Ganaxolone is the first product to receive regulatory approval for reduction of seizures specifically in patients with CDD. Patients with CDD are generally on a number of anti-epileptic medications and physicians' determining whether to prescribe ZTALMY to their CDD patients may add ZTALMY to existing regimens for patients or make changes in their patients' current medications to introduce ZTALMY. If we are unable to achieve significant differentiation for ZTALMY against these other products and treatments or future treatments, including on the basis of efficacy, safety and tolerability profile, reliability, convenience of administration, price and reimbursement, the opportunity for ZTALMY to be commercialized successfully would be adversely affected.

ZTALMY is our first commercial product. If our sales and marketing capabilities to market and sell ganaxolone are not effective, we may be unable to generate meaningful revenue.

ZTALMY is our first commercial product and we have hired experienced commercial leadership in our organization to support commercialization of ZTALMY, including sales, marketing, sales operations, a field force, market access professionals, and supply chain and distribution professionals. We compete for commercial talent with companies that have extensive, well-funded marketing and sales operations and successful products in the market. We have a limited track record with the early launch for ZTALMY in CDD which may make it more difficult to attract and retain effective commercial talent . If we are unable to maintain an effective commercial team, we may not be

successful in generating meaningful revenue. Even if we are able to maintain an effective commercial team, we may be unable to compete successfully against more established companies.

While ZTALMY has received favorable reimbursement determinations to date from third party payers for its approved indication, adverse changes in reimbursement or failure to obtain favorable reimbursement for future indications, if approved, could harm our business.

Our ability to successfully commercialize ganaxolone depends, in part, on the extent to which coverage and adequate reimbursement for ganaxolone is available from government health administration authorities, including Medicaid, which we expect will be a significant portion of patients prescribed ZTALMY, private health insurers and other organizations. Government authorities and commercial third-party payers, such as private health insurers and health maintenance organizations, determine which medications they will cover, the process for making such decisions, and the reimbursement levels for those medications. Obtaining formulary coverage and favorable reimbursement levels for ZTALMY from government authorities or other third-party commercial payers can be a time consuming and costly process, especially in the early years of regulatory approval. It is expected that we will be required to provide supporting clinical scientific and economic evidence in the form of cost-effectiveness and real-world data, outcomes beyond the data required to obtain marketing approval. We may not be able to gain acceptance from government health authorities, third-party payers or employer sponsored plans and, even if we are able to do so, the timing and the consistency in payer formulary placement or utilization management may vary greatly from government health authorities, third-party payers and by employer sponsored plans.

A primary trend in the U.S. healthcare industry and elsewhere is budget predictability and cost containment. Government authorities and third-party payers have attempted to control costs by limiting access through utilization management controls, formulary placement and reimbursement amounts for particular medications and procedures. Increasingly, third-party payers are requiring that drug companies provide them with predetermined utilization discounts from list prices and are challenging the prices charged for drugs. Third-party payers or PMBs may also seek additional clinical and economic evidence, beyond the data required to obtain marketing approval, which may include demonstrating clinical benefits and value in specific patient populations before covering ganaxolone for those patients. We cannot be sure that formulary placement, coverage and adequate reimbursement will be available for ganaxolone and, if such is available, what hurdles may be put in place for prescribing physicians to navigate. Coverage and reimbursement may impact physician or institutional demand for ganaxolone, and that demand may vary by region or by payer segment. If coverage and reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize ganaxolone.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may only be temporary. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on formulary coverage or reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs will be reduced by mandatory discounts or rebates required by Medicaid government healthcare programs and may be reduced by private payers and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the U.S.. Third-party payers and PBMs often rely upon Medicare coverage policy and payment limitations in setting their own coverage and reimbursement policies. Any challenges in obtaining coverage and favorable reimbursement rates from both government-funded and private payers for ZTALMY could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

If the market opportunities for ZTALMY in CDD and other indications for which we obtain regulatory approval, if any, are smaller than we believe they are, our results of operations may be adversely affected and our business may suffer.

We focus our research and product development on therapeutics to treat patients suffering from seizure disorders. Our projections of both the number of people who have these disorders, as well as the subset of people with these diseases who have the potential to benefit from treatment with ganaxolone, are based on estimates. These estimates may prove to be incorrect and new studies or clinical trials may change the estimated incidence or prevalence of these disorders. The number of patients in the U.S. and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with ganaxolone, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business. ZTALMY has received regulatory approval in the U.S. for CDD and our estimates of the market for ZTALMY in CDD may be incorrect. Our ability to obtain market information is limited since ZTALMY is the first drug to be approved specifically for use in seizures associated with CDD and the ICD10 code for CDD was established in 2020, and there is limited market data available for CDD.

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

We plan to seek regulatory approval for ganaxolone outside of the U.S., 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;
- 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;
- viable pricing awarded in international markets to support commercial investment is required;
- 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 abroad;
- foreign taxes, including with respect to our Irish subsidiary;
- 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 U.S.;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and

- business interruptions resulting from geo-political actions, including war and terrorism, as well as from pandemics, including the COVID-19 pandemic.

These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of ganaxolone or other future product candidates that we may develop.

We face an inherent risk of product liability exposure related to the testing of ganaxolone by us or our investigators in human clinical trials and will face an even greater risk now that ZTALMY has received FDA approval and we enter the commercial market. Product liability claims may be brought against us by patients enrolled in our clinical trials, patients, healthcare providers or others using, administering or selling ganaxolone. If we cannot successfully defend ourselves against claims that ganaxolone caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in, for example:

- decreased demand for ganaxolone;
- termination of clinical trial sites, entire clinical trials or development programs;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial patients;
- significant costs to defend the related litigation;
- substantial monetary awards to patients;
- loss of revenue;
- diversion of management and scientific resources from our business operations;
- the inability to commercialize ganaxolone; and
- increased scrutiny and potential investigation by, among others, the FDA, the DOJ, the HHS OIG, state attorneys general, members of Congress and the public.

We currently have product liability insurance coverage, which may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. We have expanded our product liability insurance coverage to include commercial sales of ZTALMY, but we may be unable to obtain commercially reasonable limits for product liability insurance. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could have a material adverse effect on our business and financial condition.

If the FDA or other applicable regulatory authorities approve generic products that compete with any of our products or product candidates, it could reduce our sales of those products or product candidates.

In the U.S., after an NDA is approved, the product generally becomes a “listed drug” which can, in turn, be relied upon by potential competitors in support of approval of an ANDA. The Federal Food, Drug, and Cosmetic Act, FDA regulations and other applicable regulations and policies provide incentives to manufacturers to create generic, non-infringing versions of a drug to facilitate the approval of an ANDA. These manufacturers might show that their product has the same active ingredients, dosage form, strength, route of administration, conditions of use, and labeling as

our product candidate and might conduct a relatively inexpensive study to demonstrate that the generic product is absorbed in the body at the same rate and to the same extent as, or is bioequivalent to, our product. These generic equivalents would be significantly less costly than ours to bring to market, and companies that produce generic equivalents are generally able to offer their products at lower prices. Thus, after the introduction of a generic competitor, a significant percentage of the sales of any branded product are typically lost to the generic product. Accordingly, competition from generic equivalents to our products would substantially limit our ability to generate revenues and therefore to obtain a return on the investments we have made in our products.

Risks Related to Clinical Development and Regulatory Approval of our Product Candidates

Our future success is dependent on the successful clinical development, regulatory approval and commercialization of ganaxolone, which is being studied in several indications and will require significant capital resources and years of additional clinical development effort.

We have only recently received FDA approval of ZTALMY in CDD, and we plan to develop ganaxolone in several additional indications in oral and IV formulations. As a result, our business is dependent on our ability to successfully complete clinical development, scale-up manufacturing, obtain regulatory approval, and, if approved, commercialize ganaxolone in a timely manner. We cannot commercialize ganaxolone in the U.S. in any other indication without first obtaining regulatory approval from the FDA; similarly, we cannot commercialize ganaxolone outside of the U.S. without obtaining regulatory approval from comparable foreign regulatory authorities. Before obtaining regulatory approvals for the commercial sale of ganaxolone for a target indication, we must demonstrate with substantial evidence gathered in preclinical studies and clinical trials and, with respect to approval in the U.S., to the satisfaction of the FDA, that ganaxolone is safe and effective for use for that target indication and that the manufacturing facilities, processes and controls are adequate.

Ganaxolone is metabolized extensively in animals and humans. During the development of CDD, one major metabolite (M2) was present in plasma of humans that was not found in plasma of rats or dogs. The chemical structure of M2 has been identified. An activity assay, dose range finding study in rats and an in vivo micronucleus with comet analysis for the detection of genotoxicity have been conducted and the results submitted to the FDA. Results from additional preclinical studies are required to the FDA as post-marketing requirement(s). These include: 2-year carcinogenicity studies of ganaxolone and the major human unconjugated plasma metabolite, M2, in rats; a 26-week carcinogenicity of ganaxolone in transgenic mice; a juvenile animal toxicity study of the major human unconjugated plasma metabolite, M2, in rats; a CNS distribution study of the M47 metabolite in rats; and in vitro studies to assess the drug interaction potential of M47 metabolite. Additional post-marketing requirements include: phase 1 renal and hepatic impairment studies and a thorough QTc study; and extractable/leachable study results on the container closure system. The Phase 1 renal impairment study commitment was completed and submitted to the FDA in May 2022. The Phase 1 hepatic impairment study and the thorough QTc study were completed and submitted to the FDA in December 2022. We plan to complete the required FDA studies within the required FDA timeframe. However, there is a risk that the studies could take longer than expected to complete or the studies may have adverse findings which may require additional investments and have the potential to materially impact the label or our ability to market Ztalmy. In the EU, if additional studies are needed, these are usually required before or during MAA review.

We are conducting the RAISE trial in RSE, which is a life threatening medical condition involving prolonged seizure activity in seriously ill patients. The RAISE trial requires expertise in electroencephalogram (EEG) interpretation, which may be subject to variability, and the FDA or foreign regulatory authorities could find the data generated in this trial inadequate or difficult to interpret, which could delay, limit or prevent regulatory approval for this indication. There is also a risk that the Phase 3 clinical trial of ganaxolone in RAISE will generate data that is not sufficient to support regulatory approvals for this indication. Additionally, the clinical trial endpoints of the RAISE trial are based on treatment outcomes, including initiation of anesthesia for treatment of RSE. Practice variability in the use of anesthesia for SE treatment could adversely impact the ability to show a treatment effect with ganaxolone. Even if the RAISE trial shows that ganaxolone is effective, there is a risk that the FDA will require more safety data generated with IV ganaxolone at the doses given to patients in this trial before approving an NDA or require post approval commitments to generate additional safety data as a condition of approval ganaxolone for use in RSE.

We have recently reported data from the CALM trial evaluating safety and efficacy of adjunctive oral ganaxolone treatment in patients with TSC. The primary endpoint showed median 16.6% reduction in 28-day primary endpoint seizure frequency relative to the four-week baseline period. In addition, data from the Phase 2 TSC trial suggested that in patients on concomitant Epidiolex, early elevation of ganaxolone blood levels occurred and appeared to be linked to greater somnolence. A formal Phase 1 drug-drug interaction trial was completed, demonstrating a lack of significant interaction between ganaxolone and Epidiolex. Additionally, the titration schedule for all subjects in the Phase 3 TSC trial has been adjusted to maximize tolerability. Undesirable side effects could delay clinical trials and result in the FDA or other regulatory authorities requiring us to conduct additional studies or trials for our product candidate either prior or post-approval, such as additional drug-drug interaction studies or safety or efficacy studies, or it may object to elements of our clinical development program. There is also a risk that the Phase 3 clinical trial of ganaxolone in TSC will generate data that is not sufficient to support regulatory approvals for this indication.

Even if ganaxolone were to obtain approval from the FDA and comparable foreign regulatory authorities for TSC, RSE, or any other indication under development, any approval might contain significant limitations, such as restrictions as to specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval trial or risk management requirements. If we are unable to obtain regulatory approval for ganaxolone in these additional indications in one or more jurisdictions, or any approval contains significant limitations, we may not be able to obtain sufficient funding or generate sufficient revenue to continue the development of any other indications for ganaxolone or any other product candidate that we may in-license, develop or acquire in the future. Furthermore, even with regulatory approval for ganaxolone, we will still need to develop a commercial organization, establish commercially viable pricing and obtain adequate reimbursement from third-party and government payers. If we are unable to successfully commercialize ganaxolone, we may not be able to earn sufficient revenue to continue our business.

We are conducting clinical development activities for ganaxolone across multiple indications, and such clinical development activities may not produce favorable results, which could adversely impact our ability to achieve regulatory approval for ganaxolone in such indications.

We are conducting clinical development activities for ganaxolone across multiple indications. Success in preclinical studies and early clinical trials in one indication does not ensure that later clinical trials in such indication or other indications will generate adequate data to demonstrate the efficacy and safety of ganaxolone in one or more indications. Furthermore, unfavorable clinical trial results in one ganaxolone indication may adversely impact our ability to continue to develop such indication or other ganaxolone indications. A number of companies in the pharmaceutical and biotechnology industries, including those with greater resources and experience, have suffered significant setbacks in clinical trials, even after seeing promising results in earlier studies and clinical trials. For example, while ganaxolone showed statistical separation from placebo in a Phase 2 clinical trial in adjunctive treatment of adults with focal onset seizures, ganaxolone failed to show a similar statistically significant separation in a Phase 3 clinical trial for the same indication. As a result, we discontinued our program in adult focal onset seizures and began to focus our efforts on advancing ganaxolone in RSE and pediatric orphan genetic epilepsy indications. We do not know whether the clinical trials we may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market ganaxolone in any particular jurisdiction or indication. If clinical trials underway or conducted in the future do not produce favorable results, our ability to achieve regulatory approval for ganaxolone in those indications may be adversely impacted. Further, even if we believe the data collected from our clinical trials of ganaxolone are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Pre-clinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us, which could delay, limit or prevent regulatory approval.

Ganaxolone may cause undesirable side effects or have other properties, such as abuse potential, that could delay or prevent its regulatory approval in indications under clinical development, limit the commercial profile of an approved label, or result in significant negative consequences following any marketing approval.

Undesirable side effects caused by ganaxolone could cause us, an institutional review board (IRB), or regulatory authorities to interrupt, delay or halt clinical trials and could result in a restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign regulatory authority. More than 2,300 individuals have

received oral formulations of ganaxolone for durations from one day to more than two years at doses of 50 to 2,000 mg/day. The majority of AEs were non-serious and resolved upon discontinuation of therapy. The most common side effects with oral ganaxolone relate to sedation or somnolence. Somnolence and sedation have appeared early during treatment and were generally dose related. In the oral ganaxolone safety database there are no trends of medically important changes in blood chemistry, vital signs, liver function, renal function or cardiovascular parameters in adult or pediatric populations. Additionally, over 60 patients have received the IV formulation of ganaxolone in our clinical trials to date. Although ganaxolone has generally been well-tolerated by patients in our clinical trials to date, in some cases there were side effects, and some of the side effects were severe. The most frequent side effects were dizziness, fatigue and somnolence (or drowsiness). More side effects of the CNS were categorized as severe as compared to side effects of other body systems. Antiepileptic drugs, including ganaxolone, increase the risk of suicidal thoughts or behavior. In addition, as with most antiepileptic drugs, ganaxolone should be withdrawn gradually to minimize the risk of increased seizure frequency and status epilepticus.

There were no deaths reported in the double-blind phase of the Marigold Trial. Three deaths globally have occurred during the open label extension phase of the trial, two of which were assessed by the investigators as unrelated to trial treatment. The third death was assessed by the investigator as probably related to trial medication. Given the severity of CDD and its medical complications, serious adverse events or deaths may occur which, in the absence of a control group, make determination of relatedness to treatment difficult.

If these side effects are reported in future clinical trials, or if other safety or toxicity issues are reported in our future clinical trials, we may not receive approval to market ganaxolone or approval may be limited, which could prevent us from ever generating material revenue or achieving profitability. Furthermore, although we are currently developing ganaxolone for multiple indications, negative safety findings in any one indication could force us to delay or discontinue development in other indications. Results of our clinical trials could reveal an unacceptably high severity and prevalence of side effects. In such an event, our clinical trials could be suspended or terminated, and the FDA or comparable foreign regulatory authorities could order us to cease further development, or deny approval, of ganaxolone for any or all targeted indications. Drug-related side effects could affect trial subject recruitment or the ability of enrolled patients to complete our future clinical trials and may result in potential product liability claims. Additionally, in our clinical trials ganaxolone is added to the standard of care, which includes many antiseizure medications. Drug interactions with any of the medications could result in safety concerns or reduce the population in which ganaxolone may be used. For example, in our recently completed clinical trial of ganaxolone in TSC, we reported data that suggested that in patients on concomitant Epidiolex, early elevation of ganaxolone blood levels occurred and appeared to be linked to greater somnolence. A formal Phase 1 drug-drug interaction trial was completed, demonstrating a lack of significant interaction between ganaxolone and Epidiolex. Additionally, the titration schedule for all subjects in the Phase 3 TSC trial has been adjusted to maximize tolerability. Undesirable side effects could delay clinical trials and result in the FDA or other regulatory authorities requiring us to conduct additional studies or trials for our product candidate either prior to or post-approval, such as additional drug-drug interaction studies or safety or efficacy studies or trials, or it may object to elements of our clinical development program.

Ganaxolone is metabolized extensively in animals and humans. During the development of CDD, one major metabolite (M2) was present in plasma of humans that was not found in plasma of rats or dogs. The chemical structure of M2 has been identified. An activity assay, dose range finding study in rats and an in vivo micronucleus with comet analysis for the detection of genotoxicity have been conducted and the results submitted to the FDA. Results from additional preclinical studies are required to the FDA as post-marketing requirement(s). These include: 2-year carcinogenicity studies of ganaxolone and the major human unconjugated plasma metabolite, M2, in rats; a 26-week carcinogenicity of ganaxolone in transgenic mice; a juvenile animal toxicity study of the major human unconjugated plasma metabolite, M2, in rats; a CNS distribution study of the M47 metabolite in rats; and in vitro studies to assess the drug interaction potential of M47 metabolite. Additional post-marketing requirements include: phase 1 renal and hepatic impairment studies and a thorough QTc study; and extractable/leachable study results on the container closure system. The Phase 1 renal impairment study commitment was completed and submitted to the FDA in May 2022. The Phase 1 hepatic impairment study and the thorough QTc study were completed and submitted to the FDA in December 2022. We plan to complete the required FDA studies within the required FDA timeframe. However, there is a risk that the studies could take longer than expected to complete or the studies may have adverse findings which may require additional

investments and have the potential to materially impact our marketing of Ztalmy. In the EU, if additional studies are needed, these are usually required before or during MAA review.

If we or others identify undesirable side effects caused by ganaxolone after receiving marketing approval, a number of potentially significant negative consequences could result, including:

- we may be forced to suspend marketing of ganaxolone;
- regulatory authorities may withdraw their approvals of ganaxolone;
- regulatory authorities may require additional warnings on the label that could diminish the usage or otherwise limit the commercial success of ganaxolone;
- we may be required to conduct post-marketing trials;
- we may be required to develop a Risk Evaluation and Mitigation Strategy (REMS) for ganaxolone or if a REMS is already in place, to incorporate additional requirements under the REMS, and comparable regulatory authorities outside the U.S. may require similar risk management strategies;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of ganaxolone.

The FDA recommended scheduling of ganaxolone as a controlled substance based on the abuse liability assessment conducted for the NDA submission. As such, the U.S. DEA reviewed ganaxolone and following the review classified ganaxolone as a Schedule V drug. As a controlled substance, the manufacture, import, export, distribution, storage, sale, dispensing, prescribing, and use will be subject to a significant degree of additional regulation by the DEA as well as state regulatory authorities. The restrictive nature of these regulations could also limit commercialization and market acceptance of ganaxolone.

The therapeutic efficacy and safety of ganaxolone in indications other than CDD have not been established by regulatory authorities, and we may not be able to successfully develop and commercialize ganaxolone in the other indications under clinical development in the future.

Our ability to generate revenue from ganaxolone in other indications we have under clinical development such as RSE and TSC will depend on our successful development and commercialization after regulatory approval in those indications, which is subject to many potential risks and may not occur. Ganaxolone may interact with human biological systems in unforeseen, ineffective or harmful ways. If ganaxolone is associated with undesirable side effects or has characteristics that are unexpected in these indications, we may need to limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that are approved in one indication fail to achieve regulatory approvals in additional indications. As a result of these and other risks described herein that are inherent in the development of novel therapeutic agents, we may never successfully develop ganaxolone in additional indications, which would significantly decrease the commercial potential of ganaxolone overall.

Clinical development of product candidates involves a lengthy and expensive process with an uncertain outcome.

Clinical trials are expensive, can take many years to complete, and are inherently uncertain as to outcome. Failure can occur at any time during the clinical development process.

We may experience delays in our ongoing or future clinical trials and we do not know whether planned clinical trials will begin or enroll patients on time, need to be redesigned or be completed on schedule, if at all. There can be no assurance that the FDA or other foreign regulatory authorities will not put clinical trials of ganaxolone on clinical hold now or in the future. Clinical trials may be delayed, suspended or prematurely terminated for a variety of reasons, such as:

- delay or failure in reaching agreement with the FDA or a comparable foreign regulatory authority on a trial design that we are able to execute;
- delay or failure in obtaining authorization to commence a trial or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical trial;
- delay or failure in reaching agreement on acceptable terms with prospective clinical research organizations (CROs) and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- lack of adequate essential functions and staff, including related to the COVID-19 pandemic, which may impact the ability of site staff to conduct assessments, or result in delays to the conduct of the assessments, as part of our clinical trial protocols, or the ability to enter assessment results into clinical trial databases in a timely manner;
- delay or failure in obtaining IRB approval or the approval of other reviewing entities, including comparable foreign regulatory authorities, to conduct a clinical trial at each site;
- withdrawal of clinical trial sites from our clinical trials as a result of changing standards of care or the ineligibility of a site to participate in our clinical trials;
- delay or failure in recruiting and enrolling suitable trial patients to participate in a trial;
- delay or failure in trial patients completing a trial or returning for post-treatment follow-up;
- clinical sites and investigators deviating from a trial protocol, failing to conduct the trial in accordance with regulatory requirements, or dropping out of a trial;
- inability to identify and maintain a sufficient number of trial sites, many of which may already be engaged in other clinical trial programs, including some that may be for competing product candidates with the same indication;
- failure of our third-party clinical trial managers to satisfy their contractual duties or meet expected deadlines;
- limitations on our or our third-party clinical trial managers' ability to access and verify clinical trial data captured at clinical trial sites through monitoring and source document verification;
- delay or failure in adding new clinical trial sites;
- ambiguous or negative interim results or results that are inconsistent with earlier results;
- feedback from the FDA or a comparable regulatory authority outside the U.S., IRBs, or data safety monitoring boards, or results from earlier stage or concurrent preclinical studies and clinical trials, that might require modification to the protocol for the trial;

- decision by the FDA or a comparable regulatory authority outside the U.S., an IRB or us, or a recommendation by a data safety monitoring board to suspend or terminate clinical trials at any time for safety issues or for any other reason;

- unacceptable risk-benefit profile, unforeseen safety issues or adverse side effects or AEs associate with a product candidate;

- failure of a product candidate to demonstrate any or enough of a benefit;

- difficulties in manufacturing or obtaining from third parties sufficient quantities of a product candidate for use in clinical trials that meet internal and regulatory standards;

- lack of adequate funding to continue the clinical trial, including the incurrence of unforeseen costs due to enrollment delays, requirements to conduct additional clinical trials or increased expenses associated with the services of our CROs and other third parties;

- political developments that affect our ability to develop and obtain approval for ganaxolone or impair our license rights to develop and obtain approval for ganaxolone in other countries; or

- changes in governmental regulations or administrative actions.

Trial subject enrollment, which significantly impacts the timing of clinical trials, is affected by many factors including the size and nature of the subject population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, ability to obtain and maintain patient consents, risk that enrolled patients will drop out before completion, 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 or product candidates that may be studied in competing clinical trials for the indications we are investigating. Some of our clinical trials are directed at small patient populations. Patient enrollment in these trials could be particularly challenging. In the past, we have experienced delays in enrolling patients in trials directed at small patient populations. We rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and while we have agreements governing their committed activities, we have limited influence over their actual performance.

If we experience delays in the completion of any clinical trial of ganaxolone, the commercial prospects of ganaxolone may be harmed, and our ability to generate product revenue from ganaxolone, if approved, will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our development and approval process for ganaxolone and jeopardize our ability to commence product sales and generate revenues. In addition, many of the factors that could cause a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of ganaxolone.

We may not be able to obtain or maintain orphan drug exclusivity for ganaxolone across all indications and markets, which could limit the potential profitability of ganaxolone.

Regulatory authorities in some jurisdictions, including the U.S. and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 people in the U.S. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for an indication for which it receives the designation, then the product is entitled to a period of marketing exclusivity that precludes the FDA from approving another marketing application for the same drug for the same indication for the exclusivity period except in limited situations. For purposes of small molecule drugs, the FDA defines "same drug" as a drug that contains the same active moiety and is intended for the same use as the drug in question. A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation.

We have received orphan drug designation in the U.S. for treating Infantile Spasms, SE, CDD, TSC, PCDH19-RE and Fragile X Syndrome with ganaxolone and expect that we may in the future pursue orphan drug designations for ganaxolone for one or more additional indications. However, obtaining an orphan drug designation can be difficult and we may not be successful in doing so for additional ganaxolone indications. Orphan drug exclusivity for a product candidate may not effectively protect the product from the competition of different drugs for the same condition, which could be approved during the exclusivity period. In addition, after an orphan drug is approved, the FDA could subsequently approve another application for the same drug for the same indication if the FDA concludes that the later drug is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusive marketing rights in the U.S. also may be lost if the FDA later 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. The failure to obtain or maintain an orphan drug designation for any indication of ganaxolone that we may develop, or the inability to obtain or maintain orphan drug exclusivity could reduce our ability to make sufficient sales of ganaxolone to balance our expenses incurred to develop it, which would have a negative impact on our operational results and financial condition.

In the EU, we have received orphan designation for treating CDD with ganaxolone. Orphan designation would entitle us to receive ten years of orphan market exclusivity in the EU, but only if the product continues to meet the orphan designation criteria when the marketing authorization is granted. If a similar medicinal product (i.e., a medicinal product with an identical active substance, or an active substance with the same principal molecular structural features and which acts via the same mechanism) receives marketing authorization for the same indication before we receive marketing authorization, the other product's orphan market exclusivity may prevent ganaxolone from receiving marketing authorization, unless we are able to demonstrate that ganaxolone is safer, more effective or otherwise clinically superior. In the EU, if we obtain and maintain orphan designation for ganaxolone upon marketing authorization, the European Commission could subsequently approve a similar medicinal product for the same indication if the European Commission, after assessment by the EMA, concludes that the similar medicinal product is safer, more effective or otherwise clinically superior. Orphan market exclusivity rights in the EU may also be lost if we are unable to supply sufficient quantities of the product.

The failure to obtain or maintain an orphan drug designation for any indication of ganaxolone that we may develop, or the inability to obtain or maintain orphan drug exclusivity could reduce our ability to make sufficient sales of ganaxolone to balance our expenses incurred to develop it, which would have a negative impact on our operational results and financial condition.

Failure to obtain regulatory approval in international jurisdictions would prevent ganaxolone from being marketed in these jurisdictions.

In order to market and sell our products in the EU and many other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the U.S. generally includes all of the risks associated with obtaining FDA approval. In addition, many countries outside the U.S. require that a product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the U.S. on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the U.S. does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA.

In the European Union, we submitted an MAA for ganaxolone in CDD to the EMA on October 11, 2021 and currently expect the EMA CHMP's opinion on the MAA by the second quarter of 2023. The Day 180 report and LoOI we received on January 26, 2023 contain a number of outstanding major objections and other concerns, including a major objection related to the choice of our regulatory starting material (RSM). The CHMP has indicated that the proposed RSM is not acceptable and should be redefined further upstream. If we are unable to address the CHMP's outstanding major objections and other concerns, including the major objection related to the RSM, the European Commission may not approve our MAA for ganaxolone in CDD even though we have received FDA approval for CDD in the United States. If we are unable to obtain approval of ganaxolone by regulatory authorities in the EU or another

country or jurisdiction, the commercial prospects of ganaxolone may be significantly diminished and our business prospects could decline.

ZTALMY is regulated as a controlled substance, which means the making, use, sale, importation, exportation, and distribution of which is subject to significant regulation by the DEA and other regulatory agencies.

The DEA regulates ganaxolone as a schedule V drug. The DEA regulates controlled substances as Schedule I, II, III, IV or V substances. Schedule I substances by definition have no established medicinal use, and may not be marketed or sold in the U.S. A pharmaceutical product may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest risk of abuse and Schedule V substances the lowest relative risk of abuse among such substances. Because ganaxolone is a schedule V controlled substance, the manufacturing, shipping, distribution, import, export, packaging, storing, prescribing, dispensing, selling and use of ganaxolone will be subject to additional regulations, including under the CSA and DEA regulations. Regulations associated with controlled substances also govern production and procurement quotas, recordkeeping, reporting, handling, and disposal. Additionally, because ganaxolone is a controlled substance, facilities conducting research, manufacturing, distributing, importing or exporting, or dispensing ganaxolone must be registered (licensed) to perform these activities and have the security, control, recordkeeping, reporting and inventory mechanisms required by the DEA. All these facilities must renew their registrations annually, except dispensing facilities, which must renew every three years. The DEA, and some states, also conduct periodic inspections of registered establishments that handle controlled substances. These regulations increase the personnel needs and the expense associated with commercialization of products. Because of their restrictive nature, these laws and regulations could also limit commercialization of ganaxolone. Failure to comply with these laws and regulations could also result in loss of DEA registrations, disruption in manufacturing and distribution activities, consent decrees, criminal and civil penalties and state actions, among other consequences.

Various states also independently regulate controlled substances. Though state controlled substances laws often mirror federal law, because states are separate jurisdictions, they may separately schedule drugs as well. While some states automatically schedule a drug when the DEA does so, in other states there must be a rulemaking or a legislative action. State scheduling may delay commercial sale of ganaxolone and adverse scheduling could impair the commercial attractiveness of ganaxolone. Many states require separate state registrations in order to be able to obtain, manufacture, handle, distribute and dispense controlled substances for clinical trials or commercial sale, and failure to meet applicable regulatory requirements could lead to enforcement and sanctions from the states in addition to those from the DEA or otherwise arising under federal law.

Risks Related to Our Dependence on Third Parties

We rely on third parties to conduct our preclinical studies and clinical trials. If these third parties do not successfully carry out their duties in compliance with contractual terms and/or regulatory requirements or meet expected timelines, our development plans may be adversely affected and we may not be able to obtain regulatory approval for ganaxolone in indications other than CDD.

We rely on third-party CROs to monitor and manage data for our ongoing preclinical and clinical programs. We also rely on third parties to assist in conducting our preclinical studies in accordance with GLP and the Animal Welfare Act requirements, where applicable. We and our CROs are required to comply with federal regulations and GCP, which are international requirements meant to protect the rights and health of patients that are enforced by the FDA, the competent authorities of the EU Member States and comparable foreign regulatory authorities for ganaxolone. Regulatory authorities enforce GCP through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable GCP, 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. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP requirements. In addition, our clinical trials must be conducted with product produced under cGMP requirements. Failure to comply with these regulations may require us to repeat or conduct additional preclinical studies and clinical trials, which would delay the regulatory approval process.

Although we depend heavily on these parties and have contractual agreements governing their activities, we cannot control them and therefore, we cannot be assured that these third parties will devote sufficient time and resources to our ongoing clinical, nonclinical and preclinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements or for other reasons, our preclinical studies and clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize ganaxolone. As a result, our results of operations and the commercial prospects for ganaxolone would be harmed, our costs could increase and our ability to generate revenue could be delayed.

For example, we announced in February 2022 a product supply interruption for our IV ganaxolone clinical supplies. Routine monitoring of stability batches of IV clinical supply material showed visible particulates of aluminum phosphate in the drug solution, which led to a pause in recruitment for the RAISE trial. In May 2022, we announced that the RAISE trial had resumed utilizing new batches of the current IV formulation of ganaxolone. We have also worked with our third-party manufacturers to implement improvements in the IV formulation with a goal of achieving a 24-month shelf life for our IV formulation, including a change in the buffer in our IV formulation to increase stability of the formulation. We are highly dependent on our third-party manufacturers to continue to supply IV ganaxolone for our ongoing clinical trials and to manufacture new supplies of IV ganaxolone. We do not control our third-party manufacturers and there is a risk that they could take longer than expected to supply IV ganaxolone or to reformulate ganaxolone as planned.

If any of our relations terminate, switching or adding additional CROs would involve additional cost and require management time and focus. Identifying, qualifying and managing performance of third-party service providers can be difficult, time consuming and cause delays in our development programs. In addition, there is a natural transition period when a new CRO commences work. If any of our relationships with our third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms. As a result, delays my occur, which can materially impact our ability to meet our desired development timelines.

In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated. We currently have a small number of employees, which limits the internal resources we have available to monitor our third-party providers. To the extent we are unable to identify and successfully manage the performance of third-party service providers, our business may be adversely affected. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

We have multiple ganaxolone drug products in development, and until such products are approved by regulatory authorities, there remains the risk that the drug product quality requirements may not support continued clinical investigation and result in delays or termination of such clinical studies, and product approvals.

We currently have multiple ganaxolone drug products in clinical development, including an oral suspension, IV solution and a new formulation which is in Phase 1 clinical trials. While we strive to develop a full understanding of manufacturing processes used, as well as the resultant product quality attributes, there is a risk that problems may arise over the course of development which could render a given drug product non-viable. Such problems could relate to manufacturing reproducibility, scale-up challenges, drug product chemical or physical stability issues. Related quality requirements may not support continued clinical investigation and result in delays or termination of such clinical studies and product approvals. Such quality requirements can include physical and chemical attributes of the drug product, stability and shelf life, microbial and other contamination, including adverse impact of drug product packaging and administration devices. These problems could result in unacceptable manufacturing economics, or direct concerns related to drug product safety or efficacy. For example, we announced in February 2022 a product supply interruption for our IV ganaxolone clinical supplies. Routine monitoring of stability batches of IV clinical supply material showed visible particulates of aluminum phosphate in the drug solution, which led to a pause in recruitment for the RAISE trial. In May 2022, we announced that the RAISE trial had resumed utilizing new batches of the current IV formulation of ganaxolone. In connection with the resumption of the trial and in consultation with the FDA, we have implemented a 12-month shelf life; refrigerated storage conditions for the entire duration of clinical use, including

storage at the clinical sites; and frequent testing for visible particles. If we experience issues with product quality requirements and we are unable to resolve these issues in a timely manner or at all, we may need to delay or terminate our RAISE or other clinical trials with the current IV formulation, which could further delay our clinical development plans and future product approvals.

Our experience manufacturing ganaxolone is limited to the needs of our preclinical studies and clinical trials, as well as initial, limited commercial supplies following FDA approval of ZTALMY for CDD in the US. We have limited experience manufacturing ganaxolone on a commercial scale and have no manufacturing facility. We are dependent on third-party manufacturers for the manufacture of ganaxolone drug substance and drug products as well as on third parties for our supply chain, and if we experience problems with any such third parties, the manufacturing and supply of ganaxolone could be delayed.

We do not own or operate facilities for the manufacture of ganaxolone. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. We currently rely on CMOs for the manufacture of ganaxolone APIs and other CMOs for the production of ganaxolone drug products, including capsules, liquid oral suspension and IV solution, and we rely on CMOs for the manufacture of ganaxolone for commercial use. To meet our projected needs for preclinical and clinical supplies to support our activities for commercial manufacturing, the CMOs with whom we currently work will need to increase the scale of production. We may need to identify additional CMOs for continued production of supply for ganaxolone. Although alternative third-party suppliers with the necessary manufacturing and regulatory expertise and facilities exist, it could be expensive and take a significant amount of time to arrange for alternative suppliers. If we are unable to arrange for alternative third-party manufacturing sources on commercially reasonable terms, in a timely manner or at all, we may not be able to complete development of ganaxolone, or market or distribute ganaxolone.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured ganaxolone ourselves, including reliance on the third party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control, including a failure to synthesize and manufacture ganaxolone, and the possibility of termination or nonrenewal of the manufacturing agreement by the third party, based on its own business priorities, at a time that is costly or damaging to us. In addition, the FDA and other regulatory authorities will require that ganaxolone be manufactured according to cGMP and similar foreign standards. Any failure by our third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of ganaxolone in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of ganaxolone. In addition, such failure could be the basis for the FDA or other regulatory authorities to issue a warning letter, withdraw approvals for ganaxolone previously granted to us, or take other regulatory or legal action, including recall or seizure of outside supplies of ganaxolone, total or partial suspension of production, suspension of ongoing clinical trials, refusal to approve pending applications or supplemental applications, detention of product, refusal to permit the import or export of products, injunction, or imposing civil and criminal penalties.

Any significant disruption in our supplier relationships could harm our business. Any significant delay in the supply of ganaxolone or its key raw materials for an ongoing preclinical study or clinical trial could considerably delay completion of such preclinical study or clinical trial, product testing and potential regulatory approval of ganaxolone. If our manufacturers or we are unable to purchase these key raw materials after regulatory approval has been obtained for ganaxolone, the commercial launch of ganaxolone would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of ganaxolone.

Ganaxolone drug substance is manufactured by a CMO in Taiwan, and key starting materials, including the regulatory starting material (RSM) are sourced from China. While the FDA has accepted our RSM, the EMA may ultimately not accept the RSM, which could result in a significant delay to EU approvals. In addition, the current drug substance supply chain sourcing API supply from China and Taiwan is subject to the geopolitical environment, which is difficult to predict and may become less stable, which may put our API supply from that region at risk.

We have entered into and may enter into additional collaboration or out-license agreements with third parties for the development or commercialization of ganaxolone in jurisdictions outside of the United States (OUS). We will depend

on these third parties for the development and/or commercialization of ganaxolone in such jurisdictions. If these collaborations or out-licenses are not successful, we may not be able to capitalize on the market potential of ganaxolone.

On July 30, 2021, we entered into a collaboration agreement (Orion Collaboration Agreement) with Orion Corporation (Orion) whereby Orion received exclusive rights to commercialize the oral and intravenous (IV) dose formulations of ganaxolone in the European Economic Area, United Kingdom and Switzerland in multiple seizure disorders, including CDD, TSC and refractory status epilepticus (RSE). On November 16, 2022, we entered into a collaboration and supply agreement (Tenacia Collaboration Agreement) with Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia), whereby Tenacia received exclusive rights to develop, commercialize and otherwise exploit certain products incorporating certain oral and IV formulations of ganaxolone in Mainland China, Hong Kong, Macau and Taiwan for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions, including CDD, TSC and status epilepticus (SE) (including RSE and Established Status Epilepticus (ESE)). The timing and amount of any milestone and royalty payments we receive under either of these agreements will depend in part on the applicable licensee's efforts. We will also depend on the applicable licensee under each agreement to comply with all applicable laws relative to the development or commercialization of ganaxolone in the specified jurisdictions subject to the applicable agreement. We do not control the individual efforts of any of our licensees, and any failure by any such licensee to devote sufficient time and effort to the development or commercialization of ganaxolone could have a material adverse impact on our financial results and operations, such as a failure by such licensee to meet its obligations to us. In addition, if a licensee were to violate, or was alleged to have violated, any laws or regulations during the performance of its obligations for us, it is possible that we could suffer financial and reputational harm or other negative outcomes, including possible legal consequences.

Any termination, breach or expiration of the Orion Collaboration Agreement, Tenacia Collaboration Agreement or any other collaboration or out-license agreements could have a material adverse effect on our financial position by reducing or eliminating the potential for us to receive milestones and royalties. In such an event, we may be required to devote additional efforts and to incur additional costs associated with pursuing the development or commercialization of ganaxolone in the applicable jurisdictions. If we breach our obligations under the Orion Collaboration Agreement, Orion may terminate the agreement and retain all rights to commercialize ganaxolone in the applicable jurisdictions with no obligation to make any additional milestone or royalty payments to us.

In addition, OUS collaborations and licenses involving ganaxolone pose a number of risks, including the following:

- collaborators or licensees have significant discretion in determining the amount and timing of efforts and resources that they will apply to these collaborations or licenses;
- collaborators or licensees may not perform their obligations as expected;
- collaborators or licensees may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon ganaxolone, repeat or conduct new clinical trials or require a new formulation of ganaxolone for clinical testing;
- collaborators or licensees may not pursue commercialization and development of ganaxolone if ganaxolone receives marketing approval or may elect not to continue or renew commercialization or development programs based on clinical trial results, changes in any such collaborator's or licensee's strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators or licensees could independently develop, or develop with third parties, products that compete directly or indirectly with ganaxolone if the collaborators or licensees believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

- product candidates discovered under the collaboration or license with us may be viewed by our collaborators or licensees as competitive with their own product candidates or products, which may cause collaborators or licensees to cease to devote resources to the commercialization of our product candidates;

- collaborators or licensees with marketing and distribution rights to ganaxolone may not, upon achieving regulatory approval, commit sufficient resources to the marketing and distribution of ganaxolone;

- collaborators or licensees could become involved in a business combination, which might deemphasize or terminate the commercialization or development of ganaxolone licensed to it by us;

- disagreements with collaborators or licensees, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of ganaxolone, might lead to additional responsibilities for us with respect to ganaxolone, or might result in litigation or arbitration, any of which would divert management attention and resources, be time-consuming and expensive;

- collaborators or licensees 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 potential litigation;

- collaborators or licensees may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and

- commercialization collaborations or licenses may be terminated for the convenience of the collaborator or licensee and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of ganaxolone.

OUS collaboration agreements and licenses may not lead to commercialization or development of ganaxolone in the most efficient manner, or at all. If any collaborations or licenses that we enter into do not result in the successful commercialization and development of products or if one of our collaborators or licensees terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration or license.

Additionally, if one of our collaborators or licensees terminates its agreement with us, we may find it more difficult to attract new collaborators or licensees and our perception in the business and financial communities could be harmed.

If we are unable to reach agreements with suitable new collaborators or licensees on a timely basis, on acceptable terms, or at all, we may have to curtail the development of a ganaxolone, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund and undertake development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop ganaxolone or bring ganaxolone to market or continue to develop ganaxolone.

Government funding for certain aspects of our programs adds uncertainty to our research efforts with respect to those programs and may impose requirements that increase the costs of commercialization and production of product candidates developed under those government-funded programs.

In September 2020, we entered into a contract (BARDA Contract) with the Biomedical Advanced Research and Development Authority (BARDA), a division of the U.S. Department of Health and Human Services' Office of the Assistant Secretary for Preparedness and Response. Under the BARDA Contract, we received an award of up to an

estimated $51 million for development of IV-administered ganaxolone for the treatment of RSE. The BARDA Contract provides for funding to support, on a cost-sharing basis, the completion of a Phase 3 clinical trial of IV-administered ganaxolone in patients with RSE, which covers the RAISE trial, funding of pre-clinical studies to evaluate IV-administered ganaxolone as an effective treatment for RSE due to chemical nerve gas agent exposure, and funding of certain ganaxolone manufacturing scale-up and regulatory activities. In March 2022, we entered into an amendment with BARDA to extend the end date of our base performance period for funding under the BARDA Contract from September 1, 2022 to December 31, 2023. In September 2022, we entered into an amendment with BARDA that, among other things, (i) provides for the exercise of BARDA's option under the BARDA Contract to support U.S. onshoring of the manufacturing capabilities for ganaxolone API (Option 2), (ii) changes the end date of our performance period under Option 2 from December 31, 2026 to July 31, 2025, (iii) increases the government cost share amount under Option 2 from approximately $11.5 million to approximately $12.3 million, and (iv) increases our cost share amount under Option 2 from approximately $4.9 million to approximately $5.3 million.

The BARDA Contract consists of an approximately two-year base period, which was extended through December 31, 2023, during which BARDA will provide up to approximately $21 million of funding for the RAISE trial on a cost share basis and funding of additional preclinical studies of ganaxolone in nerve agent exposure models. Following successful completion of the RAISE trial and preclinical studies in contract period, the BARDA Contract provides for approximately $31 million of additional BARDA funding for three options in support of ganaxolone manufacturing, supply chain, clinical, regulatory and toxicology activities, including the $12.3 million exercise of Option 2 as described above. Under the BARDA Contract, we will be responsible for cost sharing in the amount of approximately $33 million and BARDA will be responsible for approximately $52 million, if all development options are completed. The contract period-of-performance (base period plus option exercises) is up to approximately five years.

Programs funded by the U.S. government and its agencies include provisions that confer on the government substantial rights and remedies, many of which are not typically found in commercial contracts, including powers of the government to:

- terminate agreements, in whole or in part, for any reason or no reason;
- reduce or modify the government's obligations under such agreements without the consent of the other party;
- claim rights, including intellectual property rights, in products and data developed under such agreements;
- audit contract-related costs and fees, including allocated indirect costs;
- suspend the contractor from receiving new contracts pending resolution of alleged violations of procurement laws or regulations;
- impose U.S. manufacturing requirements for products that embody inventions conceived or first reduced to practice under such agreements;
- suspend or debar the contractor from doing future business with the government; and
- control and potentially prohibit the export of products.

We may not have the right to prohibit the U.S. government from using or allowing others to use certain technologies developed by us, and we may not be able to prohibit third-party companies, including our competitors, from using those technologies in providing products and services to the U.S. government. The U.S. government generally obtains the right to royalty-free use of technologies that are developed under U.S. government contracts.

In addition, government contracts normally contain additional requirements that may increase our costs of doing business, reduce our profits, and expose us to liability for failure to comply with these terms and conditions. These requirements include, for example:

- specialized accounting systems unique to government contracts;
- mandatory financial audits and potential liability for price adjustments or recoupment of government funds after such funds have been spent;
- public disclosures of certain contract information, which may enable competitors to gain insights into our research program; and
- mandatory socioeconomic compliance requirements, including labor standards, non-discrimination and affirmative action programs and environmental compliance requirements.

If we fail to maintain compliance with these requirements, we may be subject to potential contract liability and to termination of our contracts. In addition, if an audit or review uncovers any improper or illegal activity, we may be subject to civil and criminal penalties and administrative sanctions, including termination of our contracts, forfeiture of profits, suspension of payments, fines and suspension or prohibition from doing business with the U.S. government. We could also suffer serious harm to our reputation if allegations of impropriety were made against us. In addition, under U.S. government purchasing regulations, some of our costs may not be reimbursable or allowed under the BARDA Contract. Further, as a U.S. government contractor, we are subject to an increased risk of investigations, criminal prosecution, civil fraud, whistleblower lawsuits and other legal actions and liabilities as compared to private sector commercial companies.

Further, changes in government budgets and agendas may result in a decreased and de-prioritized emphasis on supporting the RSE development program. Any reduction or delay in BARDA funding may force us to seek alternative funding in order to progress our RSE program, which may not be available on non-dilutive terms, terms favorable to us or at all.

We may elect to enter into license or collaboration agreements to partner ganaxolone in territories currently retained by us. Our dependence on such relationships may adversely affect our business.

Because we have limited resources, we have and expect that we will continue to enter into license or collaboration agreements with other pharmaceutical or biotechnology companies. Any failure by our partners to perform their obligations or any decision by our partners to terminate these agreements could negatively impact our ability to successfully develop, obtain regulatory approvals for and commercialize ganaxolone. In the event we grant exclusive rights to such partners, we would be precluded from potential commercialization of ganaxolone within the territories in which we have a partner. In addition, any termination of our license or collaboration agreements will terminate the funding we may receive under the relevant license or collaboration agreement and may impair our ability to fund further development efforts and our progress in our development programs.

Our commercialization strategy for ganaxolone may depend on our ability to enter into agreements with partners to obtain assistance and funding for the development and potential commercialization of ganaxolone in the territories in which we seek to partner. Despite our efforts, we may be unable to secure license or collaboration agreements or other arrangements that are necessary for us to further develop and commercialize ganaxolone. Supporting diligence activities conducted by potential licensees or collaborators and negotiating the financial and other terms of a license or collaboration agreement are long and complex processes with uncertain results. Even if we are successful in entering into one or more license or collaboration agreements, such agreements may involve greater uncertainty for us, as we would have less control over certain aspects of our partnered programs than we do over our un-partnered programs. We may determine that continuing a license or collaboration under the terms provided is not in our best interest, and we may terminate the license or collaboration. Our potential future partners could delay or terminate their agreements, and as a result ganaxolone may never be successfully commercialized.

Further, our potential future partners may develop alternative products or pursue alternative technologies either on their own or in collaboration with others, including our competitors, and the priorities or focus of our partners may shift such that ganaxolone receives less attention or resources than we would like, or they may be terminated altogether. Any such actions by our potential future partners may adversely affect our business prospects and ability to earn revenue. In addition, we could have disputes with our potential future partners, such as the interpretation of terms in our agreements. Any such disagreements could lead to delays in the development or commercialization of ganaxolone or could result in time-consuming and expensive litigation or arbitration, which may not be resolved in our favor.

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

Our research and development activities involve the controlled use of potentially hazardous substances, including chemical and biological materials by our third-party manufacturers. Our manufacturers are subject to federal, state and local laws and regulations in the U.S. governing the use, manufacture, storage, handling and disposal of medical, radioactive and hazardous materials. 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.

Risks Related to Regulatory Compliance

Currently enacted and future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain marketing approval of and commercialize ganaxolone and affect the prices we may obtain.

The regulations that govern, among other things, marketing approvals, coverage, pricing and reimbursement for new drug products vary widely from country to country. In the U.S. and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of ganaxolone, restrict or regulate post-approval activities and affect our ability to successfully sell ganaxolone, if we obtain marketing approval.

In the U.S., there have been and continue to be a number of legislative and regulatory changes and proposed changes to contain healthcare costs. For example, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (Medicare Modernization Act) changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by eligible beneficiaries and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In recent years, Congress has considered reductions in Medicare reimbursement for drugs administered by physicians. The Centers for Medicare & Medicaid Services (CMS) also has the authority to revise reimbursement rates and to implement coverage restrictions for drugs. Cost reduction initiatives and changes in coverage implemented through legislation or regulation could decrease utilization of, and reimbursement for, any approved products, which in turn would affect the price we can receive for those products. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our drugs. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for ganaxolone, if approved, or additional pricing pressures.

The Affordable Care Act is intended to reduce the cost of, improve the quality of, and expand access to healthcare, among other things. Among other things, the Affordable Care Act expanded manufacturers' Medicaid rebate liability to include covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations,

increased the minimum rebate due for innovator drugs from 15.1% of average manufacturer price (AMP) to 23.1% of AMP and capped the total rebate amount for innovator drugs at 100.0% of AMP. The Affordable Care Act and subsequent legislation also changed the definition of AMP (which cap is set to be lifted on January 1, 2024). Furthermore, the Affordable Care Act imposed a significant annual, nondeductible fee on companies that manufacture or import certain branded prescription drug products.

