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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, 2020

☐ **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. ☐

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, 2020) was $303,163,851, based on the closing price reported on the Nasdaq Global Market on June 30, 2020.

The total number of shares of the registrant's common stock, par value $0.001 per share, outstanding as of March 8, 2021 was 36,578,460.

Documents Incorporated by Reference

Certain portions of the registrant's Definitive Proxy Statement for its 2021 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, 2020, 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 ability to develop and commercialize ganaxolone;
- the status, timing and results of preclinical studies and clinical trials;
- 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;
- the timing of seeking marketing approval of ganaxolone;
- our ability to obtain and maintain marketing approval;
- our estimates of expenses and future revenue and profitability;
- our estimates regarding our capital requirements and our needs for additional financing;
- our plans to develop and market ganaxolone and the timing of our development programs;
- our estimates of the size of the potential markets for ganaxolone;
- our selection and licensing of ganaxolone;
- our ability to attract collaborators with acceptable development, regulatory and commercial expertise;
- the benefits to be 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 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 and our other product candidates;
- our expectation that our cash, cash equivalents and investments as of December 31, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2022;
- our expectation that we submit a New Drug Application (NDA) to FDA for ganaxolone for treatment of CDD in mid-2021 and a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in the third quarter of 2021;

- the possibility that we receive a priority review voucher from the FDA if an NDA for ganaxolone in CDD is approved;
- our expectation to announce top-line data from our Phase 3 clinical trial evaluating IV ganaxolone for the treatment of RSE in the first half of 2022;
- our expectation to report top-line data from our Phase 2 open-label trial (CALM Study) in the third quarter of 2021;
- the potential for us to develop ganaxolone in one or more additional rare refractory epilepsy indications or to in-license complementary products to leverage development and commercial investment for ganaxolone for such indications;
- our ability to create an effective sales and marketing infrastructure if we elect to market and sell ganaxolone directly;
- the rate and degree of market acceptance of ganaxolone;
- the timing and amount of reimbursement for ganaxolone;
- the success of other competing therapies that may become available;
- the manufacturing capacity for ganaxolone;
- our intellectual property position;
- our ability to maintain and protect our intellectual property rights;
- our results of operations, financial condition, liquidity, prospects, and growth strategies;
- the industry in which we operate;
- 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 trends that 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 have incurred significant losses since our inception and anticipate that we will continue to incur losses in the future.
- We have not generated any 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.
- We will require additional capital to fund our operations and if we fail to obtain necessary financing, we may be unable to complete the development and commercialization of ganaxolone.
- 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 our sole clinical stage product candidate, ganaxolone, and may fail to capitalize on other technologies or product candidates that may be more profitable or for which there may be a greater likelihood of success.
- 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.

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 four clinical trials and will require significant capital resources and years of additional clinical development effort.
- We plan to submit an NDA for ganaxolone for the treatment of CDKL5 Deficiency Disorder in mid 2021 and we may not receive regulatory approval of our application.
- Because the results of preclinical studies or earlier clinical trials are not necessarily predictive of future results, ganaxolone may not have favorable results in later clinical trials or receive regulatory approval.
- Ganaxolone may cause undesirable side effects or have other properties that could delay or prevent its regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following any marketing approval.
- The therapeutic efficacy and safety of ganaxolone are unproven, and we may not be able to successfully develop and commercialize ganaxolone in the future.
- Clinical development of product candidates involves a lengthy and expensive process with an uncertain outcome.
- Even if ganaxolone receives regulatory approval, we will still face regulatory difficulties.
- We may not be able to obtain orphan drug exclusivity for ganaxolone, which could limit the potential profitability of ganaxolone.
- Even though we have received Rare Pediatric Disease (RPD) Designation for ganaxolone for the treatment of CDD, we may not receive a rare pediatric disease priority review voucher.
- Failure to obtain regulatory approval in international jurisdictions would prevent ganaxolone from being marketed in these jurisdictions.

Risks Related to the Commercialization of Our Product

- Our commercial success depends upon attaining significant market access and acceptance of ganaxolone, if approved, among physicians, patients, government and private payers and others in the medical community and attaining sufficient reimbursement for ganaxolone.
- We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

- If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell ganaxolone, we may be unable to generate any revenue.
- Even if we are able to commercialize ganaxolone, it may not receive coverage and adequate reimbursement from third-party payers, which could harm our business.
- If the market opportunities for ganaxolone are smaller than we believe they are, our results of operations may be adversely affected and our business may suffer.

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 and/or regulatory requirements or meet expected deadlines, our development plans may be adversely affected and we may not be able to obtain regulatory approval for or commercialize ganaxolone.
- Our experience manufacturing ganaxolone is limited to the needs of our preclinical studies and clinical trials. We have no experience manufacturing ganaxolone on a commercial scale and have no manufacturing facility. We are dependent on third-party manufacturers for the manufacture of ganaxolone as well as on third parties for our supply chain, and if we experience problems with any such third parties, the manufacturing of ganaxolone could be delayed.
- Government funding for certain 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.

Risks Related to Regulatory Compliance

- Recently 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 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 adversely affect our business and our ability to conduct and complete clinical trials.

Risks Related to Ownership of Our Common Stock

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

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

We are a clinical stage pharmaceutical company focused on developing and commercializing innovative therapeutics to treat patients suffering from rare seizure disorders. Our clinical stage product candidate, ganaxolone, is a positive allosteric modulator of $GABA_A$ that is being developed in formulations for two different routes of administration: intravenous (IV) and oral. Ganaxolone is a synthetic analog of allopregnanolone, an endogenous neurosteroid. 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. Ganaxolone acts at both synaptic and extrasynaptic $GABA_A$ receptors, a target known for its anti-seizure, antidepressant and anxiolytic potential.

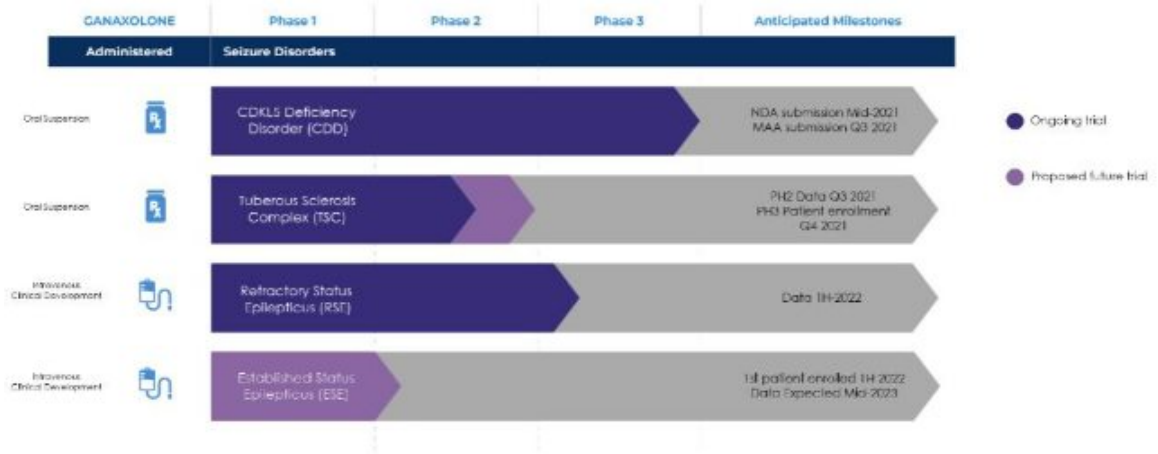
COVID-19

In December 2019, an outbreak of a novel strain of coronavirus (COVID-19) was identified in Wuhan, China. This virus was declared a pandemic by the World Health Organization in March 2020 and has spread to nearly every country in the world, including the U.S. Efforts to contain the spread of COVID-19 have intensified and many countries, including the U.S., have implemented severe travel restrictions, business shutdowns and social distancing measures that have impacted clinical development through supply chain shortages and clinical trial enrollment difficulties as hospitals reduce and redeploy staff, divert resources to patients suffering COVID-19 and limit hospital access for non-patients. The pandemic poses the risk that we, our contractors, suppliers, or other partners may be prevented from conducting normal business activities for an indefinite period of time, including those due to shutdowns that may be requested or mandated by governmental authorities.

The continued global spread of COVID-19 has affected our operations but did not have a material impact on our business, operating results, financial condition or cash flows as of and for the year ended December 31, 2020. For example, several of our Phase 1 trials of oral ganaxolone to support the CDD indication have continued enrollment and are expected to be completed by the end of the second quarter of 2021, despite experiencing delays in enrollment due to COVID-19. Further, in response to COVID-19, for our ongoing clinical trials, we have implemented multiple measures consistent with guidance of the FDA on the conduct of clinical trials of medical products during the COVID-19 pandemic, including implementing remote site monitoring and remote visits using telemedicine where needed. However, COVID-19 may still adversely impact our clinical trials. For example, our RAISE Trial in refractory status epilepticus (RSE) is being conducted in hospitals, and resources related to the COVID-19 pandemic may divert staffing in hospitals, taking resources away from our clinical trial. Our ganaxolone clinical trials in the outpatient setting may be negatively impacted if patients and their caregivers do not want to participate in a clinical trial while the COVID-19 pandemic continues to severely affect the world. Although operations were not materially affected by the COVID-19 pandemic as of and for the year ended December 31, 2020, we are unable to predict the impact that COVID-19 will have in the future on our business, financial position, operating results and cash flows due to numerous uncertainties. The duration and severity of the pandemic and its long-term impact on our business are uncertain at this time, and our ability to raise sufficient additional financing depends on many factors beyond our control, including the current volatility in the capital markets as a result of the COVID-19 pandemic.

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)

Status epilepticus (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 established SE (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. Patients with RSE unresponsive to one or more second-line AEDs may be given an IV anesthetic to terminate seizures and prevent neuronal injury and other complications. SE that recurs following an attempted wean of IV anesthesia is classified as super refractory status epilepticus (SRSE). We estimate the number of cases of SE in the United States and Europe to be approximately 156,000 per year, with approximately 50% progressing to ESE and approximately 50% of those patients further progressing to ESE. In April 2016, we were granted FDA orphan drug designation for IV formulation of ganaxolone for the treatment of SE.

In September 2019, we announced positive top-line results in our open-label, dose-finding Phase 2 clinical trial evaluating IV ganaxolone in patients with RSE. The trial enrolled 17 medically heterogeneous patients who received an infusion of IV ganaxolone for up to 96 hours added to standard-of-care In addition to the target dose (713 mg/day), patients were enrolled into low dose (500 mg/day) and medium dose (650 mg/day) groups. Patients in the trial had failed a mean of 2.1 second-line IV AEDs (a mean of 2.9 total AED medications, including benzodiazepines) administered at therapeutic dose levels for an average of four hours prior to ganaxolone treatment.

In this clinical trial, ganaxolone met the primary endpoint with no patients (n=17) progressing to IV anesthetics within 24 hours of treatment initiation. The following table summarizes efficacy data through the four-week post treatment follow-up visit:

Cohort	No escalation to IV anesthetics within 24 hrs from infusion initiation(Primary Endpoint)	Status-free through 24 hrs from infusion initiation*	No escalation to additional IV AEDs or IV anesthetics for status relapse at any time through 24 hrs after ganaxolone discontinuation	No SE relapse during the 4-wk follow up period
Target (713 mg/day) *(n=8)*	**100%** (8 of 8)	**88%** (7 of 8)	**100%** (8 of 8)	**100%** (6 of 6)
Medium (650 mg/day) *(n=4)*	**100%** (4 of 4)	**100%** (4 of 4)	**75%** (3 of 4)	**67%** (2 of 3)
Low (500 mg/day) *(n=5)*	**100%** (5 of 5)	**100%** (5 of 5)	**60%** (3 of 5)	**50%** (1 of 2)

*Investigator determination

In addition, the median time to status cessation across all dose cohorts was five minutes. Additional long-term data demonstrate that patients in the target dose cohort who were assessed at the end of a four-week follow up period (n=6) did not experience status relapse. An independent retrospective central review of continuous electroencephalography (EEG) data demonstrated that the target dose level provided sustained reductions in seizure burden (greater than 80%) throughout the entire analysis window.

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 RSE. The BARDA Contract provides funding to support, on a cost-sharing basis, the completion of a Phase 3 clinical trial of IV-administered ganaxolone in patients with RSE (our Phase 3 clinical trial evaluating IV ganaxolone for the treatment of RSE (RAISE Trial)), funding of pre-clinical studies to provide support that IV-administered ganaxolone could be an effective treatment for RSE due to chemical nerve gas agent exposure, and funding of certain manufacturing scale-up and regulatory activities.

The BARDA Contract consists of a base period of approximately a two year duration during which BARDA will provide 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 base period, the BARDA Contract provides for approximately $30 million of additional BARDA funding for three options in support of manufacturing, supply chain, clinical, regulatory and toxicology activities. 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 $51 million, if all development options are completed. The contract period-of-performance (base period plus option exercises) is up to approximately five years.

In January 2021, we enrolled the first patient in the RAISE Trial. The RAISE Trial is a randomized, double-blind, placebo-controlled clinical trial in patients with RSE. Approximately 80 trial sites in hospitals across the U.S. will participate. The 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 percent power to detect a 30 percent 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 the treatment of RSE, and (2) proportion of patients with no progression to IV anesthesia for 36 hours following treatment initiation. We plan to announce top-line data from the trial in the first half of 2022. Planning continues for a separate RSE trial to be conducted in Europe. We are meeting with the EMA to discuss trial design in the first quarter of 2021 and a registration trial is planned to commence in the first half of 2022.

In addition to the RAISE Trial, we have also provided IV ganaxolone under Emergency Investigational New Drug (eIND) applications for use in treating patients with SRSE and plans are also underway for a future SE clinical trial focusing on earlier intervention in ESE. The trial will be conducted in emergency rooms under exception from informed consent guidelines and will enroll patients with convulsive SE. We are making preparations to commence a Phase 2 clinical trial in ESE that is planned to begin in the first half of 2022. We expect that the trial will be composed of two stages, the initial open-label, dose optimization stage and subsequent double-blind placebo-controlled stage. We anticipate that during the open-label portion of the trial, multiple sequential cohorts of patients will be assessed to determine the bolus dose and subsequent IV infusion rate and infusion duration to be used in the double-blind second stage of the trial. We expect that this double-blind placebo-controlled phase will enroll approximately 80 ESE patients equally distributed among two arms of the trial who, in addition to standard of care, will receive either IV ganaxolone or placebo. We also expect that the primary efficacy endpoint of the trial 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 plan to announce top-line data from the trial in the middle of 2023.

CDKL5 Deficiency Disorder (CDD)

CDD is a serious and rare genetic disorder that is caused by a mutation of the cyclin-dependent kinase-like 5 (CDKL5) gene, located on the X chromosome. It predominantly affects females and is characterized by early-onset, difficult-to-control seizures and severe neuro-developmental impairment. The CDKL5 gene encodes proteins essential for normal brain function. Most children affected by CDD cannot walk normally, talk, or care for themselves. Many also suffer from scoliosis, visual impairment, gastrointestinal difficulties or sleep disorders. There are no medications approved specifically for the treatment of CDD. Genetic testing is available to determine if a patient has a mutation in the CDKL5 gene. To our knowledge, no previous late-stage clinical trials have been conducted in this patient population and we estimate the CDD population to be approximately 12,500 patients in the United States and Europe. In June 2017, we were granted FDA orphan drug designation for ganaxolone for the treatment of CDKL5 Disorder. Additionally, in November 2019, the EMA Committee for Orphan Medicinal Products (COMP) granted orphan drug designation for ganaxolone for the treatment of CDKL5 Disorder.

In September 2020, we announced that ganaxolone achieved the primary endpoint in a Phase 3 clinical trial (Marigold Study) in children and young adults with CDD. The Marigold Study was a global, double-blind, placebo-controlled trial that enrolled 101 patients between the ages of 2 and 21 with a confirmed disease-related CDKL5 gene variant. Patients underwent a six-week prospective baseline period to collect seizure data, followed by a 17-week double-blind treatment phase during which study treatment was added to existing AED therapy. Patients randomized to ganaxolone titrated over four weeks to a dose of up to 600 mg of oral liquid suspension three times a day and maintained that dose for the following 13 weeks. Following the double-blind treatment phase, all patients had the opportunity to receive ganaxolone in the open label phase of the trial. The primary efficacy endpoint was percent change in 28-day seizure frequency for major motor seizures (tonic-clonic, tonic, atonic and clonic seizures). Patients given ganaxolone showed a 32.2% median reduction in 28-day major motor seizure frequency, compared to a 4.0% reduction for those receiving the placebo, a statistically significant difference for the primary endpoint ($p=0.002$). Ganaxolone was generally well-tolerated, demonstrating a safety profile consistent with previous clinical trials. The most frequent adverse event was somnolence.

We have received feedback from the U.S. Food and Drug Administration (FDA) that the efficacy and safety data resulting from the Marigold Study appear capable of supporting the filing of an NDA for ganaxolone in the treatment of CDD and a pre-NDA meeting with the FDA is scheduled for March 2021 to gain FDA alignment on the proposed format and content of the NDA. Subject to the feedback from the FDA, we plan to submit an NDA for ganaxolone in the treatment of CDD to the FDA in mid-2021 and a Marketing Authorization Application (MAA) for ganaxolone for the treatment of CDD to the European Medicines Agency (EMA) at the end of the third quarter of 2021.

We continue to execute on our pre-commercial development plans for CDD, while simultaneously exploring commercialization opportunities for ganaxolone in CDD with third parties to maximize patient access. As such, we have launched an Expanded Access Program (EAP), which will allow patients who were not able to participate in the

Marigold Study to begin receiving ganaxolone under a treatment protocol in advance of its potential commercial availability.

In July 2020, the FDA granted Rare Pediatric Disease (RPD) Designation for ganaxolone in the treatment of CDD. FDA grants RPD Designation for diseases that affect fewer than 200,000 people in the U.S. in which serious or life-threatening manifestations occur primarily in individuals 18 years of age and younger. If an NDA for ganaxolone in CDD is approved, we may be eligible to receive a priority review voucher from the FDA, which can be redeemed by us for priority review in a subsequent marketing application or potentially monetized by transferring such voucher to a third party. The Consolidated Appropriations Act, 2021, which was enacted on December 27, 2020, extended the priority review voucher program such that drugs designated for a rare pediatric disease by September 30, 2024 can receive a voucher if the drug is submitted and approved by September 30, 2026.