Certain provisions of the Affordable Care Act have been subject to judicial challenges as well as efforts to modify them or to alter their interpretation or implementation. Additional legislative changes, regulatory changes, and judicial challenges related to the Affordable Care Act remain possible, but the nature and extent of such potential changes or challenges are uncertain at this time. The implications of the Affordable Care Act, and efforts to modify or invalidate the Affordable Care Act or its implementing regulations, or portions thereof, and the political uncertainty surrounding any efforts to further reform health care for our business and financial condition, if any, are not clear. We will continue to evaluate the effect that the Affordable Care Act as well as its possible modification or invalidation and other healthcare reform measures, has on our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. The Budget Control Act of 2011, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee did not achieve its targeted deficit reduction, which triggered the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to healthcare providers of, on average, 2.0% per fiscal year, starting in 2013 and continuing through 2031. Sequestration is currently set at 2% and will increase to 2.25% for the first half of fiscal year 2030, to 3% for the second half of fiscal year 2030, and to 4% for the remainder of the sequestration period that lasts through the first six months of fiscal year 2031.Additionally, the American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several categories of healthcare providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. If we ever obtain regulatory approval and commercialization of ganaxolone, these laws may result in reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

Further, on August 16, 2022, President Biden signed into law the Inflation Reduction Act of 2022, or IRA, which, among other things, establishes a Medicare Part B inflation rebate scheme, under which, generally speaking, manufacturers will owe rebates if the average sales price of a Part B drug increases faster than the pace of inflation. Failure to timely pay a Part B inflation rebate is subject to a civil monetary penalty. The IRA also establishes a Medicare Part D inflation rebate scheme, under which generally speaking manufacturers will owe rebates if the average manufacturer price of a Part D drug increases faster than the pace of inflation. The IRA also creates a drug price negotiation program under which the prices for Medicare units of certain high Medicare spend drugs and biologics without generic or biosimilar competition will be capped by reference to, among other things, a specified non-federal average manufacturer price, starting in 2026. Failure to comply with requirements under the drug price negotiation program is subject to an excise tax and/or a civil monetary penalty. The IRA further makes changes to the Medicare Part D benefit, including a limit on annual out-of-pocket costs, and a change in manufacturer liability under the program which could negatively affect the profitability of our product candidates. Failure to pay a discount under this new program will be subject to a civil monetary penalty. Congress continues to examine various policy proposals that may result in pressure on the prices of prescription drugs in the government health benefit programs. The IRA or other legislative changes could impact the market conditions for our product candidates.

In addition, legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of ganaxolone may be.

In the U.S., the EU and other potentially significant markets for ganaxolone, there has been increasing legislative, regulatory, and enforcement interest with respect to drug pricing practices. There have been several U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug 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 drugs. Individual states in the U.S. have also increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including by requiring pharmaceutical manufacturers to report to state agencies when they introduce new drugs to market with prices over a certain threshold, or when they increase the price of a drug over a certain threshold. Government authorities and third-party payers are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Furthermore, the increased emphasis on managed healthcare in the U.S. and on country and regional pricing and reimbursement controls in the EU will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for ganaxolone in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, which could negatively impact the revenue we are able to generate from the sale of the product in that particular country. Adverse pricing limitations may hinder our ability to recoup our investment in ganaxolone even if ganaxolone obtains marketing approval.

We participate in the Medicaid Drug Rebate Program and if we fail to comply with our reporting and payment obligations under that program or other governmental pricing programs that we participate in, we could be subject to additional reimbursement requirements, penalties, sanctions and fines, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We participate in the Medicaid Drug Rebate Program. Under the Medicaid Drug Rebate Program, we are required to pay a rebate to each state Medicaid program for our covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state Medicaid program as a condition of having federal funds being made available for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data that we report on a monthly and quarterly basis to CMS, the federal agency that administers the Medicaid Drug Rebate Program. These data include the average manufacturer price and, in the case of single-source and innovator multiple-source products, the best price for each drug which, in general, represents the lowest price available from the manufacturer to any entity in the United States in any pricing structure, calculated to include all sales and associated rebates, discounts and other price concessions subject to certain exclusions. The Affordable Care Act made significant changes to the Medicaid Drug Rebate Program, and CMS issued a final regulation, which became effective on April 1, 2016, to implement the changes to the Medicaid Drug Rebate program under the Affordable Care Act. On December 21, 2020, CMS issued a final regulation that (i) modified existing Medicaid Drug Rebate Program regulations to permit reporting multiple Best Price figures with regard to value-based purchasing arrangements and (ii) provided definitions for “line extension,” “new formulation,” and related terms with the practical effect of expanding the scope of drugs considered to be line extensions, with such changes taking effect in 2022). Our failure to comply with the aforementioned price reporting and rebate payment obligations, as well as pharmacy benefit manager (PBM) “accumulator” programs, could negatively impact our financial results. In addition, statutory and regulatory changes or other agency action regarding the Medicaid Drug Rebate Program could negatively affect our financial results or expand our rebate liability. For example, Congress could enact legislation that would extend rebates under the Medicaid Drug Rebate Program to all Children’s Health Insurance Program or CHIP utilization.

Federal law requires that any company that participates in the Medicaid Drug Rebate Program also participate in the 340B program in order for federal funds to be available for the manufacturer’s drugs under Medicaid and Medicare Part B. The 340B program, which is administered by the Health Resources and Services Administration (HRSA), requires participating manufacturers to agree to charge statutorily defined covered entities no more than the 340B “ceiling price” for the manufacturer’s covered outpatient drugs. These 340B covered entities include a variety of community health clinics and other entities that receive health services grants from the Public Health Service, as well as certain hospitals that serve a disproportionate share of low-income patients. The Affordable Care Act expanded the list of covered entities to include certain children’s hospitals, free-standing cancer hospitals, critical access hospitals, rural referral centers and sole

community hospitals, but exempted "orphan drugs" from the ceiling price requirements for these covered entities. The 340B ceiling price is calculated using a statutory formula based on the average manufacturer price and unit rebate amount for the covered outpatient drug as calculated under the Medicaid Drug Rebate Program, and in general, products subject to Medicaid price reporting and rebate liability are also subject to the 340B ceiling price calculation and discount requirement. Any additional future changes to the definition of average manufacturer price and the Medicaid unit rebate amount under the Affordable Care Act or other legislation or regulation could affect our 340B ceiling price calculations and negatively impact our results of operations if we successfully commercialize one or more products for which we receive regulatory approval.

HRSA issued a final regulation, effective January 1, 2019, regarding the calculation of the 340B ceiling price and the imposition of civil monetary penalties on manufacturers that knowingly and intentionally overcharge covered entities. HRSA also has implemented a ceiling price reporting requirement, pursuant to which manufacturers must report the 340B ceiling prices for their covered outpatient drugs to HRSA on a quarterly basis. HRSA then publishes those prices to 340B covered entities. Moreover, under a final regulation effective January 13, 2021, HRSA newly established an administrative dispute resolution (ADR) process for claims by covered entities that a manufacturer has engaged in overcharging, including claims that a manufacturer has limited the covered entity's ability to purchase covered outpatient drugs at or below the 340B ceiling price, and by manufacturers that a covered entity violated the prohibitions against diversion or duplicate discounts, including claims that an individual does not qualify as a patient for 340B Program purposes and claims that a covered entity is not eligible for the 340B Program. Such claims are to be resolved through an ADR panel of government officials rendering a decision that can be appealed to a federal court. An ADR proceeding could subject a manufacturer to onerous procedural requirements and could result in additional liability. In addition, changes to legislation, regulations, or guidance could modify 340B program compliance or expand discount liability.

Federal law also requires that a company report average sales price information each quarter to CMS for certain categories of drugs that are payable under the Medicare Part B program. Manufacturers calculate the average sales price based on a statutorily defined formula as well as regulations and interpretations of the statute by CMS. CMS uses these submissions to determine payment rates for drugs under Medicare Part B. Beginning in 2023, manufacturers must pay refunds to Medicare for single source drugs or biologics, or biosimilar biological products, reimbursed under Medicare Part B and packaged in single-dose containers or single-use packages, for units of discarded drug reimbursed by Medicare Part B in excess of 10% of total allowed charges under Medicare Part B for that drug (or a percentage established for drugs with unique circumstances). Manufacturers that fail to pay refunds could be subject to civil monetary penalties of 125% of the refund amount. The IRA establishes a Medicare Part B inflation rebate scheme, under which, generally speaking, manufacturers will owe rebates if the average sales price of a Part B drug increases faster than the pace of inflation. Failure to timely pay a Part B inflation rebate is subject to a civil monetary penalty.

In addition, manufacturers are required to provide to CMS a 70% discount on brand name prescription drugs utilized by Medicare Part D beneficiaries when those beneficiaries are in the coverage gap phase of the Part D benefit design. The IRA replaces the coverage gap discount program with a new manufacturer discount program beginning in 2025. Under either program, civil monetary penalties could be applied if a manufacturer fails to provide these discounts in the amount of 125% of the discount that was due. Moreover, the IRA also establishes a Medicare Part D inflation rebate scheme, under which generally speaking manufacturers will owe rebates if the average manufacturer price of a Part D drug increases faster than the pace of inflation. Failure to timely pay a Part D inflation rebate is subject to a civil monetary penalty.

Pricing and rebate calculations vary among products and programs. The calculations are complex and are often subject to interpretation by the manufacturer, governmental or regulatory agencies, and the courts. Such interpretation can change and evolve over time. In the case of Medicaid pricing data, if a manufacturer becomes aware that its reporting for a prior quarter was incorrect, or has changed as a result of recalculation of the pricing data, the manufacturer is obligated to resubmit the corrected data for up to three years after those data originally were due. Such restatements and recalculations increase costs for complying with the laws and regulations governing the Medicaid Drug Rebate program and could result in an overage or underage in our rebate liability for past quarters. Price recalculations also may affect the ceiling price at which a manufacturer is required to offer its products under the 340B program. Retroactive Medicaid rebates and 340B program refunds could become due as a result of these restatements. It is unclear how these restatements will impact our liability with respect to the Part B and Part D inflation rebates.

In addition, if we are found to have knowingly submitted false average manufacturer price or best price information to the government, we may be liable for significant civil monetary penalties per item of false information. Our failure to submit monthly/quarterly average manufacturer price and best price data on a timely basis also could result in a significant civil monetary penalty per day for each day the information is late beyond the due date. And if we are found to have knowingly misclassified a drug (i.e., by knowingly classifying it as a generic drug for Medicaid Drug Rebate Program purposes, which are subject to lower rebates, instead of a single-source or innovator multiple-source drug), we could be subject to civil monetary penalties no greater than two times the difference between the rebates we should have paid and the rebates we actually paid, which penalties are in addition to the penalties discussed previously. Such failures also could be grounds for CMS to terminate our Medicaid drug rebate agreement, pursuant to which we would be participating in the Medicaid Drug Rebate Program. In the event that CMS terminates our rebate agreement, no federal payments would be available under Medicaid or Medicare Part B for our covered outpatient drugs.

If we are found to have made a misrepresentation in the reporting of our average sales price, the Medicare statute provides for significant civil monetary penalties for each misrepresentation for each day in which the misrepresentation was applied. Civil monetary penalties can also be applied if we are found to have knowingly and intentionally charged 340B covered entities more than the statutorily mandated ceiling price. A covered entity or association representing covered entities can also bring claims against us through HRSA's 340B ADR process. HRSA could terminate our 340B program Pharmaceutical Pricing Agreement for good cause, which would cause our Medicaid National Drug Rebate Agreement to be terminated, rendering federal funds for our covered outpatient drugs unavailable under Medicaid and Medicare Part B. Finally, we note again that civil monetary penalties could apply if a manufacturer fails to provide discounts under the Medicare Part D coverage gap discount program in the amount of 125% of the discount that was due and similar civil monetary penalties will apply with respect to the new manufacturer discount program established under the IRA.

CMS and the HHS OIG have pursued manufacturers that were alleged to have failed to report these data to the government in a timely manner. Governmental agencies may also make changes in program interpretations, requirements or conditions of participation, some of which may have implications for amounts previously estimated or paid. If we participate in the Medicaid Drug Rebate Program and consequently the 340B program, we cannot assure you that our submissions will not be found to be incomplete or incorrect.

In order to be eligible to have our products that we successfully commercialize paid for with federal funds under the Medicaid and Medicare Part B programs and purchased by certain federal agencies and grantees, we also would have to participate in the Department of Veterans Affairs (VA) Federal Supply Schedule (FSS) pricing program. As part of this program, we would be obligated to make our products available for procurement on an FSS contract under which we would be required to comply with standard government contract terms and conditions and charge a price that is no higher than the statutory Federal Ceiling Price (FCP) to four federal agencies (VA, Department of Defense (DOD), Public Health Service, and U.S. Coast Guard).

The FCP is based on the Non-Federal Average Manufacturer Price (Non-FAMP), which we would be required to calculate and report to the VA on a quarterly and annual basis. Pursuant to applicable law and related contract terms, knowing provision of false information in connection with a Non-FAMP filing can subject a manufacturer to significant civil monetary penalties for each item of false information. The FSS pricing program and contract also contain extensive disclosure and certification requirements.

If we successfully commercialize one or more products for which we receive regulatory approval, we also would participate in the Tricare Retail Pharmacy program, under which we would be required to pay quarterly rebates on utilization of innovator products that are dispensed through the Tricare Retail Pharmacy network to Tricare beneficiaries. The rebates are calculated as the difference between the annual Non-FAMP and FCP. We would be required to list our innovator products on a Tricare Agreement in order for them to be eligible for DOD formulary inclusion. If it were concluded that we had overcharged the government in connection with our FSS contract or Tricare Agreement, whether due to a misstated FCP or otherwise, we would be required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges could result in allegations against us under the FCA and /or other laws and regulations. Unexpected refunds to the government, and/or having to respond to a government

investigation or enforcement action, could be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Laws and regulations governing international operations may preclude us from developing, manufacturing and selling product candidates outside of the U.S. and require us to develop and implement costly compliance programs.

As we seek to expand our operations outside of the U.S., we must comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The creation and implementation of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The Foreign Corrupt Practices Act (FCPA) prohibits any U.S. individual or business from paying, offering, 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 U.S. to comply with certain accounting provisions requiring such companies 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. The anti-bribery provisions of the FCPA are enforced primarily by the DOJ. The SEC is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the U.S., or the sharing with certain foreign nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Our expanding presence outside of the U.S. will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling ganaxolone outside of the U.S., which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

Our relationships with customers and third-party payers will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and others will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with healthcare professionals, third-party payers, patients and others will expose us to broadly applicable fraud and abuse, anti-kickback, false claims, and other healthcare laws and regulations that may affect the business or financial arrangements and relationships through which we would market, sell and distribute our products. Even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payers, federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. Restrictions under applicable federal and state healthcare laws and regulations that may affect our operations (including our

marketing, promotion, educational programs, pricing, and relationships with healthcare providers or other entities, among other things) and expose us to areas of risk including the following:

- the federal Anti-Kickback Statute (AKS) prohibits, among other things, knowingly and willfully soliciting, offering, or receiving remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, or arranging for the purchase, lease, or order of, any healthcare item or service, for which payment may be made under a federal healthcare program such as Medicare & Medicaid;

- the FCA prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, a false or fraudulent claim for payment of government funds, or knowingly making, using, or causing to be made or used a false record or statement material to an obligation to pay money to the government, or knowingly and improperly avoiding, decreasing or concealing an obligation to pay money to the federal government;

- other federal false claims laws, including, among others, federal criminal healthcare fraud and false statement statutes that extend to non-government health benefit programs;

- the Health Insurance Portability and Accountability Act of 1996 and its implementing regulations (collectively, HIPAA) imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program, including private third-party payers, and also prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services;

- the federal Physician Payments Sunshine Act, implemented as the Open Payments Program, requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or Children's Health Insurance Program, to report annually to CMS information related to payments and other transfers of value to physicians, and teaching hospitals, and starting in 2022 certain other health care professionals, and ownership and investment interests held by physicians and their immediate family members; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payers, including private insurers, as well as other state laws and regulations governing pharmaceutical manufacturers; and

- state and foreign laws and regulations govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties are compliant with applicable healthcare laws and regulations will involve the expenditure of appropriate, and possibly significant, resources. Nonetheless, it is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion from government funded healthcare programs, such as Medicare & Medicaid, and the curtailment or restructuring of our operations. If any physicians or other healthcare providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

We may be subject to data privacy and security regulations by both the federal government and the states in which we conduct our business, which could impose significant regulatory hurdles on our business.

HIPAA imposes requirements relating to the privacy, security and transmission of individually identifiable health information. HIPAA imposes privacy and security obligations on covered entity health care providers, health plans, and health care clearinghouses, as well as their "business associates"—certain persons or entities that create, receive, maintain or transmit protected health information in connection with providing a specified service on behalf of a covered entity. We may obtain health information from third parties (including research institutions from which we obtain clinical trial data) that are subject to privacy and security requirements under HIPAA. Although we are not directly subject to HIPAA, other than potentially with respect to providing certain employee benefits, we could potentially be subject to criminal penalties if we, our affiliates, or our agents knowingly receive individually identifiable health information maintained by a HIPAA-covered entity in a manner that is not authorized or permitted by HIPAA. In addition, numerous other federal and state laws and regulations govern privacy and security, including state data breach notification laws, state health information and/or genetic privacy laws, and federal and state consumer protection laws (e.g., Section 5 of the FTC Act, and the California Consumer Privacy Act (CCPA), many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. Compliance with these laws is difficult, constantly evolving, and time consuming. Federal regulators, state attorneys general, and plaintiffs' attorneys have been and will likely continue to be active in this space.

Furthermore, states are constantly adopting new laws or amending existing laws relating to the data privacy and security and consumer protection, requiring attention to frequently changing regulatory requirements. For example, in California, the CCPA establishes certain requirements for data use and sharing transparency and creates new data privacy rights for California residents. The CCPA and its implementing regulations have already been amended multiple times since their enactment. In November 2020, California voters approved the California Privacy Rights Act (CPRA) ballot initiative which introduced significant amendments to the CCPA and established and funded a dedicated California privacy regulator, the California Privacy Protection Agency (CPPA). The amendments introduced by the CPRA go into effect on January 1, 2023, and new implementing regulations are expected to be introduced by the CPPA. Failure to comply with the CCPA may result in, among other things, significant civil penalties and injunctive relief, or potential statutory or actual damages. In addition, California residents have the right to bring a private right of action in connection with certain types of incidents. These claims may result in significant liability and potential damages. We implemented processes to manage compliance with the CCPA and continue to assess the impact of the CPRA, and other state legislation, on our business as additional information and guidance becomes available.

The Federal Trade Commission (FTC) also sets expectations for failing to take appropriate steps to keep consumers' personal information secure, or failing to provide a level of security commensurate to promises made to individual about the security of their personal information (such as in a privacy notice) may constitute unfair or deceptive acts or practices in violation of Section 5(a) of the Federal Trade Commission Act (FTC Act). The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business, and the cost of available tools to improve security and reduce vulnerabilities. Individually identifiable health information is considered sensitive data that merits stronger safeguards. With respect to privacy, the FTC also sets expectations that companies honor the privacy promises made to individuals about how the company handles consumers' personal information; any failure to honor promises, such as the statements made in a privacy policy or on a website, may also constitute unfair or deceptive acts or practices in violation of the FTC Act. While we do not intend to engage in unfair or deceptive acts or practices, the FTC has the power to enforce promises as it interprets them, and events that we cannot fully control, such as data breaches, may be result in FTC enforcement. Enforcement by the FTC under the FTC Act can result in civil penalties or enforcement actions.

Similarly, there are a number of legislative proposals in the EU, the U.S. (at both the federal and state level), as well as in other jurisdictions that could change existing obligations, and/or impose new obligations or limitations in areas affecting our business. These laws and regulations are evolving and subject to interpretation, and may impose limitations on our activities or otherwise adversely affect our business. In addition, some countries are considering or have passed legislation implementing data protection requirements or requiring local storage and processing of data or similar requirements that could increase the cost and complexity of delivering our services and research activities. These laws and regulations, as well as any associated claims, inquiries, or investigations or any other government actions may lead

to unfavorable outcomes including increased compliance costs, delays or impediments in the development of new products, negative publicity, increased operating costs, diversion of management time and attention, and remedies that harm our business, including fines or demands or orders that we modify or cease existing business practices.

If we, our agents, or our third party partners fail to comply or are alleged to have failed to comply with these or other applicable data protection and privacy laws and regulations, or if we were to experience a data breach involving personal information, we could be subject to government enforcement actions or private lawsuits. Any associated claims, inquiries, or investigations or other government actions could lead to unfavorable outcomes that have a material impact on our business including through significant penalties or fines, monetary judgments or settlements including criminal and civil liability for us and our officers and directors, increased compliance costs, delays or impediments in the development of new products, negative publicity, increased operating costs, diversion of management time and attention, or other remedies that harm our business, including orders that we modify or cease existing business practices.

In addition, the EU's legislative and regulatory landscape for privacy and data security continues to evolve. There has been increased attention to privacy and data security issues that could potentially affect our business, including the EU General Data Protection Regulation (GDPR), which imposes penalties up to 4% of annual global turnover for breaches of related obligations.

In the event we enroll patients in our ongoing or future clinical trials in the European Economic Area (EEA), we will be subject to the additional privacy restrictions imposed by the GDPR, including restrictions relating to the collection, use, storage, transfer, and other processing of personal data, including personal health data, regarding individuals in the EEA as governed by the GDPR and the related national data protection laws of the individual EEA countries. The GDPR imposes several requirements on companies that process personal data, with especially strict rules on the transfer of personal data out of the EEA, including to the U.S, and fines and penalties for failure to comply with the requirements of the GDPR and the related national data protection laws of the individual EEA countries. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. The obligations under the GDPR may be onerous and adversely affect our business, financial condition, results of operations and prospects. Compliance with the GDPR will be a rigorous and time-intensive process that may increase our cost of doing business or require us to change our business practices, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation, and reputational harm in connection with any EEA activities. Further, the United Kingdom's exit from the EU, often referred to as Brexit, has created uncertainty with regard to future data protection regulation in the United Kingdom. The European Commission has adopted an Adequacy Decision concerning the level of data protection in the United Kingdom. Personal data may now flow freely from the EEA to the United Kingdom, however, the European Commission may suspend the Adequacy Decision if it considers that the United Kingdom no longer provides for an adequate level of data protection.

Because of the remote work policies we implemented due to the COVID-19 pandemic, information that is normally protected, including company confidential information, may be less secure. Cybersecurity and data security threats continue to evolve and raise the risk of incidents, including breaches, that could affect our operations or compromise our business information or sensitive personal information, including health data. With the ever-changing threat landscape, and while we have implemented security measures to protect our information technology systems and infrastructure, there can be no assurance that such measures will prevent service interruptions or security breaches that could adversely affect our business.

We may also need to collect more extensive health-related information from our employees to manage our workforce. If we or our third party partners fail to comply or are alleged to have failed to comply with applicable data protection and privacy laws and regulations, and related employment rules, or if we were to experience a data breach involving personal information, we could be subject to government enforcement actions, fines, or private lawsuits.

In addition, our business could be adversely impacted if our ability to transfer personal data outside of the EEA or Switzerland is restricted, which could adversely impact our operating results. For example, in July 2020, the Court of Justice of the European Union, or the Court of Justice, declared the Privacy Shield to be an invalid data transfer mechanism and confirmed that the Model Clauses remain valid and in June 2021, the European Commission published

updated versions of the Model Clauses, which must be incorporated into new and existing agreements within prescribed timeframes in order to continue to lawfully transfer personal data outside of the EU. This Decision (Decision 2018/1250) invalid, which could adversely impact our ability to transfer personal data from the EU to the U.S. The Court of Justice further ruled that in order to transfer data outside of the EU, under the existing mechanism known as the Standard Contractual Clauses (SCCs), the exporter and the importer must ensure that the importer may guarantee a level of personal data protection in the importing country's level of protection must be adequate that is essentially equivalent to that of the EEA. Compliance with data transfer obligations involves documenting detailed analyses of data access and protection laws in the countries in which data importers are located, which can be costly and time-consuming. Data importers must also expend resources in analyzing their ability to comply with transfer obligations, including implementing new safeguards and controls to further protect personal data.

On September 8, 2020, the Federal Data Protection and Information Commissioner (FDPIC) of Switzerland issued an opinion concluding that the Swiss-U.S. Privacy Shield Framework does not provide an adequate level of protection for data transfers from Switzerland to the U.S. The FDPIC also found that SCCs may still be legally adequate at an individual level provided that they can pass a risk assessment conducted by the FDPIC. If the level of protection in the U.S. or any other importing country is called into question under the SCCs, this could further impact our ability to transfer data outside of the EU or Switzerland.

The UK withdrew from the EU on January 31, 2020, commonly referred to as Brexit. Marketing authorizations granted through the EU centralized procedure continue to be valid in Northern Ireland by virtue of the Northern Ireland Protocol, but such EU marketing authorizations are not valid in the rest of the UK (England, Wales and Scotland, or collectively Great Britain). EU marketing authorizations existing as of the end of the Brexit transition period on December 31, 2020, were automatically converted into Great Britain marketing authorizations as of January 1, 2021. Until the end of 2023, a marketing authorization for Great Britain can be applied for on an expedited timetable through the UK European Commission Decision Reliance Procedure, after having received a positive opinion from the EMA's Committee for Medicinal Products for Human Use. It is not yet known whether the UK European Commission Decision Reliance Procedure will remain available after 2023. A Great Britain marketing authorization can alternatively be applied for separately through the standard national level procedure.

Although the body of the UK-EU Trade and Cooperation Agreement includes general terms which apply to medicinal products, greater detail on sector-specific issues is provided in an Annex to the Agreement. The Annex provides a framework for the recognition of GMP inspections and for the exchange and acceptance of official GMP documents. The regime does not, however, extend to procedures such as batch release certification. Among the changes that will now occur are that Great Britain, comprised of England, Scotland and Wales, will be treated as a third country. Northern Ireland will, with regard to EU regulations, continue to follow the EU regulatory rules. As part of the UK-EU Trade and Cooperation Agreement, the EU and the UK will recognize GMP inspections carried out by the other Party and the acceptance of official GMP documents issued by the other Party. The UK-EU Trade and Cooperation Agreement also encourages, although it does not oblige, the parties to consult one another on proposals to introduce significant changes to technical regulations or inspection procedures. Among the areas of absence of mutual recognition are batch testing and batch release. The EU continues to apply EU laws that require batch testing and batch release to take place in the EU territory. This means that medicinal products that are tested and released in the UK must be retested and re-released when entering the EU market for commercial use.

Risks Related to Intellectual Property

If we are unable to protect our intellectual property rights or if our intellectual property rights are inadequate for our technology and product candidates, our competitive position could be harmed.

Our commercial success will depend in large part on our ability to obtain and maintain patent and other intellectual property protection in the U.S. and other countries with respect to our technology and products. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. We seek to protect our proprietary position by filing and prosecuting patent applications in the U.S. and abroad related to our novel technologies and products that are important to our business.

The patent positions of biotechnology and pharmaceutical companies generally are highly uncertain, involve complex legal and factual questions and have in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patents, including those patent rights licensed to us by third parties, are highly uncertain. The steps we or our licensors have taken to protect our proprietary rights may not be adequate to preclude misappropriation of our proprietary information or infringement of our intellectual property rights, both inside and outside the U.S. Further, the examination process may require us or our licensors to narrow the claims for our pending patent applications, which may limit the scope of patent protection that may be obtained if these applications issue. The rights already granted under any of our currently issued patents or those licensed to us and those that may be granted under future issued patents may not provide us with the protection or competitive advantages we are seeking. If we or our licensors are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficient, our competitors could develop and commercialize technology and products similar or superior to ours, and our ability to successfully commercialize our technology and products may be adversely affected. It is also possible that we or our licensors will fail to identify patentable aspects of inventions made in the course of our development and commercialization activities before it is too late to obtain patent protection on them.

With respect to patent rights, the ganaxolone compound and its original synthesis were published in the early 1990s and we do not own or license patent rights on the ganaxolone compound. We seek patent protection in the U.S. and internationally for synthetic methods for making ganaxolone, ganaxolone nanoparticles, which are used in certain oral solid, oral liquid, and IV dose formulations, other injectable and oral ganaxolone formulations, and methods of treatment using ganaxolone. We do not know whether any of our granted or issued patents will, or if any of our pending patent applications will grant as patents that will, effectively prevent others from commercializing competitive technologies and products. There is a risk that others, including companies that make generic pharmaceuticals, may develop ganaxolone for the same as similar uses as us, and that our patents will not effectively prevent them from commercializing their ganaxolone products. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing or in some cases not at all, until they are issued as a patent. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

Our pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, issued patents that we own or have licensed from third parties may be challenged in the courts or patent offices in the U.S. and abroad. Such challenges may result in the loss of patent protection, the narrowing of claims in such patents or the invalidity or unenforceability of such patents, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection for our technology and products. Protecting against the unauthorized use of our or our licensors' patented technology, trademarks and other intellectual property rights is expensive, difficult and may in some cases not be possible. In some cases, it may be difficult or impossible to detect third-party infringement or misappropriation of our intellectual property rights, even in relation to issued patent claims, and proving any such infringement may be even more difficult.

Competitors may infringe our patents or misappropriate or otherwise violate our intellectual property rights. To counter infringement or unauthorized use, litigation may be necessary in the future to enforce or defend our intellectual property rights, to protect our trade secrets or to determine the validity and scope of our own intellectual property rights or the proprietary rights of others. Also, third parties may initiate legal proceedings against us to challenge the validity or scope of intellectual property rights we own or control. These proceedings can be expensive and time consuming. Many of our current and potential competitors have the ability to dedicate substantially greater resources to defend their intellectual property rights than we can. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating our intellectual property. Litigation could result in substantial costs and diversion of management resources, which could harm our business and financial results. In addition, in an infringement proceeding, a court may decide that a patent owned or controlled by us is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated,

held unenforceable or interpreted narrowly. 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 shares of our common stock.

Third parties, such as Ovid Therapeutics, Inc., may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability to develop, manufacture, market and sell our products, all of which contain ganaxolone, if approved, and to use our related technologies. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to one or more of our products, including interference or derivation proceedings before the U.S. Patent and Trademark Office (USPTO). Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue commercializing one or more of our products. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Under certain circumstances, we could be forced, including by court order, to cease commercializing one or more of our products. In addition, in any such proceeding or litigation, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing one or more of our products or force us to cease some of our business operations, which could materially harm our business. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar negative impact on our business.

While our product candidates are in preclinical studies and clinical trials, we believe that the use of our product candidates in these preclinical studies and clinical trials falls within the scope of the exemptions provided by 35 U.S.C. Section 271(e) in the U.S., which exempts from patent infringement liability activities reasonably related to the development and submission of information to the FDA (Federal Development Patent Infringement Exemption). As our product candidates progress toward commercialization, the possibility of a patent infringement claim against us increases. While ganaxolone itself is off patent, we attempt to ensure that our product candidates and the methods we employ to manufacture ganaxolone do not infringe other parties' patents and other proprietary rights. There can be no assurance they do not, however, and competitors or other parties may assert that we infringe their proprietary rights in any event.

On July 26, 2022, the USPTO issued a patent to Ovid Therapeutics, Inc. (Ovid) with claims that encompass our product candidate for the treatment of SE. Ovid may file a lawsuit against us alleging infringement of its patents and/or we may challenge the validity of Ovid's patents with the USPTO or through the courts. Any such proceeding, regardless of its outcome, would likely result in the expenditure of significant financial resources and the diversion of management's time and resources. In addition, any such proceeding may cause negative publicity, adversely impact patients, and we may be prohibited from marketing or selling ganaxolone for SE, RSE and ESE during such proceedings or if we are not successful in such proceedings. If Ovid does decide to bring an infringement lawsuit, we do not expect that it will be filed before a commercial launch of ganaxolone for SE, RSE or ESE based upon the "safe harbor" provisions of the Hatch-Waxman Act. We may need to acquire or obtain a license to the Ovid patents to market or sell ganaxolone for SE, RSE or ESE, which may not be available on commercially acceptable terms or at all. If we are not able to acquire the Ovid patents or negotiate a license on acceptable terms, and if our product is determined to infringe Ovid's patents and the patents are determined to be valid, then we may be forced to pay Ovid royalties, damages and costs, or we may be prevented from commercializing ganaxolone for SE, RSE and ESE altogether, which would have a material adverse impact on our business.

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

Filing, prosecuting and defending patents on our product candidates and any future product candidates throughout the world would be prohibitively expensive, and our or our licensors' intellectual property rights in some countries outside the U.S. can be less extensive than those in the U.S. In addition, the laws and practices of some foreign countries, particularly those relating to pharmaceuticals, do not protect intellectual property rights to the same extent as

federal and state laws in the U.S. For example, novel formulations and methods of medical treatment and manufacturing processes may not be patentable in certain jurisdictions, and the requirements for patentability may differ in certain countries, particularly developing countries. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our patents, requiring us to engage in complex, lengthy and costly litigation or other proceedings. Generic drug manufacturers may develop, seek approval for, and launch generic versions of our products. Many countries, including EU countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under certain circumstances 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 our 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 our intellectual property.

We may not be able to prevent third parties from practicing our inventions in all countries outside the U.S., or from selling or importing products made using our inventions into or within the U.S. or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products, and may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong as that in the U.S. These products may compete with our products in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing with us in these jurisdictions.

Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost 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 our patent applications at risk of not issuing 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.

Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time.

Given the amount of time required for the development, testing and regulatory review of new product candidates, such as our product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. For example, certain patents licensed to us by CyDex that relate to Captisol®, which is used in some of our product candidates, have expired, and sulfobutylether beta-cyclodextrin compounds that are similar to CyDex's Captisol® are available from other suppliers. It is possible that others may seek to develop ganaxolone formulations using sulfobutylether beta-cyclodextrin compounds obtained from such other suppliers.

We expect to seek extensions of patent terms in the U.S. and, if available, in other countries where we are prosecuting patents. In the U.S., the Drug Price Competition and Patent Term Restoration Act of 1984 permits under certain circumstances a patent term extension of up to five years beyond the normal expiration of a patent. However, the applicable authorities, including the FDA and the USPTO in the U.S., and any analogous regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case. For example, we have a pending patent term extension application of a granted U.S. patent that covers ZTALMY. This application requests an extension of five (5) years, which, if the full extension is granted, this U.S. patent would be extended to November 28, 2031. It is possible that we will not obtain patent term extension for this U.S. patent, or if we obtain such an extension, it may be for a shorter period than we had sought.

Changes in patent laws could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

As is the case with other pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the pharmaceutical industry involve technological and legal

complexity, and obtaining and enforcing pharmaceutical patents is costly, time-consuming, and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries may diminish the value of our patents or narrow the scope of our patent protection. For example, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. 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 Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce existing patents and patents we may obtain in the future. Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

In addition, the Leahy-Smith America Invents Act (Leahy-Smith Act) includes a number of provisions that affect the way patent applications are prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the U.S. transitioned to a "first to file" system in which the first inventor to file a patent application is entitled to the patent. Third parties are allowed to submit prior art before the issuance of a patent by the USPTO and may become involved in derivation, reexamination, inter-partes review or post-grant review proceedings challenging our patent rights or the patent rights of our licensors. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate patent rights, which could adversely affect our competitive position.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submissions, 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.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. 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 application process. While an inadvertent lapse can in many cases 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. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

We may be subject to claims by third parties asserting that we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Some of our employees were previously employed at universities or at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Some of these employees, including each member of our senior management, executed proprietary rights, non-disclosure and non-competition agreements, or similar agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

We rely on government funding for certain aspects of our research and development activities and we may develop intellectual property through such activities and therefore may be subject to federal regulations such as "march-in" rights, certain reporting requirements and a preference for U.S. based companies. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.

In September 2020, we entered into the BARDA Contract for the completion of pre-clinical and clinical development activities for IV administered ganaxolone for the treatment of RSE. We may generate intellectual property rights through the use of this U.S. government funding and are therefore subject to certain federal regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act of 1980 (Bayh-Dole Act), and implementing regulations. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive, or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention; (ii) government action is necessary to meet public health or safety needs; or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to these inventions if we to disclose the invention to the government and fail to file an application to register the intellectual property in the specified manner and within specified time limits. These time limits have recently been changed by regulation, and may change in the future. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us to expend substantial resources. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the U.S. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the U.S. or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

- others may be able to make compounds or ganaxolone formulations that are similar to our product candidates but that are not covered by the claims of the patents that we own or control;
- we or any strategic partners might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or control;
- we might not have been the first to file patent applications covering certain of our inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;
- it is possible that our pending patent applications will not lead to issued patents;
- issued patents that we own or control may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;
- our competitors might conduct research and development activities in the U.S. and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as

well as 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 may not develop additional proprietary technologies that are patentable; and
- the patents of others may have an adverse effect on our business.

Risks Related to our Business Operations and Ownership of our Common Stock

The COVID-19 pandemic could continue to adversely affect our business and our ability to conduct and complete clinical trials.

The continued global spread of COVID-19, including the Omicron variant, has impacted our clinical operations and timelines. For example, our Phase 3 RAISE trial is conducted in hospitals, including intensive care units and academic medical centers, which have experienced high rates of COVID-19 admissions. Several academic medical centers and intensive care units participating in the RAISE trial have experienced COVID-related difficulties, including staff turnover and the need to devote significant resources to patients with COVID-19, which has resulted in site initiation and enrollment delays for the RAISE trial. Given these COVID-19-related challenges and our recent interruption in drug supply as described above, we now expect our top-line data readout for the RAISE trial to be available in the second half of 2023. Several of the sites participating in the RAISE trial continue to encounter COVID-related setbacks, including staff turnover and the need to devote significant resources for patients with COVID-19. In addition, our ganaxolone clinical trials in the outpatient setting may be negatively impacted if patients and their caregivers do not want to participate while the COVID-19 pandemic persists. The duration and severity of the pandemic and its long-term impact on our business are uncertain at this time.

If a patient participating in one of our clinical trials contracts COVID-19, this could negatively impact the data readouts from these trials; for example, the patient may be unable to participate further (or may have to limit participation) in our clinical trial, the patient may show a different efficacy assessment than if the patient had not been infected, or the patient could experience an AE that could be attributed to our product candidate.

There is also a risk that clinical supplies of our product candidates may be significantly delayed or may become unavailable as a result of COVID-19 and the resulting impact on our suppliers' labor forces and operations, including as a result of governmental restrictions on business operations and the movement of people and goods in an effort to curtail the spread of the virus. There can be no assurance that we would be able to timely implement any mitigation plans. Disruptions in our supply chain, whether as a result of restricted travel, quarantine requirements or otherwise, could negatively impact clinical supplies of our product candidates, which could materially adversely impact our clinical trial and development timelines.

The global spread of COVID-19 has also led to severe disruption and volatility in the global capital markets, which could increase our cost of capital and adversely affect our ability to access the capital markets in the future. The potential exists for the continued spread of COVID-19 to cause an economic slowdown or recession or cause other unpredictable events, each of which could adversely affect our business, results of operations or financial condition.

The extent to which COVID-19 impacts our business will depend on future developments, which are highly uncertain and cannot be predicted, including new information which may emerge concerning the severity of the COVID-19 outbreak and the actions to contain the outbreak or treat its impact, among others. Moreover, the COVID-19 outbreak has begun to have indeterminable adverse effects on general commercial activity and the world economy, and our business and results of operations could be adversely affected to the extent that COVID-19 or any other pandemic harms the global economy generally.

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

As of December 31, 2022, we had 151 full-time employees and two part-time employees. As our development and commercialization plans and strategies develop, or as a result of any future acquisitions, we will need additional

managerial, operational, sales, marketing, financial and other resources. In addition, it may become more cost effective to bring in house certain resources currently outsourced to consultants and other third-parties. Our management, personnel and systems currently in place may not be adequate to support our future growth. Future growth would impose significant added responsibilities on members of management, including:

- growing our commercial operations;
- managing our clinical trials effectively;
- identifying, recruiting, maintaining, motivating and integrating additional employees;
- managing our internal development efforts effectively while complying with our contractual obligations to licensors, licensees, contractors and other third parties;
- improving our managerial, development, operational and finance systems; and
- expanding our facilities.

As our operations expand, we will need to manage additional relationships with various strategic partners, suppliers and other third parties. Our future financial performance and our ability to commercialize ZTALMY and, if approved, ganaxolone in other indications we are currently developing, and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to manage our development efforts and clinical trials effectively and hire, train and integrate additional management, administrative and sales and marketing personnel. Our failure to accomplish any of these tasks could prevent us from successfully growing our company.

Our business and operations would suffer in the event of computer system failures, cyberattacks or a deficiency in our cybersecurity or those of any business partners.

Despite the implementation of security measures, our internal computer systems, and those of our CROs and other third parties on which we rely, are vulnerable to damage from, among other things, computer viruses, unauthorized access, natural disasters, fire, terrorism, war, telecommunication and electrical failures, cyberattacks or cyber-intrusions over the Internet, loss of funds or information from phishing or other fraudulent schemes, or attachments to emails. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments, and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. Such an event could cause interruption of our operations or loss of our funds and have a negative financial consequence on our business. In addition, our systems safeguard important confidential personal data regarding patients enrolled in our clinical trials. If a disruption event were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. For example, the loss of data relating to completed, ongoing or planned clinical trials could result in delays in our regulatory approval efforts and cause us to incur significant additional costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, misappropriation of funds to unintended recipients, or inappropriate disclosure of confidential, proprietary or personal information, we could incur material legal claims and liabilities and damage to our reputation and the further development of ganaxolone could be delayed. Additionally, breach remediation costs may be significant.

The market price of our stock has been, and may continue to be, highly volatile, and you could lose all or part of your investment.

Historically, the trading price of our common stock has been highly volatile, and it is likely that such price will continue to be volatile in the future. The trading price of our common stock could be subject to wide fluctuations in response to various factors, some of which are beyond our control. In addition to the factors discussed elsewhere in this "Risk Factors" section, these factors could include:

- the success of competitive products or technologies;
- regulatory actions with respect to our products or our competitors' products;
- actual or anticipated changes in our growth rate relative to our competitors;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the level of expenses related to our clinical development programs;
- the results of our efforts to in-license or acquire additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or our other stockholders;
- changes in the structure of healthcare payment systems; and
- other events or factors, many of which are beyond our control.

In addition, the stock market in general, the Nasdaq Global Market and pharmaceutical and biotechnology 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. The realization of any of these risks or any of a broad range of other risks, including those described in these "Risk Factors," could have a dramatic and material adverse impact on the market price of our common stock.

Insiders have substantial influence over us and could delay or prevent a change in corporate control.

We estimate that our executive officers, directors and holders of 5% or more of our capital stock collectively beneficially own approximately 39.8% of our voting stock. Upon conversion of all our outstanding convertible preferred stock and pre-funded warrants, as of December 31, 2022, our executive officers, directors and holders of 5% or more of our capital stock collectively would beneficially own approximately 37.6% of our voting stock. This concentration of

ownership could harm the market price of our common stock by delaying, deferring or preventing a strategic transaction, even if such a transaction would benefit other stockholders. The interests of this group of stockholders may not always coincide with your interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might negatively affect the prevailing market price for our common stock.

Our operating results may fluctuate significantly in the future, which may cause our results to fall below the expectations of securities analysts, stockholders and investors.

Our operating results may fluctuate significantly in the future as a result of a variety of factors, many of which are outside of our control. These factors include, but are not limited to:

- the timing, implementation and cost of our research, preclinical studies and clinical trials;
- our ability to attract and retain personnel with the necessary strategic, technical and creative skills required for effective operations;
- introduction of new technologies;
- product liability litigation, class action and derivative action litigation, or other litigation;
- the amount and timing of capital expenditures and other costs relating to the expansion of our operations;
- the state of the debt and/or equity capital markets at the time of any proposed offering we choose to initiate;
- our ability to successfully integrate new acquisitions into our operations;
- government regulation and legal developments regarding ganaxolone in the U.S. and in the foreign countries in which we may operate in the future; and
- general economic conditions.

As a strategic response to changes in the competitive environment, we may from time to time make pricing, service, technology or marketing decisions or business or technology acquisitions that could have a material adverse effect on our operating results. Due to any of these factors, our operating results may fall below the expectations of securities analysts, stockholders and investors in any future period, which may cause our stock price to decline.

Some provisions of our charter documents and Delaware law may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would be beneficial to our stockholders and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our fourth amended and restated certificate of incorporation, as amended (Certificate of Incorporation) and amended and restated bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders, or remove our current management. These include provisions that:

- permit our board of directors to issue up to 25,000,000 shares of preferred stock, with any rights, preferences and privileges as it may designate, of which 4,300 shares of Series A Preferred Stock are outstanding;
- provide that all vacancies on our board of directors, including as a result of newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;

- establish a classified board of directors such that only one of three classes of directors is elected each year;
- provide that directors can only be removed for cause;
- require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;
- provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide advance notice in writing, and also specify requirements as to the form and content of a stockholder's notice;
- not provide for cumulative voting rights, thereby allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election; and
- provide that special meetings of our stockholders may be called only by the chairperson of the board of directors, the chief executive officer or the board of directors.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, who are responsible for appointing the members of our management. Because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law (DGCL), which prohibits, with some exceptions, stockholders owning in excess of 15.0% of our outstanding capital stock from merging or combining with us.

Our Certificate of Incorporation contains exclusive forum provisions, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers, employees or agents.

Our Certificate of Incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware shall be the sole and exclusive forum for (a) any derivative action or proceeding brought on our behalf, (b) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or agents to us or our stockholders, (c) any action asserting a claim arising pursuant to any provision of the DGCL, or (d) any action asserting a claim that is governed by the internal affairs doctrine, in each such case subject to such Court of Chancery's having personal jurisdiction over the indispensable parties named as defendants therein.

For the avoidance of doubt, the exclusive forum provisions described above do not apply to any claims arising under the Securities Act or under the Exchange Act. Section 27 of the Exchange Act creates exclusive federal jurisdiction over all suits brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder, and Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder.

The choice of forum provisions in our Certificate of Incorporation may limit our stockholders' ability to bring a claim in a judicial forum that they find favorable for disputes with us or our directors, officers, employees or agents, which may discourage such lawsuits against us and our directors, officers, employees and agents even though an action, if successful, might benefit our stockholders. The applicable courts may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring the action, and such judgments or results may be more favorable to us than to our stockholders. With respect to the provision making the Court of Chancery the sole and exclusive forum for certain types of actions, stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near Delaware. Finally, if a court were to find these provisions of our Certificate of Incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions

or proceedings, we may incur additional costs associated with resolving such matters in other jurisdictions, which could have a material adverse effect on the company.

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

Our principal offices occupy approximately 22,500 square feet of leased office space in Radnor, Pennsylvania pursuant to a lease agreement that expires in 2025. We believe that our facilities are suitable and adequate to meet our current needs. We may add new facilities or expand existing facilities as we add employees, and we believe that suitable additional or substitute space will be available as needed to accommodate any such expansion of our operations.

Item 3. Legal Proceedings.

From time to time, we may become subject to litigation and claims arising in the ordinary course of business. We are not currently a party to any material legal proceedings, and we are not aware of any pending or threatened legal proceedings against us that we believe could have a material adverse effect on our business, operating results or financial condition.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

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

Market Information

Our common stock is listed on the Nasdaq Global Market under the symbol "MRNS."

Holders of Record

As of March 3, 2023, there were approximately 15 holders of record of shares of our common stock. This number does not reflect the beneficial holders of our common stock who hold shares in street name through brokerage accounts or other nominees.

Securities Authorized for Issuance under Equity Compensation Plans

Information regarding securities authorized for issuance under equity compensation plans is incorporated by reference into the information in Part III, Item 12 of this Form 10-K.

Recent Sales of Unregistered Securities

We did not issue any equity securities during the year ended December 31, 2022 that were not registered under the Securities Act and that have not otherwise been described in a Quarterly Report on Form 10-Q or a Current Report on Form 8-K.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

The following table provides certain information with respect to purchase of our common stock during the three months ended December 31, 2022:

Period	Total Number of Shares Purchased (1)	Average Price Paid Per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs	Maximum Number (or Approximate Dollar Value) of Shares That May Yet Be Purchased Under the Plans or Programs
October 1, 2022 through October 31, 2022	-	-	-	-
November 1, 2022 through November 30, 2022	1,505	4.70	-	-
December 1, 2022 through December 31, 2022	-	-	-	-
Total	1,505	$ 4.70	-	$ -

(1) Represents shares of common stock withheld to satisfy taxes associated with the vesting of restricted stock

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 financial statements and the related notes appearing at the end of this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. You should read "Cautionary Note Regarding Forward-Looking Statements" and Item 1A. Risk Factors of this Annual Report on Form 10-K for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a commercial-stage pharmaceutical company dedicated to the development of innovative therapeutics for the treatment of seizure disorders, including rare genetic epilepsies and status epilepticus. On March 18, 2022, the U.S. Food and Drug Administration (FDA) approved our new drug application (NDA) for the use of ZTALMY (ganaxolone) oral suspension for the treatment of seizures associated with Cyclin-dependent Kinase-like 5 Deficiency Disorder (CDD) in patients 2 years of age and older. In June 2022, the U.S. Drug Enforcement Administration (DEA) published an interim final rule in the Federal Register placing ganaxolone and its salts in schedule V (CV) of the Controlled Substances Act (CSA), which rule became final on December 9, 2022. ZTALMY, our first FDA approved product, became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022. We plan to develop ganaxolone for the treatment of other rare genetic epilepsies, including Tuberous Sclerosis Complex (TSC), and for the treatment of status epilepticus (SE). We are developing ganaxolone in formulations for two different routes of administration: intravenous (IV) and oral. The different formulations are intended to maximize potential therapeutic applications of ganaxolone for adult and pediatric patient populations, in both acute and chronic care, and for both in-patient and self-administered settings. While the precise mechanism by which ganaxolone exerts its therapeutic effects in the treatment of seizures associated with CDD is unknown, its anticonvulsant effects are thought to result from positive allosteric modulation of the gamma-aminobutyric acid type A ($GABA_A$) receptor in the central nervous system (CNS). Ganaxolone is a synthetic analog of allopregnanolone, an endogenous neurosteroid. Ganaxolone acts at both synaptic and extrasynaptic $GABA_A$ receptors, a target known for its anti-seizure, antidepressant and anxiolytic potential.

Our operations to date have consisted primarily of organizing and staffing our company, developing ganaxolone, including conducting preclinical studies and clinical trials, and raising capital. We have funded our operations primarily through sales of equity and debt securities. We recorded $2.9 million of net ZTALMY sales in the year ended December 31, 2022. At December 31, 2022, we had cash and cash equivalents of $240.6 million. Since inception, other than for the three months ended September 30, 2022 due to a one-time net gain from the sale of our Priority Review Voucher (PRV), we have incurred net losses and negative cash flows from our operations. We have generated limited product revenues, and there is no assurance that profitable operations will be achieved in the future, and if achieved, could be sustained on a continuing basis. We incurred a net loss of $19.8 million for the year ended December 31, 2022. Our accumulated deficit as of December 31, 2022 was $430.5 million, and we expect to continue to incur substantial losses in future periods. We anticipate that our operating expenses will increase substantially as we carry out all of our planned commercialization and continued research and development activities with respect to ganaxolone.

We anticipate that our expenses will increase substantially as we:

- conduct multiple later stage clinical trials in targeted indications;
- continue the research, development and scale-up manufacturing capabilities to optimize ganaxolone and dose forms for which we may obtain regulatory approval;
- establish and implement sales, marketing and distribution capabilities to commercialize ganaxolone;

- conduct other preclinical studies and clinical trials to support the filing of NDAs with the FDA, marketing authorization applications (MAAs) with the European Medicines Agency (EMA) and other marketing authorization filings with regulatory agencies in other countries;
- acquire the rights to other product candidates and fund their development;
- maintain, expand and protect our global intellectual property portfolio;
- hire additional clinical, manufacturing, scientific and commercial personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our drug development efforts.

We had cash and cash equivalents of $240.6 million at December 31, 2022. We believe that our existing cash and cash equivalents as of December 31, 2022, will be sufficient to fund our operating expenses and capital expenditure requirements, as well as maintain the minimum cash balance required under our debt facility, into the second half of 2024. However, we will need to secure additional funding in the future, from one or more equity or debt financings, government funding, collaborations, licensing transactions, other commercial transactions or other sources in order to carry out all of our commercialization and planned research and development activities with respect to ganaxolone.

COVID-19

The continued global spread of COVID-19 has impacted our clinical operations and timelines. For example, our Phase 3 Randomized Therapy In Status Epilepticus Trial (RAISE trial) in refractory status epilepticus (RSE) is conducted in hospitals, primarily intensive care units in academic medical centers, which have experienced high rates of COVID-19 admissions. Several of these sites participating in the RAISE trial have experienced COVID-related difficulties, including staff turnover and the need to devote significant resources to patients with COVID-19, which has resulted in site initiation and enrollment delays for the RAISE trial. Given these COVID-19-related challenges and interruption in drug supply in mid-2022, we previously adjusted the expectation for our top-line data readout for the RAISE trial to the second half of 2023. In May 2022, we resumed screening and recruitment for the RAISE trial. Several of the sites participating in the RAISE trial continue to encounter COVID-related setbacks, including staff turnover and the need to devote significant resources for patients with COVID-19. In addition, our ganaxolone clinical trials in the outpatient setting may be negatively impacted if patients and their caregivers do not want to participate while the COVID-19 pandemic persists. The duration and severity of the pandemic and its long-term impact on our business are uncertain at this time.

Financial Overview

Product Revenue, net

Our first FDA approved product, ZTALMY, became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022. We have one customer, Orsini Pharmaceutical Services, LLC (Orsini), a specialty pharmacy that dispenses ZTALMY directly to patients. Our contract with Orsini has a single performance obligation to deliver ZTALMY upon receipt of a purchase order, which is satisfied when Orsini receives ZTALMY. We recognize ZTALMY revenue at the point in time when control of ZTALMY is transferred to Orsini, which is upon delivery to Orsini. The transaction price that we recognize for ZTALMY revenue includes an estimate of variable consideration. Shipping and handling costs to Orsini are recorded as selling, general and administrative expenses. The components of variable consideration include:

Trade Discounts and Allowances. We provide an incentive prompt payment discount to Orsini as explicitly stated in the contract with Orsini. This discount is recorded as a reduction of ZTALMY revenue and accounts receivable in the period in which the related ZTALMY revenue is recognized. We estimate the amount of variable consideration for discounts and allowances using the expected value method.

Product Returns and Recall. We provide for ZTALMY returns in accordance with our Return Good Policy. We estimate the amount of ZTALMY that may be returned using the expected value method, and we present this amount as a reduction of ZTALMY revenue in the period the related ZTALMY revenue is recognized. In the event of a recall, we will promptly notify Orsini and will reimburse Orsini for direct administrative expenses incurred in connection with the recall as well as the cost of replacement product.

Government Rebates. We are subject to discount obligations under state Medicaid programs and Medicare. We estimate reserves related to these discount programs and record these obligations in the same period the related revenue is recognized, resulting in a reduction of product revenue.

Patient Assistance. We offer a voluntary co-pay patient assistance program intended to provide financial assistance to eligible patients with a prescription drug co-payment required by payors and coupon programs for cash payors. The calculation of the current liability for this assistance is based on an estimate of claims and the cost per claim that we expect to receive associated with ZTALMY that has been recognized as revenue but remains in the distribution channel inventories at the end of each reporting period.

Federal Contract Revenue

In September 2020, we entered into a contract (BARDA Contract) with the Biomedical Advanced Research and Development Authority (BARDA), a division of the U.S. Department of Health and Human Services' Office of the Assistant Secretary for Preparedness and Response. Under the BARDA Contract, we received an award of up to an estimated $51 million for development of IV-administered ganaxolone for the treatment of RSE. The BARDA Contract provides for funding to support, on a cost-sharing basis, the completion of a Phase 3 clinical trial of IV-administered ganaxolone in patients with RSE, which covers the RAISE trial, funding of pre-clinical studies to evaluate IV-administered ganaxolone as an effective treatment for RSE due to chemical nerve gas agent exposure, and funding of certain ganaxolone manufacturing scale-up and regulatory activities. In March 2022, we entered into an amendment with BARDA to extend the end date of our base performance period for funding under the BARDA Contract from September 1, 2022 to December 31, 2023. In September 2022, we entered into an amendment with BARDA that, among other things, (i) provides for the exercise of BARDA's option under the BARDA Contract to support U.S. onshoring of the manufacturing capabilities for ganaxolone API (Option 2), (ii) changes the end date of our performance period under Option 2 from December 31, 2026 to July 31, 2025, (iii) increases the government cost share amount under Option 2 from approximately $11.5 million to approximately $12.3 million, and (iv) increases our cost share amount under Option 2 from approximately $4.9 million to approximately $5.3 million.

The BARDA Contract consists of an approximately two-year base period, which was extended through December 31, 2023, during which BARDA will provide up to approximately $21 million of funding for the RAISE trial on a cost share basis and funding of additional preclinical studies of ganaxolone in nerve agent exposure models. Following successful completion of the RAISE trial and preclinical studies in the contract period the BARDA Contract provides for approximately $31 million of additional BARDA funding for three options in support of ganaxolone manufacturing, supply chain, clinical, regulatory and toxicology activities, including the $12.3 million exercise of Option 2 as described above. Under the BARDA Contract, we will be responsible for cost sharing in the amount of approximately $33 million and BARDA will be responsible for approximately $52 million, if all development options are completed. The contract period-of-performance (base period plus option exercises) is up to approximately five years.

We recognize federal contract revenue from the BARDA Contract in the period in which the allowable research and development expenses are incurred. We expect federal contract revenue to increase as the costs associated with our RAISE trial increase.

Collaboration Revenue

In July 2021, we entered into a collaboration agreement (Orion Collaboration Agreement) with Orion Corporation (Orion). Under the terms of the Orion Collaboration Agreement, we granted Orion an exclusive, royalty-bearing, sublicensable license to certain our intellectual property rights with respect to commercializing

biopharmaceutical products incorporating ganaxolone (Licensed Products) in the European Economic Area, the United Kingdom and Switzerland (collectively, the Territory) for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions (Field), initially in the indications of CDD, tuberous sclerosis complex (TSC) and RSE.

Under the terms of the Orion Collaboration Agreement, we received a €25.0 million ($29.6 million) upfront payment from Orion in July 2021. We are eligible to receive up to an additional €97 million in R&D reimbursement and cash milestone payments based on specific clinical and commercial achievements. Also, as part of the overall arrangement, we have agreed to supply the Licensed Products to Orion at an agreed upon price.

We identified the following commitments under the arrangement: (i) exclusive rights to develop, use, sell, have sold, offer for sale and import any product comprised of Licensed Product (License); (ii) development and regulatory activities (Development and Regulatory Activities); and (iii) requirement to supply Orion with the Licensed Product at an agreed upon price (Supply of Licensed Product). We determined that these three commitments represent distinct performance obligations for purposes of recognizing revenue and will recognize license and collaboration revenue or a reduction of expense as we fulfill each performance obligation.

On November 16, 2022, we entered into a Collaboration and Supply Agreement (Tenacia Collaboration Agreement) with Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia). Under the terms of the Tenacia Collaboration Agreement, we granted Tenacia an exclusive, royalty-bearing, sublicensable license to certain of our intellectual property rights to develop, commercialize and otherwise exploit certain products incorporating certain oral and intravenous formulations of the our product candidate ganaxolone (Licensed Products) in Mainland China, Hong Kong, Macau and Taiwan (collectively, Territory) for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions (Field), initially for the treatment of CDD, TSC and SE (including refractory and established SE) (collectively, Initial Indications). The collaboration can be expanded to include additional indications and formulations of ganaxolone pursuant to a right of first negotiation.

Under the terms of the Tenacia Collaboration Agreement, Tenacia agreed to pay us an upfront cash payment of $10 million (Upfront Fee) within forty-five (45) days after the Effective Date, which was received in December 2022. In addition to the Upfront Fee, Tenacia has agreed to make cash payments to us upon the achievement of certain development, regulatory and sales-based milestones related to (i) the Initial Indications and (ii) the first new formulation or pro-drug of ganaxolone or any back-up compound of ganaxolone in a new indication (Selected Product) for which the parties amend the Tenacia Collaboration Agreement in connection with Tenacia's exercise of its right of first negotiation and for which there is no other Licensed Product approved in China (for clarity, the milestone payments under this clause (ii) will only apply to one Selected Product), up to an aggregate amount of $256 million. Of the milestones, $15 million relates to regulatory approvals with separate milestones related to each of oral and intravenous formulations and the Selected Product, and an aggregate of $241 million of sales-based milestones are connected to annual revenue thresholds specific to each of the oral, intravenous and Selected Product formulations of ganaxolone. Tenacia has further agreed to pay us tiered royalty payments based on annual net sales of Licensed Products ranging from the low double digits to the mid-teens for each of the oral formulation, intravenous formulation and Selected Product formulation of Licensed Products. Tenacia's obligations to pay royalties to us with respect to sales of a Licensed Product in each particular jurisdiction of the Territory will commence on the date of first commercial sale in such jurisdiction and expire upon the latest of (i) ten years following the first commercial sale of such Licensed Product in such jurisdiction, (ii) the expiration of the last-to-expire valid claim of any licensed patent rights that covers such Licensed Product in such jurisdiction and (iii) the expiration of all regulatory exclusivities for such Licensed Product in such jurisdiction. Royalty payments are subject to reduction in specified circumstances as set forth in the Tenacia Collaboration Agreement, including if net sales decrease by a certain percentage after the introduction of a generic product.

Tenacia will be primarily responsible for the development of Licensed Products in the Territory and regulatory interactions related thereto, including conducting and sponsoring clinical studies in the Field in the Territory to support regulatory filings in the Territory. All regulatory approvals filed by Tenacia in the Territory will be in the name of and owned by us unless otherwise required by applicable law, in which case such regulatory approvals would be in the name of and owned by Tenacia for the benefit of us. We and Tenacia have agreed to enter into clinical and commercial supply agreements pursuant to which we will supply Tenacia with its requirements of Licensed Products necessary for Tenacia to develop and commercialize Licensed Products in the Field in the Territory. The parties agreed to enter into the clinical

supply agreement no later than ninety (90) days after the Effective Date and the commercial supply agreement no later than twelve (12) months prior to Tenacia's good faith estimate of the date of first commercial sale of the Licensed Product. The parties agreed that the commercial supply agreement would include a commercial supply price adjustment mechanism pursuant to the terms set forth in the Collaboration Agreement. The supply agreements are also expected to contain delivery, acceptance, payment, termination, forecasting, and other terms consistent with the Tenacia Collaboration Agreement, as well as certain quality assurance, indemnification, liability and other standard industry terms. Tenacia will be responsible for, at Tenacia's sole cost and expense, obtaining regulatory approval and commercializing the Licensed Product in the Field in Mainland China.

The term of the Tenacia Collaboration Agreement extends for so long as royalties are payable anywhere in the Territory. Subject to the terms of the Tenacia Collaboration Agreement, (i) for a specified period of time after the Effective Date, Tenacia may terminate the Tenacia Collaboration Agreement in its entirety for any or no reason upon written notice to us, and (ii) either party may terminate the Tenacia Collaboration Agreement for the other party's material breach following a cure period or insolvency.

In accordance with the guidance, we identified the following commitments under the arrangement: (i) grant to Tenacia the exclusive rights to develop, commercialize and otherwise exploit Licensed Product in the Field in the Territory (License) and (ii) requirement to supply Tenacia with the Licensed Product at an agreed upon price (Supply of Licensed Product). We determined that these two commitments represent distinct performance obligations for purposes of recognizing revenue or reducing expense, which it will recognize such revenue or expense, as applicable, as it fulfills these performance obligations.

Research and Development Expenses

Our research and development expenses consist primarily of costs incurred for the development of ganaxolone, which include:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;
- expenses incurred under agreements with clinical research organizations (CROs) and investigative sites that conduct our clinical trials and preclinical studies;
- the cost of acquiring, developing and manufacturing clinical trial materials;
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies;
- costs associated with preclinical activities and regulatory operations; and
- costs associated with developing new formulations and prodrugs of ganaxolone.

We expense research and development costs when we incur them. We record costs for some development activities, such as clinical trials, based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations and information our vendors provide to us.

We have and will incur substantial costs beyond our present and planned clinical trials in order to file an NDA and Supplemental NDAs, or MAAs outside the U.S., for ganaxolone for various clinical indications, and in each case, the nature, design, size and cost of further clinical trials and other studies will depend in large part on the outcome of preceding studies and trials and discussions with regulators. It is difficult to determine with certainty the costs and duration of our current or future clinical trials and preclinical studies, or if, when or to what extent we will generate revenue from the commercialization and sale of ganaxolone if we obtain regulatory approval. We may never succeed in achieving regulatory approval for ganaxolone. The duration, costs and timing of clinical trials and development of

ganaxolone will depend on a variety of factors, including the uncertainties of future clinical trials and preclinical studies, uncertainties in clinical trial enrollment rate and significant and changing government regulation.

In addition, the probability of success for our clinical programs will depend on numerous factors, including competition, manufacturing capability and commercial viability. See "Risk Factors." Our commercial success depends upon attaining significant market acceptance, if approved, among physicians, patients, healthcare payers and the medical community. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success, as well as an assessment of commercial potential.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist principally of salaries and related costs for executive, commercial and other administrative personnel and consultants, including stock-based compensation and travel expenses. Other selling, general and administrative expenses include professional fees for commercial, legal, patent review, consulting and accounting services. Selling, general and administrative expenses are expensed when incurred.

Cost of Product Revenue

Cost of product revenue includes the cost of inventory sold, which includes direct manufacturing and supply chain costs. Also included in cost of product revenue are royalty payments owed to Purdue Neuroscience Company (Purdue) and Ovid in accordance with the respective license agreements. We began capitalizing inventory related to ZTALMY subsequent to the March 2022 FDA approval of ZTALMY, as the related costs were expected to be recoverable through the commercialization and subsequent sale of ZTALMY. Prior to FDA approval of ZTALMY, costs estimated at approximately $2 million for commercially saleable product and materials were incurred and included in research and development expenses. As a result, cost of product revenues related to ZTALMY will initially reflect a lower average per unit cost of materials into approximately the first half of 2024, as previously expensed inventory is utilized for commercial production and sold to customers.

Cost of Collaboration Revenue

Cost of collaboration revenue represents the amortization of one-time fees paid to Purdue pursuant to our license agreement with Purdue and in connection with our collaboration agreements with Orion and Tenacia.

Cost of IP License Fee

In March 2022, we entered into an exclusive patent license agreement (License Agreement) with Ovid. Under the License Agreement, we have an exclusive, non-transferable (except as provided in the License Agreement), royalty-bearing, sublicensable license under certain of Ovid's patent(s) and patent applications to develop, make, have made, commercialize, promote, distribute, sell, offer for sale and import, ganaxolone, including any analogues or derivatives, including its salts, and pharmaceutical formulations of the foregoing (Licensed Products), in the U.S., the member states of the EU, Iceland, Lichtenstein, Norway, the United Kingdom, and Switzerland (Territory) for the treatment of CDD in humans (Field). Under the License Agreement, we have the sole right and responsibility for, and control over, all development, manufacturing, and commercialization activities, including all regulatory activities, with respect to the Licensed Products in the Field in the Territory. In addition, all regulatory approvals and related filings with respect to the Licensed Products in the Field in the Territory will be in the name of, and be owned solely by, us. We were required, at Ovid's option exercisable in accordance with the License Agreement, to (i) pay to Ovid the sum of $1.5 million in cash; or (ii) issue to Ovid 123,255 shares of our common stock, which option to obtain shares of our common stock was exercisable within the five-business day period following the filing of our Annual Report on Form 10-K for the year ended December 31, 2021 on March 24, 2022. On March 29, 2022, we issued 123,255 shares of our common stock to Ovid, per Ovid's option in accordance with the License Agreement. As such, we recorded $1.2 million of IP license fee expenses related to the Ovid License Agreement in the year ended December 31, 2022.

The License Agreement also provides for payment of royalties by us to Ovid in the low single digits on net sales by us, our affiliates and sublicensees, of Licensed Products in the Field in the Territory. Such royalties are subject

to reduction in the event of generic competition in accordance with the License Agreement. We may terminate the License Agreement at any time without cause on thirty days' prior written notice. Either party may terminate the License Agreement for the other party's material breach or insolvency subject to certain cure periods. Also, Ovid has the right to terminate the License Agreement if there has not been a first commercial sale of any Licensed Products in the Field in the Territory on or before June 30, 2025. In the event of termination, all licenses granted under the License Agreement will terminate.

Interest Income

Interest income consists principally of interest income earned on cash and cash equivalents.

Interest Expense

Interest expense consists of interest expense and amortization of debt discount related to our Notes Payable and our Revenue Interest Financing Payable.

Gain from Sale of Priority Review Voucher, net

In the year ended December 31, 2022, we recognized a one-time gain of $107.4 million, net of transaction costs, from the sale of the Rare Pediatric Disease Priority Review Voucher (PRV) to Novo Nordisk, Inc. Refer to Notes 1 and 2 in the accompanying notes to consolidated financial statements for further details.

Other (Expense) Income, net

Other income and expense consists principally of gains or losses on disposal of fixed assets held for sale, foreign currency translation, and fair value adjustments.

Provision for Income Taxes

Due to the one-time receipt of gross proceeds from the sale of the PRV of $110.0 million in the third quarter of 2022 and the impact of the Internal Revenue Code Section 174, effective December 31, 2022, we generated taxable net income for the year ended December 31, 2022. As a result, we have recorded current income tax expense of $2.4 million for the year ended December 31, 2022 attributable to state income taxes. Also included in our current income tax expense for the year ended December 31, 2022 is $0.9 million of China withholding tax incurred in connection with the Tenacia collaboration agreement.

Results of Operations

Product Revenue, net

We recognized $2.9 million of net product revenue related to ZTALMY sales for the year ended December 31, 2022. As ZTALMY, our first FDA approved product, became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022, we did not recognize any product revenue in the year ended December 31, 2021.

Federal contract revenue

We recognized $6.9 million and $6.4 million in federal contract revenue in the years ended December 31, 2022 and 2021 respectively, as a result of the BARDA Contract.

Collaboration Revenue

Collaboration revenue was $15.7 million in the year ended December 31, 2022 as a result of $12.7 million of revenue recognition related to the previously refundable upfront payment pursuant to the Orion Collaboration

Agreement and $3.0 of revenue recognition related to the upfront payment pursuant to the Tenacia Collaboration Agreement. In connection with the upfront fee related to the Orion Collaboration Agreement, we agreed to provide Orion with the results of an ongoing genotoxicity study. In May 2022, the final study report showed that no genotoxicity was found, as measured by formation of micronuclei in the bone marrow or comet morphology in the liver. As a result of the study's findings, we are not required to refund Orion any of the upfront fee and Orion does not have the right to terminate the Orion Collaboration Agreement based on the study outcome. During the year ended December 31, 2022, we allocated the previously refundable portion of the upfront payment to the transaction price and recognized the related revenue. We recognized $9.0 million of collaboration revenue for the year ended December 31, 2021 upon entering into the Orion Collaboration Agreement.

Research and Development Expenses

We record direct research and development expenses, consisting principally of external costs, such as fees paid to investigators, consultants, central laboratories and CROs in connection with our clinical trials, and costs related to manufacturing, to specific product development programs. We do not allocate costs related to purchasing clinical trial materials, employee and contractor-related costs, costs associated with our facility expenses, including depreciation or other indirect costs, to specific product programs because these costs are deployed across multiple product programs under research and development and, as such, are not separately classified. The table below shows our research and development expenses incurred with respect to each active program, in thousands. The primary drivers of our research and development expenditures are currently in our product development programs in CDD, RSE, TSC and ESE. We did not allocate research and development expenses to any other specific product development programs during the periods presented (in thousands):

	Year Ended December 31,	
	2022	2021
CDKL5 deficiency disorder (1)	$ 4,034	$ 8,116
PCDH19-related epilepsy (2)	1,193	2,638
Tuberous Sclerosis (3)	10,013	3,816
Drug Development - Suspension (4)	4,568	5,854
Oral Indications Subtotal	19,808	20,424
Status epilepticus (5)	9,319	5,098
Drug Development - IV (6)	6,040	6,611
IV Indications Subtotal	15,359	11,709
Other research and development (7)	9,383	11,000
Indirect research and development (8)	35,362	30,387
Total	$ 79,912	$ 73,520

(1) The decrease in the year ended December 31, 2022 compared to 2021 was due primarily to more significant regulatory and statistical analysis expenses associated with our NDA filing preparation in 2021 than in 2022 and reduced clinical trial activity in 2022.

(2) The decrease in the year ended December 31, 2022 compared to 2021 was due to reduced clinical activity, specifically completion of the open label extension portion of the PCDH-19 trial.

(3) The increase in the year ended December 31, 2022 compared to 2021 was due primarily to increased activity in 2022 from the initiation and ongoing costs associated with the Phase 3 TSC, as compared to more limited Phase 2 activities in 2021.

(4) The decrease in the year ended December 31, 2022 compared to 2021 was due primarily to higher manufacturing costs related to pre-validation and registration batches in 2021 compared to 2022.

(5) The increase in the year ended December 31, 2022 compared to 2021 was due to increased costs related to the RESET trial, with no comparable costs in 2021, as well as increased costs related to the RAISE trial.

(6) The decrease in the year ended December 31, 2022 compared to 2021 was due to lower manufacturing costs in 2022, which included set-up fees for a new third party manufacturer.

(7) Other research and development expenses include external expenses associated with preclinical and clinical development of ganaxolone, including safety studies, stability studies, preclinical studies, including animal toxicology and pharmacology studies, and other professional fees. The decrease in the year ended December 31, 2022 compared to 2021 was due primarily to lower volume of activities associated with the Phase 1 clinical trials and toxicology and other safety study activities.

(8) The increase in the year ended December 31, 2022 compared to 2021 is related to increased personnel costs in support of our increased activity in preclinical, clinical, and manufacturing activities.

Selling, General and Administrative Expenses

Selling, General and administrative expenses increased to $56.8 million for the year ended December 31, 2022 compared to $37.3 million for the year ended December 31, 2021. The primary drivers of the increase were $8.9 million in increased personnel costs, $6.5 million in increased commercial activities, $2.4 million in consulting costs, $1.1 million in increased software related expenses, $0.8 million in increased travel and meeting costs and $0.9 million in other administrative costs, including legal fees and other professional fees. These increases were partially offset by a decrease of $1.1 million contract acquisition costs related to the Orion Collaboration Agreement in the year ended December 31, 2021.

Interest Income

Interest income was $2.4 million and $0.1 million for the years ended December 31, 2022 and 2021, respectively, and consisted of interest earned on cash and cash equivalents in each year. The 2022 increase over 2021 was due to increased cash and cash equivalents and higher interest rates in the year ended December 31, 2022.

Interest Expense

Interest expense was $10.7 million and $2.6 million for the years ended December 31, 2022 and 2021, respectively. Interest expense for the year ended December 31, 2022 included $7.9 million of interest paid, $1.7 million of debt amortization, $0.1 million related to commitment fees paid in connection with our Notes Payable (Note 9 in accompanying notes to the consolidated financial statements) as well as $1.0 million of non-cash interest expense related to our revenue interest financing payable (Note 10 in accompanying notes to the consolidated financial statements). Interest expense for the year ended December 31, 2021 included $2.0 million of interest paid and $0.6 million of non-cash interest expense related to the amortization of debt issuance costs related to our Notes Payable.

Liquidity and Capital Resources

Since inception, other than for the three months ended September 30, 2022 due to a one-time net gain from the sale of our PRV, we have incurred net losses and negative cash flows from our operations. We incurred a net loss of $19.8 million for the year ended December 31, 2022. Our cash used in operating activities was $112.9 million for year ended December 31, 2022 compared to $55.5 million for the year ended December 31, 2021. Historically, we have financed our operations principally through the sale of common stock, notes payable, preferred stock and convertible debt.

In July 2022, we entered into the PRV Asset Purchase Agreement to sell our PRV, pursuant to which Novo Nordisk, Inc. paid us $110.0 million upon the closing of the transaction. In August 2022, we received a letter from Purdue in which Purdue claimed that it was owed $5.5 million by us from the sale of the PRV pursuant to the Purdue

License Agreement. Our position communicated to Purdue is that we do not owe Purdue any of the proceeds from the sale of the PRV. No associated payment by us has been made, and Purdue has not filed a specific claim to date.

In November 2022, in connection with an underwritten public offering of 10,526,316 shares of our common stock, pre-funded warrants to purchase 2,105,264 shares of common stock and the exercise of an option of 1,894,737 shares of common stock, we received approximately $64.5 million in total net proceeds after taking into account the exercise of the underwriters' option, in each case deducting the underwriting discounts and commissions and after deducting offering expenses paid or payable by us. Additionally, in November 2022, we received an upfront payment of $32.5 million pursuant to the revenue interest financing agreement with Sagard, and in December 2022, we received an upfront payment of $10.0 million in connection with the Tenacia Collaboration Agreement. At December 31, 2022, we had cash and cash equivalents of $240.6 million.

We believe that our existing cash and cash equivalents as of December 31, 2022, will be sufficient to fund our operating expenses and capital expenditure requirements, as well as maintain the minimum cash balance required under our debt facility, into the second half of 2024. However, we will need to secure additional funding in the future, from one or more equity or debt financings, government funding, collaborations, licensing transactions, other commercial transactions or other sources in order to carry out all of our commercialization and planned research and development activities with respect to ganaxolone.

Orion Commercialization Agreement

On July 30, 2021, we entered into the Orion Collaboration Agreement, whereby Orion received exclusive rights to commercialize the oral and IV dose formulations of ganaxolone in the European Economic Area, United Kingdom and Switzerland in multiple seizure disorders, including CDD, TSC and RSE. Under the agreement, we received a €25 million ($29.6 million) upfront fee. We are eligible to receive up to an additional €97 million in research and development reimbursement and cash milestone payments based on specific clinical and commercial achievements, as well as tiered royalty payments based on net sales ranging from the low double-digits to high teens for the oral programs and the low double-digits to low 20s for the IV program. Also, as part of the overall arrangement, we have agreed to supply the Licensed Products to Orion at an agreed upon price.

Tenacia Commercialization Agreement

On November 16, 2022, we entered into a collaboration and supply agreement with Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia), pursuant to which we granted Tenacia an exclusive, royalty-bearing, sublicensable license to certain of our intellectual property rights to develop, commercialize and otherwise exploit certain products incorporating certain oral and intravenous formulations of our product candidate ganaxolone (Licensed Products) in Mainland China, Hong Kong, Macau and Taiwan (collectively, Territory) for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions (Field), initially for the treatment of cyclin-dependent kinase-like 5 deficiency disorder, tuberous sclerosis complex and SE (including refractory and established SE) (collectively, Initial Indications). The collaboration can be expanded to include additional indications and formulations of ganaxolone pursuant to a right of first negotiation.

In connection with the execution of the Collaboration Agreement, Tenacia agreed to pay us an upfront cash payment of $10 million (Upfront Fee) within forty-five (45) days after the Effective Date and payment was received in December 2022. In addition to the Upfront Fee, Tenacia has agreed to make cash payments to us upon the achievement of certain development, regulatory and sales-based milestones related to (i) the Initial Indications and (ii) the first new formulation or pro-drug of ganaxolone or any back-up compound of ganaxolone in a new indication (Selected Product) for which the parties amend the Collaboration Agreement in connection with Tenacia's exercise of its right of first negotiation and for which there is no other Licensed Product approved in China (for clarity, the milestone payments under this clause (ii) will only apply to one Selected Product), up to an aggregate amount of $256 million. Of the milestones, $15 million relates to regulatory approvals with separate milestones related to each of oral and intravenous formulations and the Selected Product, and an aggregate of $241 million of sales-based milestones are connected to annual revenue thresholds specific to each of the oral, intravenous and Selected Product formulations of ganaxolone.

Tenacia has further agreed to pay us tiered royalty payments based on annual net sales of Licensed Products ranging from the low double digits to the mid-teens for each of the oral formulation, intravenous formulation and Selected Product formulation of Licensed Products. Tenacia's obligations to pay royalties to us with respect to sales of a Licensed Product in each particular jurisdiction of the Territory will commence on the date of first commercial sale in such jurisdiction and expire upon the latest of (i) ten years following the first commercial sale of such Licensed Product in such jurisdiction, (ii) the expiration of the last-to-expire valid claim of any licensed patent rights that covers such Licensed Product in such jurisdiction and (iii) the expiration of all regulatory exclusivities for such Licensed Product in such jurisdiction. Royalty payments are subject to reduction in specified circumstances as set forth in the Collaboration Agreement, including if net sales decrease by a certain percentage after the introduction of a generic product.

Oaktree Credit Agreement

On May 11, 2021 (Closing Date) and as amended on May 17, 2021, May 23, 2022 and October 28, 2022 (Credit Agreement), we entered into the Credit Agreement with Oaktree Fund Administration, LLC as administrative agent (Oaktree) and the lenders party thereto (collectively, Lenders) that provides for a five-year senior secured term loan facility in an aggregate original principal amount of up to $100.0 million, available to us in four tranches (collectively, Term Loans). As of December 31, 2022, we had drawn on three tranches, and the following tranche remained available to us:

- Through December 31, 2023, $25.0 million of Tranche C Term Loans will be available for draw if we complete one or more financings (including through the issuance of common stock, convertible debt, subordinated debt, a synthetic royalty or a sublicense) resulting in gross proceeds to us of at least $40.0 million and net proceeds to us of at least $36.0 million (Qualified Financing Condition). However, the availability of this tranche is also subject to either our current Phase 3 trial in RSE or a Phase 3 trial in TSC achieving statistical significance (p value < 0.05) across all primary endpoints and ganaxolone must be generally well tolerated, with a safety profile generally consistent with previous clinical trials.

We received $15.0 million of Tranche A-1 Term Loans on the Closing Date, $30.0 million of Tranche A-2 Term Loans in September 2021 after formal acceptance by the FDA of an NDA filing relating to the use of ganaxolone in the treatment of CDD, and $30.0 million of Tranche B Term Loans in March 2022 after FDA approval of ZTALMY for CDD. We satisfied the Qualified Financing Condition in connection with our November 2022 underwritten public offering, however, if we are unable to satisfy the remaining condition, we would not be able to draw down the remaining tranche of loans and may not be able to obtain alternative financing on commercially reasonable terms or at all.

The Term Loans mature on May 11, 2026 (Maturity Date). The Term Loans bear interest at a fixed per annum rate (subject to increase during an event of default) of 11.50%, and we are required to make quarterly interest payments until the Maturity Date. We are also required to make quarterly principal payments beginning on June 30, 2024 in an amount equal to 5.0% of the aggregate amount of the Term Loans outstanding on June 30, 2024, and continuing until the Maturity Date. On the Maturity Date, we are required to pay in full all outstanding Term Loans and other amounts owed under the Credit Agreement.

At the time of borrowing any tranche of the Term Loans, we are required to pay an upfront fee of 2.0% of the aggregate principal amount borrowed at that time.

In connection with the Revenue Interest Financing Agreement, on October 28, 2022, we entered into the Credit Agreement Amendment to, among other things, allow for the consummation of the Revenue Interest Financing Agreement and the transactions thereunder, and paid $0.3 million in administrative fees in connection with the execution of the Credit Agreement Amendment. In addition, the Credit Agreement Amendment increased the exit fee due by us upon any repayment, whether as a prepayment or a scheduled repayment, of the principal of the loans under the Credit Agreement from 2.00% to 2.67%.

Sagard Financing Agreement

In October 2022, we entered into a revenue interest financing agreement (the Revenue Interest Financing Agreement) with Sagard Healthcare Royalty Partners, LP (Sagard) pursuant to which we received $32.5 million.

In exchange for the Investment Amount, we have agreed to make quarterly payments to Sagard (Payments) as follows: (i) for each calendar quarter from and after the closing date of such financing through and including the quarter ended June 30, 2026, an amount equal to 7.5% of (a) our U.S. net sales of ZTALMY and all other pharmaceutical products that contain ganaxolone (Net Sales), in each case with any dosage form, dosing regimen, or strength, or any improvements related thereto (collectively, Included Products) and (b) certain other payments received by us in connection with the manufacture, development and sale of the Included Products in the U.S. (Other Included Payments, and, together with Net Sales, Product Revenue); and (ii) for each calendar quarter following the calendar quarter ended June 30, 2026, an amount equal to (x) 15.0% of the first $100 million in annual Product Revenue of the Included Products and (y) 7.5% of annual Product Revenue of the Included Products in excess of $100 million.

The Payments are subject to a hard cap equal to 190% ($61.8 million) of the Investment Amount (Hard Cap). Sagard's right to receive payments will terminate when Sagard has received payments in respect of the Included Products, including any additional payments described below, equal to the Hard Cap. Further, we have the right to make voluntary prepayments to Sagard, and such payments will be credited against the Hard Cap.

If Sagard has not received aggregate payments equaling at least 100% of the Investment Amount by December 31, 2027 or at least 190% of the Investment Amount by December 31, 2032 (each, Minimum Amount), then we will be obligated to make a cash payment to Sagard in an amount sufficient to gross up Sagard up to the applicable Minimum Amount within a specified period of time after each reference date.

BARDA Contract

In September 2020, we and BARDA entered into the BARDA Contract, under which we received an award of up to an estimated $51 million for development of IV-administered ganaxolone for the treatment of RSE. The BARDA Contract provides for funding to support, on a cost-sharing basis, the completion of a Phase 3 clinical trial of IV-administered ganaxolone in patients with RSE, which covers the RAISE trial, funding of pre-clinical studies to evaluate IV-administered ganaxolone as an effective treatment for RSE due to chemical nerve gas agent exposure, and funding of certain ganaxolone manufacturing scale-up and regulatory activities. In March 2022, we entered into an amendment with BARDA to extend the end date of our base performance period for funding under the BARDA Contract from September 1, 2022 to December 31, 2023. In September 2022, we entered into an amendment with BARDA that, among other things, (i) provides for the exercise of BARDA's option under the BARDA Contract to support U.S. onshoring of the manufacturing capabilities for ganaxolone API (Option 2), (ii) changes the end date of our performance period under Option 2 from December 31, 2026 to July 31, 2025, (iii) increases the government cost share amount under Option 2 from approximately $11.5 million to approximately $12.3 million, and (iv) increases our cost share amount under Option 2 from approximately $4.9 million to approximately $5.3 million.

The BARDA Contract consists of an approximately two-year base period, which was extended through December 31, 2023, during which BARDA will provide up to approximately $21 million of funding for the RAISE trial on a cost share basis and funding of additional preclinical studies of ganaxolone in nerve agent exposure models. Following successful completion of the RAISE trial and preclinical studies in the contract period, the BARDA Contract provides for approximately $31 million of additional BARDA funding for three options in support of ganaxolone manufacturing, supply chain, clinical, regulatory and toxicology activities, including the $12.3 million exercise of Option 2 as described above. Under the BARDA Contract, we will be responsible for cost sharing in the amount of approximately $33 million and BARDA will be responsible for approximately $52 million if all development options are completed. The contract period-of-performance (base period plus option exercises) is up to approximately five years.

Equity Financings

In connection with the closing of an equity financing in November 2022 and the December 2022 exercise of the related underwriters' option, we issued a total of 12,421,053 shares of common stock and 2,105,264 pre-funded warrants to purchase common stock in an underwritten public offering resulting in aggregate net proceeds of $64.5 million, after deducting the underwriting discounts and commissions and offering expenses paid or payable by us.

Equity Distribution Agreement

On July 9, 2020, we entered into an Equity Distribution Agreement (EDA) with JMP Securities LLC (JMP) to create an at the market equity program under which we from time to time may offer and sell shares of our common stock having an aggregate offering price of up to $60 million. Subject to the terms and conditions of the EDA, JMP will be entitled to a commission of up to 3.0% of the gross proceeds from each sale of shares of our common stock. We did not sell any shares of our common stock during the years ended December 31, 2022 and 2021 under the EDA.

IP License Agreement

In March 2022, we entered into the License Agreement with Ovid. Under the License Agreement, we have an exclusive, non-transferable (except as provided in the License Agreement), royalty-bearing, sublicensable license under certain of Ovid's patent(s) and patent applications to develop, make, have made, commercialize, promote, distribute, sell, offer for sale and import, ganaxolone, including any analogues or derivatives, including its salts, and pharmaceutical formulations of the foregoing (Licensed Products), in the U.S., the member states of the EU, Iceland, Lichtenstein, Norway, the United Kingdom, and Switzerland (Territory) for the treatment of CDD in humans (Field). Under the License Agreement, we have the sole right and responsibility for, and control over, all development, manufacturing, and commercialization activities, including all regulatory activities, with respect to the Licensed Products in the Field in the Territory. In addition, all regulatory approvals and related filings with respect to the Licensed Products in the Field in the Territory will be in the name of, and be owned solely by, us. We were required, at Ovid's option exercisable in accordance with the License Agreement, to (i) pay to Ovid the sum of $1.5 million in cash; or (ii) issue to Ovid 123,255 shares of our common stock, which option to obtain shares of our common stock was exercisable within the five-business day period following the filing of our Annual Report on Form 10-K for the year ended December 31, 2021 on March 24, 2022. On March 29, 2022, we issued 123,255 shares of our common stock to Ovid, per Ovid's option in accordance with the License Agreement. As such, we recorded $1.2 million of IP license fee expenses related to the Ovid License Agreement in the year ended December 31, 2022.

The License Agreement also provides for payment of royalties by us to Ovid in the low single digits on net sales by us, our affiliates and sublicensees, of Licensed Products in the Field in the Territory. Such royalties are subject to reduction in the event of generic competition in accordance with the License Agreement. We may terminate the License Agreement at any time without cause on thirty days' prior written notice. Either party may terminate the License Agreement for the other party's material breach or insolvency subject to certain cure periods. Also, Ovid has the right to terminate the License Agreement if there has not been a first commercial sale of any Licensed Products in the Field in the Territory on or before June 30, 2025. In the event of termination, all licenses granted under the License Agreement will terminate.

Cash Flows

Operating Activities. Cash used in operating activities increased to $112.9 million for the year ended December 31, 2022 compared to $55.5 million for the same period in 2021. Excluding the noncash impacts primarily related to depreciation and amortization, debt issuance costs, interest accretion, stock-based compensation, cost of license agreement and changes in the net contract assets/liabilities related to the Orion and Tenacia Collaboration Agreements, the change in cash used in operating activities in the year ended December 31, 2022 compared to the year ended December 31, 2021 was primarily the result of decreases in the changes in accounts payable and accrued expenses and an increase in operating expenses.

Investing Activities. Cash provided by investing activities during the year ended December 31, 2022 represents net proceeds of $107.4 million from the sale of the PRV, partially offset by $1.8 million in purchases of property and equipment. Cash used in investing activities during the year ended December 31, 2021 represents $2.9 million related to investments in property and equipment, partially offset by $1.5 million in maturities of short-term investments.

Financing Activities. Cash provided by financing activities during the year ended December 31, 2022 primarily includes $64.5 million in total net proceeds from the November 2022 public offering, $29.9 million in net proceeds from the Sagard revenue interest financing agreement, $28.6 million in proceeds from notes payable net of issuance costs, and $1.8 million in proceeds from the exercise of stock options. Cash provided by financing activities during the year ended December 31, 2021 primarily includes $40.3 million in proceeds from notes payable net of issuance costs and $1.2 million in proceeds from the exercise of stock options.

Funding Requirements

Since inception, other than for the three months ended September 30, 2022 due to a one-time net gain from the sale of our PRV, we have incurred net losses and negative cash flows from our operations. We incurred a net loss of $19.8 million for the year ended December 31, 2022. We have generated limited product revenues, and there is no assurance that profitable operations will be achieved in the future, and if achieved, could be sustained on a continuing basis.

We believe that our existing cash and cash equivalents as of December 31, 2022 will be sufficient to fund our operating expenses and capital expenditure requirements, as well as maintain the minimum cash balance required under our debt facility, into the second half of 2024. However, we will need to secure additional funding in the future, from one or more equity or debt financings, government funding, collaborations, licensing transactions, other commercial transactions or other sources in order to carry out all of our commercialization and planned research and development activities with respect to ganaxolone. In order to meet these additional cash requirements, we may seek to sell additional equity or convertible debt securities that may result in dilution to our stockholders, or engage in federal contracts or other partnerships. If we raise additional funds through the issuance of convertible debt securities, these securities could have rights senior to those of our common stock and could contain covenants that restrict our operations. There can be no assurance that we will be able to obtain additional equity or debt financing on terms acceptable to us, if at all. Further, the continued spread of COVID-19 has also led to severe disruption and volatility in the global capital markets, which could increase our cost of capital and adversely affect our ability to access the capital markets in the future. Our failure to obtain sufficient funds on acceptable terms when needed could have a negative impact on our business, results of operations, and financial condition.

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

- the effects of the COVID-19 pandemic on our business, the medical community and the global economy;
- the results of our preclinical studies and clinical trials;
- the development, formulation and commercialization activities related to ganaxolone, including ZTALMY;
- the scope, progress, results and costs of researching and developing ganaxolone, including ZTALMY, or any other future product candidates, and conducting preclinical studies and clinical trials;
- the timing of, and the costs involved in, obtaining regulatory approvals for ganaxolone, including ZTALMY, or any other future product candidates;
- the cost of commercialization activities for ZTALMY in CDD in the U.S., including marketing, sales and distribution costs;

- the cost of commercialization activities for ZTALMY, ganaxolone in any other indications, or any other future product candidates, are approved for sale, including marketing, sales and distribution costs;
- the cost of manufacturing and formulating ganaxolone, or any other future product candidates, to internal and regulatory standards for use in preclinical studies, clinical trials and, if approved, commercial sale;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;
- our ability to receive funding under the BARDA Contract;
- our expectations regarding the amount and timing of milestone and royalty payments pursuant to our exclusive license agreements with Orion, Tenacia and NovaMedica.
- any product liability, infringement or other lawsuits related to ZTALMY or other indications being developed for ganaxolone and, if approved, products;
- capital needed to attract and retain skilled personnel;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation; and
- the timing, receipt and amount of sales of, or royalties on, ZTALMY in CDD and on future approved products, if any.

Please see "Risk Factors" for additional risks associated with our substantial capital requirements.

Critical Accounting Policies and Estimates

We base this management's discussion and analysis of our financial condition and results of operations on our financial statements, which we have prepared in accordance with accounting principles generally accepted in the U.S. (GAAP). The preparation of our financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses and the disclosure of contingent assets and liabilities in our financial statements. We evaluate our estimates and judgments, including those related to accrued clinical trial expenses on an ongoing basis. We base our estimates on historical experience, known trends and events and various other factors that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. You should consider your evaluation of our financial condition and results of operations with these policies, judgments and estimates in mind.

While we describe our significant accounting policies in the notes to our financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe the following accounting policies are the most critical to the judgments and estimates we use in the preparation of our financial statements.

Clinical Trial Expenses

As part of the process of preparing our financial statements, we are required to estimate our clinical trial expenses. Our clinical trial accrual process seeks to account for expenses resulting from our obligations under contracts with vendors, consultants and CROs and clinical site agreements in connection with conducting clinical trials. The financial terms of these contracts are subject to negotiations, which vary from contract to contract and may result in payment flows that do not match the periods over which materials or services are provided to us under such contracts. Our objective is to reflect the appropriate clinical trial expenses in our financial statements by matching the appropriate

expenses with the period in which services and efforts are expended. We account for these expenses according to the progress of the trial as measured by subject progression and the timing of various aspects of the trial.

We determine accrual estimates based on estimates of the services received and efforts expended that take into account discussion with applicable personnel and outside service providers as to the progress or state of completion of trials. During the course of a clinical trial, we adjust our clinical expense recognition if actual results differ from our estimates. We make estimates of our accrued expenses and prepaid assets as of each balance sheet date in our financial statements based on the facts and circumstances known to us at that time. Our clinical trial accrual and prepaid assets are dependent, in part, upon the receipt of timely and accurate reporting from CROs and other third-party vendors. Although we do not expect our estimates to differ materially from amounts we actually incur, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low for any particular period.

Orion Collaboration Agreement

The recognition of revenue related to the Orion Collaboration Agreement requires significant judgement and estimates. As discussed in Note 11 to the Consolidated Financial Statements, we are required to identify distinct performance obligations and subsequently allocate a portion of the transaction price to each performance obligation. We will recognize such revenue or expense, as applicable, as we fulfill these performance obligations. We identified three commitments in the Orion Collaboration Agreement that represent distinct performance obligations for purposes of recognizing revenue or reducing expense: (i) exclusive rights to develop, use, sell, have sold, offer for sale and import any product comprised of Licensed Product (License) (ii) development and regulatory activities (Development and Regulatory Activities), and (iii) requirement to supply Orion with the Licensed Product at an agreed upon price (Supply of Licensed Product). We allocated the transaction price to the three performance obligations based on the estimated stand-alone selling prices at contract inception and we reevaluate the transaction price and the total estimated costs expected to be incurred to satisfy the performance obligations and adjust the deferred revenue at the end of each reporting period. Such changes will result in a change to the amount of collaboration revenue recognized and deferred revenue.

Significant estimates were used in the determination of the stand-along selling prices. The stand-alone selling price of the License was based on a discounted cash flow approach and considered several factors including, but not limited to, discount rate, development timeline, regulatory risks, estimated market demand and future revenue potential using an adjusted market approach. The stand-alone selling price of the Development and Regulatory Activities and the Supply of Licensed Product was estimated using the expected cost-plus margin approach.

Tenacia Collaboration Agreement

The recognition of revenue related to the Tenacia Collaboration Agreement requires significant judgement and estimates. As discussed in Note 11 to the Consolidated Financial Statements, we are required to identify distinct performance obligations and subsequently allocate a portion of the transaction price to each performance obligation. We identified the following two commitments under the arrangement: (i) grant to Tenacia the exclusive rights to develop, commercialize and otherwise exploit Licensed Product in the Field in the Territory (License) and (ii) requirement to supply Tenacia with the Licensed Product at an agreed upon price (Supply of Licensed Product). We will recognize such revenue or expense, as applicable, as we fulfill these performance obligations. We allocated the transaction price to the two performance obligations based on the estimated stand-alone selling prices at contract inception and we reevaluate the transaction price and the total estimated costs expected to be incurred to satisfy the performance obligations and adjust the deferred revenue at the end of each reporting period. Such changes will result in a change to the amount of collaboration revenue recognized and deferred revenue.

Significant estimates were used in the determination of the stand-along selling prices. The stand-alone selling price of the License was based on a discounted cash flow approach and considered several factors including, but not limited to, discount rate, development timeline, regulatory risks, estimated market demand and future revenue potential

using an adjusted market approach. The stand-alone selling price of the Supply of Licensed Product was estimated using the expected cost-plus margin approach.

Revenue Interest Financing Agreement

In October 2022, we recognized a liability related to the Revenue Interest Financing Agreement with Sagard Healthcare Royalty Partners, LP (Sagard) under ASC 470-10 *Debt* and ASC 835-30 *Interest - Imputation of Interest.* The initial funds received by us from Sagard pursuant to the terms of the Revenue Interest Financing Agreement were recorded as a liability and will be accreted under the effective interest method upon the estimated amount of future royalty payments to be made pursuant to the Revenue Interest Financing Agreement. The issuance costs were recorded as a direct deduction to the carrying amount of the liability and will be amortized under the effective interest method over the estimated period the liability will be repaid. We estimated the total amount of future product revenue to be generated over the life of the Revenue Interest Financing Agreement, and a significant increase or decrease in these estimates could materially impact the liability balance and the related interest expense. If the timing or amounts of any estimated future revenue and related payments change, we will prospectively adjust the effective interest and the related amortization of the liability and related issuance costs.

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

We are a smaller reporting company as defined by Rule 12b-2 of the Securities Exchange Act of 1934 and are not required to provide the information under this item.

Item 8. Financial Statements and Supplementary Data.

Our financial statements, accompanying notes and Reports of Independent Registered Public Accounting Firm are included in this Annual Report on Form 10-K beginning on page F-1, which are incorporated in this Item 8 by reference.

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 of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures (as such term is 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 on Form 10-K to ensure that the information required to be disclosed by us in the 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 information required to be disclosed in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, to allow timely decisions regarding required disclosures. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost benefit relationship of possible controls and procedures. Based on such evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of December 31, 2022.

Management's Annual Report on Internal Control Over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act.

Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles in the U.S. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Management utilized the criteria established in the Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) to assess the effectiveness of our internal control over financial reporting as of December 31, 2022. Based on the assessment, management has concluded that, as of December 31, 2022, our internal control over financial reporting was effective.

Changes in Internal Control Over Financial Reporting

There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the quarter ended December 31, 2021 that has materially affected, or is 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 and Executive Officers and Corporate Governance.

We incorporate the information required by this Item 10 by reference to the definitive proxy statement for our 2023 annual meeting of shareholders, to be filed with the SEC.