Tuberous Sclerosis Complex (TSC)

TSC is a rare genetic disorder that affects many organs and causes non-malignant tumors in the brain, skin, kidney, heart, eyes, and lungs. The condition is caused by inherited mutations in either the *TSC1* gene or the *TSC2* gene. TSC occurs with a frequency of 1:6,000 live births and a mutation is found in 85% of patients. While the disease phenotype can be extremely variable, epilepsy occurs in up to 85% of TSC patients. 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.

We are conducting a Phase 2 open-label trial (CALM Study) to evaluate the safety and tolerability of adjunctive ganaxolone treatment in patients with TSC. The trial is expected to enroll approximately 25 patients ages 2 to 65 and consists of a four-week baseline period followed by a 12-week treatment period and a 24-week extension. Patients will receive up to 600 mg of ganaxolone (oral liquid suspension) three times a day. The primary endpoint is the percent change in 28-day seizure frequency during the treatment period relative to baseline. We expect to report top-line data in the third quarter of 2021 and also plan to further explore whether allopregnanolone sulfate levels represent a response biomarker. The company's interim evaluation of the dataset in the CALM Study supports our plan to move to a Phase 3 trial in the third quarter of 2021. An End of Phase 2 meeting with the FDA is targeted for the second quarter of 2021 and a meeting with the EMA is targeted for the third quarter of 2021 with the first patient enrolled in the fourth quarter of 2021.

PCDH19-Related Epilepsy (PCDH19-RE)

PCDH19-RE is a rare epileptic syndrome characterized by early-onset seizures, cognitive and sensory impairment, and psychiatric and behavioral disturbances. Seizures occur in clusters lasting from several hours to days. The disorder is caused by a mutation in the PCDH19 gene on the X-chromosome. Unlike other X-linked disorders, it selectively affects females with very few cases reported in males. The gene encodes a protein involved in cell adhesion that is widely expressed in the central nervous system. There are no drugs approved specifically for the treatment of seizures associated with PCDH19-RE.

We conducted a Phase 2 proof-of-concept (POC) clinical trial (Violet Study) of ganaxolone treatment in patients with PCDH19-RE, in which enrollment is stratified based on allopregnanolone sulfate, a potential biomarker for the antiepileptic efficacy of ganaxolone. We enrolled 21 patients in one of two strata based on baseline allopregnanolone sulfate levels. The trial consists of a 12-week prospective baseline period, followed by a 17-week double-blind treatment phase. Patients were titrated over four weeks to a dose of up to 600 mg of ganaxolone oral liquid suspension or matching placebo three times daily and maintain that dose for the following 13 weeks. Patients are expected to be offered the opportunity to remain on ganaxolone following the completion of the protocol-specified treatment.

The primary efficacy endpoint was the percent change in 28-day primary seizure frequency during the 17-week double-blind phase relative to the baseline. The primary seizure types were defined in the protocol as countable focal seizures that included progressive hypotonia and impaired awareness, or any countable focal or generalized seizure with a clear motor component. Focal and generalized nonmotor seizures and myoclonic seizures did not count as the primary

seizure types for the primary efficacy endpoint. The analyses of the primary endpoint were performed on the sum of the individual countable seizures and each series of continuous uncountable seizures (each contributes one to the sum).

In March 2021, we announced top-line data from the Violet Study. In the trial, patients on ganaxolone experienced a median 61.5% reduction in primary-endpoint seizure frequency compared to a median 24.0% reduction in patients on placebo (p=0.17). Ganaxolone was generally well tolerated in the Violet Study, with one patient discontinuing on the ganaxolone arm due to a serious adverse event (SAE), psychogenic nonepileptic seizures, judged by the investigator to be ganaxolone-related. There was no allopregnanolone-sulphate biomarker signal.

Orphan Designations

The FDA has granted orphan drug designation to ganaxolone for the treatment of SE, CDD and PCDH19-RE. 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 United States. 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, antiepileptic and sedative activity. Unlike allopregnanolone, ganaxolone cannot be 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 $GABA_A$ receptor-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 treating patients who have developed tolerance to benzodiazepines and barbiturates, such as occurs during RSE.

Safety Overview

Oral Safety

More than 1,600 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. No drug-related deaths occurred in any of these clinical trials and the majority of adverse events (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 the adult or pediatric populations.

In the Marigold Study, ganaxolone was generally well tolerated with a safety profile consistent with previous clinical trials. The most frequent AE was somnolence.

IV Safety

In 2016, we completed a Phase 1 dose-escalation trial with IV ganaxolone that enrolled 36 patients, designed to determine the pharmacokinetics (PK), pharmacodynamics (PD), and safety of IV ganaxolone administered as an

ascending bolus dose (Stage 1) or continuous infusion (Stage 2). Four subject cohorts were enrolled in Stage 1 and one cohort was enrolled in Stage 2.

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 study 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 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. Furthermore, neither ganaxolone nor its metabolites have a ketone ring at the 3-position, a requirement for hormonal activity. In binding 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. We are working to establish the chemical structure of M2. In October, the FDA communicated that the characterization of the activity of the M2 metabolite would need to be included in the NDA submission. Results from any additional studies that may be required based on an evaluation of these or additional data sets could be submitted during the review or, if unavailable, in response to a post-marketing requirement(s) if the application is approved. We have recently completed an activity assay for the M2 metabolite and believe the results are consistent with our expectations and supportive of a mid-year 2021 filing.

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.

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 hospital-based rare and underserved indications for ganaxolone.*** We believe that hospitalized SE patients who do not respond to available first- and second-line treatment options are significantly underserved with severely limited treatment options and are at high risk of morbidity and mortality. Due to its activity at extrasynaptic $GABA_A$ receptors, ganaxolone may provide a therapeutic benefit as second-line therapy for patients whose SE is refractory to treatment with benzodiazapines. To that end, and based on our recent Phase 2 trial results, we are conducting the RAISE Trial in RSE patients and may in the future study similar and other hospital-based patient populations that could benefit from ganaxolone's mechanism of action. If our clinical trials are successful, we may in-license complementary assets to leverage our development and commercial investments.
- ***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 GABAergic tone with ganaxolone could provide 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, PCDH19-RE, 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 development and commercial investment if we elect to commercialize ganaxolone for these indications, if approved.
- ***Pursuing targeted depression and other neuropsychiatric disorder indications for ganaxolone.*** Due to its mechanism of action, we believe ganaxolone has potential for therapeutic benefit in a variety of targeted neuropsychiatric disorders. Data from preclinical studies and clinical trials demonstrate that treatment with ganaxolone could benefit patients with depression, anxiety, mood, sleep and other neuropsychiatric disorders. We may also explore development of ganaxolone in other targeted depression-related, neuropsychiatric conditions. We have currently deferred further development of ganaxolone for the treatment of depression and other neuropsychiatric disorders in order to focus our efforts and our resources on our ongoing development of ganaxolone for SE and orphan refractory epilepsy indications.
- ***Building on our product pipeline.*** We intend to expand and diversify our product pipeline through further development of ganaxolone in additional indications and/or acquisition of additional drug candidates that fit our business strategy. In addition, we may expand the targeted indication footprint and explore new potential formulations for our ganaxolone franchise.

Intellectual Property

The proprietary nature of and protection for our product candidates, discovery programs and know-how are important to our business. We have sought patent protection in the United States 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 11 different patent families, filed in various jurisdictions around the world.

Nanoparticle Ganaxolone Formulations. We own two 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 United States 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 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. For more information regarding the DRI and NovaMedica agreements, please see "Intellectual Property –

Licenses and Collaborations." A second patent family directed to injectable nanoparticle neurosteroid formulations consists of one granted United States patent and one pending United States application. The 20-year term of this patent family runs through 2036, absent any available patent term adjustments.

Process for Manufacturing Ganaxolone. Our patent portfolio contains patents issued in Australia, Canada, China, Europe, Hong Kong, India, Israel Japan, Mexico, New Zealand, South Korea, and the United States 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. A corresponding foreign patent application is pending in Brazil.

Intravenous Ganaxolone Formulations. We own three 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 United States 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 pending international application filed under the Patent Cooperation Treaty (PCT) that is directed to certain therapeutic regimens for the treatment of SE using IV 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 international application will run through 2040, absent any available patent term adjustments. A third patent family currently consists of a pending U.S. provisional application directed to certain therapeutic uses of IV ganaxolone. We intend to file an international patent application under the PCT before the applicable deadline.

Additional Therapeutic Uses. We own five patent families directed to certain therapeutic uses of ganaxolone, including for treating genetic epilepsy disorders, such as, CDD and PCDH19-RE, TSC, and depressive disorders. One of the patent families includes pending applications filed in Australia, Canada, China, Eurasia, Europe, India, Japan, Korea, Malaysia, New Zealand, Singapore, Vietnam, and the United States 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. We also own two patent families directed to certain methods of treating depressive disorders. One patent family consists of two granted United States patents and one allowed US patent directed to certain methods of using ganaxolone for treating postpartum depression and related disorders. The 20-year patent term of this family runs through 2037, absent any available patent term adjustments. A second patent family currently includes one pending international patent application filed under the PCT that claims certain methods of using ganaxolone for treating postpartum depression using ganaxolone. The 20-year term of this family runs through 2039, absent any available patent term extensions. Our fifth patent family currently comprises one pending United States provisional application directed to certain new therapeutic regimens for treating epileptic disorders. We intend to file an international patent application under the PCT before the applicable deadline.

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.

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 (the 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 FDA approval, 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

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 has 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 December 31, 2020, we had not met any additional milestones under the License Agreement and had not made any additional payments to CyDex other than the upfront fee; however we achieved a milestone in the first quarter of 2021 with a payment now due. 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.

NovaMedica

In December 2012, we entered into a Technology Transfer Agreement (Transfer Agreement) with DRI. Pursuant to the Transfer Agreement, in exchange for a payment of $100,000, we assigned to DRI certain patents and patents applications in Armenia, Azerbaijan, Belarus, Georgia, Kazakhstan, Kyrgyzstan, Moldova, Russia, Tajikistan, Turkmenistan, Ukraine and Uzbekistan (the Covered Territory), and granted to DRI an exclusive, royalty-free, irrevocable and assignable license under our know-how to develop and commercialize ganaxolone and other products that would infringe our patent rights or use our know-how (the Covered Products) in the Covered Territory, in the field

of uses for any human or animal disease or condition excluding the treatment of unpleasant sensory or emotional experience associated with actual or potential tissue damage or described in terms of such damage (the Field). DRI subsequently transferred all of its rights and obligations under the Transfer Agreement to NovaMedica. Under the terms of the Transfer Agreement, NovaMedica, or its permitted transferees or assignees, has the exclusive right within the Covered Territory to manufacture the Covered Products solely for development and commercialization in the Covered Territory in the Field. Until the first commercial sale of a Covered Product within the Covered Territory, NovaMedica will have the right to purchase supplies of the Covered Product from us on a cost-plus basis, subject to certain limitations. The Transfer Agreement also provides that we will enter into the Supply Agreement with NovaMedica to supply ganaxolone and/or Covered Product for development in the Covered Territory at a future date.

In June 2013, we entered into a Clinical Development and Collaboration Agreement (the Collaboration Agreement) with NovaMedica, pursuant to which we agreed to assist NovaMedica in the development and commercialization of Covered Products in the Covered Territory in the Field. The Collaboration Agreement requires the formation of committees consisting of our representatives and NovaMedica representatives to oversee the general development, day-to-day development work and commercialization of Covered Products in the Field in the Covered Territory. NovaMedica is required to reimburse us for any out-of-pocket expenses incurred by us in providing this assistance, except for expenses incurred in our participation on the joint committees. Pursuant to the Collaboration Agreement and the Transfer Agreement, we have agreed to use commercially reasonable efforts to include sites in the Russian Federation in our clinical trial programs for the first indications of the Covered Products at our sole expense. Under the Transfer Agreement, at least 36 months prior to the first commercial sale of a product candidate in the Covered Territory, the parties have agreed to negotiate in good faith a supply agreement pursuant to which we or a third party contract manufacturer authorized by us to manufacture and supply the Covered Products, will supply needed quantities of Covered Product to NovaMedica solely for commercialization of Covered Products in the Covered Territory, on commercially fair and reasonable terms. Such purchases will be made on a cost-plus basis. The Collaboration Agreement expires on the earlier of three years following the first commercial sale of a product candidate in the Covered Territory or the termination of the Transfer Agreement. NovaMedica also has the right to terminate the Collaboration Agreement at any time at its convenience upon 90 days' prior written notice.

Purdue Neuroscience Company (Purdue)

In September 2004, we entered into a license agreement with Purdue, which was amended and restated in May 2008, 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.

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 and institutional experience. Compared to us, many of our competitors have significantly greater financial, technical and human resources.

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 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 established SE (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, and there are no other companies currently conducting clinical trials in SE patients.

CDD, TSC and PCDH19-RE

There are no drugs approved specifically for the treatment of CDD or PCDH19-RE, 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, PCDH19-RE 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 development for the treatment of CDD (Ovid Therapeutics, Inc.'s OV935), no other ongoing clinical trials in PCDH19-RE, and no other ongoing clinical trials in TSC.

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 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 active pharmaceutical ingredient and finished product will be capable of providing sufficient quantities of each to meet our clinical trial supply needs. Other CMOs may be used in the future for clinical supplies and, subject to approval, 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

If we obtain FDA approval for ganaxolone, we intend to build sales and marketing infrastructures to reach high prescribing neurologist, critical care, epilepsy specialists and other target physician populations in the United States. We believe a focused sales and marketing organization could be leveraged to market ganaxolone across multiple epilepsy, neurology or psychiatry indications if we are able to obtain regulatory approval for those other indications. We may seek co-promotion partners for our sales efforts to reach other United States physician groups, such as primary care physicians. We believe that there could also be significant market opportunities for ganaxolone in epilepsy and other neurological and psychiatric conditions outside of the United States. In order to capitalize on such opportunities, we plan to seek collaborations with pharmaceutical companies that have greater reach and resources by virtue of their size and experience in the field.

Government Regulation

As a clinical stage pharmaceutical company that operates in the United States, we are subject to extensive regulation by the FDA, and other federal, state, and local regulatory agencies. The Federal Food, Drug, and Cosmetic Act (the 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 United States, 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 United States, 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 United States, 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 United States 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 United States 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, pharmacokinetic properties (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 market approval for the product in specified indications.

New Drug Applications

In order to obtain approval to market a drug in the United States, 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 patient dependence and abuse are classified as controlled substances under the Controlled Substances Act and similar state and foreign laws. In the United States, for new chemical entities under development for therapeutic use, FDA makes recommendations about whether a drug should be scheduled as a controlled substance, and the Drug Enforcement Administration (DEA) makes the final determination. In the case of a new drug approved by the FDA, the final DEA scheduling determination generally occurs several months, and in some cases longer, after FDA approval of the NDA. 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 its potential for dependence and abuse and its medicinal uses. 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 special DEA licenses in order to prescribe controlled substances.

Breakthrough Therapy Designation

In the United States, 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.

In July 2020, the FDA granted 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. in which serious or life-threatening manifestations occur primarily in individuals 18 years of age and younger. If an NDA for ganaxolone in CDD is approved, we may be eligible to receive a priority review voucher from the FDA, which can be redeemed for priority review in a subsequent marketing application.

Post-Approval Regulation

After regulatory approval of a drug is obtained, a sponsor is required to comply with a number of 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.

Manufacturing must continue to conform to cGMP after approval, and the FDA periodically inspects manufacturing facilities to assess compliance with cGMP. 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. 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 result in restrictions on a product, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action that could delay or prohibit further marketing.

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 United States Department of Justice (DOJ) or the Office of the Inspector General of the United States 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.

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 and Norway) 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.

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 United States, or, if the disease or condition affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making the drug would be recovered from sales in the United States. 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 in addition 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 United States, 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, which means 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 sufficiently profitable not 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, which 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.

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, which 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.

The Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act (FCPA) prohibits any United States individual or business from paying, offering, or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring 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 United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Some countries outside of the United States 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 Trial Directive, the details of which may vary per EU Member State. In addition, when conducting a clinical trial in the EU, the processing of personal data, including pseudonymized data, would have to 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 product 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 is 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 2020, 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, 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, EMA ensures that the opinion of the CHMP is given within 150 days.

Healthcare Reform

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (the 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 payment 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;

- 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 repeal, replace, or otherwise modify them or to alter their interpretation and implementation. For example, the Tax Cuts and Jobs Act (the 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 repeal, replace, or otherwise modify or invalidate, the Affordable Care Act, 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 of an amount greater than $1.2 trillion for the years 2013 through 2021, triggering 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 2030 (with the exception of a temporary suspension from May 1, 2020 through March 31, 2021) unless additional Congressional action is taken. 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.

We anticipate that the Affordable Care Act 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 United States 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 will likely negotiate discounted prices for our products. Federal, state and local governments in the United States continue to consider legislation to limit the growth of healthcare costs, including the cost of prescription drugs.

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 United States 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 (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. For example, in November 2020, the U.S. Department of Health and Human Services finalized a previously abandoned proposal to amend the discount safe harbor regulation of the AKS in a purported effort to create incentives to manufacturers to lower their list prices, and to lower federal program beneficiary out-of-pocket costs. The rule, which is currently slated to take full effect January 1, 2023, revises the AKS discount safe harbor to exclude manufacturer rebates to Medicare Part D plans, either directly or through pharmacy benefit managers (PBMs), creates a new safe harbor for point-of-sale price reductions that are set in advance and are available to the beneficiary at the point-of-sale, and creates a new safe harbor for service fees paid by manufacturers to PBMs for services rendered to the manufacturer. It is too early to know whether the U.S. administration

under President Biden will further delay, rewrite, or allow the rule to go into effect, and if so, what the effect of the rule will be on negotiations of coverage for our products with Medicare Part D plans, or whether the rule will affect our coverage arrangements with commercial insurers. It is also unclear whether the rule will have the intended effect of reducing net prices and beneficiary out-of-pocket costs without also increasing Medicare Part D premiums, which may impact the willingness of Part D plans to cover our products and the price concessions or other terms the plans or their PBMs may seek from us. 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. Beginning in 2022, applicable manufacturers also will be 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"—independent contractors or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. 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 obtain, use, or disclose 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 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, and time consuming. Federal regulators, state attorneys general, and plaintiffs' attorneys have been and will likely continue to be active in this space.