We have adopted a written Code of Business Conduct and Ethics (Code of Business Conduct) that applies to all of our employees, officers and directors. This Code of Business Conduct is designed to ensure that our business is conducted with integrity and in compliance with SEC regulations and Nasdaq listing standards. The Code of Business Conduct covers adherence to laws and regulations as well as professional conduct, including employment policies, conflicts of interest and the protection of confidential information. The Code of Business Conduct is available under "Governance Documents" within the "Investors & Media – Governance" section of our website at www.marinuspharma.com.

We intend to disclose any future amendments to, or waivers from, the Code of Business Conduct and Ethics that affect our directors or senior financial and executive officers within four business days of the amendment or waiver by posting such information on the website address and location specified above

Item 11. Executive Compensation.

We incorporate the information required by this Item 11 by reference to the definitive proxy statement for our 2023 annual meeting of shareholders, to be filed with the SEC.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

We incorporate the information required by this Item 12 by reference to the definitive proxy statement for our 2023 annual meeting of shareholders, to be filed with the SEC.

Item 13. Certain Relationships and Related Transactions and Director Independence.

We incorporate the information required by this Item 13 by reference to the definitive proxy statement for our 2023 annual meeting of shareholders, to be filed with the SEC.

Item 14. Principal Accountants Fees and Services.

We incorporate the information required by this Item 14 by reference to the definitive proxy statement for our 2023 annual meeting of shareholders, to be filed with the SEC.

PART IV

Item 15. Exhibits and Financial Statement Schedules.

(a) Documents filed as part of this report:

1. Financial Statements. The financial statements as set forth under Item 8 of this Annual Report on Form 10-K are incorporated herein.

2. Financial Statement Schedules. All financial statement schedules have been omitted because they are not applicable, not required, or the information is shown in the financial statements or related notes.

3. Exhibits. See (b) below.

Exhibit No.	Description of Exhibit
3.1	Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on August 7, 2014.)
3.2	Certificate of Amendment of the Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on April 2, 2020.)
3.3	Certificate of Amendment of the Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on May 27, 2020.)
3.4	Certificate of Amendment of the Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on September 22, 2020.)
3.5	Certificate of Amendment of the Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.2 to Form 8-K current report filed on September 22, 2020.)
3.6	Amended and Restated By-laws. (Incorporated by reference to Exhibit 3.2 to Form 8-K current report filed on August 7, 2014.)
3.7	Certificate of Designations, Preferences and Rights of Series A Participating Convertible Preferred Stock. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on December 13, 2019.)
3.8	Delaware Certificate of Change of Registered Agent. (Incorporated by reference to Exhibit 3.8 to Form 10-Q quarterly report filed on May 12, 2022.)
4.1	Specimen Certificate evidencing shares of Marinus Pharmaceutical Inc.'s common stock. (Incorporated by reference to Exhibit 4.1 to Form S-1/A registration statement filed on July 18, 2014.)
4.2	Description of the Registrant's Securities. (Incorporated by reference to Exhibit 4.3 to Form 10-K annual report filed on March 24, 2022.)

Exhibit No.	Description of Exhibit
4.3	Form of Pre-funded Warrant to Purchase Common Stock. (Incorporated by reference to Exhibit 4.1 to Form 8-K current report filed on November 10, 2022.)
10.1+	Marinus Pharmaceuticals, Inc. 2005 Stock Option and Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.1 to Form S-1 registration statement filed on May 12, 2014.)
10.2+	Forms of Stock Option Agreement under the 2005 Stock Option and Incentive Plan. (Incorporated by reference to Exhibit 10.2 to Form S-1 registration statement filed on May 12, 2014.)
10.3+	Employment Agreement, effective as of April 12, 2021, between Marinus Pharmaceuticals, Inc. and Steven Pfanstiel (Incorporated by reference to Exhibit 10.4 to Form 10-K annual report filed on March 9, 2021.)
10.4+	First Amendment to Executive Employment Agreement, by and between Marinus Pharmaceuticals, Inc. and Steven Pfanstiel, MBA, CMA dated April 9, 2021. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on April 13, 2021.)
10.5+	Employment Agreement, effective November 9, 2020, between Marinus Pharmaceuticals, Inc. and Christina Shafer (filed herewith).
10.6	Form of Amended and Restated Indemnification Agreement (VC Directors). (Incorporated by reference to Exhibit 10.10 to Form S-1 registration statement filed on May 12, 2014.)
10.7	Form of Amended and Restated Indemnification Agreement (Non-VC Directors). (Filed herewith.)
10.8*	Amended and Restated Agreement dated as of May 23, 2008 between Marinus Pharmaceuticals, Inc. and Purdue Neuroscience Company. (Incorporated by reference to Exhibit 10.12 to Form S-1 registration statement filed on May 12, 2014.)
10.9+	Marinus Pharmaceuticals, Inc. Change in Control Severance Plan effective November 7, 2016. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on November 8, 2016.)
10.10	First Amendment to Lease agreement dated as of December 28, 2015 between Radnor Properties-SDC, L.P. and Marinus Pharmaceuticals, Inc. amending Lease agreement dated as of October 14, 2014 between Radnor Center Associates and Marinus Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on January 4, 2016.)
10.11	License Agreement by and between Marinus Pharmaceuticals, Inc. and CyDex Pharmaceuticals, Inc., dated March 31, 2017. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on April 6, 2017.)
10.12	Supply Agreement by and between Marinus Pharmaceuticals, Inc. and CyDex Pharmaceuticals, Inc., dated March 31, 2017. (Incorporated by reference to Exhibit 10.2 to Form 8-K current report filed on April 6, 2017.)
10.13	Second Amendment to Lease agreement dated as of December 7, 2018 between Radnor Properties-SDC, L.P., Radnor Center Associates and Marinus Pharmaceuticals, Inc, amending Lease agreement, as amended, dated as of December 28, 2015 between Radnor Properties-SDC, L.P. and Marinus Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on December 7, 2018.)
10.14+	Amended and Restated Employment Agreement dated as of August 6, 2019, between Marinus Pharmaceuticals, Inc. and Scott Braunstein, M.D. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on August 8, 2019).
10.15+	Employment Agreement dated as of October 25, 2019, between Joe Hulihan, M.D. and Marinus Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on October 29, 2019).
10.16+	Employment Agreement dated as of June 15, 2020 between Marinus Pharmaceuticals, Inc. and Martha Manning. (Incorporated by reference to Exhibit 10.22 to Form 10-K annual report filed on March 9, 2021).
10.17	Securities Purchase Agreement, dated December 11, 2019, by and between the Company and the Investors listed therein. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on December 13, 2019).
10.18	Equity Distribution Agreement, dated July 9, 2020, by and between Marinus Pharmaceuticals, Inc. and JMP Securities LLC. (Incorporated by reference to Exhibit 1.1 to Form S-3 registration statement filed on July 9, 2020.)

Exhibit No.	Description of Exhibit
10.19*	Contract, dated September 8, 2020, by and between Marinus Pharmaceuticals, Inc. and the Biomedical Advanced Research and Development Authority, a division of the U.S. Department of Health and Human Services' Office of the Assistant Secretary for Preparedness and Response. (Incorporated by reference to Exhibit 10.3 to Form 10-Q quarterly report filed on November 9, 2020.)
10.20	Credit Agreement and Guaranty, dated May 11, 2021, by and among Marinus Pharmaceuticals, Inc., as Borrower, Oaktree Fund Administration, LLC, as Administrative Agent, and the other lenders party thereto. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on May 17, 2021.)
10.21	Security Agreement, dated May 11, 2021, by and among Marinus Pharmaceuticals, Inc., as Grantor, and Oaktree Fund Administration, LLC, as Administrative Agent. (Incorporated by reference to Exhibit 10.2 to Form 10-Q quarterly report filed on May 17, 2021.)
10.22	Letter Agreement, dated May 17, 2021, by and among Marinus Pharmaceuticals, Inc., as Borrower, Oaktree Fund Administration, LLC, as Administrative Agent, and the other lenders party thereto. (Incorporated by reference to Exhibit 10.3 to Form 10-Q quarterly report filed on May 17, 2021.)
10.23*	Collaboration Agreement, dated as of July 30, 2021, by and between Marinus Pharmaceuticals, Inc. and Orion Corporation. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on November 9, 2021.)
10.24+	Form of Incentive Stock Option Agreement for Employees under the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.2 to Form 10-Q quarterly report filed on November 9, 2021.)
10.25+	Form of Nonqualified Stock Option Agreement for Employees under the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.3 to Form 10-Q quarterly report filed on November 9, 2021.)
10.26+	Form of Nonqualified Stock Option Agreement for Non-Employee Directors under the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.4 to Form 10-Q quarterly report filed on November 9, 2021.)
10.27+	Form of Nonqualified Stock Option Agreement for Employees granted as an Inducement Award. (Incorporated by reference to Exhibit 10.5 to Form 10-Q quarterly report filed on November 9, 2021.)
10.28+	Form of Restricted Stock Unit Agreement for Employees under the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.6 to Form 10-Q quarterly report filed on November 9, 2021.)
10.29+	Form of Restricted Stock Unit Agreement for Non-Employee Directors under the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.7 to Form 10-Q quarterly report filed on November 9, 2021.)
10.30+	Form of Restricted Stock Unit Agreement containing a Sell to Cover Election for Employees under the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.34 to Form 10-K annual report filed on March 24, 2022.)
10.31+	Form of Restricted Stock Unit Agreement for Employees granted as an Inducement Award, under the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.35 to Form 10-K annual report filed on March 24, 2022.)
10.32+	Form of Restricted Stock Unit Agreement containing a Sell to Cover Election for Employees granted as an Inducement Award, under the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.36 to Form 10-K annual report filed on March 24, 2022.)
10.33*	License Agreement, dated March 1, 2022, by and between Marinus Pharmaceuticals, Inc. and Ovid Therapeutics Inc. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on March 3, 2022.)
10.34	Amendment, dated March 29, 2022, by and between Marinus Pharmaceuticals, Inc. and the Biomedical Advanced Research and Development Authority, a division of the U.S. Department of Health and Human Services' Office of the Assistant Secretary for Preparedness and Response. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on March 31, 2022.)
10.35+	Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended. (Incorporated by reference to Exhibit 10.1 to Form S-8 registration statement filed on August 10, 2021.)

Exhibit No.	Description of Exhibit
10.36	Letter Agreement re: Amendment to Tranche C Commitment, dated May 23, 2022, by and among Marinus Pharmaceuticals, Inc., as Borrower, Oaktree Fund Administration, LLC, as Administrative Agent and the other lenders party thereto. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on May 25, 2022.)
10.37*^	Asset Purchase Agreement, dated July 13, 2022, by and between Marinus Pharmaceuticals, Inc. and Novo Nordisk Inc. (Incorporated by referenced to Exhibit 10.1 to Form 8-K current report filed on July 14, 2022.)
10.38^	Amendment No. 2, dated September 21, 2022, by and between Marinus Pharmaceuticals, Inc. and the Biomedical Advanced Research and Development Authority, a division of the U.S. Department of Health and Human Services' Office of the Assistant Secretary for Preparedness and Response. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on September 23, 2022.)
10.39^	Revenue Interest Financing Agreement, dated October 28, 2022, by and between Marinus Pharmaceuticals, Inc. and Sagard Healthcare Royalty Partners, LP. (Incorporated by reference to Exhibit 10.3 to Form 10-Q quarterly report filed on November 7, 2022.)
10.40^	Security Agreement dated October 28, 2022, by and among Marinus Pharmaceuticals, Inc. and Sagard Healthcare Royalty Partners, LP. (Incorporated by reference to Exhibit 10.4 to Form 10-Q quarterly report filed on November 7, 2022.)
10.41^	Limited Consent and First Amendment to Credit Agreement, dated October 28, 2022, by and among Marinus Pharmaceuticals, Inc., as Grantor, and Oaktree Fund Administration, LLC, as Administrative Agent. (Incorporated by reference to Exhibit 10.5 to Form 10-Q quarterly report filed on November 7, 2022.)
10.42*	Collaboration and Supply Agreement, dated November 16, 2022, between Marinus Pharmaceuticals, Inc. and Tenacia Biotechnology (Shanghai) Co., Ltd. (Filed herewith.)
21	Subsidiaries of the Registrant. (Filed herewith.)
23.1	Consent of Ernst & Young LLP. (Filed herewith.)
31.1	Certification Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002. (Filed herewith.)
31.2	Certification Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002. (Filed herewith.)
32.1	Certification Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. (Furnished herewith.)
101.INS	XBRL Instance Taxonomy – the instance document does not appear in the Interactive Data file because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File formatted as Inline XBRL and contained in Exhibit 101

+ Indicates management contract or compensatory plan.

* Portions of this exhibit (indicated by asterisks) have been omitted in compliance with Item 601 of Regulation S-K.

^ 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.

(c) None.

Item 16. Form 10-K Summary

None.

SIGNATURES

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

Marinus Pharmaceuticals, Inc.

Date: March 9, 2023

By: /s/ Scott Braunstein
Scott Braunstein
Chief Executive Officer and Director

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

Name	Capacity	Date
/s/ Scott Braunstein, M.D. Scott Braunstein, M.D.	President, Chief Executive Officer (Principal Executive Officer) and Chairman of the Board and Director	March 9, 2023
/s/ Steven Pfanstiel Steven Pfanstiel	Chief Operating Officer, Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)	March 9, 2023
/s/ Charles Austin Charles Austin	Director	March 9, 2023
/s/ Elan Ezickson Elan Ezickson	Director	March 9, 2023
/s/ Seth H.Z. Fischer Seth H.Z. Fischer	Director	March 9, 2023
/s/ Tim M. Mayleben Tim M. Mayleben	Director	March 9, 2023
/s/ Saraswathy V. Nochur, Ph.D. Saraswathy V. Nochur, Ph.D.	Director	March 9, 2023
/s/ Christine B. Silverstein Christine B. Silverstein	Director	March 9, 2023

CONSOLIDATED FINANCIAL STATEMENTS

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

INDEX TO FINANCIAL STATEMENTS

CONTENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

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

Opinion on Financial Statements

We have audited the accompanying consolidated balance sheets of Marinus Pharmaceuticals, Inc. and subsidiaries (the Company) as of December 31, 2022 and 2021, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for the years then ended, 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, 2022 and 2021, and the results of its operations and its cash flows for the years then ended, 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.

Critical Audit Matters

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

Clinical Trial Prepaid and Accrued Expenses

Description of the Matter	As disclosed in Note 2 to the consolidated financial statements, the Company expenses research and development costs as incurred, which include costs relating to contracts with vendors, clinical research organizations and consultants and under clinical site agreements. The Company estimates the prepaid and accrued expenses based on the services received and efforts expended in relation to amounts invoiced by and paid to contract research organizations and other third-party vendors at the balance sheet date. The Company's clinical trial prepaid expenses of $3.9 million at December 31, 2022 are included in prepaid expenses and other current assets on the consolidated balance sheet, the Company's clinical trial accrued expenses at December 31, 2022 of $5.7 million are included in accrued expenses on the consolidated balance sheet, and the Company's related 2022 clinical trial expenses are included in research and development expenses of $79.9 million on the consolidated statement of operations and comprehensive loss for the year ended December 31, 2022. Auditing the Company's clinical trial prepaid and accrued expenses involved complex and subjective auditor judgment due to the estimation required by management in determining the progress to completion of services that have been performed by the service providers and the associated costs that will be invoiced by the service providers subsequent to the date that the financial statements are issued.
How We Addressed the Matter in Our Audit	To test the clinical trial prepaid and accrued expenses, our audit procedures included, among others, reviewing a sample of agreements with the service providers to corroborate key financial and contractual terms, and testing the accuracy and completeness of the underlying data used in the prepaid and accrued expense computations. We also evaluated management's estimates of the progress of a sample of clinical trials by making direct inquiries of the Company's operations personnel that oversee the clinical trials and obtaining information directly from a sample of service providers. To evaluate the completeness of the prepaid and accrued expenses, we also examined subsequent invoices from the service providers and cash disbursements to the service providers, to the extent such invoices were received, or payments were made prior to the date that the financial statements were issued.

Revenue Interest Financing Payable

Description of the Matter	As described in Note 10 to the consolidated financial statements, the Company entered into a royalty purchase agreement (RPA) with Sagard Healthcare Partners. Pursuant to the RPA, the Company received gross proceeds of $32.5 million in exchange for the obligation to make royalty payments on U.S. net sales of ganaxolone, including the recently approved ZTALMY (ganaxolone) oral suspension CV. The Company recorded the RPA as a liability (revenue interest financing payable) on the consolidated balance sheet at a carrying value of $30.9 million as of December 31, 2022 and imputed interest expense associated with this liability during 2022 using the effective interest rate method. The effective interest rate is calculated based on the rate that would enable the liability 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 RPA depending on a number of factors, including the level and timing of forecasted net sales and resulting royalty payments. The Company utilizes the prospective method to account for the revenue interest financing payable, under which a new effective interest rate is determined at each balance sheet date based on the Company's current estimates of the amount and timing of expected future royalty payments. Auditing the revenue interest financing payable involved complex and subjective auditor judgment due to the estimation uncertainty involved in determining the effective interest rate. The Company's effective interest rate model includes actual revenues recorded and royalties paid to-date, as well as estimated revenue projections for which future royalties will be paid, which are sensitive to significant assumptions including the size of the addressable patient population and the Company's expected market share and the anticipated sales price of its products, among others.
How We Addressed the Matter in Our Audit	To test the revenue interest financing payable, our audit procedures included, among others, testing the significant assumptions used to develop the estimates and evaluating the completeness and accuracy of the underlying data used by the Company in its effective interest rate model. To test the estimated amount of future product sales, we compared the estimated size of the addressable patient population to industry data that tracks healthcare information, and we compared the anticipated pricing information to actual sales and a third-party market analysis. We recalculated the current year interest expense based on the Company's model, and performed sensitivity analyses to evaluate the changes in the effective interest rates, and associated interest expense, that would result from changes in the significant assumptions.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2020.

Philadelphia, Pennsylvania
March 9, 2023

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED BALANCE SHEETS

(In thousands, except share and per share amounts)

	December 31, 2022	December 31, 2021
ASSETS		
Current assets:		
Cash and cash equivalents	$ 240,551	$ 122,927
Accounts receivable, net	6,348	2,629
Inventory	77	—
Contract asset, net	—	557
Prepaid expenses and other current assets	5,402	5,565
Total current assets	252,378	131,678
Property and equipment, net	4,236	2,499
Other assets	2,904	2,663
Total assets	$ 259,518	$ 136,840
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 4,461	$ 3,126
Refund liability	—	21,233
Current portion of revenue interest financing payable	1,020	—
Accrued expenses	19,536	16,207
Total current liabilities	25,017	40,566
Notes payable, net of deferred financing costs	71,018	40,809
Revenue interest financing payable, net of deferred financing costs	29,857	—
Contract liabilities, net	16,285	—
Other long-term liabilities	1,341	1,979
Total liabilities	143,518	83,354
Stockholders' equity:		
Series A convertible preferred stock, $0.001 par value; 25,000,000 shares authorized, 4,300 shares issued and outstanding at December 31, 2022 and 4,575 shares issued and outstanding at December 31, 2021	4,043	4,302
Common stock, $0.001 par value; 150,000,000 shares authorized, 49,650,074 issued and 49,642,767 outstanding at December 31, 2022 and 36,797,561 issued and 36,790,254 outstanding at December 31, 2021	50	37
Additional paid-in capital	542,428	459,852
Treasury stock at cost, 7,307 shares at December 31, 2022 and December 31, 2021	—	—
Accumulated deficit	(430,521)	(410,705)
Total stockholders' equity	116,000	53,486
Total liabilities and stockholders' equity	$ 259,518	$ 136,840

See accompanying notes to financial statements.

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

	Year Ended December 31,	
	2022	2021
Revenue:		
Product revenue, net	$ 2,872	$ —
Federal contract revenue	6,935	6,358
Collaboration revenue	15,671	8,987
Total revenue	25,478	15,345
Expenses:		
Research and development	$ 79,912	$ 73,520
Selling, general and administrative	56,845	37,278
Cost of product revenue	190	—
Cost of collaboration revenue	150	1,478
Cost of IP license fee	1,169	—
Total expenses	138,266	112,276
Loss from operations	(112,788)	(96,931)
Interest income	2,354	80
Interest expense	(10,672)	(2,582)
Gain from sale of priority review voucher, net	107,375	—
Other (expense) income, net	(2,696)	657
Loss before income taxes	(16,427)	(98,776)
Provision for income taxes	(3,389)	—
Net loss and comprehensive loss	$ (19,816)	$ (98,776)
Net loss applicable to common shareholders	$ (19,816)	$ (98,776)
Per share information:		
Net loss per share of common stock—basic and diluted	$ (0.51)	$ (2.69)
Basic and diluted weighted average shares outstanding	39,072,599	36,697,171

See accompanying notes to financial statements.

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(In thousands, except share and per share amounts)

	Series A Convertible Preferred Stock		Common Stock		Additional Paid-in	Treasury Stock		Accumulated	Total Stockholders'
	Shares	Amount	Shares	Amount	Capital	Shares	Amount	Deficit	Equity
Balance, December 31, 2020	4,753	$ 4,469	36,578,460	$ 37	$ 444,622	7,307	$ —	$ (311,929)	$ 137,199
Stock-based compensation expense	-	-	-	-	13,867	-	-	-	13,867
Exercise of stock options	-	-	176,194	-	1,199	-	-	-	1,199
Financing costs	-	-	-	-	(3)	-	-	-	(3)
Conversion of convertible preferred stock into common	(178)	(167)	35,600	-	167				-
Net loss	-	-	-	-	-	-	-	(98,776)	(98,776)
Balance, December 31, 2021	4,575	$ 4,302	36,790,254	$ 37	$ 459,852	7,307	$ —	$ (410,705)	$ 53,486
Stock-based compensation expense	—	—	—	—	14,890	—	—	—	14,890
Issuance of common stock and pre-funded warrants in connection with follow-on public offering ($4.75 per share), net of expenses of $4,521	—	—	12,421,053	13	64,464	—	—	—	64,477
Exercise of stock options	—	—	238,312	—	1,794	—	—		1,794
Net issuance of common stock in connection with the vesting of restricted stock	—	—	14,893	—	—	—	—	—	—
Issuance of stock related to IP license agreement with Ovid	—	—	123,255	—	1,169	—	—	—	1,169
Conversion of convertible preferred stock into common	(275)	(259)	55,000	—	259	—	—	—	—
Net loss	—	—	—	—	—	—	—	(19,816)	(19,816)
Balance, December 31, 2022	4,300	$ 4,043	49,642,767	$ 50	$ 542,428	7,307	$ —	$ (430,521)	$ 116,000

See accompanying notes to financial statements.

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

	Year Ended December 31,	
	2022	2021
Cash flows from operating activities		
Net loss	$ (19,816)	$ (98,776)
Adjustments to reconcile net loss to net cash used in operating activities:		
Gain from sale of PRV, net of transaction costs	(107,375)	—
Depreciation and amortization	418	355
Amortization of debt issuance costs	1,663	549
Accretion of revenue interest financing debt	956	—
Stock-based compensation expense	14,890	13,867
Amortization of net contract asset/liability	(1,052)	(105)
Noncash lease expense	251	312
Noncash lease liability	344	309
Write off of fixed assets	828	243
Issuance of common stock for cost of license agreement	1,169	—
Unrealized loss (gain) on foreign currency transactions	930	(930)
Changes in operating assets and liabilities:		
Refund liability	(22,163)	22,163
Net contract asset/liability	17,894	(452)
Prepaid expenses and other current assets, non-current assets, inventory and accounts receivable	(5,577)	(853)
Accounts payable and accrued expenses	3,754	7,841
Net cash used in operating activities	(112,886)	(55,477)
Cash flows from investing activities		
Proceeds from sale of PRV, net of transaction costs	107,375	—
Proceeds from sale of property and equipment	171	—
Maturities of short-term investments	—	1,474
Deposit on property and equipment	—	(1,793)
Purchases of property and equipment	(1,774)	(1,096)
Net cash provided by (used in) investing activities	105,772	(1,415)
Cash flows from financing activities		
Proceeds from exercise of stock options	1,794	1,199
Proceeds from notes payable, net of fees	28,588	40,259
Proceeds from revenue interest financing agreement, net of issuance costs	29,921	—
Payments of revenue interesting financing debt	(42)	—
Financing costs, paid	—	(148)
Proceeds from equity offerings, net of offering costs	64,477	—
Net cash provided by financing activities	124,738	41,310
Net increase in cash and cash equivalents	117,624	(15,582)
Cash and cash equivalents—beginning of period	122,927	138,509
Cash and cash equivalents—end of period	$ 240,551	$ 122,927
Supplemental disclosure of cash flow information		
Debt issuance costs included in accrued expenses	$ —	$ 900
Property and equipment in accrued expenses	$ 22	$ 43
Cash paid for interest during the year	$ 7,892	$ 2,032
Property and equipment in deposits placed in service	$ 1,664	$ —

See accompanying notes to financial statements.

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Organization and Description of the Business

We are a commercial-stage pharmaceutical company dedicated to the development of innovative therapeutics for the treatment of seizure disorders, including rare genetic epilepsies and status epilepticus. On March 18, 2022, the U.S. Food and Drug Administration (FDA) approved our new drug application (NDA) for the use of ZTALMY (ganaxolone) oral suspension for the treatment of seizures associated with Cyclin-dependent Kinase-like 5 Deficiency Disorder (CDD) in patients 2 years of age and older. In June 2022, the U.S. Drug Enforcement Administration (DEA) published an interim final rule in the Federal Register placing ganaxolone and its salts in schedule V (CV) of the Controlled Substances Act (CSA), which rule became final on December 9, 2022. ZTALMY, our first FDA approved product, became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022. We plan to develop ganaxolone for the treatment of other rare genetic epilepsies, including Tuberous Sclerosis Complex (TSC), and for the treatment of status epilepticus (SE).

The continued global spread of COVID-19 has impacted our clinical operations and timelines. For example, our RAISE trial is conducted in hospitals, primarily intensive care units in academic medical centers, which have experienced high rates of COVID-19 admissions. Several of these sites participating in the RAISE trial have experienced COVID-related difficulties, including staff turnover and the need to devote significant resources to patients with COVID-19, which has resulted in site initiation and enrollment delays for the RAISE trial. Given these COVID-19-related challenges and the interruption in drug supply in mid-2022, we previously adjusted our expectation for our top-line data readout for the RAISE trial to the second half of 2023. In May 2022, we resumed screening and recruitment for the RAISE trial. Several of the sites participating in the RAISE trial continue to encounter COVID-related setbacks, including staff turnover and the need to devote significant resources for patients with COVID-19. In addition, our ganaxolone clinical trials in the outpatient setting may be negatively impacted if patients and their caregivers do not want to participate while the COVID-19 pandemic persists. The duration and severity of the pandemic and its long-term impact on our business are uncertain at this time.

Liquidity

Since inception, other than for the three months ended September 30, 2022 due to a one-time net gain from the sale of our Priority Review Voucher (PRV), we have incurred net losses and negative cash flows from our operations. We incurred a net loss of $19.8 million for the year ended December 31, 2022. We have generated limited product revenues, and there is no assurance that profitable operations will be achieved in the future, and if achieved, could be sustained on a continuing basis. In addition, development activities, clinical and preclinical testing, and commercialization of ganaxolone, in indications other than CDD in the US, will require significant additional financing. Our accumulated deficit as of December 31, 2022 was $430.5 million and we expect to incur substantial losses in future periods. We plan to finance our future operations with a combination of proceeds from the issuance of equity securities, the issuance of debt, government funding, collaborations, licensing transactions and other commercial transactions or other sources, and revenues from future product sales, if any. We have not generated positive cash flows from operations, and there are no assurances that we will be successful in obtaining an adequate level of financing for the continued development and commercialization of ganaxolone.

Management’s operating plan which underlies the analysis of our ability to continue as a going concern involves the estimation of the amount and timing of future cash inflows and outflows. Actual results could vary from the operating plan. We follow the provisions of Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) Topic 205-40, Presentation of Financial Statements—Going Concern, which requires management to assess our ability to continue as a going concern within one year after the date the financial statements are issued. In the year ended December 31, 2022 the following events occurred: in August 2022, we secured additional funding of $107.4 million in net proceeds from the closing of the PRV transaction with Novo Nordisk, Inc.; in October 2022, we entered into a royalty monetization agreement with Sagard Healthcare Partners, pursuant to which we received a total of $32.5 million upfront in return for royalty payments on U.S. net sales of ganaxolone; we entered into a collaboration and

supply agreement with Tenacia, pursuant to which we received a total upfront payment of $10.0 million; and in November 2022, we completed a public offering resulting in approximately $64.5 million in net proceeds including the full exercise of the underwriters' overallotment option after deducting underwriting discounts and commissions. As a result of these events, the going concern uncertainty that was disclosed in the financial statements for the year ended December 31, 2021 was alleviated during 2022. We believe that our existing cash and cash equivalents on hand as of December 31, 2022, will be sufficient to fund our operating expenses and capital expenditure requirements, as well as maintain the minimum cash balance required under our debt facility, into the second half of 2024. However, we will need to secure additional funding in the future, from one or more equity or debt financings, government funding, collaborations, licensing transactions, other commercial transactions or other sources in order to carry out all of our commercialization and planned research and development activities with respect to ganaxolone.

2. Summary of Significant Accounting Policies

Principles of Consolidation

The financial statements include the accounts of Marinus Pharmaceuticals, Inc. (the Company) and its wholly-owned subsidiary as of December 31, 2022. In February 2021, a wholly-owned subsidiary was established in Ireland. All intercompany accounts and transactions have been eliminated.

Use of Estimates

The preparation of financial statements in conformity with generally accepted accounting principles (GAAP) requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from such estimates.

Segment Information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. We view our operations and manage our business in one segment, which is the identification and development of innovative therapeutics to treat rare seizure disorders.

Fair Value of Financial Instruments and Credit Risk

At December 31, 2022, our financial instruments included cash equivalents, accounts payable, accrued expenses, notes payable, and royalty interest financing payable. At December 31, 2021, our financial instruments included cash equivalents, accounts payable, accrued expenses, and notes payable. The carrying amount of cash equivalents, accounts payable and accrued expenses approximated fair value, given their short-term nature. The carrying value of the notes payable and royalty interest financing payable approximates fair value as the interest rate is reflective of current market rates on debt with similar terms and conditions.

Cash equivalents subject us to concentrations of credit risk. However, we invest our cash in accordance with a policy objective that seeks to ensure both liquidity and safety of principal. The policy limits investments to instruments issued by the U.S. government, certain Securities and Exchange Commission (SEC)-registered money market funds that invest only in U.S. government obligations and various other low-risk liquid investment options, and places restrictions on portfolio maturity terms.

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

Cash and Cash Equivalents

We consider all highly liquid investments that have maturities of three months or less when acquired to be cash equivalents. As of December 31, 2022 and 2021, we invested a portion of our cash balances in money market investments, which we have included as cash equivalents on our balance sheets.

Accounts Receivable, net

Net trade receivables related to ZTALMY sales, which are recorded in net accounts receivable on the consolidated balance sheets, were approximately $1.3 million as of December 31, 2022. There were no net trade receivables related to ZTALMY as of December 31, 2021. We had no allowance for doubtful accounts as of December 31, 2022 or 2021. An allowance for doubtful accounts is determined based on our assessment of the credit worthiness and financial condition of our customer, aging of receivables, as well as the general economic environment. Any allowance would reduce the net receivables to the amount that is expected to be collected. We have one customer, Orsini Pharmaceutical Services, LLC (Orsini), a specialty pharmacy that dispenses ZTALMY directly to patients. Payment terms for Orsini are 30 days from the shipment date.

Excluding net trade receivables, accounts receivable represents amounts due to us under the BARDA contract for valid expenditures expected to be reimbursed to us under the terms of the BARDA contract and current amounts due to us from Orion Corporation (Orion) under the collaboration agreement (Note 11).

Inventory

Inventories are recorded using actual costs and may consist of raw materials (ganaxolone API), work in process and finished goods. We began capitalizing inventory related to ZTALMY subsequent to the March 2022 FDA approval of ZTALMY, as the related costs were expected to be recoverable through the commercialization and subsequent sale of ZTALMY. Prior to FDA approval of ZTALMY, costs estimated at approximately $2 million for commercially saleable product and materials were incurred and included in research and development expenses. As a result, cost of product revenues related to ZTALMY will initially reflect a lower average per unit cost of materials into approximately the first half of 2024, as previously expensed inventory is utilized for commercial production and sold to customers.

Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets generally represent payments made for goods or services to be received within one year, and are expensed as the related benefit is received.

Property and Equipment

Property and equipment consist of laboratory and office equipment and are recorded at cost. Property and equipment are depreciated on a straight-line basis over their estimated useful lives. We estimate a life of three years for computer equipment, including software, five years for office equipment and furniture, five to fifteen years for laboratory equipment, and the lesser of the lease term or useful life for leasehold improvements. When property and equipment are sold or otherwise disposed of, the cost and related accumulated depreciation are removed from the accounts and the resulting gain or loss is included in operating expenses.

Impairment of Long-Lived Assets

We review long-lived assets, including property and equipment, for impairment whenever events or changes in business circumstances indicate that the carrying amount of an asset may not be fully recoverable. If the estimated undiscounted future cash flows expected to result from the use of the asset and its eventual disposition is less than its

carrying amount an impairment loss would be recognized if the carrying value of the asset exceeded its fair value. Fair value is generally determined using discounted cash flows.

Other Assets

Other assets generally represent noncurrent capitalized contract costs, our noncurrent right-of-use asset related to our operating lease and assets held for sale or placed into storage.

Income Taxes

We recognize deferred tax assets and liabilities for temporary differences between the financial reporting basis and the tax basis of our assets and liabilities and the expected benefits of net operating loss carryforwards. The impact of changes in tax rates and laws on deferred taxes, if any, applied during the years in which temporary differences are expected to be settled, is reflected in the financial statements in the period of enactment. The measurement of deferred tax assets is reduced, if necessary, if, based on weight of the evidence, it is more likely than not that some, or all, of the deferred tax assets will not be realized. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the period that such tax rate changes are enacted. At December 31, 2022 and 2021, we have concluded that a full valuation allowance is necessary for our net deferred tax assets. We had no material amounts recorded for uncertain tax positions, interest or penalties in the accompanying financial statements.

The Tax Cuts and Jobs Act passed in 2017 included a provision which would require taxpayers to capitalize and amortize U.S.-based research & experimentation (R&E) expenses over a period of five years and non-U.S. R&E expenses over 15 years effective for tax years beginning after December 31, 2021 pursuant to Internal Revenue Code Section 174. As a result of the capitalization of R&E expenses, income generated from the sale of the PRV, and limitations related to the utilization of state net operating losses, we have a current income tax expense for the year ended December 31, 2022 attributable to state income taxes. We do not have a federal income tax liability for the year ended December 31, 2022 due to the utilization of Net Operating Losses (NOLs) after taking into consideration Internal Revenue Code Section 382 limitations related to changes in ownership.

Debt Issuance Costs

Debt issuance costs incurred in connection with our note payable (Note 9) and revenue interest financing payable (Note 10) are amortized to interest expense over the term of the respective financing arrangement using the effective-interest method. Debt issuance costs, net of related amortization are deducted from the carrying value of the related debt.

Contract Liabilities, net

When consideration is received, or such consideration is unconditionally due, from a customer prior to completing our performance obligation to the customer under the terms of a contract, a contract liability is recorded. Contract liabilities expected to be recognized as revenue or a reduction of expense within the 12 months following the balance sheet date are classified as current liabilities. Contract liabilities not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as long-term liabilities. In accordance with ASC 210-20, our contract liabilities are partially offset by our contract assets for the year ended December 31, 2022, as further discussed in Note 11. For the year ended December 31, 2021, our contract liability was offset by our contract asset.

Liability Related to Revenue Interest Financing and Non-Cash Interest Expense

In October 2022, we recognized a liability related to the Revenue Interest Financing Agreement with Sagard Healthcare Royalty Partners, LP (Sagard) under ASC 470-10 *Debt* and ASC 835-30 *Interest - Imputation of Interest.* The initial funds received by us from Sagard pursuant to the terms of the Revenue Interest Financing Agreement were

recorded as a liability and will be accreted under the effective interest method upon the estimated amount of future royalty payments to be made pursuant to the Revenue Interest Financing Agreement. The issuance costs were recorded as a direct deduction to the carrying amount of the liability and will be amortized under the effective interest method over the estimated period the liability will be repaid. We estimated the total amount of future product revenue to be generated over the life of the Revenue Interest Financing Agreement, and a significant increase or decrease in these estimates could materially impact the liability balance and the related interest expense. If the timing or amounts of any estimated future revenue and related payments change, we will prospectively adjust the effective interest and the related amortization of the liability and related issuance costs.

Product Revenue, net

We recognize ZTALMY revenue in accordance with ASC 606 – Revenue from contracts with customers. Our revenue recognition analysis consists of the following steps: (i) identification of the promised goods in the contract; (ii) determination of whether the promised goods are performance obligations, including whether they are capable of being distinct; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue as we satisfy each performance obligation.

Our first FDA approved product, ZTALMY, became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022. We have one customer, Orsini, which is a specialty pharmacy that dispenses ZTALMY directly to patients. Our contract with Orsini has a single performance obligation to deliver ZTALMY upon receipt of a purchase order, which is satisfied when Orsini receives ZTALMY. We recognize ZTALMY revenue at the point in time when control of ZTALMY is transferred to Orsini, which is upon delivery to Orsini. The transaction price that we recognize for ZTALMY revenue includes an estimate of variable consideration. Shipping and handling costs to Orsini are recorded as selling, general and administrative expenses. The components of variable consideration include:

Trade Discounts and Allowances. We provide an incentive prompt payment discount to Orsini as explicitly stated in the contract with Orsini. This discount is recorded as a reduction of ZTALMY revenue and accounts receivable in the period in which the related ZTALMY revenue is recognized. We estimate the amount of variable consideration for discounts and allowances using the expected value method.

Product Returns and Recall. We provide for ZTALMY returns in accordance with our Return Good Policy. We estimate the amount of ZTALMY that may be returned using the expected value method, and we present this amount as a reduction of ZTALMY revenue in the period the related ZTALMY revenue is recognized. In the event of a recall, we will promptly notify Orsini and will reimburse Orsini for direct administrative expenses incurred in connection with the recall as well as the cost of replacement product.

Government Rebates. We are subject to discount obligations under state Medicaid programs and Medicare. We estimate reserves related to these discount programs and record these obligations in the same period the related revenue is recognized, resulting in a reduction of product revenue.

Patient Assistance. We offer a voluntary co-pay patient assistance program intended to provide financial assistance to eligible patients with a prescription drug co-payment required by payors and coupon programs for cash payors. The calculation of the current liability for this assistance is based on an estimate of claims and the cost per claim that we expect to receive associated with ZTALMY that has been recognized as revenue but remains in the distribution channel inventories at the end of each reporting period.

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

Federal Contract Revenue

We recognize federal contract revenue from the BARDA Contract in the period in which the allowable research and development expenses are incurred, and receivables associated with this revenue are included within accounts receivable on our balance sheets. This revenue is not within the scope of Accounting Standards Codification (ASC) 606 – Revenue from contracts with customers.

Collaboration and Licensing Revenue

We may enter into collaboration and licensing arrangements for research and development, manufacturing, and commercialization activities with counterparties for the development and commercialization of our product candidates. These arrangements may contain multiple components, such as (i) licenses, (ii) research and development activities, and (iii) the manufacturing of certain material. Payments pursuant to these arrangements may include non-refundable and refundable payments, payments upon the achievement of significant regulatory, development and commercial milestones, sales of product at certain agreed-upon amounts, and royalties on product sales. The amount of variable consideration is constrained until it is probable that the revenue is not at a significant risk of reversal in a future period.

In determining the appropriate amount of revenue to be recognized as we fulfill our obligations under a collaboration agreement, we perform the following steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are capable of being distinct; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue as we satisfy each performance obligation.

We must develop estimates and assumptions that require judgment to determine the underlying stand-alone selling price for each performance obligation, which determines how the transaction price is allocated among the performance obligations. The estimation of the stand-alone selling price may include such estimates as forecasted revenues and costs, development timelines, discount rates and probabilities of regulatory and commercial success. We also apply significant judgment when evaluating whether contractual obligations represent distinct performance obligations, allocating transaction price to performance obligations within a contract, determining when performance obligations have been met, assessing the recognition and future reversal of variable consideration and determining and applying appropriate methods of measuring progress for performance obligations satisfied over time.

Research and Development

Research and development costs are expensed as incurred. Costs for certain development activities, such as clinical trials, are recognized based on an evaluation of the progress to completion of specific tasks using data such as subject enrollment, monitoring visits, clinical site activations, or information provided to us by our vendors with respect to their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the financial statements as prepaid or accrued research and development expense, as the case may be.

Clinical Trial Expenses

As part of the process of preparing our financial statements, we are required to estimate our expenses resulting from our obligations under contracts with vendors, clinical research organizations and consultants and under clinical site agreements in connection with conducting clinical trials. The financial terms of these contracts are subject to negotiations, which vary from contract to contract and may result in payment flows that do not match the periods over which materials or services are provided under such contracts. Our objective is to reflect the appropriate trial expenses in our financial statements by matching those expenses with the period in which services are performed and efforts are expended. We

account for these expenses according to the progress of the trial as measured by patient progression and the timing of various aspects of the trial. We determine accrual estimates based on estimates of services received and efforts expended that take into account discussion with applicable personnel and outside service providers as to the progress or state of consummation of trials. During the course of a clinical trial, we adjust our clinical expense recognition if actual results differ from its estimates. We make estimates of our accrued expenses as of each balance sheet date based on the facts and circumstances known at that time. Our clinical trial accruals are dependent upon the timely and accurate reporting of contract research organizations and other third-party vendors. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low for any particular period. For the years ended December 31, 2022 and 2021 there were no material adjustments to our prior period estimates of accrued expenses for clinical trials. For the year ended December 31, 2022, our accrued clinical trial expenses were $5.7 million, and our prepaid clinical expenses were $3.9 million, respectively.

Stock-Based Compensation

We account for stock-based compensation in accordance with the provisions of Accounting Standards Codification (ASC) Topic 718, *Compensation—Stock Compensation*, or ASC 718, which requires the recognition of expense related to the fair value of stock-based awards in the statements of operations. For stock options issued to employees, non-employees and members of our board of directors for their services on our board of directors, we estimate the grant-date fair value of options using the Black-Scholes option pricing model. The use of the Black-Scholes option pricing model requires management to make assumptions with respect to the expected term of the option, the expected volatility of the common stock consistent with the expected life of the option, risk-free interest rates, and, for grants prior to our initial public offering, the value of the common stock. For restricted stock awards, the grant date fair value is determined by the closing market price of our common stock on the date of grant. For awards subject to time-based vesting, we recognize stock-based compensation expense, on a straight-line basis over the requisite service period, which is generally the vesting term of the award. For awards subject to performance-based vesting conditions, we recognize stock-based compensation expense when it is probable that the performance condition will be achieved. We recognize forfeitures as they occur in accordance with the guidance in ASC Topic 718.

Loss Per Share of Common Stock

Basic loss per share is computed by dividing net loss applicable to common stockholders by the weighted average number of shares of common stock outstanding during each period. Diluted loss per share includes the effect, if any, from the potential exercise or conversion of securities, such as convertible preferred stock, stock options, and unvested restricted stock, which would result in the issuance of incremental shares of common stock. In computing the basic and diluted net loss per share applicable to common stockholders, the weighted average number of shares remains the same for both calculations due to the fact that when a net loss exists, dilutive shares are not included in the calculation. These potentially dilutive securities are more fully described in Note 7.

The following table sets forth the computation of basic and diluted earnings per share for the years ended December 31, 2022 and 2021 (in thousands, except share and per share amounts):

	Year ended December 31,	
	2022	2021
Basic and diluted net loss per share of common stock:		
Net loss applicable to common stockholders	$ (19,816)	$ (98,776)
Weighted average shares of common stock outstanding	39,072,599	36,697,171
Net loss per share of common stock—basic and diluted	$ (0.51)	$ (2.69)

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

The following potentially dilutive securities (common stock equivalents) have been excluded from the computation of diluted weighted average shares outstanding, as they would be antidilutive:

	Year Ended December 31,	
	2022	2021
Convertible preferred stock	860,000	915,000
Restricted stock awards and restricted stock units	671,976	26,025
Stock options	5,730,219	4,738,855
	7,262,195	5,679,880

The pre-funded warrants to purchase common shares issued in connection with the November 2022 offering are included in the calculation of basic and diluted net loss per share as the exercise price of $0.001 per share is non-substantive and is virtually assured. The pre-funded warrants are more fully described in Note 7. The convertible preferred stock meets the definition of a participating security; however, the holders are not obligated to share in our losses. As of December 31, 2022 and 2021, we had no other potentially dilutive securities.

3. Fair Value Measurements

FASB accounting guidance defines fair value as the price that would be received to sell an asset or paid to transfer a liability (exit price) in an orderly transaction between market participants at the measurement date. The accounting guidance outlines a valuation framework and creates a fair value hierarchy in order to increase the consistency and comparability of fair value measurements and the related disclosures. In determining fair value, we use quoted prices and observable inputs. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from independent sources.

The fair value hierarchy is broken down into three levels based on the source of inputs as follows:

- Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities.
- Level 2—Valuations based on observable inputs and quoted prices in active markets for similar assets and liabilities.
- Level 3—Valuations based on inputs that are unobservable and models that are significant to the overall fair value measurement.

If the inputs used to measure fair value fall within different levels of the hierarchy, the category level is based on the lowest priority level input that is significant to the fair value measurement of the instrument. As of December 31, 2022 and 2021, all of our financial assets and liabilities were classified as Level 1 valuations.

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

The following fair value hierarchy table presents information about each major category of our financial assets and liabilities measured at fair value on a recurring basis (in thousands):

	Level 1	Level 2	Level 3	Total
December 31, 2022				
Assets				
Cash	$ 10,569	$ —	$ —	$ 10,569
Money market funds (cash equivalents)	229,982	—	—	229,982
Total assets	$ 240,551	$ —	$ —	$ 240,551
December 31, 2021				
Assets				
Cash	$ 2,360	$ —	$ —	$ 2,360
Money market funds (cash equivalents)	120,567	—	—	120,567
Total assets	$ 122,927	$ —	$ —	$ 122,927

4. Property and Equipment

Property and equipment consisted of the following (in thousands):

	December 31, 2022	December 31, 2021
Laboratory equipment	$ 4,277	$ 2,565
Leasehold improvements	899	899
Office furniture and equipment	514	429
Total property and equipment	5,690	3,893
Less: accumulated depreciation	(1,454)	(1,394)
Total property and equipment, net	$ 4,236	$ 2,499

Depreciation expense was $0.3 million and $0.3 million for the years ended December 31, 2022 and 2021, respectively. In 2022 and 2021, we determined certain of our laboratory equipment was not required and held for sale or placed into storage, and as a result, write-downs of net equipment of approximately $0.8 million and $0.2 million, respectively, were recorded as a loss and included in Other (expense) income, net on the consolidated statement of operations for the years ended December 31, 2022 and 2021. We had assets held for sale of approximately $0.7 million and $0.3 million as of December 31, 2022 and 2021, respectively, included in other long-term assets.

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	December 31, 2022	December 31, 2021
Payroll and related costs	$ 7,061	$ 5,830
Clinical trials and drug development	5,725	8,217
Professional fees	1,417	1,311
Accrued tax provision	2,445	—
Third-party commercial expenses	1,880	—
Short-term lease liabilities	637	556
Other	371	293
Total accrued expenses	$ 19,536	$ 16,207

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6. Leases

We have entered into one operating lease for real estate. This lease has a term of 78 months, and includes renewal terms which can extend the lease term by 60 months, which is included in the lease term when it is reasonably certain that we will exercise the option. As of December 31, 2022, our operating lease had a weighted average remaining lease term of 33 months. The right-of-use (ROU) asset is included in "Other assets" on our balance sheets as of December 31, 2022 and 2021, and represents our right to use the underlying asset for the lease term. Our obligations to make lease payments are included in "Accrued expenses" and "Other long-term liabilities" on our balance sheets as of December 31, 2022 and 2021. The ROU asset was initially measured at cost, which comprises the initial amount of the lease liability adjusted for lease payments made at or before the lease commencement date, plus any initial direct costs incurred, less any lease incentives received. The ROU asset is subsequently measured throughout the lease term at the carrying amount of the lease liability, plus initial direct costs, plus (minus) any prepaid (accrued) lease payments, less the unamortized balance of lease incentives received.

As of December 31, 2022 and 2021, the ROU asset was $1.3 million and $1.7 million, respectively, and operating lease liabilities were $2.0 million and $2.5 million, respectively. We have entered into various short-term operating leases, primarily for clinical trial equipment, with an initial term of twelve months or less. These leases are not recorded on our balance sheets. All operating lease expense is recognized on a straight-line basis over the lease term. During each of the years ended December 31, 2022 and 2021, we recognized $0.6 million in total lease costs, which included less than $0.1 million in short-term lease costs related to short-term operating leases in each year.

Because the rate implicit in the lease is not readily determinable, we use our incremental borrowing rate to determine the present value of the lease payments. The weighted average incremental borrowing rate used to determine the initial value of the ROU asset and lease liability was 11.0%, derived from a corporate yield curve based on a synthetic credit rating model using a market signal analysis. We have certain contracts for real estate which may contain lease and non-lease components which we have elected to treat as a single lease component.

ROU assets for operating leases are periodically reduced by impairment losses. We use the long-lived assets impairment guidance in ASC Subtopic 360-10, Property, Plant, and Equipment – Overall, to determine whether an ROU asset is impaired, and if so, the amount of the impairment loss to recognize. As of December 31, 2022 and 2021, we have not recognized any impairment losses for our ROU assets.

Maturities of operating lease liabilities as of December 31, 2022 were as follows (in thousands):

2023	$	823
2024		840
2025		642
Thereafter		—
		2,305
Less: imputed interest		(327)
Total lease liabilities	$	1,978
Current operating lease liabilities	$	637
Non-current operating lease liabilities		1,341
Total lease liabilities	$	1,978

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7. Stockholders' Equity

In 2005, we adopted the 2005 Stock Option and Incentive Plan (2005 Plan) that authorizes us to grant options, restricted stock and other equity-based awards. As of December 31, 2022, 577 options to purchase shares of common stock were outstanding pursuant to grants in connection with the 2005 Plan. No additional shares are available for issuance under the 2005 Plan. The amount, terms of grants, and exercisability provisions are determined and set by our board of directors.

Effective August 2014, we adopted our 2014 Equity Incentive Plan, as amended (2014 Plan) that authorizes us to grant options, restricted stock, and other equity-based awards, subject to adjustment in accordance with the 2014 Plan. As of December 31, 2022, 3,755,239 options to purchase shares of common stock and 671,976 restricted shares of common stock were outstanding pursuant to grants in connection with the 2014 Plan, and 975,646 shares of common stock were available for future issuance. The amount, terms of grants, and exercisability provisions are determined and set by our board of directors. In accordance with the 2014 Plan, on January 1, 2023, the shares of common stock available for future grants under the 2014 Plan was increased to 2,995,756.