In California, the CCPA took effect on January 1, 2020. The CCPA establishes certain requirements for data use and sharing transparency and creates new data privacy rights for consumers. 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. 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 became applicable on May 25, 2018 and is directly applicable in each EEA country. The Regulation will result in a more uniform application of data privacy laws across the EEA. The GDPR imposes strict requirements and onerous accountability obligations on companies that 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.

In addition to the foregoing requirements, we expect to participate in and have certain price reporting obligations to the Medicaid Drug Rebate Program. Under the Medicaid Drug Rebate Program, if we successfully commercialize one or more products for which we receive regulatory approval, we would be 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 to the states for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data, such as average manufacturer price and best price, that we would have to 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 United States in any pricing structure, calculated to include all sales and associated rebates, discounts and other price concessions. 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 modified existing Medicaid Drug Rebate Program regulations to permit reporting multiple best price figures with regard to value-based purchasing arrangements (beginning in 2022); provide definitions for "line

extension," "new formulation," and related terms with the practical effect of expanding the scope of drugs considered to be line extensions (beginning in 2022); and revise manufacturer price and best price exclusions of manufacturer-sponsored patient benefit programs, specifically regarding inapplicability of such exclusions in the context of pharmacy benefit manager "accumulator" programs (beginning in 2023). Our failure to comply with the aforementioned price reporting and rebate payment obligations if we participate in the Medicaid Drug Rebate Program could negatively impact our financial results.

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. In addition, legislation may be introduced that, if passed, would further expand the 340B program to additional covered entities or would require participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting.

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, legislation may be introduced that, if passed, would further expand the 340B program to additional covered entities or would require participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting.

Federal law also requires that a company that participates in the Medicaid Drug Rebate Program report average sales price information each quarter to CMS for certain categories of drugs that are paid 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. In a November 20, 2020 interim final rule, CMS established a "Most Favored Nation" demonstration model that would lower Medicare Part B reimbursement of certain drugs based on international reference prices. The rule has become subject to judicial challenges, and federal courts have enjoined the rule at this time. There is also proposed legislation pending that would establish an international reference price-based payment methodology.

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 "*If we participate in the Medicaid Drug Rebate Program and 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 United States 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 United States 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 every full-time employee a cash target bonus, a comprehensive benefits package and equity compensation.

Historically, we have experienced a low turnover of employees. During 2020, our voluntary turnover rate was less than 5%.

As of December 31, 2020, we had 65 full-time employees and 7 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. 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 the Company's facilities. These include, but are not limited to, social distancing, capacity limitations, mask requirements in common areas, weekly deep cleaning and daily sanitation procedures.

Reverse stock split

On September 23, 2020, we effected a 1-for-4 reverse split of shares of our common stock (Reverse Split), as approved by our board of directors and stockholders. The par value per share of our common stock was not adjusted as a result of the Reverse Split, and our authorized shares of common stock was reduced to 150,000,000. All of the share and per share amounts included in this Annual Report on Form 10-K have been adjusted to reflect the Reverse Split.

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

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 incurred significant losses since our inception and anticipate that we will continue to incur losses in the future.

We have incurred significant operating losses since our inception, including a net loss of $67.5 million for the year ended December 31, 2020. As of December 31, 2020, we had an accumulated deficit of $311.9 million. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital. Our losses have resulted principally from costs incurred in our research and development activities. We anticipate that our operating losses will substantially increase over the next several years as we execute our plan to expand our research, development and commercialization activities, including the clinical development and planned commercialization of our product candidate, ganaxolone. In addition, if we obtain regulatory approval of ganaxolone, we may incur significant sales and marketing expenses. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or whether or when we will become profitable, if ever. The net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our

future performance. In any particular quarter or quarters, our operating results could be below the expectations of securities analysts or investors, which could cause our stock price to decline.

We have not generated any 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.

We have no products approved for commercial sale and have not generated any revenue from sales of any of our product candidates, and we do not know when, or if, we will generate revenues in the future. Our ability to generate revenue from product sales and achieve profitability will depend upon our ability to successfully gain regulatory approval and commercialize ganaxolone or other product candidates that we may develop, in-license or acquire in the future. Even if we obtain regulatory approval for ganaxolone, we do not know when we will generate revenue from product sales, if at all. Our ability to generate revenue from product sales of 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 product candidates for which we obtain regulatory approval;
- obtain market acceptance of our 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;
- 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, 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 ganaxolone, we anticipate incurring significant costs associated with commercializing ganaxolone.

Even if we are able to generate revenue from the sale of 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 complete the development and commercialization of ganaxolone.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to advance the clinical and regulatory development of ganaxolone and, if approved, commercialize ganaxolone. We will require additional capital for the further development, regulatory submission and potential commercialization of ganaxolone and may also need to raise additional funds sooner should we choose to accelerate development of ganaxolone. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

We believe that our cash, cash equivalents and investments as of December 31, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2022. 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:

- initiation, progress, timing, costs and results of preclinical studies and clinical trials, including patient enrollment in such trials, for ganaxolone or any other future product candidates;
- clinical development plans we establish for ganaxolone and any other future product candidates;
- obligation to make royalty and non-royalty sublicense receipt payments to third-party licensors, if any, under our existing or any future licensing agreements;
- our ability to receive funding under the BARDA Contract;
- number and characteristics of product candidates that we discover or in-license and develop;
- outcome, timing and cost of regulatory review by the FDA, the EMA and other comparable foreign regulatory authorities, including the potential for the FDA, the EMA or other comparable foreign regulatory authorities to require that we perform more studies or clinical trials than those that we currently expect;
- costs of filing, prosecuting, defending and enforcing any patent claims and maintaining and enforcing other intellectual property rights;
- effects of competing technological and market developments;
- costs and timing of the implementation of commercial-scale manufacturing activities; and
- costs and timing of establishing sales, marketing and distribution capabilities for ganaxolone or any other product candidates for which we may receive regulatory approval.

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.

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 our sole clinical stage product candidate, ganaxolone, and may fail to capitalize on other technologies or 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 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 activities for ganaxolone and performing research and development with respect to our preclinical and clinical programs. In addition,

as a clinical stage pharmaceutical company, 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. Nor have we demonstrated an ability to obtain regulatory approval to commercialize any product candidate. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a history of successfully developing and commercializing pharmaceutical products. Further, our budgeted expense levels are based in part on our expectations concerning the costs of 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. 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. 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, 2020, we had U.S. net operating loss, or NOL, carryforwards of approximately $212.0 million for U.S. federal income tax and approximately $209.5 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 $11.5 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 2023 if not utilized.

Our U.S. NOL and tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under Section 382, and corresponding provisions of U.S. state law, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change U.S. NOLs and other pre-change tax attributes, such as research and development tax credits, to offset its post-change income may be limited. We have completed several financings since our inception that may have resulted in "ownership changes" within the meaning of Section 382. We have not evaluated the ownership history of our company to determine if there were any ownership changes as defined under Section 382 and the effects any ownership change may have had. 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 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 clinical trials and will require significant capital resources and years of additional clinical development effort.

We do not have any products that have gained regulatory approval in any jurisdiction. Our only clinical stage product candidate is ganaxolone. 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 United States without first obtaining regulatory approval from the FDA; similarly, we cannot commercialize ganaxolone outside of the United States 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 United States, 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.

The FDA has provided guidance to the industry that the substantial evidence requirement for effectiveness, which had generally been interpreted as calling for two adequate and well-controlled clinical trials, could also be met by

a single clinical trial plus confirmatory evidence. In September 2020, we announced that ganaxolone achieved the primary endpoint in a pivotal Phase 3 clinical trial (Marigold Study), which evaluated the use of oral ganaxolone in children and young adults with CDD. We requested feedback from the FDA as to whether the Marigold Study could serve as a single pivotal efficacy study to support the approval of ganaxolone for the treatment of CDD. Based on the information we provided the FDA, which included supportive data from an earlier clinical trial, the FDA responded that the efficacy and safety data to be included in our planned NDA appear capable of supporting the filing of our planned NDA. The adequacy of these data to support an approval of ganaxolone for CDD will be a matter for FDA review of the application. There is a risk that the FDA may determine as a result of their review of our NDA, that we have not met the FDA requirements for ganaxolone approval.

In addition, 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. We are working to establish the chemical structure of M2. In October, the FDA communicated that the characterization of the activity of the M2 metabolite would need to be included at the time of submission; results from any additional studies that may be required based on an evaluation of these data could be submitted during the review or, if unavailable, in response to a post-marketing requirement(s) if the application is approved. If such additional non-clinical data indicates a safety issue, it may impact approvability or the FDA may impose serious and extensive restrictions on the commercialization of oral ganaxolone for CDD, which could have a material adverse impact on our business, results of operations and financial condition. We have recently completed an activity assay for the M2 metabolite and believe the results are consistent with our expectations and supportive of a mid-year 2021 filing. We plan to submit an NDA for oral ganaxolone in the treatment of CDD to the FDA in mid-2021 and an MAA for oral ganaxolone for the treatment of CDD to the European EMA at the end of the third quarter of 2021.

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 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. 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 ganaxolone were to obtain approval from the FDA and comparable foreign regulatory authorities, 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 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 product candidate that we may in-license, develop or acquire in the future. Furthermore, even if we obtain 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.

Because the results of preclinical studies or earlier clinical trials are not necessarily predictive of future results, ganaxolone may not have favorable results in later clinical trials or receive regulatory approval.

Success in preclinical studies and early clinical trials does not ensure that later clinical trials will generate adequate data to demonstrate the efficacy and safety of ganaxolone. 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 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, 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 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. Although ganaxolone has generally been safe and 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.

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 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.

In addition, 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. We are working to establish the chemical structure of M2. In October, the FDA communicated that the characterization of the activity of the M2 metabolite would need to be included at the time of submission; results from any additional studies that may be required based on an evaluation of these data could be submitted during the review or, if unavailable, in response to a post-marketing requirement(s) if the application is approved. If such additional non-clinical data indicates a safety issue, it may impact approvability or the FDA may impose serious and extensive restrictions on the commercialization of CDD, which could have a material adverse impact on our business, results of operations and financial condition. We have recently completed an activity assay for the M2 metabolite and believe the results are consistent with our expectations and supportive of a mid-year 2021 filing.

If ganaxolone receives marketing approval, and we or others later identify undesirable side effects caused by ganaxolone, 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 United States 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, if approved.

Additionally, the FDA may recommend scheduling of ganaxolone as a controlled substance if it determines ganaxolone has abuse potential. In such event, the DEA will need to determine the controlled substance schedule of the product, taking into account the recommendation of the FDA. The timing of the scheduling process is uncertain and may delay our ability to market ganaxolone if approved. If ganaxolone is determined to be 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, if approved.

The therapeutic efficacy and safety of ganaxolone are unproven, and we may not be able to successfully develop and commercialize ganaxolone in the future.

Ganaxolone is a novel compound and its potential therapeutic benefit is unproven. Our ability to generate revenue from ganaxolone, which we do not expect will occur for at least the next several years, if ever, will depend on our successful development and commercialization after regulatory approval, 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, we may need to abandon its development or 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 initially showed promise in early stage testing for treating the target indications for ganaxolone have later been found to cause side effects that prevented further development of the compound. 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, enter into or maintain third-party licensing or collaboration transactions with respect to, or successfully commercialize, ganaxolone, in which case we will not achieve profitability and the value of our stock may decline.

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 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;
- any shelter-in-place orders from local, state or federal governments or clinical trial site policies resulting from the COVID-19 pandemic that determine essential and non-essential functions and staff, 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 study 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 United States, 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 United States, 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.

Even if ganaxolone receives regulatory approval, we will still face regulatory difficulties.

Even if we obtain regulatory approval for ganaxolone, it would be subject to ongoing requirements by the FDA and comparable foreign regulatory authorities 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 cGMP and 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 United States 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 ganaxolone, if approved by the FDA, will be heavily scrutinized by, among others, the FDA, the DOJ, the 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 United States, will be heavily scrutinized by comparable foreign regulatory authorities.

In the United States, 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 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 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.

We may not be able to obtain orphan drug exclusivity for ganaxolone, which could limit the potential profitability of ganaxolone.

Regulatory authorities in some jurisdictions, including the United States 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 individuals in the United States. 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 United States for treating Infantile Spasms, SE, CDD, and PCDH19-RE 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 United States 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.

Even though we have received Rare Pediatric Disease (RPD) Designation for ganaxolone for the treatment of CDD, we may not receive a rare pediatric disease priority review voucher.

In July 2020, the FDA granted 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. in which serious or life-threatening manifestations occur primarily in individuals 18 years of age and younger. If an NDA for ganaxolone in CDD is approved, we may be eligible to receive a priority review voucher from the FDA, which can be redeemed for priority review in a subsequent marketing application. However, receiving an RPD Designation for ganaxolone for the treatment of CDD does not guarantee that an NDA for ganaxolone for the treatment of CDD will meet the eligibility criteria for a RPD priority review voucher at the time the application is approved. Under the FDC Act, we will need to request an RPD priority review voucher in our original NDA for ganaxolone. The FDA may determine that the NDA for ganaxolone, if approved, does not meet the eligibility criteria for an RPD priority review voucher, including for the following reasons:

- CDD no longer meets the definition of an RPD;
- ganaxolone contains an active ingredient (including any ester or salt of the active ingredient) that has been previously approved in an application;
- the NDA is not deemed eligible for priority review;
- the NDA does not rely on clinical data derived from studies examining a pediatric population and dosages of the drug intended for that population; or
- the NDA seeks approval for a different adult indication than the rare pediatric disease for which ganaxolone is designated.

The authority for the FDA to award RPD priority review vouchers for drugs after September 30, 2024 is currently limited to drugs that receive rare pediatric disease designation on or prior to September 30, 2024, and the FDA may only award RPD priority review vouchers through September 30, 2026. If the NDA for ganaxolone is not approved on or prior to September 30, 2026 for any reason, it will not be eligible for a priority review voucher. However, it is possible the authority for the FDA to award RPD priority review vouchers will be further extended by Congress.

If a priority review voucher is granted, we may use the voucher for our own FDA approval processes or decide to sell the voucher to other biotech or pharmaceutical companies. The market for priority review vouchers has a limited history and disclosed sales prices may not be indicative of the current value of vouchers, which may also fluctuate significantly. The Consolidated Appropriations Act, 2021, which was enacted on December 27, 2020, extended the priority review voucher program such that drugs designated for an RPD by September 30, 2024 can receive a voucher if the drug is submitted and approved by September 30, 2026. Further, the potential award of a voucher would trigger an obligation to market the relevant RPD product within one year from FDA approval or the FDA may revoke the voucher. Finally, a voucher award subjects us to additional post-marketing reporting obligations to the FDA.

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 United States generally includes all of the risks associated with obtaining FDA approval. In addition, many countries outside the United States 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 United States 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 United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the

FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. 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.

Ganaxolone may be regulated as a controlled substance, the making, use, sale, importation, exportation, and distribution of which is subject to significant regulation by the U.S. Drug Enforcement Administration (DEA) and other regulatory agencies.

The FDA may recommend controlled substance scheduling for ganaxolone. In such event, the DEA will need to determine the controlled substance schedule taking into account the recommendation of the FDA. 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. If ganaxolone is determined to be a controlled substance, the manufacturing, shipping, distribution, import, export, packaging, storing, prescribing, dispensing, selling and use of ganaxolone will be subject to an additional regulation, including under the CSA and DEA regulations. Regulations associated with controlled substances also govern production and procurement quotas, recordkeeping, reporting, handling, and disposal Additionally, if ganaxolone is determined to be a controlled substance, facilities conducting research, manufacturing, distributing, importing or exporting, or dispending ganaxolone must be registered (licensed) to perform these activities and have the security, control, recordkeeping, reporting and inventory mechanisms required by the DEA to prevent drug loss and intervention. 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 development commercialization of products. Because of their restrictive nature, these laws and regulations could also limit commercialization of ganaxolone, if approved. Failure to comply with these laws and regulations could also result in withdrawal of our 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, if approved, and adverse scheduling could impair the commercial attractiveness of ganaxolone. We must also obtain separate state registrations in order to be able to obtain, handle and distribute 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 the Commercialization of Our Product

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

Even if ganaxolone receives regulatory approval, it may not gain market acceptance among physicians, patients, government and private payers, or others in the medical community. Market acceptance of ganaxolone, if we receive approval, 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;
- product labeling or product insert requirements of the FDA or comparable foreign regulatory authorities;
- 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;
- relative convenience and ease of administration;
- effectiveness of our sales and marketing strategy and efforts;
- adequate commercial investment; and
- stability and continuity of product supply chains.

If ganaxolone is approved but 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 are restricted, withdrawn or recalled, or fail to be approved, as the case may be, we may not be able to generate significant revenues, which would compromise our ability to become profitable.

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 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 United States and worldwide, including the generic products levetiracetam, lamotrigine, carbamazepine, oxcarbazepine, valproic acid and topiramate. Recent market entrants include branded products developed by Lundbeck, UCB, Eisai, GW Pharmaceuticals, Zogenix, SK Biopharmaceuticals and Sunovion Pharmaceuticals. In addition, there are several drugs in development for the treatment

of pediatric orphan indications, including compounds being developed by GW Pharmaceuticals, Zogenix, Zynerba and Ovid.

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 establish sales and marketing capabilities or enter into agreements with third parties to market and sell ganaxolone, we may be unable to generate any revenue.

We do not currently have an organization for the sale, marketing and distribution of pharmaceutical products and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order to market ganaxolone, if approved by the FDA or comparable foreign regulatory authorities, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable. We will be competing with many companies that currently have extensive and well-funded sales and marketing operations. Without an internal commercial organization or the support of a third party to perform sales and marketing functions, we may be unable to compete successfully against these more established companies. To the extent we rely on third parties to commercialize ganaxolone, if approved, we may have little or no control over the marketing and sales efforts of such third parties, and our revenues from product sales may be lower than if we had commercialized ganaxolone ourselves.