In addition, during the years ended December 31, 2022 and 2021, we granted 788,885 and 772,117 options, respectively, to purchase shares of common stock outside of our 2014 Plan as inducement grants material to new employees entering into employment agreements with us pursuant to Nasdaq Listing Rule 5635(c)(4). The amount, terms of grants, and exercisability provisions of these grants are determined and set by our board of directors, and are largely consistent with the terms and exercisability provisions of grants under our 2014 Plan.

Stock Options

Total compensation cost recognized for all stock option awards in the statements of operations is as follows (in thousands):

	Year Ended December 31,	
	2022	2021
Research and development	$ 4,740	$ 4,482
Selling, general and administrative	8,429	9,106
Total	$ 13,169	$ 13,588

Options issued under both the 2005 Plan and 2014 Plan and the inducement grants have a contractual life of up to 10 years and may be exercisable in cash or as otherwise determined by the board of directors. Vesting generally

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occurs over a period of not greater than four years. A summary of stock option activity for the years ended December 31, 2022 and 2021 is presented below (in thousands, except share and per share amounts):

	Shares	Weighted-Average Exercise Price Per Share	Aggregate Intrinsic Value
Outstanding—December 31, 2020	3,507,638	$ 12.64	
Granted	1,868,066	13.49	
Exercised	(176,194)	6.80	
Forfeited	(225,726)	11.85	
Expired	(234,929)	32.81	
Outstanding—December 31, 2021	4,738,855	$ 12.23	
Granted	2,026,981	8.77	
Exercised	(238,312)	7.52	
Forfeited	(415,816)	10.49	
Expired	(381,489)	14.61	
Outstanding—December 31, 2022	5,730,219	$ 10.87	$ —
Exercisable—December 31, 2022	3,371,063	$ 11.46	$ —

The weighted average remaining contractual term of options outstanding and exercisable as of December 31, 2022 is 7.6 years and 6.8 years, respectively.

Intrinsic value in the table above was determined by calculating the difference between the market value of our common stock on the last trading day of 2022 of $3.98 per share and the exercise price, for any in-the-money options. There were no outstanding or exercisable in-the-money stock options as of December 31, 2022.

The weighted-average grant date fair value of options granted was $7.00 and $11.54 per share in 2022 and 2021, respectively, and was estimated at the date of grant using the Black-Scholes option-pricing model with the following ranges of weighted-average assumptions:

	2022		2021	
Expected stock price volatility	103 - 116	%	115 - 124	%
Expected term of options	5.7 - 6.5	years	5.0 - 6.1	years
Risk-free interest rate	1.65 - 4.46	%	0.44 - 1.33	%
Expected annual dividend yield	0	%	0	%

The weighted-average valuation assumptions were determined as follows:

- Expected stock price volatility: The expected volatility is based on historical volatility of our stock price.
- Expected term of options: We estimated the expected term of our stock options with service-based vesting using the “simplified” method, as prescribed in SAB No. 107, whereby the expected life equals the average of the vesting tranches and the original contractual term of the option due to our lack of sufficient historical data.
- Risk-free interest rate: We base the risk-free interest rate on the interest rate payable on U.S. Treasury securities in effect at the time of grant for a period that is commensurate with the assumed expected option term.

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- Expected annual dividend yield: The estimated annual dividend yield is 0% because we have not historically paid, and do not expect for the foreseeable future to pay, a dividend on our common stock.

As of December 31, 2022, there was $19.0 million of total unrecognized compensation expense related to unvested stock options. That expense is expected to be recognized over the next four years as follows, in thousands:

2023	$	10,054
2024		6,627
2025		1,974
2026		316
	$	18,971

Restricted Stock

All issued and outstanding restricted shares of common stock are time-based and become vested within two years after the grant date, pursuant to the 2014 Plan. Compensation expense is recorded ratably over the requisite service period. Compensation expense related to restricted stock is measured based on the fair value using the closing market price of our common stock on the date of the grant. As of December 31, 2022, we had 7,500 restricted shares of common stock outstanding.

During the year ended December 31, 2022, we granted 809,028 restricted share units, which vest within four years of the grant date, pursuant to the 2014 Plan. As of December 31, 2022, we had 664,476 restricted stock units outstanding.

A summary of activity for the years ended December 31, 2022 and 2021 is presented below:

	Shares	Weighted-average Grant Date Fair Value per Share
Outstanding—December 31, 2020	24,625	$ 11.41
Granted	18,400	13.48
Vested	(17,000)	12.93
Outstanding—December 31, 2021	26,025	12.75
Granted	809,028	8.49
Vested	(37,450)	11.19
Forfeited	(125,627)	9.04
Outstanding—December 31, 2022	671,976	$ 8.39

As of December 31, 2022, there was $4.2 million in unrecognized compensation cost related to unvested restricted stock and restricted stock units.

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Total compensation cost recognized for all restricted stock awards and restricted stock units in the statements of operations for the years ended December 31, 2022 and 2021 is as follows (in thousands):

	Year Ended December 31,	
	2022	2021
Research and development	$ 624	$ 3
Selling, general and administrative	1,097	276
Total	$ 1,721	$ 279

Stock Issued in Connection with Ovid License Agreement

On March 29, 2022, pursuant to an exclusive patent license agreement with Ovid Therapeutics Inc. (Ovid), we issued 123,255 shares of our common stock to Ovid. The shares were issued in reliance upon the exemption from the registration requirements of the Securities Act of 1933, as amended (Securities Act) provided by Section 4(a)(2) of the Securities Act and Regulation D thereunder as sales by an issuer not involving any public offering (see Part II, Item 2. *Unregistered Sales of Equity Securities and Use of Proceeds*). The fair value of these shares is reflected in operating expenses for the year ended December 31, 2022.

Underwritten Public Offering

In connection with an underwritten public offering in November 2022 and the closing of the related exercise of the underwriters' option in December 2022, we issued a total of 12,421,053 shares of common stock and 2,105,264 pre-funded warrants resulting in aggregate net proceeds, after underwriting discounts and commissions in the public offering and fees, of $64.5 million. The exercise price and the number of Common Shares issuable upon exercise of each Pre-Funded Warrant (the Warrant Shares) are subject to appropriate adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the Common Shares as well as upon any distribution of assets, including cash, stock or other property, to our stockholders. The Pre-funded Warrants are exercisable at any time, will not expire and are exercisable in cash or by means of a cashless exercise.

A holder of Pre-funded Warrants may not exercise such Pre-funded Warrants if the holder, together with its affiliates, would beneficially own more than 9.99% of the number of Common Shares outstanding immediately after giving effect to such exercise. A holder of Pre-funded Warrants may increase or decrease this percentage not in excess of 19.99% by providing at least 61 days' prior notice to us.

8. Convertible Preferred Stock

Concurrent with a 2019 public offering, we entered into a Securities Purchase Agreement (Purchase Agreement), by and among us and the investors listed therein. Pursuant to the terms of the Purchase Agreement, we sold to the investors an aggregate of 30,000 shares of Series A Participating Convertible Preferred Stock, par value $0.001 per share (Series A Preferred Stock), at a per share price of $1,000 in a private placement (Private Placement), and received net proceeds of $28.2 million, after deducting underwriting discounts and commissions of $1.8 million. Each share of Series A Preferred Stock is convertible into 200 shares of common stock, reflecting a conversion price equal to $5.00 per share, subject to customary anti-dilution adjustments.

During the year ended December 31, 2022, 275 shares of our Series A Preferred Stock converted into 55,000 shares of our common stock, pursuant to the terms of the Purchase Agreement. During the year ended December 31, 2021, 178 shares of our Series A Preferred Stock converted into 35,600 shares of our common stock, pursuant to the

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terms of the Purchase Agreement. As of December 31, 2022, 4,300 shares of our Series A Preferred Stock remained outstanding, convertible into 860,000 shares of our common stock.

The holders of the Series A Preferred Stock have the right to receive discretionary dividends paid to common shareholders. Except as required by law, the Series A Preferred Stock is non-voting stock. The holders of the Series A Preferred Stock each have a beneficial ownership limitation of 9.99% of total outstanding shares of common stock, including an option for the holder to increase this percentage to 19.99%.

9. Notes Payable

On May 11, 2021 (Closing Date) and as amended on May 17, 2021, May 23, 2022 and October 28, 2022 (Credit Agreement), we entered into the Credit Agreement with Oaktree Fund Administration, LLC as administrative agent (Oaktree) and the lenders party thereto (collectively, the Lenders) that provides for a five-year senior secured term loan facility in an aggregate original principal amount of up to $100.0 million, available to us in four tranches (collectively, the Term Loans).

Upon entering into the Credit Agreement in May 2021, we borrowed $15.0 million in term loans from the Lenders (Tranche A-1 Term Loans); upon receipt of written acceptance by the FDA of our NDA filing relating to the use of ganaxolone in CDD in September 2021, we borrowed $30.0 million of tranche A-2 term loans from the Lenders (Tranche A-2 Term Loans); and in March 2022, we borrowed $30.0 million in term loans from the Lenders that became available as a result of the approval by the FDA of ZTALMY oral suspension for the treatment of seizures associated with CDD in patients two years of age and older (Tranche B Term Loans). In May 2022, we entered into an amendment to extend the commitment date for the tranche C term loans (Tranche C Term Loans) commitment from June 30, 2023 to December 31, 2023, and to eliminate the commitment fees associated with the Tranche C Term Loans. Also in May 2022, we delivered to Oaktree a separate notice of commitment termination with respect to the tranche D term loans (Tranche D Term Loans) commitment. In October 2022, we entered into an amendment to, among other things, allow for the consummation of the Revenue Interest Financing Agreement with Sagard and the transactions thereunder. In addition, the Credit Agreement Amendment increases the exit fee due by us upon any repayment, whether as a prepayment or a scheduled repayment, of the principal of the loans under the Credit Agreement from 2.00% to 2.67%. Under the terms of the Credit Agreement, we may, at our sole discretion, borrow from the Lenders up to an additional $25.0 million in term loans subject to certain milestone events, as follows:

- Through December 31, 2023, $25.0 million of tranche C term loans will be available for draw if we complete one or more financings (including through the issuance of common stock, convertible debt, subordinated debt, a synthetic royalty, or a sublicense) resulting in gross proceeds to us of at least $40.0 million and net proceeds to us of at least $36.0 million (Qualified Financing Condition). However, the availability of this tranche is also subject to either our current Phase 3 trial in RSE or a Phase 3 trial in TSC achieving statistical significance (p value < 0.05) across all primary endpoints and ganaxolone must be generally well tolerated, with a safety profile generally consistent with previous clinical trials.

We satisfied the Qualified Financing Condition in connection with our November 2022 underwritten public offering, however, if we are unable to satisfy the remaining condition, we would not be able to draw down the remaining tranche of loans and may not be able to obtain alternative financing on commercially reasonable terms or at all. In addition, the Credit Agreement contains a minimum liquidity covenant that requires us to maintain cash and cash equivalents of at least $15.0 million from the funding date of the tranche B term loans until the maturity of the Term Loans.

The Term Loans will be guaranteed by certain of our future subsidiaries (Guarantors). Our obligations under the Credit Agreement are secured by a pledge of substantially all of our assets and will be secured by a pledge of substantially all of the assets of the Guarantors.

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The Term Loans mature on May 11, 2026 (Maturity Date). The Term Loans bear interest at a fixed per annum rate (subject to increase during an event of default) of 11.50%, and we are required to make quarterly interest payments until the Maturity Date. We are also required to make quarterly principal payments beginning on June 30, 2024 in an amount equal to 5.0% of the aggregate amount of the Term Loans outstanding on June 30, 2024, and continuing until the Maturity Date. On the Maturity Date, we are required to pay in full all outstanding Term Loans and other amounts owed under the Credit Agreement.

At the time of borrowing any tranche of the Term Loans, we are required to pay an upfront fee of 2.0% of the aggregate principal amount borrowed at that time. In addition, a commitment fee of 75 basis points per annum began to accrue on each of the tranche B, C, and D commitments for the period beginning 120 days after the funding date of the tranche A-2 term loans and continued until the applicable tranche was either funded or terminated, at which time the related commitment fees were due. The Tranche A-2 Term Loans were funded on September 27, 2021, and as such, we began accruing the commitment fees for tranche B, C, and D Term Loans 120 days later, on January 25, 2022. We drew down the additional $30.0 million of Tranche B Term Loans in March 2022, and paid less than $0.1 million in commitment fees related to Tranche B Term Loans. The May 2022 amendment eliminated the commitment fees related to the Tranche C Term Loans, and separately, we terminated the Tranche D Term Loans in May 2022.

We may prepay all or any portion of the Term Loans, and are required to make mandatory prepayments of the Term Loans from the proceeds of asset sales, casualty and condemnation events, and prohibited debt issuances, subject to certain exceptions. All mandatory and voluntary prepayments of the Term Loans are subject to prepayment premiums equal to (i) 4% of the principal prepaid plus a "make-whole" amount equal to the interest that would have accrued through May 11, 2023 if prepayment occurs on or before May 11, 2023, (ii) 4% of the principal prepaid if prepayment occurs after May 11, 2023 but on or before May 11, 2024, or (iii) 2% of the principal prepaid if prepayment occurs after May 11, 2024 but on or before May 11, 2025. If prepayment occurs after May 11, 2025, no prepayment premium is due.

We are also required to make mandatory prepayments of the Term Loans upon an event of default under the Credit Agreement resulting from the occurrence of a change of control. These mandatory prepayments are subject to a prepayment premium equal to (i) 12.5% of the principal prepaid if such prepayment occurs on or before May 11, 2022 or (ii) 10.0% of the principal prepaid if the prepayment occurs after May 11, 2022 but on or before May 11, 2023.

In addition, we are required to pay an exit fee in an amount equal to 2.67% of all principal repaid, whether as a mandatory prepayment, voluntary prepayment, or a scheduled repayment. Prior to the October 28, 2022 amendment to the Credit Agreement, the exit fee was 2.0%. The increase in the exit fee resulted in an additional $0.5 million of debt issuance costs that are classified as a contra-liability on the consolidated balance sheets and is being recognized as interest expense over the term of the loan using the effective interest method. In addition, we paid $0.2 million in fees, which were recorded as debt issuance costs for the year ended December 31, 2022.

In addition to the minimum liquidity covenant, we are subject to a number of affirmative and restrictive covenants under the Credit Agreement, including limitations on its ability and its subsidiaries' abilities, among other things, to incur additional debt, grant or permit additional liens, make investments and acquisitions, merge or consolidate with others, dispose of assets, pay dividends and distributions, and enter into affiliate transactions, subject to certain exceptions. As of December 31, 2022, we were in compliance with all covenants.

Upon the occurrence of certain events, including but not limited to our failure to satisfy our payment obligations under the Credit Agreement, the breach of certain of our other covenants under the Credit Agreement, the occurrence of cross defaults to other indebtedness, or defaults related to enforcement action by the FDA or other Regulatory Authority or recall of ganaxolone, Oaktree and the Lenders will have the right, among other remedies, to accelerate all amounts outstanding under the Term Loans and declare all principal, interest, and outstanding fees immediately due and payable.

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In March 2022, we borrowed $30.0 million upon the approval by the FDA of ZTALMY for CDD and incurred debt issuance costs of $1.8 million, including the exit fee of $0.6 million, that are classified as contra-liabilities on our consolidated balance sheets and are being recognized as interest expenses over the term of the loan using the effective interest method.

In September 2021, we borrowed $30.0 million upon receipt of written acceptance by the FDA of our NDA filing relating to the use of ganaxolone in the treatment of CDD and incurred debt issuance costs of $1.2 million, including the exit fee of $0.6 million, that are classified as contra-liability on our consolidated balance sheets and are being recognized as interest expense over the term of the loan using the effective-interest method.

In May 2021, we borrowed $15.0 million upon entering into the Credit Agreement and incurred debt issuance costs of $4.4 million, including the exit fee of $0.3 million, that are classified as a contra-liability on the consolidated balance sheet and are being recognized as interest expense over the term of the loan using the effective-interest method.

For the year ended December 31, 2022, we recognized interest expense of $9.5 million, of which $7.9 million was cash interest paid on the Term Loans and $1.6 million was non-cash interest expense related to the amortization of debt issuance costs. For the year ended December 31, 2021, we recognized interest expense of $2.6 million, of which $2.0 million was cash interest paid on the Term Loans and $0.6 million was non-cash interest expense related to the amortization of debt issuance costs.

The following table summarizes the composition of Notes payable as reflected on the consolidated balance sheet as of December 31, 2022 (in thousands):

Gross proceeds	$	75,000
Contractual exit fee		2,003
Unamortized debt discount and issuance costs		(5,985)
Total	$	71,018

The aggregate maturities of Notes payable as of December 31, 2022 are as follows (in thousands):

2023	$	—
2024		11,250
2025		15,000
2026		48,750
Total	$	75,000

10. Revenue Interest Financing Payable

On October 28, 2022 (Closing Date), we entered into a revenue interest financing agreement (Revenue Interest Financing Agreement) with Sagard Healthcare Royalty Partners, LP (Sagard) pursuant to which we received $32.5 million (Investment Amount) to provide funding for our development and commercialization of ganaxolone and related pharmaceutical products, including the commercial launch of ZTALMY, and for working capital and general administrative purposes.

In exchange for the Investment Amount, we have agreed to make quarterly payments to Sagard (Payments) as follows: (i) for each calendar quarter from and after the Closing Date through and including the quarter ended June 30, 2026, an amount equal to 7.5% of (a) our U.S. net sales of ZTALMY and all other pharmaceutical products that contain ganaxolone (Net Sales), in each case with any dosage form, dosing regimen, or strength, or any improvements related thereto (collectively, Included Products) and (b) certain other payments received by us in connection with the manufacture, development and sale of the Included Products in the U.S. (Other Included Payments, and, together with

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Net Sales, Product Revenue); and (ii) for each calendar quarter following the calendar quarter ended June 30, 2026, an amount equal to (x) 15.0% of the first $100 million in annual Product Revenue of the Included Products and (y) 7.5% of annual Product Revenue of the Included Products in excess of $100 million.

The Payments are subject to a hard cap equal to 190% of the Investment Amount (Hard Cap) or $61.8 million. Sagard's right to receive payments will terminate when Sagard has received payments in respect of the Included Products, including any additional payments described below, equal to the Hard Cap. Further, we have the right to make voluntary prepayments to Sagard, and such payments will be credited against the Hard Cap.

If Sagard has not received aggregate payments equaling at least 100% of the Investment Amount by December 31, 2027 or at least 190% of the Investment Amount by December 31, 2032 (each, a Minimum Amount), then we will be obligated to make a cash payment to Sagard in an amount sufficient to gross up Sagard up to the applicable Minimum Amount within a specified period of time after each reference date.

The obligations under the Revenue Interest Financing Agreement, including the Payments, will be guaranteed by certain of our future subsidiaries that are required to become a party thereto as guarantors (Guarantors). Our obligations under the Revenue Interest Financing Agreement and the guarantee of such obligations are secured, subject to customary permitted liens and other agreed upon exceptions and subject to an intercreditor agreement with Oaktree as administrative agent for the lenders under our credit agreement (as described below, the Credit Agreement), by a pledge of substantially all of our and the Guarantors' assets that relate to, or are used or held for use for, the development, manufacture, use and/or commercialization of ZTALMY and all other pharmaceutical products that contain ganaxolone in the U.S., including the Product Revenue, pursuant to the terms of the Security Agreement dated as of the Closing Date by and among us, the Guarantors from time to time party thereto, and Sagard (Security Agreement).

At any time, we have the right, but not the obligation (Call Option), to repurchase all, but not less than all, of Sagard's interest in the Payments at a repurchase price (Put/Call Price) equal to: (a) on or before the third anniversary of the Closing Date, 160% of the Investment Amount; (b) after the third anniversary but on or prior to the fourth anniversary of the Closing Date, 180% of the Investment Amount; and (c) after the fourth anniversary of the Closing Date, 190% of the Investment Amount, in each case, less the aggregate of all of our payments in respect of the Payments made to Sagard prior to such date.

The Revenue Interest Financing Agreement contains certain restrictions on our and our subsidiaries' abilities, among other things, to incur additional debt, grant or permit additional liens, make investments and acquisitions, dispose of assets, pay dividends and distributions and enter into affiliate transactions, in each case, subject to certain exceptions. In addition, the Revenue Interest Financing Agreement contains a financial covenant that requires us to maintain at all times cash and cash equivalents in certain deposit accounts in an amount at least equal to (i) from the Closing Date until the repayment of the loans under the Credit Agreement, $15.0 million and (ii) thereafter, $10.0 million.

In connection with the Revenue Interest Financing Agreement, on the Closing Date, we entered into the Credit Agreement Amendment with Oaktree which is fully described in Note 9.

Issuance costs pursuant to the Revenue Interest Financing Agreement consisted primarily of advisory and legal fees and totaled $2.6 million. These issuance costs were recorded as a direct deduction to the carrying amount of the liability and will be amortized under the effective interest method over the estimated period the liability will be repaid. For the year ended December 31, 2022, we estimated an effective annual interest rate of approximately 19%. Over the course of the Revenue Interest Financing Agreement, the actual interest rate will be affected by the amount and timing of net ZTALMY revenue recognized and changes in the timing of forecasted net ZTALMY revenue. On a quarterly basis, we will reassess the expected timing of the net ZTALMY revenue, recalculate the amortization and effective interest rate and adjust the accounting prospectively as needed.

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The following table summarizes the activity of the Revenue Interest Financing Agreement for the year ended December 31, 2022 (in thousands):

Revenue Interest Financing Agreement upon October 2022 closing	$	32,500
Issuance costs		(2,579)
Non-cash interest expense		956
Amortization of debt discount		42
Payments made in the year ended December 31, 2022		(42)
Balance at December 31, 2022	$	30,877
Current portion of revenue interest financing liability		1,020
Long-term portion of revenue interest financing liability	$	29,857

11. License and Collaboration Revenue

Orion Collaboration Agreement

In July 2021, we entered into a collaboration agreement (Orion Collaboration Agreement) with Orion. The Orion Collaboration Agreement falls under the scope of ASC Topic 808, Collaborative Arrangements (ASC 808) as both parties are active participants in the arrangement that are exposed to significant risks and rewards. While this arrangement is in the scope of ASC 808, we analogize to ASC 606 for some aspects of this arrangement, including for the delivery of a good or service (i.e., a unit of account). Revenue recognized by analogizing to ASC 606 is recorded as collaboration revenue on the consolidated statements of operations.

Under the terms of the Orion Collaboration Agreement, we granted Orion an exclusive, royalty-bearing, sublicensable license to certain of our intellectual property rights with respect to commercializing biopharmaceutical products incorporating our product candidate ganaxolone (Licensed Products) in the European Economic Area, the United Kingdom and Switzerland (collectively, the Territory) for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions (Field), initially in the indications of CDD, TSC and RSE. We will be responsible for the continued development of Licensed Products and regulatory interactions related thereto, including conducting and sponsoring all clinical trials, provided that Orion may conduct certain post-approval studies in the Territory. Orion will be responsible, at Orion's sole cost and expense, for the commercialization of any Licensed Product in the Field in the Territory.

Under the terms of the Orion Collaboration Agreement, we received a €25.0 million ($29.6 million) upfront payment from Orion in July 2021. In connection with the upfront fee, we agreed to provide Orion with the results of a planned genotoxicity study on the M2 metabolite of ganaxolone, a "Combined Micronucleus & Comet study in vivo." In May 2022, the final study report was received, which confirmed that no genotoxicity was found, as measured by formation of micronuclei in the bone marrow or comet morphology in the liver. In the event that the results of such study were positive, based on the criteria set forth in the study's protocol, Orion would have had the right to terminate the Collaboration Agreement within ninety (90) days after its receipt of the final report of such study, in which case we would have been required to refund Orion seventy-five percent (75%) of the upfront fee. We are eligible to receive up to an additional €97 million in R&D reimbursement and cash milestone payments based on specific clinical and commercial achievements, as well as tiered royalty payments based on net sales ranging from the low double-digits to high teens for the oral programs and the low double-digits to low 20s for the IV program. Also, as part of the overall arrangement, we have agreed to supply the Licensed Products to Orion at an agreed upon price.

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The Orion Collaboration Agreement shall remain effective until the date of expiration of the last to expire Royalty Term, which is defined as the period beginning on the date of the first commercial sale Licensed Product in such country and ending on the latest to occur of (a) the tenth (10th) anniversary of the first commercial sale of Licensed Product in such country, (b) the expiration of the last-to-expire licensed patent covering the manufacture, use or sale of such Licensed Product in such country, and (c) the expiration of regulatory exclusivity period, if any, for such Licensed Product in such country. The Orion Collaboration Agreement has a term of at least ten (10) years since a commercial sale has yet to occur. The Orion Collaboration Agreement allows for termination in certain specific events, such as material breach, in the event Orion challenges the validity, enforceability or scope of the licensed patent rights, termination for forecast failure, insolvency and force majeure, none of which are probable at contract inception.

In accordance with the guidance, we identified the following commitments under the arrangement: (i) exclusive rights to develop, use, sell, have sold, offer for sale and import any product comprised of Licensed Product (License) (ii) development and regulatory activities (Development and Regulatory Activities), and (iii) requirement to supply Orion with the Licensed Product at an agreed upon price (Supply of Licensed Product). We determined that these three commitments represent distinct performance obligations for purposes of recognizing revenue or reducing expense, which we will recognize such revenue or expense, as applicable, as we fulfill these performance obligations.

At contract inception, we determined that the non-refundable portion of the upfront payment plus the research and development reimbursement constitutes the transaction price as of the outset of the Orion Collaboration Agreement. The refundable portion of the upfront payment and the future potential regulatory and development milestone payments were fully constrained at contract inception as the risk of significant revenue reversal related to these amounts had not yet been resolved. During the year ended December 31, 2022, the refundable portion of the upfront payment was determined to be included in the transaction price as the final genotoxicity study on the M2 metabolite of ganaxolone was received as described above. The achievement of the future potential milestones is not within our control and is subject to certain research and development success and therefore carry significant uncertainty. We will reevaluate the likelihood of achieving these milestones at the end of each reporting period and adjust the transaction price in the period the risk is resolved. In addition, we will recognize any consideration related to sales-based milestones and royalties when the subsequent sales occur since those payments relate primarily to the License, which was delivered by us to Orion upon entering into the Orion Collaboration Agreement. We recorded $12.7 million of the $21.2 million refundable portion of the upfront payment as collaboration revenue in the year ended December 31, 2022, which, along with the transaction price allocation, relieved the refund liability reported at December 31, 2021.

The transaction price was allocated to the three performance obligations based on the estimated stand-alone selling prices at contract inception. The stand-alone selling price of the License was based on a discounted cash flow approach and considered several factors including, but not limited to, discount rate, development timeline, regulatory risks, estimated market demand and future revenue potential using an adjusted market approach. The stand-alone selling price of the Development and Regulatory Activities and the Supply of Licensed Product was estimated using the expected cost-plus margin approach.

As of the agreement date in July 2021, we allocated the transaction price to the performance obligations as described below and recorded the $9.0 million transaction price associated with the License as Revenue. During 2021, we amortized $0.1 million of the transaction price associated with the Development and Regulatory Services as a reduction of research and development costs. These reductions to transaction price resulted in a total contract liability of $6.6 million at December 31, 2021. In accordance with ASC 210-20, the contract liability of $6.6 million is offset by a contract asset of $7.2 million related to the reimbursement of research and development costs, resulting in a net Contract asset of $0.6 million at December 31, 2021.

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Transaction Price and Net Contract Asset at December 31, 2021 (in thousands):

	Transaction Price	Cumulative Collaboration Revenue Recognized as of December 31, 2021	Contract Liability
License	$ 8,987	$ 8,987	$ -
Development and Regulatory Services	2,787	106	2,681
Supply of Licensed Product	3,943	-	3,943
	$ 15,717	$ 9,093	6,624
Less Total Contract Asset			7,181
Net Contract Asset			$ 557

During the year ended December 31, 2022, the refundable portion of the upfront payment was determined to be included in the transaction price as the final genotoxicity study on the M2 metabolite of ganaxolone was received as described above. As such, the refundable portion of the upfront payment of €18.8 million ($21.2 million) was allocated to the purchase price as shown below, resulting in a total purchase price of $37.9 million. Of the $21.2 million refundable portion of the upfront payment, we recorded $12.7 million of collaboration revenue in the year ended December 31, 2022. During the year ended December 31, 2022, we amortized $1.1 million of the transaction price associated with the Development and Regulatory Services as a reduction of research and development costs. These reductions to the transaction price resulted in a total contract liability of $15.1 million at December 31, 2022. In accordance with ASC 210-20, the contract liability of $15.1 million is offset by a contract asset of $5.1 million related to the reimbursement of research and development costs, resulting in a net contract liability of $10.0 million at December 31, 2022.

Transaction Price and Net Contract Asset at December 31, 2022 (in thousands):

	Transaction Price	Cumulative Collaboration Revenue Recognized as of December 31, 2022	Contract Liability
License	$ 21,660	$ 21,660	$ -
Development and Regulatory Services	6,717	1,158	5,559
Supply of Licensed Product	9,503	-	9,503
	$ 37,880	$ 22,818	15,062
Less Total Contract Asset			5,079
Net Contract Liability			$ 9,983

In 2021, we incurred $2.0 million of incremental costs in obtaining the Orion Collaboration Agreement. These contract acquisition costs were allocated consistent with the transaction price, resulting in $1.1 million of expense recorded to General and administrative expense commensurate with the recognition of the License performance obligation and $0.9 million recorded as capitalized contract costs, included in Other current assets and Other assets, which are being amortized as Development and Regulatory Services and Supply of License Product obligations are met. Cost of collaboration revenue of $1.5 million in the year ended December 31, 2021 represents a one-time fee paid to Purdue Neuroscience Company (Purdue) related to that certain license agreement entered into in September 2004, and subsequently amended and restated in May 2008, between us and Purdue, and was paid in conjunction with the Orion Collaboration Agreement.

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We reevaluate the transaction price and the total estimated costs expected to be incurred to satisfy the performance obligations and adjust the deferred revenue at the end of each reporting period. Such changes will result in a change to the amount of collaboration revenue recognized and deferred revenue.

Tenacia Collaboration Agreement

On November 16, 2022 (Effective Date), we entered into a Collaboration and Supply Agreement (Tenacia Collaboration Agreement) with Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia). The Tenacia Collaboration Agreement falls under the scope of ASC Topic 808, Collaborative Arrangements (ASC 808) as both parties are active participants in the arrangement that are exposed to significant risks and rewards. While this arrangement is in the scope of ASC 808, we analogize to ASC 606 for some aspects of this arrangement, including for the delivery of a good or service (i.e., a unit of account). Revenue recognized by analogizing to ASC 606 is recorded as collaboration revenue on the consolidated statements of operations.

Under the terms of the Tenacia Collaboration Agreement, we granted Tenacia an exclusive, royalty-bearing, sublicensable license to certain of our intellectual property rights to develop, commercialize and otherwise exploit certain products incorporating certain oral and intravenous formulations of our product candidate ganaxolone (Licensed Products) in Mainland China, Hong Kong, Macau and Taiwan (collectively, Territory) for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions (Field), initially for the treatment of cyclin-dependent kinase-like 5 deficiency disorder, tuberous sclerosis complex and SE (including refractory and established SE) (collectively, the Initial Indications). The collaboration can be expanded to include additional indications and formulations of ganaxolone pursuant to a right of first negotiation.

Under the terms of the Tenacia Collaboration Agreement, Tenacia agreed to pay us an upfront cash payment of $10 million (the Upfront Fee) within forty-five (45) days after the Effective Date, which was paid in December 2022. In addition to the Upfront Fee, Tenacia has agreed to make cash payments to us upon the achievement of certain development, regulatory and sales-based milestones related to (i) the Initial Indications and (ii) the first new formulation or pro-drug of ganaxolone or any back-up compound of ganaxolone in a new indication (Selected Product) for which the parties amend the Collaboration Agreement in connection with Tenacia's exercise of its right of first negotiation and for which there is no other Licensed Product approved in China (for clarity, the milestone payments under this clause (ii) will only apply to one Selected Product), up to an aggregate amount of $256 million. Of the milestones, $15 million relates to regulatory approvals with separate milestones related to each of oral and intravenous formulations and the Selected Product, and an aggregate of $241 million of sales-based milestones are connected to annual revenue thresholds specific to each of the oral, intravenous and Selected Product formulations of ganaxolone. Tenacia has further agreed to pay us tiered royalty payments based on annual net sales of Licensed Products ranging from the low double digits to the mid-teens for each of the oral formulation, intravenous formulation and Selected Product formulation of Licensed Products. Tenacia's obligations to pay royalties to us with respect to sales of a Licensed Product in each particular jurisdiction of the Territory will commence on the date of first commercial sale in such jurisdiction and expire upon the latest of (i) ten years following the first commercial sale of such Licensed Product in such jurisdiction, (ii) the expiration of the last-to-expire valid claim of any licensed patent rights that covers such Licensed Product in such jurisdiction and (iii) the expiration of all regulatory exclusivities for such Licensed Product in such jurisdiction. Royalty payments are subject to reduction in specified circumstances as set forth in the Collaboration Agreement, including if net sales decrease by a certain percentage after the introduction of a generic product.

Tenacia will be primarily responsible for the development of Licensed Products in the Territory and regulatory interactions related thereto, including conducting and sponsoring clinical studies in the Field in the Territory to support regulatory filings in the Territory. All regulatory approvals filed by Tenacia in the Territory will be in the name of and owned by us unless otherwise required by applicable law, in which case such regulatory approvals would be in the name of and owned by Tenacia for the benefit of the us. We and Tenacia have agreed to enter into clinical and commercial supply agreements pursuant to which we will supply Tenacia with its requirements of Licensed Products necessary for

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Tenacia to develop and commercialize Licensed Products in the Field in the Territory. The parties agreed to enter into the clinical supply agreement no later than ninety (90) days after the Effective Date and the commercial supply agreement no later than twelve (12) months prior to Tenacia's good faith estimate of the date of first commercial sale of the Licensed Product. The parties agreed that the commercial supply agreement would include a commercial supply price adjustment mechanism pursuant to the terms set forth in the Collaboration Agreement. The supply agreements are also expected to contain delivery, acceptance, payment, termination, forecasting, and other terms consistent with the Collaboration Agreement, as well as certain quality assurance, indemnification, liability and other standard industry terms. Tenacia will be responsible for, at Tenacia's sole cost and expense, obtaining regulatory approval and commercializing the Licensed Product in the Field in Mainland China.

The term of the Collaboration Agreement extends for so long as royalties are payable anywhere in the Territory. Subject to the terms of the Collaboration Agreement, (i) for a specified period of time after the Effective Date, Tenacia may terminate the Collaboration Agreement in its entirety for any or no reason upon written notice to us, and (ii) either party may terminate the Collaboration Agreement for the other party's material breach following a cure period or insolvency.

In accordance with the guidance, we identified the following commitments under the arrangement: (i) grant to Tenacia the exclusive rights to develop, commercialize and otherwise exploit Licensed Product in the Field in the Territory (License) and (ii) requirement to supply Tenacia with the Licensed Product at an agreed upon price (Supply of Licensed Product). We determined that these two commitments represent distinct performance obligations for purposes of recognizing revenue or reducing expense, which it will recognize such revenue or expense, as applicable, as it fulfills these performance obligations.

The transaction price was allocated to the two performance obligations based on the estimated stand-alone selling prices at contract inception. The stand-alone selling price of the License was based on a discounted cash flow approach and considered several factors including, but not limited to, discount rate, development timeline, regulatory risks, estimated market demand and future revenue potential using an adjusted market approach. The stand-alone selling price of the Supply of Licensed Product was estimated using the expected cost-plus margin approach.

As of the agreement date in November 2022, we allocated the transaction price to the performance obligations as described below and recorded the $3.0 million transaction price associated with the License as revenue for the year ended December 31, 2022. This reduction to the transaction price resulted in a total contract liability of $7.0 million at December 31, 2022.

Transaction Price and Net Contract Liability at December 31, 2022:

	Transaction Price	Cumulative Collaboration Revenue Recognized as of December 31, 2022	Contract Liability
License	$ 2,998	$ 2,998	$ -
Supply of Licensed Product	7,002	-	7,002
Net Contract Liability	$ 10,000	$ 2,998	7,002
Less Total Contract Asset			700
Net Contract Liability			$ 6,302

We incurred $1.0 million of incremental costs in obtaining the Tenacia Collaboration Agreement. These contract acquisition costs were allocated consistent with the transaction price, resulting in $0.1 million of expense recorded to General and administrative expense and $0.2 million recorded to cost of collaboration revenue,

commensurate with the recognition of the License performance obligation, and $0.7 million recorded as capitalized contract costs, which will be amortized as Supply of License Product obligations are met.

We reevaluate the transaction price and the total estimated costs expected to be incurred to satisfy the performance obligations and adjust the deferred revenue at the end of each reporting period. Such changes will result in a change to the amount of collaboration revenue recognized and deferred revenue.

12. Commitments and Contingencies

Employee Benefit Plan

We maintain a Section 401(k) retirement plan for all employees. The plan allows employees to make contributions up to a specified percentage of their compensation, subject to maximum amounts allowed under law. On January 1, 2021, we began contributing 3% of compensation to each employee's 401(k) retirement account. We contributed $1.0 million and $0.7 million for the years ended December 31, 2022 and 2021, respectively. We also can make discretionary profit sharing contributions, which would vest over a period of four years from each employee's commencement of employment with us. We have not made any discretionary contributions.

License Agreements

We are obligated to pay royalties pursuant to a license agreement with Purdue Neuroscience Company (Purdue) as a percentage of net product sales for direct licensed products, such as ganaxolone. The obligation to pay royalties expires, on a country-by-country basis, 10 years from the first commercial sale of a licensed product in each country. The agreement also requires that we pay Purdue a percentage of the non-royalty consideration that we receive from a sublicensee and a percentage of milestone payments for indications other than seizure disorders and vascular migraine headaches not associated with mood disorders. Under the license agreement, we are committed to use commercially reasonable efforts to develop and commercialize at least one licensed product.

ZTALMY, our first approved product, was approved by the FDA in March 2022 for the treatment of seizures associated with CDD in patients 2 years of age and older and became available for commercial sale and shipment to patients with a prescription in the U.S. in the third quarter of 2022. We recorded net U.S. product revenue related to ZTALMY of $2.9 million in the year ended December 31, 2022. We paid royalty fees to Purdue in connection with the net product sales of ZTALMY in the year ended December 31, 2022. These fees were recorded as cost of product revenue in the year ended December 31, 2022.

In connection with the November 2022 execution of the Tenacia Collaboration Agreement, we paid to Purdue a portion of the upfront payment we received from Tenacia pursuant to the Purdue license agreement, a portion of which was recorded as a contract asset and a portion of which was recorded as cost of collaboration revenue for the year ended December 31, 2022. Details of the Tenacia Collaboration Agreement are more fully described in Note 11.

In March 2022, we entered into an exclusive patent license agreement (License Agreement) with Ovid Therapeutics Inc. (Ovid). Under the License Agreement, we have an exclusive, non-transferable (except as provided in the License Agreement), royalty-bearing, sublicensable license under certain of Ovid's patent(s) and patent applications to develop, make, have made, commercialize, promote, distribute, sell, offer for sale and import, ganaxolone, including any analogues or derivatives, including its salts, and pharmaceutical formulations of the foregoing (Licensed Products), in the U.S., the member states of the EU, Iceland, Lichtenstein, Norway, the United Kingdom, and Switzerland (Territory) for the treatment of CDD in humans (Field). Under the License Agreement, we have the sole right and responsibility for, and control over, all development, manufacturing, and commercialization activities, including all regulatory activities, with respect to the Licensed Products in the Field in the Territory. In addition, all regulatory approvals and related filings with respect to the Licensed Products in the Field in the Territory will be in the name of,

and be owned solely by, us. We were required, at Ovid's option exercisable in accordance with the License Agreement, to (i) pay to Ovid the sum of $1.5 million in cash; or (ii) issue to Ovid 123,255 shares of our common stock, which option to obtain shares of our common stock was exercisable within the five-business day period following the filing of our Annual Report on Form 10-K for the year ended December 31, 2021 on March 24, 2022. On March 29, 2022, we issued 123,255 shares of our common stock to Ovid, per Ovid's option in accordance with the License Agreement. As such, we recorded $1.2 million of IP license fee expenses related to the Ovid License Agreement in the year ended December 31, 2022. The License Agreement also provides for payment of royalties by us to Ovid in the low single digits on net sales by us, our affiliates and sublicensees, of Licensed Products in the Field in the Territory and are recorded as cost of product revenue. Such royalties are subject to reduction in the event of generic competition in accordance with the License Agreement. We may terminate the License Agreement at any time without cause on thirty days' prior written notice. Either party may terminate the License Agreement for the other party's material breach or insolvency subject to certain cure periods. Also, Ovid has the right to terminate the License Agreement if there has not been a first commercial sale of any Licensed Products in the Field in the Territory on or before June 30, 2025. In the event of termination, all licenses granted under the License Agreement will terminate.

In March 2017, we and CyDex Pharmaceuticals, Inc. (CyDex) entered into a License Agreement and a Supply Agreement. Under the terms of the License Agreement, CyDex has granted us an exclusive license to use sulfobutylether beta-cyclodextrin, CyDex's Captisol® drug formulation system, and related intellectual property in connection with the development and commercialization of ganaxolone in any and all therapeutic uses in humans, with some exceptions.

As consideration for this license, we paid an upfront fee which was recorded as research and development expense in 2017, and are required to make additional payments in the future upon achievement of various specified clinical and regulatory milestones. We will also be required to pay royalties to CyDex on sales of ganaxolone, if successfully developed, in the low-to-mid single digits based on levels of annual net sales. To date, we have achieved one milestone under the License Agreement, which occurred and was paid in the first quarter of 2021. Certain patents relating to Captisol®, including some that were licensed to us by CyDex, have expired, while other patents that are licensed to us remain in force.

Under the terms of the Supply Agreement, we are required to purchase all of our requirements for Captisol with respect to ganaxolone from CyDex, and CyDex is required to supply us with Captisol for such purposes, subject to certain limitations.

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13. Income Taxes

Income tax expense by jurisdiction as follows (in thousands):

	Year Ended December 31,	
	2022	2021
Current		
U.S. State	$ 2,445	—
Foreign	944	—
Total current	3,389	—
Deferred	—	—
Provision for income taxes	$ 3,389	$ —

Loss before income taxes is allocated as follows (in thousands):

	Year Ended December 31,	
	2022	2021
U.S. operations	$ 16,427	$ 98,776
Foreign operations	—	—
Loss before income taxes	$ 16,427	$ 98,776

As of December 31, 2022 and 2021, we had approximately $233.5 million and $303.3 million, respectively, of net operating loss (NOL) carry forwards available to offset future federal taxable income, $79.5 million of which will expire beginning in 2029, and the remaining amount can be carried forward indefinitely. As of December 31, 2022 and 2021, we had approximately $204.6 million and $253.0 million, respectively, of NOL carry forwards available to offset future state taxable income that will expire beginning in 2024. As of December 31, 2022, we also have federal research and development credit carryovers of approximately $30.2 million and state credit carryovers of approximately $0.4 million, which expire beginning in 2023.

The NOL carry forwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. NOL and tax credit carry forwards are subject to an annual limitation due to certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, as well as similar state tax provisions. This limits the amount of NOLs that we can utilize annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of our company immediately prior to an ownership change. Subsequent ownership changes may further affect the limitation in future years. In addition, U.S. tax laws limit the time during which these carry forwards may be applied against future taxes, therefore, we may not be able to take full advantage of these carry forwards for federal income tax purposes.

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The components of the net deferred tax asset are as follows (in thousands):

	December 31,	
	2022	2021
Gross deferred tax assets:		
Net operating loss carryforwards	$ 58,056	$ 81,719
Accrued expenses	124	272
Amortization of intangible	454	251
Stock-based compensation	8,091	5,944
Research and development and other credits and other carryforwards	30,627	16,162
Lease liability	440	610
Capitalized research and development expenses	22,382	11,437
Royalty/ Deferred Revenue	3,781	—
Capital Loss	94	6
Other	64	3
Total gross deferred tax assets	$ 124,113	$ 116,404
Gross deferred tax liabilities:		
ROU Asset	(291)	(399)
Depreciation	(224)	(99)
Unrealized income	(192)	(212)
Total gross deferred tax liabilities	(707)	(710)
Net deferred tax assets	123,406	115,694
Less: valuation allowance	(123,406)	(115,694)
Net deferred tax assets after valuation allowance	$ —	$ —

In assessing the realizability of deferred tax assets, management considers whether it is more likely than not that some portion or all of the deferred income tax assets will not be realized. The ultimate realization of deferred income tax assets is dependent upon the generation of future taxable income during the periods in which those temporary differences become deductible. Management considers the scheduled reversal of deferred income tax liabilities, projected future taxable income, and tax planning strategies in making this assessment. Based on consideration of these items, management has determined that enough uncertainty exists relative to the realization of the deferred income tax asset balances to warrant the application of a full valuation allowance as of December 31, 2022 and 2021. The valuation allowance increased by $7.7 million and $23.6 million during the years ended December 31, 2022 and 2021, respectively. The increase for the year ended December 31, 2022 was due primarily to our increase in research and development credits and capitalized research and development expenses, partially offset by a decrease in net operating losses. The increase for the year ended December 31, 2021 was due primarily to our increase in net operating loss carryovers and an increase in tax attributes.

We did not have unrecognized tax benefits as of December 31, 2022 and 2021, and do not expect this to change significantly over the next twelve months. We recognize tax positions in the financial statements only when it is more likely than not that the position will be sustained on examination by the relevant taxing authority based on the technical merits of the position. A position that meets this standard is measured at the largest amount of benefit that will more likely than not be realized on settlement. A liability is established for differences between positions taken in a tax return and amounts recognized in the financial statements. Accrued interest and penalties, where appropriate, are recorded in income tax expense. We did not have uncertain tax positions as of December 31, 2022 and 2021. As of December 31, 2022 and 2021, we had not accrued interest or penalties related to any uncertain tax positions.

The Tax Cuts and Jobs Act passed in 2017 included a provision which would require taxpayers to capitalize and amortize U.S.-based research & experimentation (R&E) expenses over a period of five years and non-U.S. R&E

expenses over 15 years effective for tax years beginning after December 31, 2021 pursuant to Internal Revenue Code Section 174. As a result of the capitalization of R&E expenses, income generated from the sale of the PRV, and limitations related to the utilization of state net operating losses, we have a current income tax expense of $2.4 million for the year ended December 31, 2022 attributable to state income taxes. We do not have a federal income tax liability for the year ended December 31, 2022 due to the utilization of our NOLs after taking into account Internal Revenue Code Section 382 limitations related to changes in ownership. Also included in our current income tax expense for the year ended December 31, 2022 is $0.9 million of China withholding tax incurred in connection with the Tenacia collaboration agreement.

A reconciliation of income tax (expense) benefit at the statutory federal income tax rate and income taxes as reflected in the financial statements is as follows:

	Year Ended December 31,	
	2022	2021
Federal income tax expense at statutory rate	21.0 %	21.0 %
Permanent items	(2.6)	(0.7)
State income tax, net of federal benefit	(9.0)	2.2
R&D tax credits	88.1	4.3
Change in state apportionment	(59.5)	(2.8)
Foreign withholding tax	(5.7)	—
Change in valuation allowance	(47.0)	(24.0)
Other	(5.9)	—
Effective income tax rate	(20.6)%	0.0 %

For all years through December 31, 2020, we generated research and development credits but have not conducted a study to document the qualified activities. This study may result in an adjustment to our research and development credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position for these years. A full valuation allowance has been provided against our research and development credits and, if an adjustment is required, this adjustment to the deferred tax asset established for the research and development credit carryforwards would be offset by an adjustment to the valuation allowance.

We file income tax returns in the U.S. and various state jurisdictions. The federal and state income tax returns are generally subject to tax examinations for the tax years ended December 31, 2019 through December 31, 2021. To the extent we have tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service or state tax authorities to the extent utilized in a future period.

Exhibit 10.5

EMPLOYMENT AGREEMENT

EMPLOYMENT AGREEMENT is effective on November 9, 2020, between Marinus Pharmaceuticals, Inc. (the "Company"), a Delaware corporation, and Christina Shafer (the "Employee").

Recital:

The parties desire to enter into this Agreement so as to provide for the employment of the Employee by the Company and for certain other matters in connection with such employment, all as set forth more fully in this Agreement.

NOW, THEREFORE, in consideration of the premises and covenants set forth herein, and intending to be legally bound hereby, the parties to this Agreement hereby agree as follows:

1. Duties. The Company agrees that the Employee shall be employed by the Company to serve as the Chief Commercial Officer, of the Company. The Employee shall report to the Chief Executive Officer of the Company (the "CEO"). The Employee agrees to be so employed by the Company and to devote his/her best efforts to advance the interests of the Company and to perform the duties customarily incident to the position of Chief Commercial Officer and such other duties assigned to the Employee by the CEO, provided such other duties are commensurate with the Employee's employment level at the Company.

2. Term. The Employee's employment under this Agreement shall continue in effect until terminated pursuant to Section 4 of this Agreement.

3. Compensation.

(a) Salary. During the term of the Employee's employment under this Agreement, the Employee shall be paid an annual salary at the rate of not less than $425,000 (the "Base Salary"). The Base Salary may be increased from time to time by the Board of Directors (the "Board"). The Board shall review the Base Salary at least annually at the end of each fiscal year of the Company. The Base Salary shall be paid in accordance with the Company's regular payroll practices.

(b) Annual Bonus. At the end of each fiscal year of the Company that ends during the term of this Agreement, the Board shall consider the award of a performance bonus to the Employee for such fiscal year in an amount of up to 40% of the Employee's Base Salary (the "Target Bonus") based upon the achievement of performance objectives established annually by the Board or its Compensation Committee. Whether the performance objectives for any year have been achieved by the Employee shall be determined by the Board or its Compensation Committee. Notwithstanding the foregoing, all bonuses shall be paid within two and one-half months after the close of each year.

(c) Sign-on Bonus. The Employee will receive a sign-on bonus of $40,000.00; to be paid 30 days after the first day of employment, less applicable taxes and withholding, paid in accordance with the Company's normal payroll practices.

(d) Equity Incentive Awards. On the date hereof, the Employee will be granted an inducement stock option award under the Company's 2014 Equity Incentive Plan, exercisable for the purchase of 128,000 shares of the Company's Common Stock, subject to the execution of a stock option agreement in the form approved by the Company. The exercise price of the stock option will be equal to the last reported sale price on the Nasdaq Global Market on the grant date. The stock option will vest 25% on the first anniversary and monthly thereafter in 36 substantially equal installments, provided that, no portion of the stock option that is not exercisable at the time of the Employee's termination of employment shall thereafter become exercisable. The Employee shall be eligible to participate in equity incentive programs established by the Company from time to time to provide stock options and other equity-based incentives to key employees of the Company in accordance with the terms of those programs.