Even if we are able to commercialize ganaxolone, it may not receive coverage and adequate reimbursement from third-party payers, which could harm our business.

Our ability to commercialize ganaxolone successfully will depend, in part, on the extent to which coverage and adequate reimbursement for ganaxolone and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payers, such as private health insurers and health maintenance organizations, determine which medications they will cover and establish reimbursement levels. A primary trend in the United States healthcare industry and elsewhere is cost containment. Government authorities and third-party payers have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payers are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for drugs. Third-party payers may also seek additional clinical evidence, beyond the data required to obtain marketing approval, demonstrating clinical benefits and value in specific patient populations before covering ganaxolone for those patients. We cannot be sure that coverage and adequate reimbursement will be available for ganaxolone and, if reimbursement is available, what the level

of reimbursement will be. Coverage and reimbursement may impact the demand for, or the price of, ganaxolone, if we obtain marketing approval. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize ganaxolone even if we obtain marketing approval.

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 reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or 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 United States. Third-party payers often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to obtain coverage and profitable reimbursement rates from both government-funded and private payers for any approved products that we develop 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 ganaxolone 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 rare 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 United States 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.

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 United States, 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 withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities 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 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 if ganaxolone receives regulatory approval and we subsequently commercialize it. Product liability claims may be brought against us by subjects 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 intend to expand our product liability insurance coverage to include the sale of commercial products if we obtain marketing approval for ganaxolone, but we may be unable to obtain commercially reasonable product liability insurance for ganaxolone, if approved for marketing. 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.

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 deadlines, our development plans may be adversely affected and we may not be able to obtain regulatory approval for or commercialize ganaxolone.

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.

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.

Our experience manufacturing ganaxolone is limited to the needs of our preclinical studies and clinical trials. We have no experience manufacturing ganaxolone on a commercial scale and have no manufacturing facility. We are dependent on third-party manufacturers for the manufacture of ganaxolone as well as on third parties for our supply chain, and if we experience problems with any such third parties, the manufacturing 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 chemical manufacture of raw materials and active pharmaceutical ingredients for ganaxolone and other CMOs for the production of the ganaxolone nanoparticulate formulation into capsules, liquid suspension and IV, and we plan to rely on CMOs for the manufacture of ganaxolone for commercial use, if approved. To meet our projected needs for preclinical and clinical supplies to support our activities through regulatory approval and 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.

Government funding for certain 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 (the 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 consists of an approximately two-year base period-during which BARDA will provide approximately $21 million of funding for the RSE Phase 3 clinical trial on a cost share basis and funding of additional preclinical studies of ganaxolone in nerve agent exposure models. Following successful completion of the RSE Phase 3 clinical trial and preclinical studies in the base period, the BARDA Contract provides for approximately $30 million of additional BARDA funding for three options in support of manufacturing, supply chain, clinical, regulatory and toxicology activities. 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 $51 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 United States 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 United States 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 United States 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 United States government. The United States government generally obtains the right to royalty-free use of technologies that are developed under United States 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 United States 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

Recently 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 United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of ganaxolone, restrict or regulate post-approval activities and affect our ability to successfully sell ganaxolone, if we obtain marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (the 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 further reductions in Medicare reimbursement for drugs administered by physicians. 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 payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our 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' 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. 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 repeal, replace or otherwise modify them or to alter their interpretation and 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 repeal, replace, or otherwise modify or invalidate, the Affordable Care Act or its implementing regulations, or portions thereof, or the political uncertainty surrounding any efforts to repeal, replace, or otherwise modify the Affordable Care Act 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 repeal, replacement, modification, or invalidation, in whole or in part, 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 a targeted deficit reduction of an amount greater than $1.2 trillion for the years 2013 through 2021, triggering 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 2030 (with the exception of a temporary suspension from May 1, 2020 through March 31, 2021) unless additional Congressional action

is taken. 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 additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations.

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 FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of ganaxolone may be.

In the United States, the EU and other potentially significant markets for ganaxolone, 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 United States 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.

If we participate in the Medicaid Drug Rebate Program and 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 expect to participate in and have certain price reporting obligations to the Medicaid Drug Rebate Program. Under the Medicaid Drug Rebate Program, if we successfully commercialize one or more products for which we receive regulatory approval, we would be 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 to the states for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data, such as average manufacturer price and best price, that we would have to report on a monthly and quarterly basis to the CMS, the federal agency that administers the Medicaid Drug Rebate Program. 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 United States in any pricing structure, calculated to include all sales and associated rebates, discounts and other price concessions. 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 modified existing Medicaid Drug Rebate Program regulations to permit reporting multiple Best Price figures with regard to value-based purchasing arrangements (beginning in 2022); provide definitions for “line extension,” “new formulation,” and related terms with the practical effect of expanding the scope of drugs considered to be line extensions (beginning in 2022); and revise AMP and Best Price exclusions of manufacturer-sponsored patient benefit programs, specifically regarding inapplicability of such exclusions in the context of pharmacy benefit manager “accumulator” programs (beginning in 2023). Our failure to comply with the aforementioned price reporting and rebate payment obligations if we participate in the Medicaid Drug Rebate Program could negatively impact our financial results.

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 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, legislation may be introduced that, if passed, would further expand the 340B program to additional covered entities or would require participating manufacturers to agree to provide 340B discounted pricing on drugs used in an inpatient setting.

Federal law also requires that a company that participates in the Medicaid Drug Rebate Program report average sales price information each quarter to CMS for certain categories of drugs that are paid 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. In a November 20, 2020 interim final rule, CMS established a "Most Favored Nation" demonstration model that would lower Medicare Part B reimbursement of certain drugs based on international reference prices. The rule has become subject to judicial challenges, and federal courts have enjoined the rule at this time. There is also proposed legislation pending that would establish an international reference price-based payment methodology.

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. If we participate in the Medicaid Drug Rebate Program and consequently the 340B program, we could be held liable for errors associated with our submission of pricing data. In addition to retroactive Medicaid rebates and the potential for 340B program refunds, 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. 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 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. Our failure to submit monthly/quarterly average manufacturer price and best price data on a timely basis could result in a significant civil monetary penalty per day for each day the information is late beyond the due date. Such failure 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.

CMS and the Department of Health & Human Services Office of Inspector General 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 VA 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. 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.

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 we overcharge 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 other laws and regulations. Unexpected refunds to the government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

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

As we seek to expand our operations outside of the United States, 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 United States 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 United States 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 United States, 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 United States 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 United States, 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 United States 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 United States 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 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, 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 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;

- HIPAA imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program, including private third-party payors, 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 applicable group purchasing organizations; 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 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. For a fuller discussion of the applicable anti-kickback, fraud and abuse, transparency, and other healthcare laws and regulations applicable to our business, see Item 1, "Business - Other Healthcare Laws and Compliance Requirements."

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"—independent contractors or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. 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 obtain, use, or disclose 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 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.

In California, the CCPA took effect on January 1, 2020. The CCPA establishes certain requirements for data use and sharing transparency and creates new data privacy rights for consumers. 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. 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 to laws and regulations enacted in the United States, including the new California Consumer Privacy Act of 2018, the EU the 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 entered into effect on May 25, 2018 and 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 EEA, we may be subject to additional privacy restrictions, 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 General Data Protection Regulation, or GDPR. 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 data protection regulation in the United Kingdom.

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 an incident that could affect our operations or compromise our business information or sensitive personal information, including health data.

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 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 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 importing country's level of protection must be adequate.

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 United States. 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 impact of Brexit on the on-going validity in the UK of current EU authorizations for medicinal products, whether granted through the centralized procedure, decentralized procedure, or mutual recognition, and on the future process for obtaining marketing authorization for pharmaceutical products manufactured or sold in the UK remains uncertain. 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 Good Manufacturing Practice (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 UK has unilaterally agreed to accept EU batch testing and batch release for a period of at least two years until January 1, 2023. However, 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. As regards marketing authorizations, Great Britain will have a separate regulatory submission process, approval process and a separate national MA. Northern Ireland will, however, continue to be covered by the marketing authorizations granted by the EC.

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 United States 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 United States 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 United States. 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 United States 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 United States 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 United States 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 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 United States 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 United States, 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.

In September 2020, Ovid Therapeutics, Inc. (Ovid) contacted us and disclosed that it owns two recently issued patents that include claims that encompass our product candidates for the treatment of CDD and PCDH19. 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 CDD and PCDH19, 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 CDD, or PCDH19, as applicable, 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 CDD and PCDH19, 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 CDD and PCDH19 altogether, which would have a material adverse impact on our business. We are also aware of a pending patent application by Ovid in the same patent family that includes claims that encompass our product candidate for the treatment of SE. This pending patent application is in the early stages of examination at the USPTO. It is possible that this pending patent application may issue as a patent with claims that encompass our product candidate for the treatment of RSE, in which case, the above risks would also apply to any such patent that was issued.