An award of 10,000 Restricted Stock Units to be vested at 50% on the first anniversary of hire and 50% on the second anniversary of hire.

(e) Vacation and Fringe Benefits. The Employee shall be entitled to 20 days' paid vacation accrued monthly (1.66 days/month), plus Company holidays and two discretionary holidays and two personal days, as per Company policy. The Employee shall be entitled to participate in all insurance and other fringe benefit programs of the Company to the extent and on the same terms and conditions as are accorded to other officers and key employees of the Company.

(f) Reimbursement of Expenses. The Employee shall be reimbursed for all normal items of travel, entertainment and miscellaneous business expenses reasonably incurred by the Employee on behalf of the Company, provided that such expenses are documented and submitted in accordance with the reimbursement policies of the Company as in effect from time to time.

4. Termination.

(a) Death. This Agreement shall automatically terminate effective as of the date of the Employee's death, in which event the Company shall not have any further obligation or liability under this Agreement except that the Company shall pay to the Employee's estate: (i) any portion of the Employee's Base Salary for the period up to the Employee's date of death that has been earned but remains unpaid; and (ii) any benefits that have accrued to the Employee under the terms of the employee benefit plans of the Company, which benefits shall be paid in accordance with the terms of those plans.

(b) Total Disability. The Company may terminate the employment of the Employee immediately upon written notice to the Employee in the event of the Disability (as that term is hereinafter defined) of the Employee, in which event, the Company shall not have any further obligation or liability under this Agreement except that the Company shall pay to the Employee: (i) any portion of the Employee's Base Salary for the period up to the date of termination that has been earned but remains unpaid; and (ii) any benefits that have accrued to the Employee under the terms of the employee benefit plans of the Company, which benefits

shall be paid in accordance with the terms of those plans. For purposes of this Agreement, the term "Disability" shall mean an illness, incapacity or a mental or physical condition that renders the Employee unable or incompetent to carry out the job responsibilities that the Employee held or the tasks that the Employee was assigned at the time the disability commenced, as determined by the Board and supported by the opinion of a physician. The Employee shall fully cooperate with the physician retained to furnish such opinion, including submitting to such examinations and tests as may be requested by the physician.

(c) Termination by the Company for Cause. The Company may terminate the Employee's employment hereunder upon written notice to the Employee for any of the following reasons: (i) the Employee's misuse of alcoholic beverages, controlled substances or other narcotics, which misuse has had or is reasonably likely to have a material adverse effect on the business or financial affairs of the Company or the reputation of the Company; (ii) failure by the Employee to cooperate with the Company in any investigation or formal proceeding; (iii) the commission by the Employee of, or a plea by the Employee of guilty or *nolo contendere* with respect to, or conviction of the Employee for, a felony (or any lesser included offense or crime in exchange for withdrawal of a felony indictment or charged crime that might result in a penalty of incarceration), a crime involving moral turpitude, or any other offense that results in or could result in any prison sentence; (iv) adjudication as an incompetent; (v) a breach by the Employee of any material term of this Agreement, including the Employee's failure to faithfully, diligently and adequately perform the Employee's duties under this Agreement, that is not corrected within ten days after written notice from the Company, which notice shall set forth the nature of the breach; (vi) violation in any material respect of any of the Company's rules, regulations or
policies; (vii) gross insubordination by the Employee in the performance of the Employee's duties under this Agreement; (viii) engaging in any conduct, action or behavior that, in the reasonable opinion of the Company, has had a material adverse effect on the reputation of the Company or the Employee; (ix) any continued or repeated absence from the Company, unless the absence is approved or excused by the CEO or the result of the Employee's illness, disability or incapacity (in which event the provisions of Section 4(b) hereof shall control); or (x) misappropriation of any funds or property of the Company, theft, embezzlement or fraud. For the avoidance of doubt, "Cause" shall not mean a failure to achieve scientific goals, financial goals or forecasted timelines. In the event that the Company shall discharge the Employee pursuant to this Section 4(c), the Company shall not have any further obligation or liability under this Agreement, except that the Company shall pay to the Employee: (i) any portion of the
Employee's Base Salary for the period up to the date of termination that has been earned but remains unpaid; and (ii) any benefits that have accrued to the Employee under the terms of the employee benefit plans of the Company, which benefits shall be paid in accordance with the terms of those plans.

(d) Other Termination by the Company. The Company may terminate the employment of the Employee for any reason other than one specified in Section 4(b) or 4(c) hereof immediately upon written notice to the Employee, in which event the Employee shall be entitled to receive: (i) any portion of the Employee's Base Salary for the period up to the date of termination that has been earned but remains unpaid; (ii) any benefits that have accrued to the Employee under the terms of any employee benefit plans of the Company, which benefits shall be paid in accordance with the terms of those plans; and (iii) subject to the satisfaction of the provisions of Section 4(g) and the compliance by the Employee with all terms and provisions of

this Agreement that survive the termination of the Employee's employment by the Company, (A) the Employee's Base Salary for a period of nine months, less applicable taxes and withholdings, payable in accordance with the Company's regular payroll practices, with an accelerated payment of any balance upon the occurrence of a Change in Control; provided, however, that if such termination of employment shall occur within three months before or within twelve months after the occurrence of a Change in Control (such period being referred to herein as the "Change of Control Period"), the severance payable to the Employee shall be increased to an amount equal to the Employee's Base Salary for a period of eighteen months and be payable in a single lump sum payment, less applicable taxes and withholdings; (B) payment or reimbursement (upon presentation of proof of payment) of the Employee's medical insurance premiums at the same level as was in effect on the termination date for a period of nine months, which period shall increase to eighteen months if such termination of employment shall occur within the Change in Control Period; and (C) if such termination shall occur within the Change in Control Period, an amount equal to the Employee's Target Bonus for one year plus the Target Bonus for the year in which such employment termination shall occur prorated based on the relative number of days in such year during which the Employee was employed by the Company and/or its successor in the Change in Control, payable in a single lump sum payment, less applicable taxes and withholdings. Any severance payments and lump sum payments due hereunder shall commence as soon as administratively feasible within 60 days after the date of the Employee's termination of employment provided the Employee has timely executed and returned the Release referred to in Section 4(g) and, if a revocation period is applicable, the Employee has not revoked the Release; provided, however, that if the 60-day period begins in one calendar year and ends in a second calendar year, the severance payments shall begin to be paid in the second calendar year. On the date that severance payments commence, the Company will pay the Employee in a single lump sum payment, less applicable taxes and withholding, the severance payments that the Employee would have received on or prior to such date but for the delay imposed by the immediately preceding sentence, with the balance of the severance payments to be paid as originally scheduled.

(e) Termination by the Employee for Good Reason. The Employee may terminate the Employee's employment by providing written notice to the Company of a breach constituting Good Reason. "Good Reason" shall be deemed to exist with respect to any termination of employment by the Employee for any of the following reasons: (i) reassignment of the Employee to a location outside the Greater Philadelphia area; (ii) any material failure by the Company to comply with any material term of this Agreement; (iii) the demotion of the Employee to a lesser position than described in Section 1 hereof or a substantial diminution of the Employee's authority, duties or responsibilities as in effect on the date of this Agreement or as hereafter increased; or (iv) a material diminution of the Executive's Base Salary and benefits, in the aggregate, unless such reduction is part of a Company-wide reduction in compensation and/or benefits for all of its senior executives. If the Employee shall terminate the Employee's employment hereunder for Good Reason, the Employee shall be entitled to receive the same payments and benefits on the same terms and conditions as would be applicable upon a termination of the Employee's employment by the Company without Cause, as provided in Section 4(d) and subject to the satisfaction of the other provisions of this Section 4(e). The Employee may not resign with Good Reason pursuant to this Section 4(e), and shall not be considered to have done so for any purpose of this Agreement, unless (A) the Employee, within 60 days after the initial existence of the act or failure to act by the Company that constitutes

"Good Reason" within the meaning of this Agreement, provides the Company with written notice that describes, in particular detail, the act or failure to act that the Employee believes to constitute "Good Reason" and identifies the particular clause of this Section 4(e) that the Employee contends is applicable to such act or failure to act; (B) the Company, within 30 days after its receipt of such notice, fails or refuses to rescind such act or remedy such failure to act so as to eliminate "Good Reason" for the termination by the Employee of the Employee's employment relationship with the Company, and (C) the Employee actually resigns from the employ of the Company on or before that date that is six calendar months after the initial existence of the act or failure to act by the Company that constitutes "Good Reason." If the requirements of the preceding sentence are not fully satisfied on a timely basis, then the resignation by the Employee from the employ of the Company shall not be deemed to have been for "Good Reason," the Employee shall not be entitled to any of the benefits to which the Employee would have been entitled if the Employee had resigned from the employ of the Company for "Good Reason," and the Company shall not be required to pay any amount or provide any benefit that would otherwise have been due to the Employee under this Section 4(e) had the Employee resigned with "Good Reason."

(f) Other Termination by the Employee. The Employee may terminate the Employee's employment for any reason other than one specified in Section 4(e) upon at least 30 days' prior written notice to the Company, which notice shall specify the effective date of the termination. In the event the Employee shall terminate the Employee's employment pursuant to this Section 4(f), the Company shall not have any further obligation or liability under this Agreement, except that the Company shall pay to the Employee: (i) any portion of the Employee's Base Salary for the period up to the date of termination that has been earned but remains unpaid; and (ii) any benefits that have accrued to the Employee under the terms of the employee benefit plans of the Company, which benefits shall be paid in accordance with the terms of those plans.

(g) Execution of Release. The Employee shall not be entitled to any payments or benefits under Sections 4(d) or 4(e) unless the Employee executes and does not revoke a Release and Agreement (the "Release"), as drafted at the time of the Employee's termination of employment, including, but not limited to:

(i) an unconditional release of all rights to any claims, charges, complaints, grievances, known or unknown to the Employee, against the Company, its affiliates or assigns, through the date of the Employee's termination from employment other than post-termination payments and benefits pursuant to this Agreement;

(ii) a representation and warranty that the Employee has not filed or assigned any claims, charges, complaints, or grievances against the Company, its affiliates, or assigns;

(iii) an agreement not to use, disclose or make copies of any confidential information of the Company, as well as to return any such confidential information and property to the Company upon execution of the Release;

(iv) a mutual agreement to maintain the confidentiality of the Release or disclose the reasons for any termination of employment;

(v) an agreement not to disparage the Company or its officers, directors, stockholders, products or business; and

(vi) an agreement to indemnify the Company, or its affiliates or assigns, in the event that the Employee breaches any portion of this Agreement or the Release.

Notwithstanding any provision of this Agreement to the contrary, in no event shall the timing of the Employee's execution of the Release, directly or indirectly, result in the Employee designating the calendar year of payment, and if a payment that is subject to execution of the Release could be made in more than one taxable year, payment shall be made in the later taxable year.

(h) Definition of Change in Control. As used in this Agreement, the term "Change in Control" means:

(i) any merger or consolidation in which voting securities of the Company possessing more than 50% of the total combined voting power of the Company's outstanding securities are transferred to a person or persons different from the person holding those securities immediately prior to such transaction and the composition of the Board following such transaction is such that the directors of the Company prior to the transaction constitute less than 50% of the Board membership following the transaction;

(ii) any acquisition, directly or indirectly, by a person or related group of persons (other than the Company or a person that directly or indirectly controls, is controlled by, or is under common control with, the Company) of beneficial ownership of voting securities of the Company possessing more than 50% of the total combined voting power of the Company's outstanding securities; provided, however, that, no Change in Control shall be deemed to occur by reason of the acquisition of shares of the Company's capital stock by an investor or group of investors in the Company in a capital-raising transaction; or

(iii) any sale, transfer, exclusive worldwide license or other disposition of all or substantially all of the assets of the Company; or

(iv) within any 24-month period beginning on or after the date hereof, the persons who were directors of the Company immediately before the beginning of such period (the "Incumbent Directors") shall cease (for any reason other than death) to constitute at least a majority of the Board of Directors of the Company or the board of directors of any successor to the Company, provided that any director who was not a director as of the date hereof shall be deemed to be an Incumbent Director if such director was elected to the Board by, or on the recommendation of or with the approval of, at least two-thirds of the directors who then qualified as Incumbent Directors either actually or by prior operation of this Section 4(h)(iv), unless such election, recommendation or approval was the result of an actual or threatened contested election of directors pursuant to Regulation 14A under the Securities Exchange Act of 1934 or any successor provision.

(i) Base Salary Continuation. The Base Salary continuation set forth in Sections 4(d) and (e) above shall be intended either (i) to satisfy the safe harbor set forth in the regulations issued under section 409A of the Internal Revenue Code of 1986, as amended (the "Code") (Treas. Regs. 1.409A-1(n)(2)(ii)) or (ii) be treated as a Short-term Deferral as that term is defined under Code section 409A (Treas. Regs. 1.409A-1(b)(4)). To the extent such continuation payments exceed the applicable safe harbor amount or do not constitute a Short- term Deferral, the excess amount shall be treated as deferred compensation under Code section 409A and as such shall be payable pursuant to the following schedule: such excess amount shall be paid via standard payroll in periodic installments in accordance with the Company's usual practice for its senior executives. Solely for purposes of Code section 409A, each installment payment is considered a separate payment. Notwithstanding any provision in this Agreement to the contrary, in the event that the Employee is a "specified employee" as defined in Section 409A, any continuation payment, continuation benefits or other amounts payable under this Agreement that would be subject to the special rule regarding payments to "specified employees" under Section 409A(a)(2)(B) of the Code shall not be paid before the expiration of a period of six months following the date of the Employee's termination of employment or before the date of the Employee's death, if earlier.

(j) Parachute Provisions. Notwithstanding any provisions of this Agreement to the contrary:

(i) If any of the payments or benefits received or to be received by the Employee in connection with the Employee's termination of employment in respect of a Change in Control, whether pursuant to the terms of this Agreement or any other plan, arrangement or agreement with the Company (all such payments and benefits, being hereinafter referred to as the "Total Payments"), would be subject to the excise tax (the "Excise Tax") imposed under Section 4999 of the Code, the Employee shall receive the Total Payments and be responsible for the Excise Tax; provided, however that the Employee shall not receive the Total Payments and the Total Payments shall be reduced to the Safe Harbor Amount (defined below) if (A) the net amount of such Total Payments, as so reduced to the Safe Harbor Amount (and after subtracting the net amount of federal, state and local income taxes on such reduced Total Payments) is greater than or equal to (B) the net amount of such Total Payment without such reduction (but after subtracting the net amount of federal, state and local income taxes on such Total Payments and the amount of Excise Tax to which the Employee would be subject in respect of such unreduced Total Payments). The "Safe Harbor Amount" is the amount to which the Total Payments would hypothetically have to be reduced so that no portion of the Total Payments would be subject to the Excise Tax.

(ii) For purposes of determining whether any of the Total Payments will be subject to the Excise Tax and the amount of such Excise Tax, (A) all of the Total Payments shall be treated as "parachute payments" (within the meaning of Section 280G(b)(2) of the Code) unless, in the opinion of tax counsel ("Tax Counsel") selected by the accounting firm that was, immediately prior to the Change in Control, the Company's independent auditor (the "Auditor"), such payments or benefits (in whole or in part) do not constitute parachute payments, including by reason of Section 280G(b)(4)(A) of the Code, (B) all "excess parachute payments" within the meaning of Section 280G(b)(1) of the Code shall be treated as subject to the Excise Tax unless, in the opinion of Tax Counsel, such excess parachute payments (in whole or in part)

represent reasonable compensation for services actually rendered (within the meaning of Section 280G(b)(4)(B) of the Code) in excess of the base amount (within the meaning of Section 280G(b)(3) of the Code) allocable to such reasonable compensation, or are otherwise not subject to the Excise Tax, and (C) the value of any noncash benefits or any deferred payment or benefit shall be determined by the Auditor in accordance with the principles of Sections 280G(d)(3) and (4) of the Code. If the Auditor is prohibited by applicable law or regulation from performing the duties assigned to it hereunder, then a different auditor, acceptable to both the Company and Employee, shall be selected. The fees and expenses of Tax Counsel and the Auditor shall be paid by the Company.

(iii) In the event it is determined that the Safe Harbor Amount is payable to Employee, then the severance payments provided under this Agreement that are cash shall first be reduced on a pro rata basis, and the non-cash severance payments shall thereafter be reduced on a pro rata basis, to the extent necessary so that no portion of the Total Payments is subject to the Excise Tax.

5. Non-Disclosure and Non-Competition.

(a) Non-Disclosure. The Employee acknowledges that in the course of performing services for the Company, the Employee will obtain knowledge of the Company's business plans, products, processes, software, know-how, trade secrets, formulas, methods, models, prototypes, discoveries, inventions, improvements, disclosures, names and positions of employees and/or other proprietary and/or confidential information (collectively the "Confidential Information"). The Employee agrees to keep the Confidential Information secret and confidential and not to publish, disclose or divulge to any other party, and the Employee agrees not to use any of the Confidential Information for the Employee's own benefit or to the detriment of the Company without the prior written consent of the Company, whether or not such Confidential Information was discovered or developed by the Employee. The Employee also agrees not to divulge, publish or use any proprietary and/or confidential information of others that the Company is obligated to maintain in confidence.

(b) Non-Competition. The Employee agrees that during the Employee's employment by the Company hereunder and for an additional period of twelve (12) months after the termination of the Employee's employment hereunder, the Employee will (i) not engage or assist others in engaging in any business or enterprise (whether as owner, partner, officer, director, employee, consultant, investor or otherwise) that is competitive with the Company's business, including but not limited to any business or enterprise that develops, manufactures, markets, licenses, sells or provides any product that competes with any product developed, manufactured, marketed, licensed, sold or provided, or planned to be developed, manufactured, marketed, licensed sold or provided, by the Company ("Competitive Business") while Employee was employed by the Company; or (ii) solicit, hire, contract for services or otherwise employ, directly or indirectly, any of the employees of the Company. The foregoing prohibition shall not prevent any employment or engagement of the Employee, after termination of employment with the Company, by any company or business organization not substantially engaged in a Competitive Business as long as the activities of any such employment or engagement, in any capacity, do not involve work on matters related to any product or service being developed, manufactured, marketed, distributed or planned in writing by the Company at the time of

termination of Employee's employment with the Company. The Employee's ownership of no more than 5% of the outstanding voting stock of a publicly traded company shall not constitute a violation of this Section 5(b). The Employee is entering into this covenant not to compete in consideration of the additional agreements of the Company in this Agreement, including but not limited to the rights of the Employee set forth in Sections 4(d) and 4(e).

6. Inventions and Discoveries.

(a) Disclosure. The Employee shall promptly and fully disclose to the Company, with all necessary detail, all developments, know-how, discoveries, inventions, improvements, concepts, ideas, formulae, processes and methods (whether copyrightable, patentable or otherwise) made, received, conceived, acquired or written by the Employee (whether or not at the request or upon the suggestion of the Company, solely or jointly with others), during the period of the Employee's employment with the Company that (i) result from, arise out of, or relate to any work, assignment or task performed by the Employee on behalf of the Company, whether undertaken voluntarily or assigned to the Employee within the scope of the Employee's responsibilities to the Company, or (ii) were developed using the Company's facilities or other resources or in Company time, or (iii) result from the Employee's use or knowledge of the Company's Confidential Information, or (iv) relate to the Company's business or any of the products or services being developed, manufactured or sold by the Company or that may be used in relation therewith (collectively referred to as "Inventions"). The Employee hereby acknowledges that all original works of authorship that are made by the Employee (solely or jointly with others) within the above terms and that are protectable by copyright are "works made for hire," as that term is defined in the United States Copyright Act. The Employee understands and hereby agrees that the decision whether or not to commercialize or market any Invention developed by the Employee solely or jointly with others is within the Company's sole discretion and for the Company's sole benefit and that no royalty shall be due to the Employee as a result of the Company's efforts to commercialize or market any such Invention.

(b) Assignment and Transfer. The Employee agrees to assign and transfer to the Company all of the Employee's right, title and interest in and to the Inventions, and the Employee further agrees to deliver to the Company any and all drawings, notes, specifications and data relating to the Inventions, and to sign, acknowledge and deliver all such further papers, including applications for and assignments of copyrights and patents, and all renewals thereof, as may be necessary to obtain copyrights and patents for any Inventions in any and all countries and to vest title thereto in the Company and its successors and assigns and to otherwise protect the Company's interests therein. The Employee shall not charge the Company for time spent in complying with these obligations. If the Company is unable because of the Employee's mental or physical incapacity or for any other reason to secure the Employee's signature to apply for or to pursue any application for any United States or foreign patents or copyright registrations covering Inventions or original works of authorship assigned to the Company as above, then the Employee hereby irrevocably designates and appoints the Company and its duly authorized officers and agents as the Employee's agent and attorney in fact, to act for and in the Employee's behalf and stead to execute and file any such applications and to do all other lawfully permitted acts to further the prosecution and issuance of letters patent or copyright registrations thereon with the same legal force and effect as if executed by the Employee.

(c) Company Documentation. The Employee shall hold in a fiduciary capacity for the benefit of the Company all documentation, disks, programs, data, records, drawings, manuals, reports, sketches, blueprints, letters, notes, notebooks and all other writings, electronic data, graphics and tangible information and materials of a secret, confidential or proprietary information nature relating to the Company or the Company's business that are in the possession or under the control of the Employee. The Employee agrees that in connection with any research, development or other services performed for the Company, the Employee will maintain careful, adequate and contemporaneous written records of all Inventions, which records shall be the property of the Company.

7. Injunctive Relief. The Employee acknowledges that the Employee's compliance with the agreements in Sections 5 and 6 hereof is necessary to protect the good will and other proprietary interests of the Company and that the Employee is one of the principal executives of the Company and conversant with its affairs, its trade secrets and other proprietary information. The Employee acknowledges that a breach of any of the Employee's agreements in Sections 5 and 6 hereof will result in irreparable and continuing damage to the Company for which there will be no adequate remedy at law; and the Employee agrees that in the event of any breach of the aforesaid agreements, the Company and its successors and assigns shall be entitled to injunctive relief and to such other and further relief as may be proper.

8. Full Agreement. This Agreement amends, restates and supersedes the Prior Agreement and all other consulting and employment arrangements between the Employee and the Company, but shall not supersede any existing confidentiality, nondisclosure, invention assignment or non-compete agreement between the Employee and the Company. Except as set forth in the preceding sentence, this Agreement constitutes the entire agreement of the parties concerning its subject matter and supersedes all other oral or written understandings, discussions, and agreements, and may be modified only in a writing signed by both parties. The parties acknowledge that they have read and fully understand the contents of this Agreement and execute it after having an opportunity to consult with legal counsel.

9. Amendments. Any amendment to this Agreement shall be made in writing and signed by the parties hereto.

10. Enforceability. If any provision of this Agreement shall be invalid or unenforceable, in whole or in part, then such provision shall be deemed to be modified or restricted to the extent and in the manner necessary to render the same valid and enforceable, or shall be deemed excised from this Agreement, as the case may require, and this Agreement shall be construed and enforced to the maximum extent permitted by law as if such provision had been originally incorporated herein as so modified or restricted or as if such provision had not been originally incorporated herein, as the case may be.

11. Construction. This Agreement shall be construed and interpreted in accordance with the internal laws of the Commonwealth of Pennsylvania.

12. Assignment.

(a) By the Company. The rights and obligations of the Company under this Agreement shall inure to the benefit of, and shall be binding upon, the successors and assigns of the Company. This Agreement may be assigned by the Company without the consent of the Employee.

(b) By the Employee. This Agreement and the obligations created hereunder may not be assigned by the Employee, but all rights of the Employee hereunder shall inure to the benefit of and be enforceable by the Employee's heirs, devisees, legatees, executors, administrators and personal representatives.

13. Notices. All notices required or permitted to be given hereunder shall be in writing and shall be deemed to have been given when mailed by certified mail, return receipt requested, or delivered by a national overnight delivery service addressed to the intended recipient as follows:

If to the Company:

Marinus Pharmaceuticals, Inc. 100
Matsonford Road
5 Radnor Corporate Center; Suite 500 Attention:
Chief Executive Officer

If to the Employee, to address stated on the signature page to this Agreement.

Any party may from time to time change its address for the purpose of notices to that party by a similar notice specifying a new address, but no such change shall be deemed to have been given until it is actually received by the party sought to be charged with its contents.

14. Waivers. No claim or right arising out of a breach or default under this Agreement shall be discharged in whole or in part by a waiver of that claim or right unless the waiver is supported by consideration and is in writing and executed by the aggrieved party hereto or such party's duly authorized agent. A waiver by any party hereto of a breach or default by the other party hereto of any provision of this Agreement shall not be deemed a waiver of future compliance therewith, and such provisions shall remain in full force and effect.

15. Section 409A. It is intended that this Agreement be drafted and administered in compliance with section 409A of the Code, including, but not limited to, any future amendments to Code section 409A, and any other Internal Revenue Service or other governmental rulings or interpretations (together, "Section 409A") issued pursuant to Section 409A so as not to subject the Employee to payment of interest or any additional tax under Code section 409A. The parties intend for any payments under this Agreement to either satisfy the requirements of Section 409A or to be exempt from the application of Section 409A, and this Agreement shall be construed and interpreted accordingly. In furtherance thereof, if payment or provision of any amount or benefit hereunder that is subject to Section 409A at the time specified herein would subject such amount or benefit to any additional tax under Section 409A, the payment or provision of such amount or

benefit shall be postponed to the earliest commencement date on which the payment or provision of such amount or benefit could be made without incurring such additional tax. In addition, to the extent that any Internal Revenue Service guidance issued under Section 409A would result in the Employee being subject to the payment of interest or any additional tax under Section 409A, the parties agree, to the extent reasonably possible, to amend this Agreement in order to avoid the imposition of any such interest or additional tax under Section 409A, which amendment shall have the minimum economic effect necessary and be reasonably determined in good faith by the Company and the Employee.

16. Survival of Covenants. The provisions of Sections 4, 5, 6 and 7 hereof shall survive the termination of this Agreement. Furthermore, each other provision of this Agreement that, by its terms, is intended to continue beyond the termination of the Employee's employment shall continue in effect thereafter.

(Signature page follows.)

IN WITNESS WHEREOF, this Agreement has been executed by the parties.

By: /s/ Scott Braunstein, MD
Scott Braunstein, MD
Chief Executive Officer
Date: October 26, 2020

By: /s/ Christina Shafer
Christina Shafer
Date: October 26, 2020

EXHIBIT 10.7

AMENDED AND RESTATED INDEMNIFICATION AGREEMENT

This Amended and Restated Indemnification Agreement (the "Agreement") is entered into as of , by and among **Marinus Pharmaceuticals, Inc.**, a Delaware corporation (the "Company") and the undersigned party (the "Indemnitee").

RECITALS

A. The Company and ("Director") previously entered into that certain Indemnification Agreement dated September 30, 2005 (the "Original Indemnification Agreement").

B. Section 20 of the Original Indemnification Agreement provided that the Original Indemnification Agreement could be amended by written agreement executed by each of the parties hereto.

C. The Company and the Director desire to amend and restate the Original Indemnification Agreement as set forth herein.

D. The Company and the Indemnitee recognize the substantial increase in corporate litigation in general, subjecting directors, officers, employees, controlling persons, agents and fiduciaries to expensive litigation risks at the same time as the availability and coverage of liability insurance has been severely limited.

E. The Indemnitee does not regard the current protection available as adequate under the present circumstances, and the Indemnitee and other directors, officers, employees, controlling persons, agents and fiduciaries of the Company may not be willing to serve in such capacities without additional protection.

F. The Company: (i) desires to attract and retain the involvement of highly qualified individuals and entities, such as the Indemnitee, to serve the Company and, in part, to induce the Indemnitee to be involved with the Company and (ii) wishes to provide for the indemnification and advancing of expenses to the Indemnitee to the maximum extent permitted by law.

G. Although the bylaws of the Company require indemnification of the officers and directors of the Company, and the Indemnitee may also be entitled to indemnification pursuant to the General Corporation Law of the State of Delaware (the "DGCL"), the bylaws and the DGCL expressly provide that the indemnification provisions set forth therein are not exclusive, and thereby contemplate that contracts may be entered into between the Company and members of the board of directors, officers and other persons with respect to indemnification.

H. This Agreement is a supplement to and in furtherance of the bylaws of the Company and any resolutions adopted pursuant thereto, and shall not be deemed a substitute therefor, nor to diminish or abrogate any rights of the Indemnitee thereunder.

I. In view of the considerations set forth above, the Company desires that the Indemnitee be indemnified by the Company as set forth herein.

NOW, THEREFORE, the Company and the Indemnitee hereby agree as follows:

1. Indemnification.

a. Indemnification of Expenses. The Company shall indemnify and hold harmless the Indemnitee (including his or her respective directors, officers, partners, employees, agents and spouses, if any) and each person who controls any of them or who may be liable within the meaning of Section 15 of the Securities Act of 1933, as amended (the "Securities Act"), or Section 20 of the Securities Exchange Act of 1934, as amended (the "Exchange Act") to the fullest extent permitted by law if the Indemnitee was or is or becomes a party to or witness or other participant in, or are threatened to be made a party to or witness or other participant in, any threatened, pending or completed action, suit, proceeding or alternative dispute resolution mechanism, or any

hearing, inquiry or investigation that the Indemnitee believes might lead to the institution of any such action, suit, proceeding or alternative dispute resolution mechanism, whether civil, criminal, administrative, investigative or other (hereinafter a "Claim") (i) by reason of (or arising in part or in whole out of) any event or occurrence related to the fact that the Indemnitee is, was or may be deemed a director, officer, stockholder, employee, controlling person, agent or fiduciary of the Company, or any subsidiary of the Company, or is, was or may be deemed to be serving at the request or consent of the Company as a director, officer, stockholder, employee, controlling person, agent or fiduciary of another corporation, partnership, limited liability company, joint venture, trust or other enterprise, or (ii) by reason of any action or inaction on the part of such Indemnitee while serving in such capacity including, without limitation, any and all losses, claims, damages, expenses and liabilities, joint or several (including any investigation, legal and other expenses incurred in connection with, and any amount paid in settlement of, any action, suit, proceeding or any claim asserted) under the Securities Act, the Exchange Act or other federal or state statutory law or regulation, at common law or otherwise, that relate directly or indirectly to the registration, purchase, sale or ownership of any securities of the Company or to any fiduciary obligation owed or alleged to be owed to the Company or its stockholders or any other constituency of the Company with respect thereto (hereinafter an "Indemnifiable Event"), against any and all expenses (including attorneys' fees and all other costs, expenses and obligations incurred in connection with investigating, defending a witness in or participating in (including on appeal), or preparing to defend, be a witness in or participate in, any such action, suit, proceeding, alternative dispute resolution mechanism, hearing, inquiry or investigation), judgments, fines, penalties and amounts paid in settlement (if such settlement is approved in advance by the Company, which approval shall not be unreasonably withheld) of such Claim and any federal, state, local or foreign taxes imposed on the Indemnitee as a result of the actual or deemed receipt of any payments under this Agreement (collectively, hereinafter "Expenses"), including all interest, assessments and other charges paid or payable in connection with or in respect of such Expenses. Such payment of Expenses shall be made by the Company as soon as practicable but in any event no later than twenty (20) days after written demand by the Indemnitee therefor is presented to the Company.

b. Reviewing Party. Notwithstanding the foregoing, (i) the obligations of the Company under Section 1(a) shall be subject to the condition that the Reviewing Party (as described in Section 10(e) hereof) shall not have determined (in a written opinion, in any case in which the Independent Legal Counsel referred to in Section 10(d) hereof is involved) that the Indemnitee would not be permitted to be indemnified under applicable law, and (ii) the Indemnitee acknowledges and agrees that the obligation of the Company to make an advance payment of Expenses to the Indemnitee pursuant to Section 2(a) (an "Expense Advance") shall be subject to the condition that, if, when and to the extent that the Reviewing Party determines that the Indemnitee would not be permitted to be so indemnified under applicable law, the Company shall be entitled to be reimbursed by the Indemnitee (who hereby agrees to reimburse the Company) for all such amounts theretofore paid; provided, however, that if the Indemnitee has commenced or thereafter commences legal proceedings in a court of competent jurisdiction to secure a determination that the Indemnitee should be indemnified under applicable law, any determination made by the Reviewing Party that the Indemnitee would not be permitted to be indemnified under applicable law shall not be binding and the Indemnitee shall not be required to reimburse the Company for any Expense Advance until a final judicial determination is made with respect thereto (as to which all rights of appeal therefrom have been exhausted or lapsed). The Indemnitee's obligation to reimburse the Company for any Expense Advance shall be unsecured and no interest shall be charged thereon. If there has not been a Change in Control (as defined in Section 10(c) hereof), the Reviewing Party shall be selected by the Board of Directors, and if there has been such a Change in Control (other than a Change in Control that has been approved by a majority of the Company's Board of Directors who were directors immediately prior to such Change in Control), the Reviewing Party shall be the Independent Legal Counsel referred to in Section 10(d) hereof. If there has been no determination by the Reviewing Party or if the Reviewing Party determines that the Indemnitee substantively would not be permitted to be indemnified in whole or in part under applicable law, the Indemnitee shall have the right to commence litigation seeking an initial determination by the court or challenging any such determination by the Reviewing Party or any aspect thereof, including the legal or factual bases therefor, and the Company hereby consents to service of process and to appear in any such proceeding. Any determination by the Reviewing Party otherwise shall be conclusive and binding on the Company and the Indemnitee.

c. Contribution. If the indemnification provided for in Section 1(a) above for any reason is held by a court of competent jurisdiction to be unavailable to an Indemnitee in respect of any losses,

claims, damages, expenses or liabilities referred to therein, then the Company, in lieu of indemnifying the Indemnitee thereunder, shall contribute to the amount paid or payable by the Indemnitee as a result of such losses, claims, damages, expenses or liabilities (i) in such proportion as is appropriate to reflect the relative benefits received by the Company and the Indemnitee, or (ii) if the allocation provided by clause (i) above is not permitted by applicable law, in such proportion as is appropriate to reflect not only the relative benefits referred to in clause (i) above but also the relative fault of the Company and the Indemnitee in connection with the action or inaction that resulted in such losses, claims, damages, expenses or liabilities, as well as any other relevant equitable considerations. In connection with the registration of the Company's securities, the relative benefits received by the Company and the Indemnitee shall be deemed to be in the same respective proportions that the net proceeds from the offering (before deducting expenses) received by the Company and the Indemnitee, in each case as set forth in the table on the cover page of the applicable prospectus, bear to the aggregate public offering price of the securities so offered. The relative fault of the Company and the Indemnitee shall be determined by reference to, among other things, whether the untrue or alleged untrue statement of a material fact or the omission or alleged omission to state a material fact relates to information supplied by the Company or the Indemnitee and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such statement or omission.

The Company and the Indemnitee agree that it would not be just and equitable if contribution pursuant to this Section 1(c) were determined by pro rata or per capita allocation or by any other method of allocation that does not take account of the equitable considerations referred to in the immediately preceding paragraph. In connection with the registration of the Company's securities, in no event shall an Indemnitee be required to contribute any amount under this Section 1(c) in excess of the lesser of: (i) that proportion of the total of such losses, claims, damages or liabilities that are indemnified against, equal to the proportion of the total securities sold under such registration statement that is being sold by the Indemnitee or (ii) the proceeds received by the Indemnitee from its sale of securities under such registration statement. No person found guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) shall be entitled to contribution from any person who was not found guilty of such fraudulent misrepresentation.

d. Survival Regardless of Investigation. The indemnification and contribution provided for in this Section 1 will remain in full force and effect regardless of any investigation made by or on behalf of any Indemnitee or any officer, director, employee, agent or controlling person of an Indemnitee.

e. Change in Control. The Company agrees that if there is a Change in Control of the Company (other than a Change in Control that has been approved by a majority of the Company's Board of Directors who were directors immediately prior to such Change in Control) then, with respect to all matters thereafter arising concerning the rights of an Indemnitee to payments of Expenses under this Agreement or any other agreement or under the Company's certificate of incorporation or bylaws as now or hereafter in effect, Independent Legal Counsel (as defined in Section 10(d) hereof) shall be selected by the Indemnitee and approved by the Company (which approval shall not be unreasonably withheld). Such counsel, among other things, shall render its written opinion to the Company and the Indemnitee as to whether and to what extent the Indemnitee would be permitted to be indemnified under applicable law. The Company agrees to abide by such opinion and to pay the reasonable fees of the Independent Legal Counsel referred to above and to fully indemnify such counsel against any and all expenses (including attorneys' fees), claims, liabilities and damages arising out of or relating to this Agreement or its engagement pursuant hereto.

f. Mandatory Payment of Expenses. Notwithstanding any other provision of this Agreement, to the extent that an Indemnitee has been successful on the merits or otherwise, including, without limitation, the dismissal of an action without prejudice, in the defense of any action, suit, proceeding, inquiry or investigation referred to in Section 1(a) hereof or in the defense of any claim, issue or matter therein, the Indemnitee shall be indemnified against all Expenses incurred by the Indemnitee in connection herewith.

2. Expenses; Indemnification Procedure.

a. Advancement of Expenses. The Company shall advance all Expenses incurred by an Indemnitee. The advances to be made hereunder shall be paid by the Company to the Indemnitee as soon as

practicable but in any event no later than twenty (20) days after written demand by the Indemnitee therefor to the Company.

b. Notice/Cooperation by the Indemnitee. The Indemnitee shall give the Company notice in writing as soon as practicable of any Claim made against the Indemnitee for which indemnification will or could be sought under this Agreement. Notice to the Company shall be directed to the Chief Executive Officer of the Company at the Company's address (or such other address as the Company shall designate in writing to the Indemnitee).

c. No Presumptions; Burden of Proof. For purposes of this Agreement, the termination of any Claim by judgment, order, settlement (whether with or without court approval) or conviction, or upon a plea of nolo contendere, or its equivalent, shall not create a presumption that the Indemnitee did not meet any particular standard of conduct or have any particular belief or that a court has determined that indemnification is not permitted by applicable law. In addition, neither the failure of the Reviewing Party to have made a determination as to whether the Indemnitee has met any particular standard of conduct or had any particular belief, nor an actual determination by the Reviewing Party that the Indemnitee has not met such standard of conduct or did not have such belief, prior to the commencement of legal proceedings by the Indemnitee to secure a judicial determination that the Indemnitee should be indemnified under applicable law, shall be a defense to the Indemnitee's claim or create a presumption that the Indemnitee has not met any particular standard of conduct or did not have any particular belief. In connection with any determination by the Reviewing Party or otherwise as to whether an Indemnitee is entitled to be indemnified hereunder, the burden of proof shall be on the Company to establish that the Indemnitee is not so entitled.

d. Notice to Insurers. If, at the time of the receipt by the Company of a notice of a Claim pursuant to Section 2(b) hereof, the Company has liability insurance in effect that may cover such Claim, the Company shall give prompt notice of the commencement of such Claim to the insurers in accordance with the procedures set forth in each of the policies. The Company shall thereafter take all necessary or desirable action to cause such insurers to pay, on behalf of the Indemnitee, all amounts payable as a result of such Claim in accordance with the terms of such policies.

e. Selection of Counsel. If the Company shall be obligated hereunder to pay the Expenses of any Claim, the Company shall be entitled to assume the defense of such Claim, with counsel approved by the Indemnitee (which approval shall not be unreasonably withheld), upon the delivery to the Indemnitee of written notice of its election to
do so. After delivery of such notice, approval of such counsel by the Indemnitee and the retention of such counsel by the Company, the Company will not be liable to the Indemnitee under this Agreement for any fees of counsel subsequently incurred by the Indemnitee with respect to the same Claim; provided that, (i) the Indemnitee shall have the right to employ the Indemnitee's counsel in any such Claim at the Indemnitee's expense and (ii) if (A) the employment of counsel by the Indemnitee has been previously authorized by the Company, (B) the Indemnitee shall have reasonably concluded that there is a conflict of interest between the Company and the Indemnitee in the conduct of any such defense, or (C) the Company shall not continue to retain such counsel to defend such Claim, then the fees and expenses of the Indemnitee's counsel shall be at the expense of the Company.

3. Additional Indemnification Rights; Nonexclusivity.

a. Scope. The Company hereby agrees to indemnify the Indemnitee to the fullest extent permitted by law, even if such indemnification is not specifically authorized by the other provisions of this Agreement, the Company's certificate of incorporation, the Company's bylaws or by statute. In the event of any change after the date of this Agreement in any applicable law, statute or rule that expands the right of a Delaware corporation to indemnify a member of its Board of Directors or an officer, stockholder, employee, controlling person, agent or fiduciary, it is the intent of the parties hereto that the Indemnitee shall enjoy by this Agreement the greater benefits afforded by such change. In the event of any change in any applicable law, statute or rule that narrows the right of a Delaware corporation to indemnify a member of its Board of Directors or an officer, employee, agent or fiduciary, such change, to the extent not otherwise required by such law, statute or rule to be applied to this Agreement, shall have no effect on this Agreement or the parties' rights and obligations hereunder except as set forth in Section 8(a) hereof.

b. Nonexclusivity. The indemnification provided by this Agreement shall be in addition to any rights to which the Indemnitee may be entitled under the Company's certificate of incorporation, its bylaws, any agreement, any vote of stockholders or disinterested directors, the DGCL, or otherwise. The indemnification provided under this Agreement shall commence upon the date an Indemnitee first serves in an indemnified capacity and shall continue as to the Indemnitee for any action the Indemnitee took or did not take while serving in an indemnified capacity even though the Indemnitee may have ceased to serve in such capacity. The Company hereby acknowledges that the Indemnitee may have other sources of indemnification or insurance, whether currently in force or established in the future (collectively, the "Outside Indemnitors"). The Company hereby agrees: (i) that it is the indemnitor of first resort (i.e., its obligations to the Indemnitee are primary and any obligation of the Outside Indemnitors to advance expenses or to provide indemnification for the same expenses or liabilities incurred by the Indemnitee are secondary); (ii) that it shall be required to advance the full amount of expenses incurred by the Indemnitee and shall be liable in full for all indemnifiable amounts to the extent legally permitted and as required by the certificate of incorporation and bylaws (or any agreement between the Company and the Indemnitee), without regard to any rights the Indemnitee may have against the Outside Indemnitors and (iii) that it irrevocably waives, relinquishes and releases the Outside Indemnitors from any and all claims against the Outside Indemnitors for contribution, subrogation or any other recovery of any kind in respect thereof. The Company further agrees that no advancement or payment by the Outside Indemnitors on behalf of the Indemnitee with respect to any claim for which the Indemnitee have sought indemnification from the Company shall affect the foregoing and the Outside Indemnitors shall have a right of contribution and/or be subrogated to the extent of such advancement or payment to all of the rights of recovery of the Indemnitee against the Company. The Company and the Indemnitee agree that the Outside Indemnitors are express third party beneficiaries of the terms hereof.

4. No Duplication of Payments. Except as otherwise set forth in Section 3(b) above, the Company shall not be liable under this Agreement to make any payment in connection with any Claim made against an Indemnitee to the extent the Indemnitee has otherwise actually received payment (under any insurance policy, certificate of incorporation, bylaw or otherwise) of the amounts otherwise indemnifiable hereunder.

5. Partial Indemnification. If an Indemnitee is entitled under any provision of this Agreement to indemnification by the Company for any portion of Expenses incurred in connection with any Claim, but not, however, for all of the total amount thereof, the Company shall nevertheless indemnify the Indemnitee for the portion of such Expenses to which the Indemnitee is entitled.

6. Mutual Acknowledgement. The Company and the Indemnitee acknowledge that in certain instances, Federal law or applicable public policy may prohibit the Company from indemnifying its directors, officers, employees, controlling persons, agents or fiduciaries under this Agreement or otherwise. The Indemnitee understands and acknowledges that the Company has undertaken or may be required in the future to undertake with the Securities and Exchange Commission to submit the question of indemnification to a court in certain circumstances for a determination of the Company's rights under public policy to indemnify an Indemnitee.

7. Liability Insurance. To the extent the Company maintains liability insurance applicable to directors, officers, employees, control persons, agents or fiduciaries, the Indemnitee shall be covered by such policies in such
a manner as to provide the Indemnitee the same rights and benefits as are accorded to the most favorably insured (i) of the Company's directors, if the Indemnitee is a director, or (ii) of the Company's officers, if the Indemnitee is not a director of the Company but is an officer; or (iii) of the Company's key employees, controlling persons, agents or fiduciaries, if the Indemnitee is not an officer or director but is a key employee, agent, control person or fiduciary.

8. Exceptions. Any other provision herein to the contrary notwithstanding, the Company shall not be obligated pursuant to the terms of this Agreement:

a. Claims Initiated by an Indemnitee. To indemnify or advance expenses to an Indemnitee with respect to Claims initiated or brought voluntarily by the Indemnitee and not by way of defense, except: (i) with respect to actions or proceedings to establish or enforce a right to indemnify under this Agreement or any other agreement or insurance policy or under the Company's certificate of incorporation or bylaws now or hereafter in effect relating to Claims for Indemnifiable Events; (ii) in specific cases if the Board of Directors has

approved the initiation or bringing of such Claim; or (iii) as otherwise required under Section 145 of the DGCL, regardless of whether the Indemnitee ultimately is determined to be entitled to such indemnification, advance expense payment or insurance recovery, as the case may be; or

b. Claims Under Section 16(b). To indemnify an Indemnitee for expenses and the payment of profits arising from the purchase and sale by the Indemnitee of securities in violation of Section 16(b) of the Exchange Act or any similar successor statute; or

c. Claims Excluded Under Section 145 of the DGCL. To indemnify the Indemnitee if: (i) the Indemnitee did not act in good faith and in a manner reasonably believed to be in or not opposed to the best interests of the Company or (ii) with respect to any criminal action or proceeding, the Indemnitee had reasonable cause to believe the conduct was unlawful or (iii) the Indemnitee shall have been adjudged to be liable to the Company unless and only to the extent the court in which such action was brought shall permit indemnification as provided in Section 145(b) of the DGCL.

9. Period of Limitations. No legal action shall be brought and no cause of action shall be asserted by or in the right of the Company against an Indemnitee or an Indemnitee's estate, spouse, heirs, executors or personal or legal representatives after the expiration of five (5) years from the date of accrual of such cause of action, and any claim or cause of action of the Company shall be extinguished and deemed released unless asserted by the timely filing of a legal action within such five (5)-year period; provided, however, that if any shorter period of limitations is otherwise applicable to any such cause of action, such shorter period shall govern.

10. Construction of Certain Phrases.

a. For purposes of this Agreement, references to the "Company" shall include, in addition to the resulting corporation, any constituent corporation (including any constituent of a constituent) absorbed in a consolidation or merger that, if its separate existence had continued, would have had power and authority to indemnify its directors, officers, stockholders, employees, agents or fiduciaries, so that if an Indemnitee is, was or may be deemed a director, officer, stockholder, employee, agent, control person, or fiduciary of such constituent corporation, or is or was serving at the request of such constituent corporation as a director, officer, employee, control person, agent or fiduciary of another corporation, partnership, limited liability company, joint venture, employee benefit plan, trust or other enterprise, the Indemnitee shall stand in the same position under the provisions of this Agreement with respect to the resulting or surviving corporation as the Indemnitee would have with respect to such constituent corporation if its separate existence had continued.

b. For purposes of this Agreement, references to "other enterprises" shall include employee benefit plans; references to "fines" shall include any excise taxes assessed on an Indemnitee with respect to an employee benefit plan; and references to "serving at the request of the Company" shall include any service as a director, officer, employee, agent or fiduciary of the Company that imposes duties on, or involves services by, such director, officer, employee, agent or fiduciary with respect to an employee benefit plan, its participants or its beneficiaries; and if an Indemnitee acted in good faith and in a manner the Indemnitee reasonably believed to be in the interest of the participants and beneficiaries of an employee benefit plan, the Indemnitee shall be deemed to have acted in a manner "not opposed to the best interests of the Company" as referred to in this Agreement.

c. For purposes of this Agreement a "Change in Control" shall be deemed to have occurred if: (i) any "person" (as such term is used in Sections 13(d)(3) and 14(d)(2) of the Exchange Act), other than a trustee or other fiduciary holding securities under an employee benefit plan of the Company or a corporation owned directly or indirectly by the stockholders of the Company in substantially the same proportions as their ownership of stock of the Company, (A) who is or becomes the beneficial owner, directly or indirectly, of securities of the Company representing twenty percent (20%) or more of the combined voting power of the Company's then outstanding Voting Securities, increases his beneficial ownership of such securities by five percent (5%) or more over the percentage so owned by such person, or (B) becomes the "beneficial owner" (as defined in Rule 13d-3 under said Exchange Act), directly or indirectly, of securities of the Company representing more than thirty percent (30%) of the total voting power represented by the Company's then outstanding Voting Securities, (ii) during any period of two (2) consecutive years, individuals who at the beginning

of such period constitute the Board of Directors of the Company and any new director whose election by the Board of Directors or nomination for election by the Company's stockholders was approved by a vote of at least two-thirds (2/3) of the directors then still in office who either were directors at the beginning of the period or whose election or nomination for election was previously so approved, cease for any reason to constitute a majority thereof, or (iii) the stockholders of the Company approve a merger or consolidation of the Company with any other corporation other than a merger or consolidation that would result in the Voting Securities of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into Voting Securities of the surviving entity) at least eighty percent (80%) of the total voting power represented by the Voting Securities of the Company or such surviving entity outstanding immediately after such merger or consolidation, or the stockholders of the Company approve a plan of complete liquidation of the Company or an agreement for the sale or disposition by the Company of (in one transaction or a series of transactions) all or substantially all of the Company's assets.

d. For purposes of this Agreement, "Independent Legal Counsel" shall mean an attorney or firm of attorneys, selected in accordance with the provisions of Section 2(e) hereof, who shall not have otherwise performed services for the Company or the Indemnitee within the last three (3) years (other than with respect to matters concerning the right of the Indemnitee under this Agreement, or of other indemnitees under similar indemnity agreements).

e. For purposes of this Agreement, a "Reviewing Party" shall mean any appropriate person or body consisting of a member or members of the Company's Board of Directors or any other person or body appointed by the Board of Directors who is not a party to the particular Claim for which the Indemnitee is seeking indemnification, or Independent Legal Counsel.

f. For purposes of this Agreement, "Voting Securities" shall mean any securities of the Company that vote generally in the election of directors.

11. Counterparts. This Agreement may be executed in one or more counterparts, each of which shall constitute an original.

12. Binding Effect; Successors and Assigns. This Agreement shall be binding upon and inure to the benefit of and be enforceable by the parties hereto and their respective successors, assigns, including any direct or indirect successor by purchase, merger, consolidation or otherwise to all or substantially all of the business and/or assets of the Company, spouses, heirs and personal and legal representatives. The Company shall require and cause any successor (whether direct or indirect by purchase, merger, consolidation or otherwise) to all, substantially all, or a substantial part, of the business and/or assets of the Company, by written agreement in form and substance satisfactory to the Indemnitee, expressly to assume and agree to perform this Agreement in the same manner and to the same extent that the Company would be required to perform if no such succession had taken place. This Agreement shall continue in effect with respect to Claims relating to Indemnifiable Events regardless of whether the Indemnitee continues to serve as a director, officer, employee, agent, controlling person or fiduciary of the Company or of any other enterprise, including subsidiaries of the Company, at the Company's request.

13. Attorneys' Fees. In the event that any action is instituted by an Indemnitee under this Agreement or under any liability insurance policies maintained by the Company to enforce or interpret any of the terms hereof or thereof, the Indemnitee shall be entitled to be paid all Expenses incurred by the Indemnitee with respect to such action if the Indemnitee is ultimately successful in such action, and shall be entitled to the advancement of Expenses with respect to such action, except, in the case of both payment and advancement of Expenses, as and solely to the extent of Expenses incurred with respect to a material assertion made by the Indemnitee as a part of such action which a court of competent jurisdiction over such action determines was not made in good faith or was frivolous. In the event of an action instituted by or in the name of the Company under this Agreement to enforce or interpret any of the terms of this Agreement, an Indemnitee shall be entitled to be paid Expenses incurred by such Indemnitee in defense of such action (including costs and expenses incurred with respect to his or its counterclaims and cross-claims made in such action), and shall be entitled to the advancement of Expenses with respect to such action, except, in the case of both payment and advancement of Expenses, as and solely to the extent of Expenses incurred with respect to a material assertion made by the Indemnitee as a part of

such action which a court of competent jurisdiction over such action determines was not made in good faith or was frivolous.

14. Notice. All notices and other communications required or permitted hereunder shall be in writing, shall be effective when given, and shall in any event be deemed to be given: (a) five (5) days after deposit with the U.S. Postal Service or other applicable postal service, if delivered by first class mail, postage prepaid; (b) upon delivery, if delivered by hand; (c) one (1) business day after the business day of deposit with Federal Express or similar overnight courier, freight prepaid; or (d) one (1) day after the business day of delivery by facsimile transmission, if deliverable by
facsimile transmission, with copy by first class mail, postage prepaid, and shall be addressed if to an Indemnitee, at the Indemnitee's address as set forth beneath the Indemnitee's signature to this Agreement and if to the Company at the address of its principal corporate offices (attention: Secretary) or at such other address as such party may designate by ten (10) days' advance written notice to the other party hereto.

15. Consent to Jurisdiction. The Company and the Indemnitee each hereby irrevocably consent to the jurisdiction of the courts of the State of Delaware for all purposes in connection with any action or proceeding that arises out of or relates to this Agreement and agree that any action instituted under this Agreement shall be commenced, prosecuted and continued only in the Court of Chancery of the State of Delaware in and for New Castle County, which shall be the exclusive and only proper forum for adjudicating such a claim.

16. Severability. The provisions of this Agreement shall be severable in the event that any of the provisions hereof (including any provision within a single section, paragraph or sentence) are held by a court of competent jurisdiction to be invalid, void or otherwise unenforceable, and the remaining provisions shall remain enforceable to the fullest extent permitted by law. Furthermore, to the fullest extent possible, the provisions of this Agreement (including, without limitations, each portion of this Agreement containing any provision held to be invalid, void or otherwise unenforceable, that is not itself invalid, void or unenforceable) shall be construed so as to give effect to the intent manifested by the provision held invalid, illegal or unenforceable.

17. Choice of Law. This Agreement shall be governed by and its provisions construed and enforced in accordance with the laws of the State of Delaware, as applied to contracts between Delaware residents, entered into and to be performed entirely within the State of Delaware, without regard to the conflict of laws principles thereof.

18. Subrogation. In the event of payment under this Agreement, the Company shall be subrogated to the extent of such payment to all of the rights of recovery of the Indemnitee who shall execute all documents required and shall do all acts that may be necessary to secure such rights and to enable the Company effectively to bring suit to enforce such rights.

19. Amendment and Termination. No amendment, modification, termination or cancellation of this Agreement shall be effective unless it is in writing signed by all parties hereto. No waiver of any of the provisions of this Agreement shall be deemed or shall constitute a waiver of any other provisions hereof (whether or not similar) nor shall such waiver constitute a continuing waiver.

20. Integration and Entire Agreement. This Agreement sets forth the entire understanding between the parties hereto and supersedes and merges all previous written and oral negotiations, commitments, understandings and agreements relating to the subject matter hereof between the parties hereto.

21. No Construction as Employment Agreement. Nothing contained in this Agreement shall be construed as giving the Indemnitee any right to be retained in the employ of the Company or any of its subsidiaries.

22. Board and Stockholder Approval. The Company represents that this Agreement has been approved by the Company's board of directors and stockholders.

23. Amendment and Restatement. Effective and contingent upon execution of this Agreement, the Company and the Director agree that the Original Indemnification Agreement is hereby amended and restated in its entirety to read as set forth in this Agreement, and the Company and the parties hereto hereby agree to be bound by the provisions hereof.

[*Remainder of page intentionally left blank*]

IN WITNESS WHEREOF, the parties hereto have executed this Amended and Restated Indemnification Agreement on and as of the day and year first above written.

COMPANY:

MARINUS PHARMACEUTICALS, INC.,
a Delaware corporation

By: _____
Chief Executive Officer and President

Address for Notice:

5 Radnor Corporate Center, Suite 500
100 Matsonford Rd
Radnor, PA 19087

INDEMNITEE:

_____

Address: _____

_____

Signature Page to Amended and Restated Indemnification Agreement

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Schedule of Material Differences to Exhibit 10.7

The following directors and executive officers are parties to an Indemnification Agreement with the Company, each of which are substantially identical in all material respects to the representative Indemnification Agreement filed herewith as Exhibit 10.7 except as to the name of the signatory and the date of each signatory's Indemnification Agreement. The name of each signatory is listed below. The actual Indemnification Agreements are omitted pursuant to Instruction 2 to Item 601 of Regulation S-K.

Indemnitee

Scott Braunstein Chief Executive Officer and Chairman of the Board
Chuck Austin, Director
Elan Ezickson, Director
Seth H.Z. Fischer, Director
Timothy Mayleben, Director
Saraswathy Nochur, Director
Christine B. Silverstein, Director

Exhibit 10.42

Final Execution Copy

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

Collaboration and Supply Agreement

between

Marinus Pharmaceuticals, Inc.

and

Tenacia Biotechnology (Shanghai) Co., Ltd.

COLLABORATION AND SUPPLY AGREEMENT

This **COLLABORATION AND SUPPLY AGREEMENT** is made and entered into as of November 16, 2022 ("Effective Date") between **Marinus Pharmaceuticals, Inc.** ("Marinus"), a Delaware corporation with principal offices at 5 Radnor Corporate Center, Suite 500, 100 Matsonford Road, Radnor, Pennsylvania 19087 USA, and **Tenacia Biotechnology (Shanghai) Co.**, Ltd. ("Company"), a company organized and existing under the laws of PRC, located at Room 368, Part 302, 211 North Fute Road, China (Shanghai) Pilot Free Trade Zone. Marinus and Company may be referred herein individually as a "Party" or collectively as the "Parties."

INTRODUCTION

WHEREAS, Marinus and Company desire to enter into a collaborative relationship to develop and commercialize certain Marinus proprietary formulations of ganaxolone (as defined below) in the Territory in the Field (both as defined below);

WHEREAS, Marinus Controls (defined below) certain intellectual property, including Patents (defined below), related to Licensed Products, and has the right to grant certain rights thereunder as set forth herein;

WHEREAS, Company has certain expertise in the development, registration and commercialization of pharmaceutical products in the Territory, and wishes to obtain exclusive rights to develop, market, distribute and sell Licensed Products in the Territory; and

WHEREAS, Marinus wishes to convey such rights to Company, subject to the terms and conditions of this Agreement.

NOW, THEREFORE, in consideration of the mutual promises, covenants and agreements hereinafter set forth, the sufficiency of which is hereby acknowledged, the Parties to this Agreement mutually agree as follows:

Article I
Definitions

For purposes of this Agreement, the following initially capitalized terms in this Agreement, whether used in the singular or plural, shall have the following meanings:

1.1 "**Affiliate**" shall mean any corporation, company, partnership, joint venture and/or firm which controls, is controlled by, or is under common control with a specified person or entity (Marinus or Company). "Control" as used in this definition shall mean (a) in the case of corporate entities, direct or indirect ownership of more than fifty percent (50%) of the stock or shares having the right to vote for the election of directors, and (b) in the case of non-corporate entities, direct or indirect ownership of more than fifty percent (50%) of the equity interest with the power to direct the management and policies of such non-corporate entities.

1.2 "**Agreement**" shall mean this Agreement together with all exhibits, schedules, and appendices attached to this Agreement, all as respectively amended, modified or supplemented by the Parties in accordance with the terms of this Agreement.

1.3 "**Business Day**" shall mean any day on which banking institutions in Beijing, PRC, and Philadelphia, Pennsylvania, the USA, are open for business.

1.4 "**Calendar Quarter**" means the period beginning on the Effective Date and ending on the last day of the calendar quarter in which the Effective Date falls, and thereafter each successive period of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31; provide that the final Calendar Quarter shall end on the last day of the term of this Agreement.

1.5 "**Calendar Year**" means the period beginning on the Effective Date and ending on December 31 of the calendar year in which the Effective Date falls, and thereafter each successive period of twelve (12) months commencing on January 1 and ending on December 31; provided that, the final Calendar Year shall end on the last day of the term of this Agreement.

1.6 "**China Data Protection Laws**" means any relevant law, statute, declaration, decree, directive, legislative enactment, order, ordinance, regulation, rule or other binding instrument which implements, replaces, adds to, amends, extends, reconstitutes or consolidates such laws from time to time, including without limitation, the Personal Information Protection Law, the Prescribed Agreement on Cross-border Transfer Data and Guidelines for Cross-Border Data Transfer Security Assessment, in each case as amended, consolidated, re-enacted or replaced from time to time.

1.7 "**Clinical Data**" means data generated by Company in any Clinical Studies conducted by or on behalf of Company.

1.8 "**Clinical Study**" means the clinical study of a Licensed Product in human subjects, as applicable, as described in the ICH Harmonised Tripartite Guideline entitled "General Considerations for Clinical Trials," as amended.

1.9 "**Commercialization Plan**" means, with respect to a Licensed Product, the written high-level strategic and tactical plans for the commercialization activities (including, without limitation, compassionate and named patient use), inclusive of [***], for such Licensed Product to be conducted in the Territory.

1.10 "**Commercially Reasonable Efforts**" means, (a) where applied to carrying out specific tasks and obligations of a Party under this Agreement, expending (on its own or acting through any of its Affiliates, sublicensees or agents) reasonable, diligent, good faith efforts and resources to accomplish such task or obligation as a similarly situated biopharmaceutical company would normally use to accomplish a similar task or obligation under similar circumstances in accordance with applicable laws; and (b) with respect to a

Party's obligations or activities under this Agreement for a Licensed Product, the application of such diligent and good faith efforts and resources, in an active and ongoing program, as are commensurate with those commonly used by a similarly situated biopharmaceutical company for a product at a similar stage in its development or product life cycle and of similar market potential and intellectual property protection as the Product, taking into account all relevant factors, including the competitiveness of the marketplace and the proprietary position, regulatory status, and relative safety and efficacy of such product, and any other relevant scientific, technical, regulatory or commercial factors.

1.11 "**Company Clinical Study**" means any Clinical Study conducted in the Territory by Company.

1.12 "**Company Development Data**" means any non-clinical, clinical, CMC, or other data related to Licensed Compounds or Licensed Products generated by or for Company.

1.13 "**Company Inventions**" means inventions and Improvements discovered, conceived or reduced to practice by or for Company in exercising its rights or performing its obligations under this Agreement or utilizing Marinus Confidential Information and insofar as they relate to a Licensed Product during the term of this Agreement. For clarity, Marinus' rights, pursuant to this Agreement, to practice Company Inventions shall be limited solely to Licensed Product.

1.14 "**Company Patents**" means Patents obtained for Company Inventions.

1.15 "**Company Technology**" means to the extent discovered, conceived or reduced to practice by or for Company in exercising its rights or performing its obligations under this Agreement or utilizing Marinus Confidential Information and any and all information, intellectual property and proprietary material including but not limited to data, technical information, methods of manufacture, processes, techniques, know-how, experience, inventions, discoveries, trade secrets, compositions of matter and methods, whether or not patentable or confidential, and materials, that are either Controlled by Company or any of its Affiliates, insofar as they relate to the structure, composition, formulation, ingredients, preparation, presentation, means of delivery, dosage, packaging or development and use of Licensed Product, or of specific formulations of Licensed Product but excluding Clinical Data. For clarity, Marinus' rights, pursuant to this Agreement, to practice Company Technology shall be limited to Licensed Product.

1.16 "**Confidential Information**" of a Party means all know-how, inventions, materials, and other proprietary, scientific, marketing, financial, or commercial information that is: (a) disclosed by or on behalf of such Party or any of its Affiliates or otherwise made available to the other Party or any of its Affiliates, whether made available orally, in writing, or in electronic form; or (b) learned by the other Party pursuant to this Agreement. The existence and terms of this Agreement are the Confidential Information of both Parties. All information disclosed by a Party or any of its Affiliates under the Mutual Confidentiality

Agreement between the Parties dated [***] is deemed the Confidential Information of such Party under this Agreement.

1.17 "**Controlled**" or "**Controls**", when used in reference to intellectual property, means the legal authority or right of a Party hereto (or any of its Affiliates) to grant rights to practice such intellectual property rights to another Party, or to otherwise disclose proprietary or trade secret information to such other Party, without breaching the terms of any agreement with a Third Party.

1.18 "**Costs of Goods**" or "**COGS**" means [***].

1.19 "**Development Plan**" means the Territory Development Plan, which shall include, among other things, timelines and responsibilities to be undertaken by each Party for development and registration of a Licensed Product, including but not limited to Clinical Studies, planned to be conducted in the Field throughout the Territory.

1.20 "**Dollars**" means the lawful currency of the USA.

1.21 "**Effective Date**" shall have the meaning given to it in the preamble.

1.22 "**English Trademarks**" shall have the meaning given to it in Section 5.5.

1.23 "**FCPA**" shall have the meaning given to it in Section 13.7.

1.24 "**Field**" means application and administration of Licensed Product, for all diagnostic, prophylactic and therapeutic uses in humans in the Initial Indications and any other indications as to which Company exercises its right of first refusal pursuant to Section 2.4 of this Agreement.

1.25 "**First Commercial Sale**" means the first shipment of a Licensed Product to a Third Party by Company or an Affiliate of Company for monetary value for distribution, use or consumption in the Territory after Regulatory Approval has been granted with respect to such Licensed Product.

1.26 "**GAAP**" means the generally accepted accounting principles of the U.S., consistently applied.

1.27 "**Ganaxolone**" means 3-alpha-hydroxy-3-beta-methyl 5-alpha-pregnan-20-one, including any analogues or derivatives thereof, including its salts, or derivatives but not its salts, congeners or other derivatives which involve forming or breaking a covalent bond with or of such compound.

1.28 "**Generic Version**" of a Licensed Product means any pharmaceutical product (other than a product which was initially sold as a Licensed Product hereunder), approved by a Regulatory Authority, containing Licensed Compound and sold by a Third Party that has

not obtained the rights to market or sell such product in the Territory from Company, its Affiliates or sublicensees.

1.29 "**GCP**" means the current good clinical practice regulations promulgated by, as applicable, the ICH, US Food and Drug Administration, and NMPA, as such regulations may be amended.

1.30 "**Governmental Authority**" means any national, international, federal, state, provincial or local government, or political subdivision thereof, or any multinational organization or any authority, agency or commission entitled to exercise any administrative, executive, judicial, legislative, police, regulatory or taxing authority or power, any court or tribunal (or any department, bureau or division thereof, or any governmental arbitrator or arbitral body).

1.31 "**HKIAC**" shall have the meaning given to it in Section 12.3(a).

1.32 "**Hong Kong**" means the Hong Kong Special Administrative Region of the PRC.

1.33 "**ICH**" means the International Conference on Harmonization (of Technical Requirements for Registration of Pharmaceuticals for Human Use).

1.34 "**Improvements**" means, (i) in relation to Marinus, any inventions, discoveries, improvements or enhancements relating to Licensed Product, whether patented, patentable or not, conceived or first reduced to practice during the term of this Agreement and any and all intellectual property rights therein and thereto and (ii) in relation to Company, any inventions, discoveries, improvements or enhancements made by or for Company in exercising its rights or performing its obligations under this Agreement or utilizing Marinus Confidential Information relating to Licensed Product, whether patented, patentable or not, conceived or first reduced to practice during the term of this Agreement and any and all intellectual property rights therein and thereto.

1.35 "**Initial Indications**" means cyclin-dependent kinase-like 5 (CDKL5) deficiency disorder, tuberous sclerosis complex (TSC), established status epilepticus (ESE), refractory status epilepticus (RSE) and status epilepticus (SE).

1.36 "**IND**" means an investigational new drug application filed with a Regulatory Authority which is required to commence human Clinical Studies in a country or Jurisdiction (such as an application for a Clinical Study authorization in the Territory), and all supplements, amendments and extensions that may be filed with respect to the foregoing.

1.37 "**Indemnified Party**" shall have the meaning given to it in Section 11.3.

1.38 "**Indemnifying Party**" shall have the meaning given to it in Section 11.3.

1.39 "**Infringement Claim**" shall have the meaning given to it in Section 7.2.

1.40 "**JSC**" shall have the meaning given to it in Section 3.2.

1.41 "**Know-How**" means any proprietary scientific or technical information, results and data of any type whatsoever, in any tangible or intangible form whatsoever, including databases, safety information, practices, methods, techniques, specifications, formulations, formulae, knowledge, know-how, skill, experience, test data including pharmacological, medicinal chemistry, biological, chemical, biochemical, toxicological and clinical test data, analytical and quality control data, stability data, studies and procedures, manufacturing process information and data (but only in the event of Product Localization) and development information, results and data.

1.42 "**Licensed Compound**" shall mean any of (a) Ganaxolone, (b) any next-generation formulation of Ganaxolone or any prodrug of Ganaxolone, (c) any back-up compounds included in the Licensed Program and (d) any solvates, hydrates, stereoisomers, metabolites, isomers, positional isomers, enantiomers, tautomers, polymorphs, salts, esters, analogs or derivatives of any of the compounds described in clauses (a), (b) or (c) above.

1.43 "**Licensed Product**" shall mean any oral or intravenous (IV) product, dosage or formulation that incorporates or is comprised of a Licensed Compound (alone or with one or more active ingredients) in the Field.

1.44 "**Licensed Program**" shall mean activities conducted by Marinus for development of Licensed Products. For clarity, such activities with respect to (i) formulations of Ganaxolone in clinical development at the Effective Date of this Agreement, (ii) each next generation formulation, (iii) each prodrug, shall be considered a separate Licensed Program.

1.45 "**Local Trademarks**" shall have the meaning given to it in Section 5.5.

1.46 "**Loss**" shall have the meaning given to it in Section 11.1.

1.47 "**Mainland China**" means People's Republic of China (or "**PRC**"), not including the Hong Kong Special Administrative Region, Macau Special Administrative Region, or Taiwan for the purpose of this Agreement.

1.48 "**Marinus Clinical Study**" shall mean any Clinical Study sponsored by or for the benefit of (a) Marinus, its Affiliates, or (b) any of their respective licensees or sublicensees to the extent that Marinus has rights to the data generated, in each case, (a) or (b), for a Licensed Product (in the Field and outside the Field), including, without limitation, all global Clinical Studies for Licensed Products.

1.49 "**Marinus IP**" shall mean all Marinus Patents, Marinus Know-How, Marinus Trademarks and Marinus Improvements.

1.50 "**Marinus Know-How**" shall mean any and all Know-How that is (a) Controlled by Marinus or its Affiliates as of the Effective Date or thereafter during the term of the Agreement and (b) is necessary or useful to Exploit the Licensed Compounds or Licensed Products in accordance with the terms of this Agreement, or (c) is required in order for Company to fulfill its obligations to Marinus in accordance with the terms of this Agreement. For clarity, the data package and know-how licensed to Marinus by Purdue Neuroscience Company ("Purdue") pursuant to the Amended and Restated Agreement between Marinus and Purdue effective May 23, 2008, is not included in Marinus Know-How.

1.51 "**Marinus Patents**" shall mean any and all Patents (a) Controlled by Marinus or its Affiliates as of the Effective Date or thereafter during the term of the Agreement and (b) cover, or are otherwise necessary or useful to Exploit, according to the terms of this Agreement, the Licensed Compounds or Licensed Products, including those Patents listed in **Schedule 1.51**.

1.52 "**Marinus Study Data**" shall mean any and all non-clinical, clinical, CMC, or other data related to Licensed Compounds or Licensed Products generated by or for Marinus.

1.53 "**Marinus Trademarks**" shall mean trademarks, trade names, service marks, slogans and logos developed by or on behalf of or Controlled by Marinus or its Affiliates as of the Effective Date or during the term of the Agreement and used or intended by Marinus to be used in connection with the Licensed Products and all intellectual property rights therein, including those listed in **Exhibit A**. Marinus Trademarks shall include English Trademarks.

1.54 "**NDA**" or "New Drug Application" shall mean a new drug application filed with the NMPA or other applicable Regulatory Authorities in the Territory, for the purposes of requesting marketing authorization of the applicable Licensed Product in the Territory.

1.55 **"NDA Approval**" shall mean all authorizations by the appropriate Regulatory Authorities necessary for commercial sale of a Licensed Product in the Territory including, without limitation and where applicable, approval of labeling, price, reimbursement and manufacturing.

1.56 "**Net Sales**" shall mean, with respect to any Licensed Product, the gross amounts invoiced by Company, its Affiliates, or sublicensees for sales of such Licensed Product to a Third Party, less the sum of the following items:

(a) taxes (including sales, excise taxes and custom duties) actually incurred, paid or remitted by the selling party, but not including income taxes;

(b) refunds, allowances or credit for returns or recalled Licensed Product, including those granted on account of price adjustments, rejected goods, and damaged goods and returns;

(c) normal and customary trade, quantity and cash discounts (including early payment discounts) actually allowed;

(d) costs of transportation, freight, postage and insurance allowances;

(e) bad debt written off under the applicable accounting standard, with reasonable collection efforts and added back if collected, [***]; and

(f) rebates actually paid to individuals, group purchasers, or wholesalers, administrative fees in lieu of rebates paid to managed care and similar institutions, chargebacks and retroactive price adjustments.

No deductions shall be made for commissions paid to individuals whether they are with independent sales agencies or regularly employed by Company, or its Affiliates and on its or their payroll, or for cost of collections. A Licensed Product shall be considered "sold" when billed out or invoiced. Sale or transfer to an Affiliate or sublicensee for re-sale by such Affiliate or sublicensee shall not be considered a sale for the purpose of this provision, but the resale by such Affiliate or sublicensee to a Third Party shall be a sale for such purposes.

No deduction will be made for any item of cost incurred by Company and/or its Affiliates or sublicensees except as permitted pursuant to clauses (a) to (e) above; provided that Licensed Products transferred in connection with Development or testing of a License Product or transferred to a entity in reasonable quantities in connection with Clinical Studies, patient assistance programs, compassionate use, expanded access programs, named patient sales, indigent programs, or promotional sampling, in each case, will not give rise to Net Sales, further provided that to the extent that Company or its Affiliates or sublicensees invoice amounts or receive payments in connection with compassionate use, expanded access programs, or named patient sales, the portion of such amounts in excess of Company's, its Affiliate's, or sublicensee's supply price paid for such Licensed Product shall be consider Net Sales. If a single item falls into more than one of the categories set forth in clauses (a)-(e) above, then such item may not be deducted more than once.

Net Sales will also include and be deemed to have been made with respect to any Licensed Product used by Company or any of its Affiliates, or sublicensees for its own commercial purposes, or transferred to any Third Party for [***]; and Net Sales in all such cases will be deemed to have been made at the prices therefor at which such Licensed Product is [***]. For clarity, in the event a Licensed Product is sold in an arms-length transaction to a governmental agency, a group purchase entity and/or any other entity having the bargaining power to negotiate the purchase price below normal retail price in transactions of lesser volume, Net Sales shall be calculated based on the actual price negotiated and agreed to for such agency and/or entity and not be based on the price charged in other arms-length sales transactions.

Where a Licensed Product is sold in the form of a combination product containing a Licensed Product that is co-formulated or co-administered with one or more active ingredients that is not a Licensed Compound (such other components, the "Combination Components" and such co-formulated or co-administered combination product, a "Combination Product"), the Net Sales applicable to such Combination Product shall be calculated by multiplying the total Net Sales of such Combination Product by the fraction A/(A+B), where A is the weighted average per unit sale price of the Licensed Product in the same dosage amount or quantities in the applicable jurisdiction during the applicable Calendar Quarter if sold separately, and B is the sum of the weighted average per unit sale price of the Combination Components, in the same dosage amount or quantities in the applicable jurisdiction during the applicable Calendar Quarter if sold separately. If A or B cannot be determined because values for the Licensed Product or Combination Components are not available separately in a particular jurisdiction, then Marinus and Company shall discuss an appropriate allocation for the fair market value of the Licensed Product and Combination Components to mutually determine in good faith Net Sales for the relevant transactions based on an equitable method of determining the same.

1.57 "**NMPA**" shall mean the National Medical Product Administration, formerly known as the China Food and Drug Administration, and local or provincial counterparts thereto, and any successor agency(ies) or authority thereto having substantially the same function.

1.58 "**Patent Challenge**" shall have the meaning given to it in Section 10.5.

1.59 "**Patents**" shall mean patents and patent applications, including improvement patents, continuations, continuations-in-part, divisions, provisionals, substitutions, patents of addition, reissues, reexamination, renewals or extensions thereof (including any supplemental patent certificates) and any confirmation patents or registration patent and all foreign counterparts of any of the forgoing, which are granted by competent authorities with claims generally directed to Licensed Product.

1.60 "**Person**" shall mean any individual, corporation, company, association, joint-stock company, trust, unincorporated organization or government or political subdivision thereof.

1.61 "**Product Localization**" shall mean localizing within the Territory particular supply, manufacturing or regulatory activities related to Licensed Product.

1.62 "**Rare Epilepsies**" shall mean an epilepsy disorder that with a prevalence of less than 1/5,000.

1.63 "**Regulatory Approval**" means, with respect to a particular regulatory jurisdiction, any approval of a marketing authorization application or other approval, product, or establishment license, registration, or authorization of any Regulatory Authority necessary

for the development, manufacture, use, storage, import, transport, promotion, marketing, distribution, sale, offer for sale, or other commercialization or exploitation of a pharmaceutical or biologic product in such regulatory jurisdiction, excluding reimbursement approvals.

1.64 "**Regulatory Authority**" means any applicable Governmental Authority with jurisdiction or authority over the development, manufacture, use, storage, import, transport, promotion, marketing, distribution, sale, offer for sale, or other commercialization or exploitation (including Regulatory Approval, pricing approval and reimbursement approval) of pharmaceutical or biologic products in a particular country or other regulatory jurisdiction, including the NMPA, and any corresponding national or regional regulatory authorities.

1.65 "**Regulatory Filing**" shall mean all applications, submissions, registrations or filings made for and procurement of Regulatory Approval, including but not limited to price reimbursement approval for the marketing and sale of Products from the relevant Regulatory Authorities.

1.66 "**Territory**" means the following, each a separate "Jurisdiction" for the purposes of this Agreement: Mainland China, the Hong Kong Special Administrative Region, Macau Special Administrative Region, and Taiwan.

1.67 "**Territory Development Plan**" shall mean the development plan for the Territory attached hereto as **Schedule 1.67**, as it may be amended from time to time.

1.68 "**Third Party**" shall mean any Person other than Marinus, Company or their respective Affiliates.

1.69 "**Upstream Licenses**" means any and all agreements between Marinus or any of its Affiliates, on the one hand, and any Third Party (the "Upstream Licensor"), on the other hand, pursuant to which Marinus has (a) in-licensed any Patent, Know-How, or trademarks owned or Controlled by such Third Party that are included as part of the Marinus IP, or (b) agreed to provisions that would require Company to make any payments (including royalties or as part of COGS) or to undertake or observe any restrictions or obligations with respect to the Exploitation of any Licensed Compound or Licensed Product in the Field.

1.70 "**USA**" shall mean the United States of America.

1.71 "**Valid Claim**" shall mean any claim of a pending Patent application (that has not been pending for more than [***] from its earliest priority date) or an issued and unexpired Patent, in each case, which (i) has not been found to be unpatentable, unenforceable or invalid by a court or other governmental agency of competent jurisdiction, in an unappealable decision or a decision that is no longer appealable because the time for filing an appeal has passed, (ii) has not been disclaimed or admitted to be unpatentable, invalid or unenforceable through disclaimer or otherwise (but not through a reissue while the reissue is pending), and (iii) provided that Regulatory Approval has been granted to one or

more Generic Versions of a Product, prevents the commercialization of each such Generic Version or blocks the entry of all Generic Versions in a jurisdiction in the Territory.

Article II
Grant of Rights

2.1 Grant to Company. Subject to the terms and conditions of this Agreement, Marinus hereby grants Company an exclusive license under the Marinus IP to (a) carry out its rights and obligations set forth in this Agreement, and (b) package, label, import, market, promote, use, sell, offer for sale, research, develop and commercialize (collectively, "Exploit," or "Exploitation") the Licensed Products in the Field in the Territory. Marinus retains all rights to Marinus IP except to the extent explicitly granted to Company hereunder. Company may sublicense (through multiple tiers) its rights under Marinus IP to (x) (i) Company Affiliates and (ii) Third Party contractors in connection with the performance of its obligations under this Agreement, in each case (i) or (ii) without requiring prior consent from Marinus, provided that the execution of an agreement or arrangement with any Affiliate or Third Party contractor shall not in any way diminish, reduce or eliminate any of Company's obligations under this Agreement, and Company shall remain primarily liable for such obligations; and (y) to other Third Parties only upon the prior written consent of Marinus, which consent shall not be unreasonably withheld, conditioned or delayed.

2.2 Disclosure of Know-How. Promptly following the Effective Date, and in any event no later than sixty (60) days from the Effective Date, Marinus shall disclose to Company all Marinus Know-How existing as of the Effective Date that is reasonably necessary for Company to conduct any required Clinical Studies or for obtaining and IND or NDA Approval and consultations with Regulatory Authorities.

2.3 Right of First Refusal. In the event Marinus its Affiliates, or any of their respective licensees or sublicensees intends to file or amend an IND to initiate one or more Marinus Clinical Studies intended to generate clinical data for any indication for a Licensed Product other than in an Initial Indication including [***], Marinus hereby grants Company a right of first refusal to Exploit such Licensed Product in the Territory in accordance with the terms of this Agreement and shall (a) promptly after developing the information required in such notice as set forth below, provide written notice to Company, which notice shall include in reasonable detail a description of the Clinical Study, including [***]which would be studied pursuant to such Clinical Studies, and a summary of any clinical data supporting Marinus' decision to pursue such Clinical Studies and any information and calculation that Marinus may have showing the reasons that Marinus believes it is commercially desirable to conduct such Clinical Studies, and (b) promptly after completion of a [***] provide Company with a notice of such completion which notice shall include [***] (the "[***]Notice"). Within [***], Company shall notify Marinus as to whether or not it is interested in amending this Agreement to include such new indication in the Field. If Company notifies such interest to Marinus, Company shall have the exclusive right to negotiate with Marinus, and the Parties shall have [***] days, from the date of Marinus'

receipt of Company's notice to negotiate in good faith an amendment to this Agreement for Company to obtain the right to commercialize Licensed Products in such indication, including conducting development as part of a global Marinus Clinical Trial, sharing of development costs and any additional milestone or other payments in respect of such indication. If either (x) Company does not notify Marinus of its interest within the time period set forth in this Section 2.4, or (y) if the Parties are not able to reach agreement on an amendment to this Agreement within such [***]day period and either Party terminates negotiations by notice to the other Party after [***] day period, then Marinus will have no further obligation to Company with respect to such indication of Licensed Product and Company will have no rights to commercialize Licensed Product for such indication in the Territory.

Article III
Territory Development

3.1 Know-How Transfer Support. During the term of the Agreement, Marinus shall promptly provide Company with additional Marinus Know-How, to the extent such Marinus Know-How comes to Marinus' attention (or are reasonably requested by Company) and have not previously been provided to Company. Marinus shall provide reasonable consultation or other assistance as Company may reasonably request to assist Company in becoming familiar with such Know-How in order for Company to undertake Exploitation of the Licensed Products in the Territory or to fulfill its obligations to Marinus, provided that, Company shall reimburse Marinus for all reasonable out-of-pocket external costs reasonably incurred by or on behalf of Marinus in connection with such assignments, consultation and assistance within [***] after receiving Marinus' invoice therefor.

3.2 Joint Steering Committee. Except as provided below, Company shall have sole authority and control over all matters relating to Exploitation of the Licensed Products in the Territory (including regulatory strategy and execution within the Territory with respect to the Licensed Product), subject to the oversight of the JSC and its subcommittees as set forth in this Section 3.2. Promptly after the Effective Date, the parties shall form a Joint Steering Committee ("JSC"), which shall consist of [***] representatives of each Party, with expertise in such disciplines as clinical development, regulatory affairs, marketing and commercialization. The JSC may, in its discretion, establish (i) a joint development committee (JDC) to leverage synergies, oversee the Development Plan and exchange information with respect to the global development of Licensed Products and (ii) other subcommittees as it deems to be necessary or desirable. The JSC shall be responsible for (a) reviewing and discussing the overall strategy for the Exploitation of the Licensed Products in the Field in the Territory, (b) providing a forum for the discussion and coordination of the Parties' activities pursuant to this Agreement, (c) directing and overseeing the operation of the JDC and any other joint subcommittee established by the JSC, including resolving any disputed matter of the JDC or other subcommittees. The Development Plans and any amendments thereto and final protocol design shall be subject to the review and approval of the JSC or, at the discretion of the JSC, the JDC. If formed

by the JSC, the JDC's responsibilities shall include (a) providing a forum for information sharing, including planned development activities, and communications between the Parties with respect to the development of the Licensed Products in their respective territories; (b) reviewing, discussing and approving the Development Plan and amendments thereto, (c) discussing and determining, with respect to each global Clinical Study for Licensed Product and on an indication-by-indication basis, whether to include clinical sites in the Territory in such Clinical Study and whether to jointly participate in such Clinical Study, and (d) performing such other functions as may be appropriate to further the purposes of the development of the Licensed Products in the Territory, as directed by the JSC. All decisions of the JSC shall be made by unanimous vote, provided, however, that any dispute shall be resolved in the following manner: (a) appropriate senior officers of Marinus and Company shall meet within [***] days and attempt to resolve the dispute, and (b) if the dispute cannot be resolved by the senior officers, then Company shall have the final decision-making authority with respect to all matters pertaining solely to the Exploitation of Licensed Products in the Field in the Territory, provided that Marinus will have final decision-making authority with respect to all matters determined in good faith by Marinus to be reasonably expected to have a material adverse effect on Marinus' clinical development, registration or commercialization of Licensed Compound (including Licensed Products); and further provided that Company will, acting in good faith, use Commercially Reasonable Efforts to align, where practicable, its Exploitation plan pertaining to the applicable Licensed Product within the Territory with the global commercialization plan of Marinus for the same Licensed Product. The JSC and JDC (if established) shall meet regularly (but in no event less than semi-annually) at such times and locations and in a manner as shall be mutually agreed by the parties. The JSC and JDC shall review and oversee the Development Plan, including protocol design, conduct and monitoring of Clinical Studies in the Territory, regulatory strategy and activities of the Parties to apply for and obtain Regulatory Approvals. The JSC shall review and discuss the commercialization of Products after Regulatory Approval in the Territory. The Parties will use Commercially Reasonable Efforts to coordinate commercialization activities to facilitate consistent marketing, promotional, positioning, and sales approaches with those in other major markets outside the Territory. At least [***] prior to each regularly scheduled meeting of the JSC or JDC, each Party shall provide a proposed agenda of matters to be addressed at the meeting. Minutes shall also be provided of each meeting of the JSC or JDC, which shall include at a minimum: [***].

As soon as reasonably practicable after the Effective Date, each Party will provide the other, in writing, with the name of its "Alliance Manager." The Alliance Managers will act as the primary liaison in coordinating the activities under this Agreement including facilitating the communications of the Parties, the JSC and the JDC.

3.3 Development Plan. Within [***] after the Effective Date, the Company shall submit an initial Development Plan to the JSC for its consideration. The JSC or JDC will review and agree on the suitability of any amendments proposed to be made to the Development Plan, which will provide that (a) Company will run separate Company Clinical Studies in the

Territory (separate from a global or other Marinus Clinical Studies to be conducted in whole or in part in the Territory) specifically as required to support Regulatory Filings in the Territory and will be responsible for managing study operations, qualifications and compliance and submission of clinical study permission and other documentation, and (b) Company shall act as Marinus' regulatory agent in the Territory for all Company Clinical Studies and be responsible for managing interactions with Regulatory Authorities in the Territory, subject to consultation with and participation of the JSC or JDC.

Each Party shall be responsible for costs associated with completing their corresponding activities in the Development Plan, including conducting Clinical Studies.

3.4 Regulatory Approvals. Company shall be responsible for the preparation of regulatory documents and any other studies, including Company Clinical Studies, necessary to achieve Regulatory Approvals in the Territory. Prior to Product Localization, all Regulatory Approvals filed by Company in the Territory shall be in the name of and owned by Marinus, including PRC import drug licenses, except to the extent required by applicable law such Regulatory Approvals shall be in the name of and owned by Company.

3.5 Product Localization. The Parties agree that Product Localization of a Licensed Product in the Territory may occur if and when they mutually agree that it would be desirable due to then prevailing market conditions or regulatory requirements within the Territory. The JSC (or JDC if formed) shall make all decisions with respect to Product Localization by unanimous agreement. The Parties agree that they do not envision pursuing any consideration of Product Localization of any particular Licensed Product prior to Regulatory Approval being granted in the Territory for such Licensed Product as an imported drug product and availability to the Parties of commercially sufficient manufacturing capacity and infrastructure. Upon mutual agreement of the Parties to pursue Product Localization with respect to a particular Licensed Product, (i) the license grant under Section 2.1 of this Agreement will automatically be amended to provide Company with appropriate manufacturing and other rights within the Territory as are necessary to accomplish the Product Localization, (ii) Marinus will undertake to complete any additional technology transfer and transition activities as are necessary to enable Company to pursue such Regulatory Approvals as are required for Product Localization and to commercially launch such Licensed Product as a domestic drug product (iii) if required by applicable law, Marinus shall assign to Company all Regulatory Filings in the Territory related solely to the applicable localized Licensed Products in the Territory and (iv) if applicable law requires that for a localized Licensed Product an entity incorporated in the Territory serve as the regulatory sponsor and market authorization holder, Company will serve as the regulatory sponsor and market authorization holder within the Territory in relation to such localized Licensed Product to be manufactured in the Territory.

3.6 Engagement of Third Parties. In the course of performing its development obligations under this Agreement, if Company engages Affiliates or Third Parties to perform certain development activities, Company shall (i) inform Marinus of the use and identity of Third

Parties for the relevant activity, and (ii) obtain contractual undertakings from such Third Party including in particular with respect to quality assurance, compliance with law, and confidentiality which are customary in the pharmaceutical industry for the relevant activity. All development and regulatory activities shall be conducted either directly by Company, a qualified subcontractor of Company (such as a contract research organization), by an Affiliate of Company, or a sublicensee. For clarity, Marinus will manage Clinical Study sites within the Territory for Marinus Clinical Studies conducted outside the Field, provided that in the event that Marinus wishes to conduct such a Clinical Study, Marinus shall notify Company in writing prior to conducting such Clinical Study and the Parties shall discuss and coordinate regulatory activities relating to such Clinical Study on an ongoing basis.

3.7 Development Information.

(a) General. Each Party will be entitled to (i) receive, keep and use for regulatory and commercialization purposes all clinical protocols, registration applications, and other substantive regulatory documents including, but not limited to, all toxicological and clinical data, and (ii) access and reference all regulatory dossiers and filings, produced by the other Party and its Affiliates, and to the extent the other Party has rights to provide it, by licensees, sublicensees and sub-contractors, pertaining to a Licensed Product. Each Party shall retain ownership and all intellectual property rights to clinical data generated by or for it. For clarity, to the extent permitted under applicable law, Company shall provide to Marinus all Company Development Data and Marinus, including its Affiliates and sublicensees, will have the exclusive right to use and reference such data for development, regulatory and commercialization purposes outside the Territory. For clarity, to the extent permitted by applicable law, Marinus shall provide to Company all Marinus Study Data and Company, including its Affiliates and sublicensees, will have the exclusive right to use and reference such data for Exploitation of Licensed Products in the Territory. For clarity, Company shall only have the right to use all such materials in the Territory and shall not transfer any such materials, other than to Marinus, for use outside the Territory. Marinus shall keep Company reasonably informed of the status of Regulatory Authority review and approval of Regulatory Filings controlled by Marinus with respect to the Licensed Products in the Field outside the Territory and provide all such documents to Company reasonably promptly after Marinus comes to possess such documents. In addition, Marinus undertakes to cooperate with Company's efforts to maximize the scope and/or period of data exclusivity as permitted by the relevant Regulatory Authorities (in particular NMPA) with respect to Licensed Products in the Territory. Upon a Party's request, the other Party shall execute and submit such letters of authorization or other instruments as are necessary to effect such rights of reference.

(b) Company Regulatory Communications and Filings. With respect to Company Clinical Studies, Company shall (i) permit Marinus to participate with Company in material communications with regulatory officials in the Territory, (ii) immediately (but in no case later than [***] after receipt) inform Marinus, and provide copies to Marinus of any material regulatory communications received by Company, (iii) provide an English summary of final draft Regulatory Filings and an English translation of that portion of such final draft Regulatory Filings solely developed and prepared by Company (the "Independent Portion") to Marinus for review and comment, which shall be provided within [***], provided that for a final draft clinical study report or protocol, Company shall provide the English translation of the Independent Portion within [***] of the submission of such report or protocol, (iv) Company shall incorporate any comments received from Marinus on draft Regulatory Filings where required under any applicable law and shall consider in good faith any other comments received from Marinus on such Regulatory Filings (v) provide Marinus with a copy of the final Regulatory Filings within [***] of the submission.

With respect to Company Clinical Studies, Company shall (i) inform Marinus during the JSC meetings prospective material communications with regulatory officials in the Territory and (ii) consider in good faith Marinus' input in preparation for such material communications.

(c) Notice of Pharmaceutical Side-Effects.

(i) Marinus and Company will enter into a pharmacovigilance agreement pursuant to which each Party will provide to each other the information necessary to monitor the safety of Licensed Products and to meet in a timely manner, for all countries where either Party has responsibility for the Licensed Products, all regulatory requirements for reporting adverse reactions and adverse events. Prior to the enrollment of the first subject in the first Company Clinical Study in the Territory, the Parties will enter into a pharmacovigilance agreement and quality agreement under usual and customary industry terms. In the event that Company at any time discontinues development of a Licensed Product in the Territory, to the extent permitted under applicable law, Company shall promptly provide to Marinus a copy of all Company Development Data related to that Licensed Product. In order to facilitate data exchange, the Parties will enter into a data agreement within [***] after the Effective Date.

(ii) Each Party shall forward to the other on a regular basis information on adverse events and any material difficulties associated with clinical use, studies, investigations, tests and prescriptions of Licensed Products.

(iii) For the purposes of this Agreement, definitions stated in the ICH documents from E2A (Clinical Safety Data Management Definitions and Standards for Expedited Reporting) and E2C (Clinical Safety Data Management Periodic Safety Update Report for Marketed Drugs) will be used.

(iv) The Parties will exchange expedited and non-expedited case reports from all sources, changes in product labeling, actions taken by local regulators and other information pertinent to human safety of the Licensed Products. The Parties further agree to prepare a mutually agreeable procedure for sharing safety data.

(d) Compliance with ICH and GCP. Company and Marinus agree to comply with ICH and GCP guidelines. Company will notify Marinus within [***] of learning that a participant in a Company Clinical Study has failed to comply with GCP standards and will, within [***] after such notice agree upon a plan for audit and GCP breach notifications (if needed) to Regulatory Authorities in and outside the Territory, all as defined in the Quality Agreement.

3.8 Efforts in Development and Commercialization. During the term of this Agreement, Company will use Commercially Reasonable Efforts to obtain Regulatory Approvals for, and after Regulatory Approval, commercialize, [***] in Mainland China.

Article IV
Manufacture and Supply

4.1 Manufacture of Product Candidates and Products. Except to the extent otherwise specifically required by law or government regulations, Marinus will be responsible for timely manufacturing and supplying Licensed Product as necessary for the conduct of Company Clinical Studies and for all commercial purposes in the Territory and Company will purchase all of its clinical and commercial requirements thereof from Marinus.

4.2 Terms of Supply. Marinus will supply to Company Licensed Products pursuant to a written clinical supply agreement and commercial supply agreement to be agreed by the Parties in good faith. All Licensed Products will be supplied [***], provided that, Marinus shall be responsible for completing relevant export clearance procedures and Company shall be responsible for completing relevant import clearance procedures, further provided, that upon the responsible Party's reasonable request, the other Party shall provide timely support, as reasonably needed, to the responsible Party with respect to export or import clearance, as applicable, of the supplied Licensed Product. The Parties will (a) exercise their Commercially Reasonable Efforts to agree on all other terms and conditions of supply of such Licensed Products, (b) agree upon and enter into a written clinical supply agreement including all such additional terms and conditions shall be agreed by the Parties no later than [***] after the Effective Date; and (c) agree upon and enter into a written commercial supply agreement including all such additional terms and conditions shall be agreed by the Parties no later than [***].

(a) Development Supply: Marinus will supply Licensed Product to Company for all Company Clinical Studies (set forth in the Development Plan that has been approved by the JSC) at [***]. Licensed Product may be supplied as labelled for use or un-labelled primary packaged (bulk supply) upon mutual agreement of both Parties.

(b) Commercial Supply: Commercial Licensed Products will be supplied by Marinus at [***]. Marinus shall invoice Company for the applicable Licensed Product upon delivery in accordance with the supply agreement. Company shall, subject to the terms of the supply agreement, pay the invoiced amounts with respect to such Licensed Product within the timeframe set forth in the supply agreement. Marinus shall supply to Company free-of-charge reasonable and customary quantities of Licensed Products (to be agreed in good faith and in writing by the Parties) for quality assurance purposes and, as agreed by the Parties and to the extent allowed under applicable laws, for use as samples and donations, provided that Company will be responsible for [***]. Licensed Products may be supplied as labelled for use in secondary packaging or un-labelled primary packaged (bulk supply) upon mutual agreement of both Parties.

4.3 Commercial Supply Price Adjustment. [***].

Article V
Commercialization.

5.1 Commercialization Plan. Company shall be responsible for and will use Commercially Reasonable Efforts to commercialize in each Jurisdiction in the Territory each Licensed Product in the Field for which the applicable Regulatory Approval has been granted in such Jurisdiction in the Territory. Company will conduct all commercialization (including compassionate and named patient use programs) of the Licensed Products in the Territory in accordance with the Commercialization Plan for such Licensed Products, at its sole cost and expense, and subject to the terms of this Agreement. Without limiting the foregoing, Company shall achieve the First Commercial Sale of a Licensed Product in Mainland China within [***] after obtaining Regulatory Approval for such Product in Mainland China. Within [***] after submission of an NDA with the NMPA or applicable Regulatory Authority in the Territory, Company shall provide to the JSC a draft Commercialization Plan for review and comment.

5.2 Marketing and Promotion. Company shall have the right to market, sell and distribute Licensed Products in the Field in the Territory. Under no circumstances will Company promote or sell Licensed Products for off label use or seek labeling that describes, names or lists any use outside of the Field, or distribute or sell outside the Territory to sell or transfer to Third Parties for sale or distribution outside of the Territory.

5.3 Competing Products. During the term of this Agreement, each Party agrees that it will not, independently or with or through a Third Party (including through the grant of any license

or similar rights to a Third Party), develop or commercialize any Competing Product (other than in the case of Company, a Licensed Product) in the Territory. For purposes of this Section 5.3, "Competing Product" shall mean any product, substance or formulation that [***].

5.4 Product Markings. Each Licensed Product marketed and sold by Company under this Agreement shall be marked (to the extent not prohibited by law or regulation): (a) with a notice that such Licensed Product is sold under a license from Marinus and (b) with all Patents and other intellectual property notices relating to Marinus IP as may be required by applicable law.

5.5 Trademarks. Marinus shall cooperate with Company to develop and register trademarks for Licensed Products in the Territory, and Marinus shall own all such trademarks in English ("English Trademarks") and Company shall own all such trademarks in Chinese/other local languages ("Local Trademarks") in the relevant jurisdiction in the Territory. Company acknowledges that, to facilitate worldwide brand recognition, Company may, to the extent practicable in Company's sole discretion, market and sell the Licensed Products under the English Trademarks and/or the same trademarks as those used by Marinus outside the Territory, together with the applicable Local Trademarks in the Territory.

5.6 Commercialization Efforts

(a) For each Calendar Year following the First Commercial Sale of a Licensed Product in the Territory, no later than January 31st of the following Calendar year, Company will provide to Marinus a report summarizing the commercialization activities performed by or on behalf of Company and its Affiliates and distributors in the Territory for such Licensed Product. Each such report will contain sufficient detail to enable Marinus to assess Company's compliance with its commercialization diligence obligations set forth in this Agreement.

(b) Availability to Public. Following the First Commercial Sale of each Licensed Product in a Jurisdiction and until the expiration or termination of this Agreement, Company shall use Commercially Reasonable Efforts to cause the Licensed Product to be reasonably available to the public in such Jurisdiction.

(c) Pricing and Product Distribution. Company shall set prices for the Licensed Products in the Territory and shall obtain all pricing approvals as may be required and all reimbursement approvals as may be desirable and required. Company shall also be responsible for final packaging and distribution of the Licensed Products in the Territory.

(d) Each Party agrees that it will not, and will ensure that its Affiliates, subcontractors and distributors will not, either directly or indirectly, promote, market, distribute, import, sell, or have sold any Licensed Products to any Third Party or to any address

or Internet Protocol address or the like in the other Party's territory, including via the Internet or mail order. Neither Party will engage, nor permit its Affiliates or distributors/subcontractors to engage, in any advertising or promotional activities relating to any Licensed Products for use directed primarily to customers or other buyers or users of the Licensed Products located in any country or jurisdiction in the other Party's territory, or solicit orders from any prospective purchaser located in any country or jurisdiction in the other Party's territory. If a Party or its Affiliates or distributors/subcontractors receive any order for any Licensed Products from a prospective purchaser located in a country or jurisdiction in the other Party's territory, then such Party will immediately refer that order to the other Party and will not accept any such orders.

5.7 Standard of Conduct. Company shall perform all its commercialization activities in the Territory in an ethical manner and in compliance with applicable laws.

Article VI
Payments

6.1 Up-Front Fee. Within [***] after the Effective Date, Company will pay, or cause to be paid, to Marinus the sum of Ten Million Dollars ($10,000,000).

6.2 Milestone Payments by Company.

(a) Milestone Payments. Company will make the following payments to Marinus.

Development Milestones	Payment
[***]	$[***]
[***]	$[***]
[***]	$[***]
[***]	[***]

Commercialization Milestones – to be paid when cumulative Net Sales of specified Licensed Products in a Calendar Year in the Territory first achieve the applicable milestone.	
[***]	
[***]	$[***]

[***]	$[***]
[***]	$[***]
[***]	
[***]	$[***]
[***]	$[***]
[***]	$[***]
[***]	
[***]	$[***]
[***]	$[***]
[***]	$[***]

(b) Timing. For clarity, it is possible for more than one milestone to be achieved within a single Calendar Year and Company shall pay Marinus for all milestones achieved in accordance with the terms of this Agreement.

(c) Payments. Company shall notify Marinus promptly upon the achievement of each Development Milestone or Commercialization Milestone. After the receipt of each such notice provided by Company, Marinus shall submit to Company an invoice for the amount of milestone payment payable thereon. Company shall pay to Marinus the applicable milestone payment within [***] after receipt of the invoice from Marinus.

(d) All milestone payments are non-creditable and non-refundable and shall be payable only once upon the initial achievement of such milestone event and no amounts shall be due hereunder for subsequent or repeated achievement of such milestone, regardless of how many times such milestone event is achieved or the number of Licensed Products that achieves such milestone event.

6.3 Royalties.

(a) Earned Royalties. Company will book all sales of all Licensed Products in the Territory and will report those sales to Marinus as specified in this Section 6.3. During the applicable Royalty Term, Company shall pay Marinus tiered royalties as calculated by multiplying the applicable royalty rate set forth in the tables below by the corresponding amount of incremental, aggregated annual Net Sales on a

Licensed Product-by-Licensed Product and jurisdiction-by-jurisdiction basis in the Territory in a Calendar Year, on annual Net Sales of each Licensed Product, as follows:

[***]	
Annual Net Sales	Royalty Rate
Up to $[***] million	[***]%
$[***] million to $[***] million	[***]%
Above $[***] million	[***]%

[***]	
Annual Net Sales	Royalty Rate
Up to $[***] million	[***]%
$[***] million to $[***] million	[***]%
Above $[***] million	[***]%

[***]	
Annual Sales	Royalty Rate
Up to $[***] million	[***]%
$[***] million to $[***] million	[***]%
Above $[***] million	[***]%

(b) Royalty Term. Royalties shall be payable by Company, on a Licensed Product-by-Licensed Product and Jurisdiction-by-Jurisdiction basis, on (i) all Net Sales of Licensed Products for compassionate use, expanded access programs or named patient sales (in each case, in accordance with Section 1.56), and (ii) on all other Net Sales of Licensed Products from the First Commercial Sale of each Licensed Product in such Jurisdiction, until the later to occur of (x) the tenth (10th) anniversary of the date of First Commercial Sale of such Licensed Product in such Jurisdiction, (y) the expiration of the last Valid Claim included within the Marinus

Patents which covers the Licensed Product in such Jurisdiction, or (z) the expiration of all (if any) regulatory exclusivities for the Licensed Product in such Jurisdiction (the "Royalty Term").

6.4 Taxes. Each Party shall be solely responsible for the payment of all taxes imposed on its income arising from activities of such Party under this Agreement. The Parties agree to cooperate with one another and use reasonable efforts to avoid or reduce tax withholding or similar obligations in respect of upfront fees, royalties, milestone payments, and other payments made by Company to Marinus under this Agreement. If withholding taxes are imposed on any such payment, Company shall deduct such taxes from the payment made to Marinus. Each Party shall provide the other with reasonable assistance to enable the recovery, as permitted by applicable laws, of withholding taxes or similar obligations resulting from payments made under this Agreement, such recovery to be for the benefit of the Party bearing such withholding tax under this Section 6.4.

6.5 Blocked Currency. If by applicable laws in a Jurisdiction in the Territory, conversion into Dollars or transfer of funds of a convertible currency to the USA becomes materially restricted, forbidden or substantially delayed (collectively, "Restricted"), then Company shall promptly notify Marinus and, thereafter, such Restricted amounts accrued in such Jurisdiction under this Article VI shall be paid to Marinus (or its designee) in such Jurisdiction in local currency by deposit to an account in a local bank designated by Company and to the credit of Company, unless the Parties otherwise agree.

6.6 Royalty Payments and Reports. Beginning with the First Commercial Sale of a Licensed Product, royalties shall accrue on a Calendar Quarter basis, and Company shall within [***] after the end of each Calendar Quarter provide Marinus with a report summarizing the number, description, and aggregate sales of all Licensed Products made and the royalty payable thereon according to Section 6.3, including a description of any offsets or credits deducted from such sales, on a Licensed Product-by-Licensed Product basis during the relevant Calendar Quarter. After the receipt of each royalty report provided by Company, Marinus shall submit to Company an invoice for the amount of royalty payable thereon. Company shall pay to Marinus the royalties for such Calendar Quarter within [***] after receipt of the invoice from Marinus. For any given royalty period, Company shall pay to Marinus the royalty as calculated by multiplying the applicable royalty rate set forth in the Section 6.3 by the corresponding amount of incremental, aggregated annual Net Sales. For clarity, for the royalty payments in each Calendar Quarter, the tiered royalty rates list above apply such that the higher tiered royalty rates only apply to the portion of Net Sales in such Calendar Year that exceed the top threshold of the previous tier. Each statement provided at the end of the fourth quarter of each Calendar Year shall contain a reconciliation of actual royalty payments made during that year and the amount actually owed for such Calendar Year, and if after such reconciliation, Company determines there was an underpayment, the resulting owed amount shall be paid to Marinus at the time of such fourth quarter statement, in accordance with the terms of this Section 6.6. If after such reconciliation, Company determines there was an overpayment, Company may either

deduct such overpayment from the royalty payment due to Marinus for the next Calendar Quarter or Marinus shall pay to Company the amount of such overpayment within [***] after receiving Company's invoice therefor.

6.7 Currency; Exchange Rate. All amounts paid under this Agreement shall be paid in Dollars to Marinus by wire transfer to a financial institution to be designated by Marinus. Net Sales by Company or its Affiliates or sublicensees of Licensed Products sold in a currency other than Dollars shall be converted into Dollars using the selling rate of exchange for the currency in which the Net Sales were made as published by the Wall Street Journal, New York, N.Y., U.S.A., for the last Business Day of the quarterly period during which such Net Sales were made. All payments made by Company to Marinus for any reason, including without limitation, milestones, royalties, up-front payments, or payments for supply of Licensed Products, shall be made to Marinus by Company.

6.8 Accounting. Company agrees to keep and maintain such records as it normally generates in the ordinary course of its business for a period of [***] showing the sale, use, and other disposition of Licensed Products sold or otherwise disposed of under the license herein granted. Such records shall be kept in sufficient detail to enable the royalties payable hereunder to be determined. Company further agrees to permit its books and records to be examined by an independent certified public accountant selected by Marinus, at ordinary business hours with reasonable prior notice to Company and consent by Company (not to be unreasonably withheld or delayed), and not more than [***], to the extent necessary to verify reports provided for in Section 6.6. Such examination is to be made under appropriate confidentiality restrictions, at the expense of Marinus, except in the event that the results of the audit reveal an underreporting of royalties due Marinus of [***] or more in any Calendar Year, then the audit costs shall be paid by Company.

6.9 Interest Due. In case of any delay in payment by Company to Marinus not occasioned by Force Majeure, interest on the overdue payment shall accrue at an annual interest rate, compounded annually, equal to [***]. The foregoing interest shall be due from Company without any special notice and shall be in addition to any other remedies that Marinus may have pursuant to this Agreement.

6.10 Generic Version of a Product. [***].

Article VII
Intellectual Property

7.1 Patentable Inventions and Know-How.

(a) Ownership of intellectual Property. Except as otherwise specifically set forth in this Article VII, any intellectual property including but not limited to data, technical information, methods, processes, techniques, know-how, experience, inventions, discoveries, trade secrets, compositions of matter and methods, whether or not patentable or confidential, and materials, characterized, conceived, developed,

derived, discovered, generated or identified during the term of and pursuant to the activities conducted under or pursuant to this Agreement and that is solely conceived and/or developed by employees or representatives of a single Party shall be owned by that Party. Inventions jointly conceived and/or developed by Marinus and Company will be jointly owned by Marinus and Company and, subject to the terms of this Agreement, each shall have rights to practice such inventions and the intellectual property associated therewith without need for further consent or approval of the other Party. For clarity, inventorship and ownership for the purpose of patent applications shall be determined in accordance with the patent law of the jurisdiction in which patent applications based on the invention are filed.

(b) Improvements. Each Party shall keep the other Party fully advised of any Improvements, inventions and know-how relating to any Licensed Product, made by or for that Party or its Affiliates during the term of this Agreement. Company grants to Marinus a paid-up, non-exclusive license to practice Company Improvements for the development and commercialization of Licensed Products outside the Territory and for the manufacture thereof inside and outside the Territory.

(c) Patent Prosecution.

(i) Company. Company shall be responsible, at its own expense, for the preparation, filing, prosecution and maintenance of all Patents filed for Company Inventions. All patent applications for Company Inventions shall be filed under the name of Company. Company shall permit Marinus to review and comment on any documents with the relevant patent authorities with respect to Company Inventions and Company Patents. Company grants to Marinus a paid-up non-exclusive license to practice Company Patents for the development and commercialization of Licensed Products outside the Territory and for the manufacture thereof inside and outside the Territory.

(ii) Marinus. Marinus shall be responsible, at its own expense, for the preparation, filing, prosecution and maintenance of all Marinus Patents and jointly owned Patents. During the term of this Agreement, Marinus will use Commercially Reasonable Efforts to cause each patent application within the Marinus Patents which exist as of the Effective Date in Mainland China to be granted. Marinus shall permit Company to review and comment on any documents with the relevant patent authorities with respect to Marinus Patents or any jointly owned patents.

(iii) Company Discontinuance. If Company does not intend to file for Patent protection or files for Patent protection for an Company Invention (on a country-by-country basis) or does not wish to continue preparation, prosecution, or maintenance of a Company Patent, then it shall give at least

[***] advance notice, and in no event less than a reasonable period of time for Marinus to act in its stead, to Marinus of any decision to cease preparation, filing, prosecution and maintenance of that Patent in any jurisdiction (a "Company Discontinued Patent"). In such case, Marinus may elect at its sole discretion to continue preparation, filing and prosecution or maintenance of the Company Discontinued Patent at its sole expense. Discontinuance may be elected on a country-by-country basis or for a patent application or patent series in total.

(iv) Marinus Discontinuance. If Marinus does not intend to continue preparation, prosecution, or maintenance of a Marinus Patent, then it shall give at least [***] advance notice, and in no event less than a reasonable period of time for Company to act in its stead, to Company of any decision to cease preparation, filing, prosecution and maintenance of that Patent in any jurisdiction (a "Marinus Discontinued Patent"). In such case, Company may elect at its sole discretion to continue preparation, filing and prosecution or maintenance of the Marinus Discontinued Patent at its sole expense. Discontinuance may be elected on a country-by-country basis or for a patent application or patent series in total.

(d) Cooperation. Each Party will consult with the other Party and will keep the other Party continuously informed of all matters relating to the preparation, filing, prosecution and maintenance of Company Patents and Marinus Patents covered by this Agreement.

(i) Company shall endeavor in good faith to coordinate its efforts with those of Marinus to minimize or avoid interference with the prosecution of Marinus Patents.

(ii) To the extent practicable, Company shall provide Marinus with a copy of any Company patent application relating to a Licensed Product, prior to filing the first of such applications in any jurisdiction, for review and comment by Marinus or its designees.

(iii) Company shall provide, at Marinus' reasonable request, copies of all material correspondence with the relevant patent office.

(iv) To the extent practicable, Marinus shall provide Company with a copy of any patent applications within the Marinus Patents, prior to filing the first of such applications in any Jurisdiction, for review and comment by Company or its designees.

(v) Marinus shall provide, at Company's reasonable request, copies of all material correspondence with the relevant patent office.

7.2 Infringement Claims by Third Parties.

(a) Notice. If the manufacture, use or sale of Licensed Product under the Marinus Patents or the Company Patents results in a claim or a threatened claim by a Third Party against a Party hereto for patent infringement or for inducing or contributing to patent infringement ("Infringement Claim"), the Party first having notice of an Infringement Claim shall promptly notify the other in writing. The notice shall set forth the facts of the Infringement Claim in reasonable detail.

(b) Third Party Licenses. In the event that practicing under the Marinus Patents in connection with use or sale of the Product in the Territory would infringe a Third Party Patent and a license to such Third Party Patent is available, the parties agree:

(i) Each Party will bear its own costs associated with acquiring any Third Party license that is required to practice any of the Marinus Patent in the Territory; and

(ii) Marinus will use Commercially Reasonable Efforts to obtain required licenses under the Third Party's Patents, with a right to sublicense to Company, under reasonable terms mutually acceptable to both Parties and taking into account any views and comments provided to Marinus by Company.

7.3 Infringement Claims against Third Parties. Each Party shall promptly inform the other of any suspected infringement of any Marinus Patent(s) by a Third Party. Company or Marinus shall have the right to institute an action for infringement of the Marinus Patent(s) in the Territory against a Third Party in accordance with the following:

(a) If the Parties agree to institute suit jointly, the suit shall be brought in both their names, the out-of-pocket costs thereof shall be borne equally and any recovery or settlement shall be shared fifty percent to Marinus and fifty percent to Company. Marinus and Company shall work together to manage such litigation with Company having the primary responsibility for controlling such suits. Marinus may, if it so desires, also be represented by separate counsel of its own selection, the fees for which counsel shall be paid by Marinus.

(b) In the absence of an agreement to institute a suit jointly as provided in (a) above, Company shall have the first right, but not the obligation, to institute suit and, at its option, let Marinus join as a party plaintiff. Company shall bear the entire cost of such litigation and shall be entitled to retain the entire amount of any recovery or settlement.

(c) In the absence of an agreement to institute a suit jointly as provided in (a) above, and if Company notifies Marinus that it has decided not to institute a suit as provided in (b) above, Marinus may institute suit, and, at its option, let Company

join as a party plaintiff. Marinus shall bear the entire cost of such litigation including attorneys' fees and shall be entitled to retain the entire amount of any recovery or settlement.

(d) Should either Party commence a suit under the provisions of Section 7.3 and thereafter elect to abandon the same, it shall give timely notice to the other Party who may, if it so desires, continue prosecution of such suit, provided, however, that the sharing of expenses and any recovery in such suit shall be agreed upon between the Parties in advance of such continuation.

7.4 Patent Term Extensions. The Parties shall cooperate in good faith with each other in gaining patent term extension wherever applicable to Marinus Patents and Company Patents covering Licensed Products.

(a) Company and Marinus shall each determine which of its Patents shall be extended.

(b) All filings for such extension shall be made by the Party responsible for prosecution and maintenance of the Patent, provided, however, that in the event that the Party who is responsible for prosecution and maintenance of the Patent elects not to file for an extension, such Party shall (i) inform the other Party of its intention not to file, and (ii) grant the other Party the right to file for such extension.

Article VIII
Representations, Warranties and Covenants

8.1 Marinus represents, warrants and covenants:

(a) Authorization. Marinus is duly organized and validly existing under the laws of the country of its incorporation. This Agreement has been duly executed and delivered by Marinus and constitutes the valid and binding obligation of Marinus, enforceable against Marinus in accordance with its terms except as enforceability may be limited by bankruptcy, fraudulent conveyance, insolvency, reorganization, moratorium and other laws relating to or affecting creditors' rights generally and by general equitable principles. The execution, delivery and performance of this Agreement have been duly authorized by all necessary action on the part of Marinus, its officers and directors.

(b) Marinus IP. Marinus has the right under the Marinus IP to grant the licenses to Company as purported to be granted pursuant to this Agreement.

(c) No Conflict. The execution, delivery and performance of this Agreement by Marinus does not conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it may be bound, and, to its knowledge, does not violate any material law or regulation of any court, governmental body or administrative or other agency having authority over it. Marinus has not granted

(and shall not grant) any right to any Third Party under the Marinus IP that would conflict with the rights granted to Company hereunder.

(d) Information. All information and data disclosed to Company by Marinus relating to the Licensed Product, and the information and data generated in the performance by or on behalf of Marinus of the development activities relating to the Licensed Product before the Effective Date is, at the time of its disclosure to Company, true and accurate in all material respects.

(e) No Infringement. To Marinus' knowledge and based on its current understanding of the Licensed Compounds and Licensed Products and their use, the development, use or sale of any Licensed Compound or Licensed Product pursuant to this Agreement does not and will not infringe, misappropriate or violate any intellectual property rights of any Third Party, and Marinus is not aware of any pending patent application that, if validly issued, would be infringed by the development, manufacture, use or sale of any Licensed Compound or Licensed Product pursuant to this Agreement. No claim or action has been brought or, to Marinus' knowledge, threatened in writing by any governmental authority or Third Party and there exists no valid basis for any claim (i) that the use of any Marinus Trademark in connection with the commercialization, marketing and sale of a Licensed Product violates the rights of a Third Party or (ii) currently challenging the enforceability or validity of any Marinus Trademark.

(f) Upstream Licenses. (i) **Schedule 1.69** sets forth a complete and accurate list of all Upstream Licenses in effect as of the Effective Date, Marinus has provided Company with a true, complete and correct copy of each Upstream License, and each Upstream License is in full force and effect; (ii) no written notice of default or termination has been received or given under any Upstream License, and to Marinus' knowledge, there is no act or omission by Marinus that would provide a right to terminate any Upstream License; (iii) during the term of this Agreement: (1) Marinus shall remain in compliance in all material respects with each Upstream License and shall not terminate, amend, waive or otherwise modify (or consent to any of the foregoing) its rights under any Upstream License in any manner that would materially adversely affect the rights or licenses granted to Company hereunder or increases or generates any new payment or other obligation under any Upstream License that would apply to Company, without Company's express written consent; and (2) other than the payment obligation of Company as part of the definition of COGS, Marinus shall be solely responsible for any and all payments payable to the respective Upstream Licensors under each applicable Upstream License.

8.2 Company represents, warrants and covenants:

(a) Authorization. Company is duly organized and validly existing under the laws of the country of its incorporation. This Agreement has been duly executed and delivered by Company and constitutes the valid and binding obligation of Company, enforceable against Company in accordance with its terms except as enforceability may be limited by bankruptcy, fraudulent conveyance, insolvency, reorganization, moratorium and other laws relating to or affecting creditors' rights generally and by general equitable principles. The execution, delivery and performance of this Agreement have been duly authorized by all necessary action on the part of Company, its officers and directors.

(b) No Conflict. The execution, delivery and performance of this Agreement by Company does not conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it may be bound, and, to its knowledge, does not violate any material law or regulation of any court, governmental body or administrative or other agency having authority over it. Company is not subject to any order, decree or injunction by a court of competent jurisdiction which prevents or materially delays the consummation of the transactions contemplated by this Agreement.

8.3 NO OTHER WARRANTIES. TO THE MAXIMUM EXTENT PERMITTED BY APPLICABLE LAW, THE LIMITED WARRANTIES CONTAINED IN THIS ARTICLE ARE THE SOLE WARRANTIES GIVEN BY THE PARTIES AND ARE MADE EXPRESSLY IN LIEU OF AND EXCLUDE ANY IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, TITLE, INFRINGEMENT OR OTHERWISE, AND ALL OTHER EXPRESS OR IMPLIED REPRESENTATIONS AND WARRANTIES PROVIDED BY COMMON LAW, STATUTE OR OTHERWISE ARE HEREBY DISCLAIMED BY BOTH PARTIES.

Article IX
Confidentiality

9.1 Confidentiality. During the term of this Agreement, and for a period of [***] thereafter, each Party shall keep all Confidential Information received from the other Party confidential and shall not disclose nor use such Confidential Information without the other Party's written consent except to the extent contemplated by this Agreement. This restriction shall not, however, prevent disclosure of the Confidential Information if and to the extent that disclosure is required by law, provided that the disclosing Party informs the other Party without delay of any such requirement, in order to allow such other Party to object to such disclosure and to seek an appropriate protective order or similar protection prior to disclosure.

9.2 Exceptions. The above obligations shall not apply or shall cease to apply to any Confidential Information which:

(a) is now, or hereafter becomes, through no act or failure to act on the part of the receiving Party, generally known or available;

(b) is known by the receiving Party at the time of receiving such information, as evidenced by its written records;

(c) is hereafter furnished to the receiving Party by a Third Party, as a matter of right and without restriction on disclosure;

(d) is independently developed by the receiving Party without any breach of this Section 10; or

(e) is the subject of a written permission to disclose provided by the disclosing Party.

9.3 Permitted Disclosures. Confidential Information may be disclosed for the purpose of filing, prosecuting and maintaining patents and patent applications and for obtaining regulatory approvals and research, development, commercialization, the packaging, labeling, manufacture (upon Product Localization), marketing, distribution or sale of Licensed Products, and to employees, agents, consultants, licensors, sublicensees, subcontractors, or suppliers of the recipient Party or its Affiliates, but only to the extent required to accomplish the purposes of this Agreement and only if such individuals are required by law, contract or otherwise not to use or disclose such information except as permitted by this Agreement. Each Party will use at least the same standard of care as it uses to protect proprietary or confidential information of its own to ensure that such employees, agents, consultants, sublicensees or suppliers do not disclose or make any unauthorized use of the Confidential Information.

9.4 Disclosure of Agreement. Except as required by law, neither Company nor Marinus shall release to any Third Party or publish in any way any non-public information with respect to the terms of this Agreement or concerning their cooperation without the prior written consent of the other, which consent will not be unreasonably withheld, conditioned or delayed; provided; however that either Party may disclose the terms of this Agreement to the extent required to comply with applicable laws, including without limitation the rules and regulations promulgated by the U.S. Securities and Exchange Commission or similar regulatory authorities in other jurisdictions. Notwithstanding any other provision of this Agreement, each Party may disclose the terms of this Agreement to lenders, investment bankers, financial advisors and other financial institutions of its choice solely for purposes of financing the business operations of such Party, or to potential investors in or acquirers of such Party either (i) upon the written consent of the other Party or (ii) if the disclosing Party uses Commercially Reasonable Efforts to obtain a signed confidentiality agreement with such intended recipient with respect to such information, upon terms substantially similar to those contained in this Section.

The Parties agree that the material financial terms of the Agreement shall be considered the Confidential Information of both Parties. Notwithstanding the foregoing, the Parties

must agree upon the initial press release(s) to announce the execution of this Agreement; thereafter, both Parties may each disclose to Third Parties the information contained in such press release(s) without the need for further approval by the other.

9.5 Employees. The Parties shall undertake to ensure that all their employees who have access to Confidential Information of the other Party are under obligations of confidentiality fully consistent with those provided in this Article.

Article X
Term and Termination

10.1 Term

(a) Term. The term of this Agreement shall extend for so long as royalties are payable pursuant to Section 6.3(b) of this Agreement anywhere in the Territory, unless earlier terminated in accordance with this Agreement. Upon the expiration of the Royalty Term for a Licensed Product in a jurisdiction, the licenses granted to Company by Marinus under this Agreement to Exploit such Licensed Product in such jurisdiction shall be fully paid-up, perpetual, irrevocable and exclusive.

(b) Accrued Obligations. Except where explicitly provided elsewhere herein, termination of this Agreement for any reason, or expiration of this Agreement, will not affect: (i) obligations, including the payment of any royalties or other sums which have accrued as of the date of termination or expiration, and (ii) rights and obligations which, from the context thereof, are intended to survive termination or expiration of this Agreement.

10.2 Termination for Convenience. Company shall have the right to terminate this Agreement in its entirety for any or no reason upon [***] written notice to Marinus, provided [***].

10.3 Termination for Insolvency. Either Party may terminate this Agreement immediately upon delivery of written notice to the other Party (a) upon the institution by or against the other Party of insolvency, receivership or bankruptcy proceedings or any other proceedings for the settlement of the other Party's debts, provided, however with respect to involuntary proceedings, that such proceedings are not dismissed within [***] (b) upon the other Party's making an assignment of substantially all of its assets for the benefit of creditors; or (c) upon the other Party's dissolution or ceasing to do business.

10.4 Material Breach. Each Party shall have the right to terminate this Agreement upon written notice to the other Party if such other Party materially breaches this Agreement and, after receiving written notice from the non-breaching Party identifying such material breach in reasonable detail (herein, "Breach Notice"), fails to cure such material breach within [***]. Notwithstanding the foregoing, if the alleged breaching Party disputes the existence or materiality of the alleged breach or asserts it has cured such breach, the other Party shall not have the right to terminate this Agreement unless and until it is determined in

accordance with Article 12 that the alleged breaching Party has materially breached this Agreement and fails to cure such breach within [***], or in the case of an assertion that the alleged breach Party has cured such breach, upon determination that such Party has not cured such breach.

10.5 Patent Challenge. Except to the extent unenforceable under the applicable law, Marinus may terminate this Agreement by providing written notice of termination to Company if Company or its Affiliates or sublicensees (individually or in association with any Person) contests or assists a Third Party in contesting the scope, validity, or enforceability of any Marinus Patent anywhere in the world in any court, tribunal, arbitration proceeding, or other proceeding, including the U.S. Patent and Trademark Office and the U.S. International Trade Commission (a "Patent Challenge"); *provided*, *however*, that Marinus shall not have the right to terminate this Agreement under this Section 10.5 if (a) [***], (b) [***], or (c) [***].

10.6 Effect of Termination.

(a) Upon the termination of this Agreement, the rights granted under this Agreement shall be treated as follows:

(i) In the event of a termination by Marinus under Sections 10.3 through 10.5, all licenses to Company under this Agreement shall terminate subject to the terms and conditions of this Section 10.6.

(ii) In the event of a termination of this Agreement by Marinus pursuant to any of the Sections 10.3 through 10.5, the parties shall cooperate to ensure that the development and commercialization of Licensed Products in the Territory continues without unreasonable delay resulting from the transfer of rights back to Marinus. In particular, Company shall grant to Marinus a paid-up, worldwide, royalty-free, non-exclusive license with right to sublicense under all Company Technology, preclinical and clinical data and regulatory documents in its control to Exploit Licensed Products (which detailed terms shall be negotiated in good faith and agreed between the Parties prior to such termination), and shall, to the extent permitted under applicable law, promptly assign or cause to be assigned to Marinus, or its designee, every granted or pending government approval, clearance, registration or permit relating to the Licensed Products obtained by Company in the Territory. In the event such assignment is not permitted by law, Company will cooperate in the cancellation of such government approval, clearance, registration or, to the extent permitted under applicable law, permit standing in its name and the reissuance of such government approval, clearance, registration or permit to Marinus or its designee. Company shall, to the extent permitted under applicable law, take all such other reasonable actions and execute such other reasonable instruments,

assignments and documents as may be necessary to effect the transfer of rights hereunder to Marinus.

(b) Exclusive Distribution. In the event of a termination of this Agreement by Marinus pursuant to any of the Sections 10.3 through 10.5, until such time as all Regulatory Approvals with respect to the Licensed Products in the Territory have been assigned and transferred to Marinus or its designee, Marinus may, in its absolute discretion, either (a) purchase and Company will sell to Marinus any and all salable inventory of Licensed Product held by Company or its Affiliates as of the effective date of termination with respect to the Licensed Products at a price equal to [***], or (b) allow Company to sell such inventory under the rights granted under Section 2.1; provided, however, that Company's obligations under this Agreement with respect to all the Products that Company sells, including the obligation to remit royalty payments to Marinus hereunder, will continue in full force and effect during such period.

(c) Assignment and Disclosure. In the event of a termination of this Agreement by Marinus pursuant to any of the Sections 10.3 through 10.5, Company shall, to the extent permitted under applicable law, upon the request of Marinus, (i) assign and transfer to Marinus or its designee all of Company's rights, title, and interests in and to all Company Clinical Study agreements and distribution agreements (to the extent assignable and not cancelled), confidentiality and other agreements, data and other Know-How (including commercial information) in Company's Control, in each case, solely to the extent relating to the Licensed Products and that are necessary or useful for the development or commercialization of the Licensed Products in the Territory, (ii) disclose to Marinus or its designee all documents, records, and materials solely related to the Licensed Products that are controlled by Company or that Company is able to obtain using reasonable efforts, and that embody the foregoing; and (iii) assign and transfer to Marinus or its designee all of Company's rights, title, and interests in and to any promotional materials, training materials, medical education materials, packaging and labeling, and all other literature or other information related solely to the Licensed Products and copyrights and any registrations for the foregoing.

To the extent that any agreement or other asset described in this Section 10.6(c) is not assignable by Company, then such agreement or other asset will not be assigned, and upon the request of Marinus, Company will use Commercially Reasonable Efforts to allow Marinus to obtain and to enjoy the benefits of such agreement or other asset, without additional payment therefor, in the form of a license or other right to the extent Company has the right and ability to do so.

(d) Know-How Transfer Support. In the event of a termination of this Agreement by Marinus pursuant to any of the Sections 10.3 through 10.5 in furtherance of the assignment of Know-How pursuant to Clause (c) above, Company will for a period

of [***] from the effective date of termination of this Agreement, provide such reasonable consultation or other assistance as Marinus may reasonably request to assist Marinus in becoming familiar with such Know-How in order for Marinus to undertake further Exploitation of the Licensed Products in the Territory, provided that, Marinus shall [***] within [***] after receiving Company's invoice therefor.

(e) Ongoing Clinical Studies. In the event of a termination of this Agreement by Marinus pursuant to any of the Sections 10.3 through 10.5, to the extent permitted under applicable laws, if, as of the effective date of the termination, Company or any of its Affiliates is conducting any Company Clinical Studies for the Licensed Products, then, at Marinus' election on a Clinical Study-by-Clinical Study basis, Company will reasonably cooperate, and will ensure that its Affiliates reasonably cooperate, with Marinus to transfer the conduct of such Clinical Study to Marinus or its designees.

(i) If Marinus so elects and to the extent permitted under applicable laws, then Company will continue to conduct such Clinical Study, at Marinus' cost, to enable such transfer to be completed without interruption of any such Clinical Study (including the assignment of all related Regulatory Filings and investigator and other agreements related to such Clinical Studies). Marinus will assume any and all liability for the conduct of such transferred Clinical Study after the effective date of such transfer (except to the extent arising prior to the transfer date or from any willful misconduct or negligent act or omission by Company, its Affiliates or their respective employees, agents and contractors). Company will provide such knowledge transfer and other training, at Marinus' cost, to Marinus or its designee as reasonably necessary for Marinus or such designee to continue such Clinical Study.

(ii) If Marinus does not elect to assume control of any such Clinical Studies for the Products, then Company will, in accordance with accepted pharmaceutical industry norms and ethical practices, wind-down the conduct of any such Clinical Study in an orderly manner.

(f) Ongoing Obligations.

(i) Upon expiration or termination of this Agreement for any reason, each Party shall immediately return to the other Party or delete or destroy all relevant records and materials in such Party's possession or control containing any Confidential Information disclosed by the other Party; provided that such Party may keep one copy of such materials for archival purposes only subject to continuing confidentiality obligations.

(ii) In the event of a termination of this Agreement by Marinus pursuant to any of the Sections 10.3 through 10.5, subject to the terms and conditions of this Section 10.6, Company shall, to the extent permitted under applicable

law, deliver to Marinus all data and information (including registration dossiers) obtained for or in pursuing Regulatory Approvals, and all Regulatory Approvals to Marinus or its designee in the Territory as permitted under the applicable law for Licensed Products in the Territory received as of such termination date.

(g) Termination by Company. Notwithstanding anything to the contrary, upon the termination of this Agreement by Company pursuant to Section 10.3 or Section 10.4, all of the provisions of Section 10.6 shall apply, except that to the extent Company is obligated to perform under any of the provisions of Section 10.6 Marinus shall reimburse Company for all reasonable costs incurred by Company in connection with such performance, including both its reasonable external costs plus its reasonable internal costs.

(h) Survival. The following provisions shall survive the termination or expiration of this Agreement for any reason: Article I (Definitions), Sections 6.2(c) and Section 6.2(d) (solely to the extent applicable with respect to a payment obligation that accrued prior to expiration or termination), Section 6.4 through Section 6.10 (solely to the extent applicable with respect to a payment obligation that accrued prior to expiration or termination), Section 7.1(a) (Ownership of Intellectual Property), the second sentence of Section 7.1(b) (Improvements), Section 8.3 (No Other Warranties), Article IX(Confidentiality) (for the time period set forth therein), Section 11.1 through Section 11.3 (Indemnification), the second sentence of Section 10.1(a) (solely in the event of expiration as set forth in that sentence and not in the event of earlier termination), Section 10.1(b)(ii), Section 10.6 (Effect of Termination), Article XII (Dispute Resolution) and Article XIII (Miscellaneous) except for Section 13.7.

Article XI
Indemnification and Insurance

11.1 Indemnification by Marinus. Marinus will indemnify, defend and hold Company and its Affiliates, and its and their respective employees, officers, agents and directors harmless against any loss, damages, action, suit, claim, demand, liability, or expense (the "Loss") to the extent resulting from any Third Party claim to the extent such Loss is based on or arises out of (a) the development, use, sale, storage or handling of a Licensed Product by Marinus or its Affiliates or their representatives, agents or subcontractors, or any actual violation of law resulting therefrom; (b) the negligent or willful misconduct of Marinus' acts with respect to the manufacture or supply of Licensed Products to Company; or (c) the material breach by Marinus of any of its covenants, representations or warranties set forth in this Agreement; provided however, that the foregoing indemnification shall not apply to any Loss to the extent such Loss arises out of any claim for which Company has an obligation to indemnify Marinus under Section 11.2.

11.2 Indemnification by Company. Company will indemnify, defend and hold Marinus and its and Affiliates, and its and their respective employees, officers, agents and directors harmless against any Loss to the extent resulting from any Third Party claim to the extent such Loss is based on or arises out of:

(a) the development, use, sale, storage or handling of a Licensed Product by Company or its Affiliates or their representatives, agents or subcontractors under this Agreement, or any actual violation of law resulting therefrom; or

(b) the material breach by Company of any of its covenants, representations or warranties set forth in this Agreement;

provided however, that the foregoing indemnification shall not apply to any Loss to the extent such Loss arises out of any claim for which Company has an obligation to indemnify Marinus under Section 11.1.

11.3 Claims Procedures. Each Party entitled to be indemnified by the other Party (an "Indemnified Party") pursuant to Section 11.1 or Section 11.2 hereof shall give notice to the other Party (an "Indemnifying Party") promptly after such Indemnified Party has actual knowledge of any threatened or asserted claim as to which indemnity may be sought, and shall permit the Indemnifying Party to assume and have the sole control of the defense of any such claim or any litigation resulting therefrom; provided that:

(a) counsel for the Indemnifying Party, who shall conduct the defense of such claim or any litigation resulting therefrom, shall be approved by the Indemnified Party (whose approval shall not unreasonably be withheld) and the Indemnified Party may participate in such defense at such Party's expense; and

(b) the failure of any Indemnified Party to give notice as provided herein shall not relieve the Indemnifying Party of its obligations under this Agreement to the extent that the failure to give notice did not result in harm to the Indemnifying Party.

(c) no Indemnifying Party, in the defense of any such claim or litigation, shall, except with the approval of each Indemnified Party, which approval shall not be unreasonably withheld, conditioned or delayed, consent to entry of any judgment or enter into any settlement which (i) would result in injunctive or other relief being imposed against the Indemnified Party; or (ii) does not include as an unconditional term thereof the giving by the claimant or plaintiff to such Indemnified Party of a release from all liability in respect to such claim or litigation.

(d) Each Indemnified Party shall furnish such information regarding itself or the claim in question as an Indemnifying Party may reasonably request in writing and shall be reasonably required in connection with the defense of such claim and litigation resulting therefrom.

11.4 Compliance. The Parties shall comply fully with all applicable laws and regulations, including without limitation, China Data Protection Laws, in connection with their respective activities under this Agreement and agree to enter into a comprehensive data protection agreement within [***] after the Effective Date.

11.5 Insurance. Each Party, at its own expense, shall maintain product liability and other appropriate insurance (or self-insure) in an amount consistent with sound business practice and reasonable in light of its obligations under this Agreement during the term of this Agreement. Each Party shall provide a certificate of insurance (or evidence of self-insurance) evidencing such coverage to the other Party upon request. The Parties agree that such insurance shall not be construed to create a limit of either Party's liability with respect to its indemnification obligations under this Article XI or other obligations under this Agreement.

Article XII
Dispute Resolution; Governing Law

12.1 Objective. The Parties recognize that disputes as to matters arising under or relating to this Agreement or either Party's rights and obligations hereunder may arise from time to time. It is the objective of the Parties to establish procedures to facilitate the resolution of such disputes in an expedient manner by mutual cooperation and without resort to litigation. To accomplish this objective, the Parties agree to follow the procedures set forth in this Article XII to resolve any such dispute if and when it arises.

12.2 Resolution by [***]. Except as otherwise provided in Section 3.2, if an unresolved dispute as to matters arising under or relating to this Agreement or either Party's rights and obligations hereunder arises, either Party may refer such dispute to the [***] of the Parties, who shall meet in person or by telephone within [***] after such referral to attempt in good faith to resolve such dispute. If such dispute cannot be resolved by discussion [***] within such [***] period, or such other time period as the Parties may agree in writing, such dispute shall be resolved in accordance with Section 12.3.

12.3 Arbitration.

(a) If the Parties do not resolve a dispute as provided in Section 12.2, and a Party wishes to pursue the matter, the dispute shall be resolved by binding arbitration administered by the Hong Kong International Arbitration Center ("HKIAC") in accordance with the HKIAC Administered Arbitration Rules in effect at the time the notice of arbitration is submitted, which Rules are deemed to be incorporated by reference into this clause. Judgment on the arbitration award may be entered in any court having jurisdiction thereof.

(b) There shall be three (3) arbitrators. The place of arbitration shall be Hong Kong, and all proceedings and communications shall be in English. Either Party may, without waiving any remedy under this Agreement, seek from any court having

jurisdiction any injunctive or provisional relief necessary to protect the rights or property of that Party pending the arbitration award.

12.4 Governing Law. This Agreement and all amendments, modifications, alterations, or supplements hereto, and the rights of the Parties hereunder shall be governed and construed in accordance with the laws of New York, exclusive of its conflicts of laws principles.

Article XIII
Miscellaneous Provisions

13.1 Waiver. The failure on the part of Company or Marinus to exercise or enforce any rights conferred upon it hereunder shall not be deemed to be a waiver of any such rights nor operate to bar the exercise or enforcement thereof at any time or times thereafter. The observance of any term of this Agreement may be waived (either generally or in a particular instance and either retroactively or prospectively) by the Party entitled to enforce such term, but any such waiver shall be effective only if in writing signed by the Party against whom such waiver is to be asserted.

13.2 Force Majeure. Neither Party shall be held liable or responsible to the other Party nor be deemed to have defaulted under or breached this Agreement for failure or delay in fulfilling or performing any term of this Agreement, other than an obligation to make a payment, when such failure or delay is caused by or results from fire, floods, embargoes, government regulations, prohibitions or interventions, pandemic, war, acts of war (whether war be declared or not), insurrections, riots, civil commotions, strikes, lockouts, acts of God, or any other cause beyond the reasonable control of the affected Party.

13.3 Limitation of Liability. NEITHER PARTY SHALL BE LIABLE TO THE OTHER PARTY FOR ANY SPECIAL, NON-COMPENSATORY, PUNITIVE, EXEMPLARY, MULTIPLIED, INDIRECT, CONSEQUENTIAL OR INCIDENTAL DAMAGES OF ANY KIND OR FOR ANY LOST PROFITS, LOST SALES, LOST REVENUE OR LOSS OF USE IN CONNECTION WITH THIS AGREEMENT, REGARDLESS OF THE FORM OF ACTION, WHETHER IN CONTRACT, TORT, NEGLIGENCE, STRICT LIABILITY, OR OTHERWISE, EVEN IF SUCH PARTY HAS BEEN INFORMED OF OR IS AWARE OF THE POSSIBILITY OF ANY SUCH DAMAGES IN ADVANCE; provided, however, that this Section 13.3 shall not be construed to limit (i) either Party's indemnification obligations under Article XI or (ii) liabilities arising from a Party's breach of its obligations under Article IX.

13.4 Severability. It is the intention of the Parties to comply with all applicable laws domestic or foreign in connection with the performance of its obligations hereunder. In the event that any provision of this Agreement, or any part hereof, is found invalid or unenforceable, the remainder of this Agreement will be binding on the Parties hereto, and will be construed as if the invalid or unenforceable provision or part thereof had been deleted, and the Agreement shall be deemed modified to the extent necessary to render the surviving provisions enforceable to the fullest extent permitted by law.

13.5 Government Acts. In the event that any act, regulation, directive, or law of a government, including its departments, agencies or courts, should make impossible or prohibit, restrain, modify or limit any material act or obligation of Company or Marinus under this Agreement, the Parties shall enter into good faith negotiations to make such modifications to this Agreement as may be necessary to fairly address the impact thereof.

13.6 Government Approvals. Each Party shall use Commercially Reasonable Efforts to obtain any government approval required to enable this Agreement to become effective, or to enable any payment hereunder to be made, or any other obligation hereunder to be observed or performed. Each Party will keep the other informed of progress in obtaining any such approvals.

13.7 FCPA. The Parties agree to strictly abide by the Foreign Corrupt Practices Act of 1977 ("FCPA") of the USA and any applicable anti-bribery or anti-corruption laws in relevant Jurisdictions in the Territory. The Parties will not offer, pay, promise to pay or authorize to pay any money, make any gift or provide anything of value to the following persons when such payment, gift, offer or promise is made for the purpose of obtaining or accelerating to obtain or retaining Regulatory Approval, or promoting the commercial sale of the Licensed Products, or securing any improper advantage:

(a) any governmental, legislative or judicial official or any candidate for such position in the PRC, the USA or any other countries of the world;

(b) any political party or political party official or any candidate for such political office; or

(c) any person while knowing or having reason to know that all or any portion of any payment or gift will be offered, given or promised directly or indirectly to any of the above.

13.8 Assignment. This Agreement may not be assigned or otherwise transferred by either Party without the prior written consent of the other Party; provided, however, that either Party may assign this Agreement, without the consent of the other Party to any of its Affiliates, if the assigning Party guarantees the full performance of its Affiliates' obligations hereunder, and either Party may assign this Agreement, without the consent of other Party in connection with the transfer or sale of all or substantially all of its assets or business or in the event of its merger or consolidation with another company. In all cases the assigning Party shall provide the other Party with prompt notice of any such assignment. Any purported assignment in contravention of this Section 13.8 shall, at the option of the non-assigning Party, be null and void and of no effect. No assignment shall release either Party from responsibility for the performance of any accrued obligation of such Party hereunder.

13.9 Performance by Affiliates. Each Party may discharge any obligations (other than the payment obligations set forth under Article 6) and exercise any right hereunder through any of its Affiliates (for so long as such entity remains an Affiliate), without notice to and

without consent from, the other Party, and each Party hereby guarantees the performance by its Affiliates of such Party's obligations under this Agreement, and shall cause its Affiliates to comply with the provisions of this Agreement in connection with such performance.

13.10 Counterparts. This Agreement may be executed in duplicate, both of which shall be deemed to be originals, and both of which shall constitute one and the same Agreement.

13.11 No Agency. Nothing herein contained shall be deemed to create an agency, joint venture, amalgamation, partnership or similar relationship between Company and Marinus. Notwithstanding any of the provisions of this Agreement, neither Party shall at any time enter into, incur, or hold itself out to Third Parties as having authority to enter into or incur, on behalf of the other Party, any commitment, expense, or liability whatsoever, and all contracts, expenses and liabilities undertaken or incurred by one Party in connection with or relating to the development, manufacture or sale of Licensed Products shall be undertaken, incurred or paid exclusively by that Party, and not as an agent or representative of the other Party.

13.12 Notice. All notices, consents or waivers under this Agreement shall be in writing and will be deemed to have been duly given when (a) scanned and converted into a portable document format file (*i.e.*, pdf file), and sent as an attachment to an e-mail message, as of the date when such message is received, as evidenced by a return e-mail sent by recipient confirming receipt, provided that a copy is promptly sent by an internationally recognized overnight delivery service (receipt requested) (although the receipt of the return e-mail message confirming receipt shall be when the notice is deemed to have been given), or (b) sent by internationally recognized overnight courier, the earlier of (i) when received by the addressee or (ii) five (5) days after it was sent, if sent by overnight courier by an internationally recognized overnight delivery service (receipt requested), in each case, (a) or (b), as applicable, to the appropriate addresses and e-mail addresses set forth below (or to such other addresses and e-mail addresses as a Party may designate by notice):

If to Marinus, at:

Marinus Pharmaceuticals, Inc.
5 Radnor Corporate Center, Suite 500
100 Matsonford Road
Radnor, PA 19087
Attention: General Counsel

If to Company, at:

BCPE Tenet CNS Cayman, Ltd.
200 Clarendon Street
Boston, MA 02116
USA

Attn: [***]
[***]
[***]

13.13 Interpretation. The paragraph headings are for convenience only and will not be deemed to affect in any way the language of the provisions to which they refer. All references in this Agreement to the singular shall include the plural where applicable. Unless otherwise specified, references in this Agreement to any Article shall include all Sections, subsections and paragraphs in such Article, references to any Section shall include all subsections and paragraphs in such Section, and references in this Agreement to any subsection shall include all paragraphs in such subsection. The word "including" and similar words means including without limitation. The word "or" means "and/or" unless the context dictates otherwise because the subject of the conjunction are mutually exclusive. The words "herein," "hereof" and "hereunder" and other words of similar import refer to this Agreement as a whole and not to any particular Section or other subdivision. All references to days in this Agreement mean calendar days, unless otherwise specified. Ambiguities and uncertainties in this Agreement, if any, shall not be interpreted against either Party, irrespective of which Party may be deemed to have caused the ambiguity or uncertainty to exist.

13.14 Authority. The undersigned represent that they are authorized to sign this Agreement on behalf of the Parties hereto. The Parties each represent that no provision of this Agreement will violate any other agreement that such Party may have with any other person or company. Each Party has relied on that representation in entering into this Agreement.

13.15 Entire Agreement. This Agreement, including the Schedules and Exhibit appended hereto, contains the entire understanding of the Parties relating to the matters referred to herein, and may only be amended by a written document, duly executed on behalf of the respective Parties.

[Signature Page Follows]

IN WITNESS WHEREOF, the Parties hereto have caused this Agreement to be executed by their duly authorized representatives as of the Effective Date.

MARINUS PHARMACEUTICALS, INC.

By:_____

Title:_____

Date:_____

Tenacia Biotechnology (Shanghai) Co., Ltd.

By:_____

Title:_____

Date:_____

Schedule 1.51

Marinus Patents

[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	
[***]	[***]	[***]	[***]	[***]	
[***]	[***]	[***]	[***]	[***]	
[***]	[***]	[***]	[***]	[***]	
[***]	[***]	[***]	[***]	[***]	
[***]	[***]	[***]	[***]	[***]	
[***]	[***]	[***]	[***]	[***]	

Schedule 1.67

Territory Development Plan

To be added upon approval by the Joint Steering Committee

Schedule 1.69

Upstream Licenses

[***]

[***]

Exhibit A

[***]

[***]

Exhibit 21

SUBSIDIARIES OF THE REGISTRANT

Marinus Pharmaceuticals Emerald Limited, an Ireland company and wholly owned subsidiary.

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. 333-239780) of Marinus Pharmaceuticals, Inc.;
2. Registration Statement (Form S-3 No. 333-237903) of Marinus Pharmaceuticals, Inc.;
3. Registration Statement (Form S-8 No. 333-258677) pertaining to the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended, and Individual Nonqualified Stock Option Awards;
4. Registration Statement (Form S-8 No. 333-239785) pertaining to the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended, and Individual Nonqualified Stock Option Awards;
5. Registration Statement (Form S-8 No. 333-233131) pertaining to the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended, and Individual Nonqualified Stock Option Awards;
6. Registration Statement (Form S-8 No. 333-219613) pertaining to the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan;
7. Registration Statement (Form S-8 No. 333-200701) pertaining to the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan; and
8. Registration Statement (Form S-8 No. 333-265865) pertaining to the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as amended, Individual Nonqualified Stock Option Awards, and Individual Restricted Stock Units;

of our report dated March 9, 2023, with respect to the consolidated financial statements of Marinus Pharmaceuticals, Inc. included in this Annual Report (Form 10-K) of Marinus Pharmaceuticals, Inc. for the year ended December 31, 2022.

/s/ Ernst & Young LLP

Philadelphia, PA
March 9, 2023

Exhibit 31.1

Certification of Chief Executive Officer Pursuant to
Exchange Act Rules 13a-14(a) or 15d-14(a)

I, Scott Braunstein, certify that:

1. I have reviewed this annual report on Form 10-K of Marinus 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, 2023

/s/ Scott Braunstein
Scott Braunstein,
Chief Executive Officer and Director
(Principal Executive Officer)

Exhibit 31.2

Certification of Chief Financial Officer Pursuant to
Exchange Act Rules 13a-14(a) or 15d-14(a)

I, Steven Pfanstiel, certify that:

1. I have reviewed this annual report on Form 10-K of Marinus 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, 2023

/s/ Steven Pfanstiel

Steven Pfanstiel,
Chief Operating Officer, Chief Financial Officer and Treasurer
(Principal Financial and Accounting Officer)

Exhibit 32.1

Certification Pursuant to 18 U.S.C. Section 1350

In connection with the annual report of Marinus Pharmaceuticals, Inc. (the "Company") on Form 10-K for the year ended December 31, 2022 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned, in the capacities and on the date indicated below, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

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

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

Date: March 9, 2023

/s/ Scott Braunstein
Chief Executive Officer and Director
(Principal Executive Officer)

Date: March 9, 2023

/s/ Steven Pfanstiel
Chief Operating Officer, Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)