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 United States can be less extensive than those in the United States. 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 United States. 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 United States, or from selling or importing products made using our inventions into or within the United States 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 United States. 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 United States and, if available, in other countries where we are prosecuting patents. In the United States, 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 United States, 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.

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 United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. For example, the United States 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 United States 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 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 United States. 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 United States 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 United States 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

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

In December 2019, an outbreak of a novel strain of coronavirus (COVID-19) was identified in Wuhan, China. This virus was declared a pandemic by the World Health Organization in March 2020 and has spread to nearly every country in the world, including the United States. Many countries, including the United States, have implemented severe travel restrictions, business shutdowns and social distancing measures in order to contain the spread of COVID-19 that

have impacted clinical development through supply chain shortages and clinical trial enrollment difficulties as hospitals reduce and redeploy staff, divert resources to patients suffering from COVID-19 and limit hospital access for non-patients. The pandemic poses the risk that we, our employees, contractors, suppliers, or other partners may be prevented from conducting normal business activities for an indefinite period of time, including those due to shutdowns that may be requested or mandated by governmental authorities.

The continued global spread of COVID-19 has impacted our operations and such impacts may continue and become material to our operations. For example, several of our Phase 1 trials of oral ganaxolone to support the CDD indication have experienced delays in enrollment due to COVID-19. Further, in response to COVID-19, for our ongoing clinical trials, we have implemented multiple measures consistent with the guidance of the FDA on the conduct of clinical trials of medical products during the COVID-19 pandemic, including implementing remote site monitoring and remote visits using telemedicine where needed. However, COVID-19 may still adversely impact our clinical trials. For example, our Phase 3 clinical trial in RSE is conducted in the hospital and resources related to the COVID-19 outbreak may divert staffing in the hospital taking resources away from our clinical trial. Our ganaxolone clinical trials in the outpatient setting may be negatively impacted if patients and their caregivers do not want to participate in a clinical trial while COVID-19 outbreaks continue.

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. It is likely that the continued spread of COVID-19 will 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, 2020, we had 65 full-time and seven 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:

- 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 ganaxolone, if approved, 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.

Risks Related to Ownership of Our Common Stock

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 42.7% of our voting stock. Upon conversion of all of our outstanding convertible preferred stock, as of December 31, 2020, our executive officers, directors and holders of 5% or more of our capital stock collectively would beneficially own approximately 40.6% 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 United States 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.

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.

If our stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline. As of March 8, 2021, we had outstanding a total of 36,578,460 shares of common stock and 4,753 shares of Series A Participating Convertible Preferred Stock, par value $0.001 per share (Series A Preferred Stock). The Series A Preferred Stock would be convertible into 950,600 shares of common stock as of March 8, 2021, subject to certain ownership limitations. In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans or otherwise will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules and either the registration of such shares under the Securities Act of 1933, as amended (Securities Act) or the application of exemptions from such registration with respect to any sales such as Rule 144 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

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 4,753 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.

General Risk Factors

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 computer viruses, unauthorized access, natural disasters, fire, terrorism, war and telecommunication and electrical failures, cyberattacks or cyber-intrusions over the Internet, loss of funds or information from phishing or other fraudulent schemes, attachments to emails, persons inside our organization, or persons with access to systems inside our organization or those with whom we do business. 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 Company 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.

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

Our operations could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce ganaxolone. Our ability to obtain clinical supplies of ganaxolone could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption. In addition, while we believe that we currently have sufficient supply of our product candidates to continue our ongoing clinical trials, some of our product candidates, or materials contained therein, come from facilities located in areas impacted by the COVID-19 pandemic. There is no guarantee that the COVID-19 pandemic, or any potential future outbreak or pandemic, would not materially impact our future supply chain. The ultimate impact on us, our significant suppliers and our general infrastructure of being in certain geographical areas is unknown, but our operations and financial condition could suffer in the event of a major earthquake, fire or other natural disaster.

Our 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 United States 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.

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 8, 2021, there were approximately 20 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, 2020 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 Periodic Report on Form 8-K.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers

We did not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

Item 6. Selected Financial Data

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 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 clinical stage pharmaceutical company focused on developing and commercializing innovative therapeutics to treat patients suffering from rare seizure disorders. Our clinical stage product candidate, ganaxolone, is a positive allosteric modulator of $GABA_A$ that is being developed in formulations for two different routes of administration: intravenous (IV) and oral. Ganaxolone is a synthetic analog of allopregnanolone, an endogenous neurosteroid. 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. 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. At December 31, 2020, we had cash, cash equivalents and investment balances of $140.0 million. We have no products currently available for sale, have incurred operating losses since inception, have not generated any product sales revenue and have not achieved profitable operations. We incurred a net loss of $67.5 million for the year ended December 31, 2020. Our accumulated deficit as of December 31, 2020 was $311.9 million, and we expect to continue to incur substantial losses in future periods. We anticipate that our operating expenses will increase substantially as we continue to advance our clinical-stage product candidate, ganaxolone.

We anticipate that our expenses will increase substantially as we:

- conduct later stage clinical trials in targeted indications, which could include SE, CDD, TSC, PCDH19-RE and possibly other indications;
- continue the research, development and scale-up manufacturing capabilities to optimize ganaxolone and dose forms for which we may obtain regulatory approval;
- conduct other preclinical studies and clinical trials to support the filing of NDAs with the FDA, MAAs with the 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 and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our drug development efforts.

We believe that our cash, cash equivalents and investment balances as of December 31, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2022. 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 planned research and development activities with respect to ganaxolone.

COVID-19

In December 2019, an outbreak of a novel strain of coronavirus (COVID-19) was identified in Wuhan, China. This virus was declared a pandemic by the World Health Organization in March 2020 and has spread to nearly every country in the world, including the U.S. Efforts to contain the spread of COVID-19 have intensified and many countries, including the U.S., have implemented severe travel restrictions, business shutdowns and social distancing measures that have impacted clinical development through supply chain shortages and clinical trial enrollment difficulties as hospitals reduce and redeploy staff, divert resources to patients suffering COVID-19 and limit hospital access for non-patients. The pandemic poses the risk that we, our contractors, suppliers, or other partners may be prevented from conducting normal business activities for an indefinite period of time, including those due to shutdowns that may be requested or mandated by governmental authorities.

The continued global spread of COVID-19 has affected our operations but did not had a material impact on our business, operating results, financial condition or cash flows as of and for the year ended December 31, 2020. For example, several of our Phase 1 trials of oral ganaxolone to support the CDD indication have continued enrollment and are expected to be completed by the end of the second quarter of 2021, despite experiencing delays in enrollment due to COVID-19. Further, in response to COVID-19, for our ongoing clinical trials, we have implemented multiple measures consistent with guidance of the FDA on the conduct of clinical trials of medical products during the COVID-19 pandemic, including implementing remote site monitoring and remote visits using telemedicine where needed. However, COVID-19 may still adversely impact our clinical trials. For example, our RAISE Trial in RSE is being conducted in hospitals, and resources related to the COVID-19 pandemic may divert staffing in hospitals, taking resources away from our clinical trial. Our ganaxolone clinical trials in the outpatient setting may be negatively impacted if patients and their caregivers do not want to participate in a clinical trial while the COVID-19 pandemic continues to severely affect the world. Although operations were not materially affected by the COVID-19 pandemic as of and for the year ended December 31, 2020, we are unable to predict the impact that COVID-19 will have in the future on our business, financial position, operating results and cash flows due to numerous uncertainties. The duration and severity of the pandemic and its long-term impact on our business are uncertain at this time, and our ability to raise sufficient additional financing depends on many factors beyond our control, including the current volatility in the capital markets as a result of the COVID-19 pandemic.

Reverse stock split

On September 23, 2020, we effected a 1-for-4 reverse split of shares of our common stock (Reverse Split), as approved by our board of directors and stockholders. The par value per share of our common stock was not adjusted as a result of the Reverse Split, and our authorized shares of common stock was reduced to 150,000,000. All of the share and per share amounts included in this Annual Report on Form 10-K have been adjusted to reflect the Reverse Split.

Financial Overview

Federal Contract Revenue

In September 2020, we entered into a contract (the 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 RSE. The BARDA Contract provides for funding to include support, on a cost-sharing basis, for completion of a Phase 3 clinical trial of IV-administered ganaxolone in patients with RSE (our Phase 3 clinical trial of IV-administered ganaxolone for the treatment of RSE (RAISE Trial)), funding of pre-clinical

studies to provide support that IV-administered ganaxolone could be an effective treatment for RSE due to chemical nerve gas agent exposure, and funding of certain manufacturing scale-up and regulatory activities.

The BARDA Contract consists of an approximately two-year base period-during which BARDA will provide 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 RSE Trial and preclinical studies in the base period, the BARDA Contract provides for approximately $30 million of additional BARDA funding for three options in support of manufacturing, supply chain, clinical, regulatory and toxicology activities. 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 $51 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.

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; and
- costs associated with preclinical activities and regulatory operations.

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 will incur substantial costs beyond our present and planned clinical trials in order to file an NDA and Supplemental New Drug Applications (sNDAs), or an MAA outside the US, 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.

General and Administrative Expenses

General and administrative expenses consist principally of salaries and related costs for executive and other administrative personnel and consultants, including stock-based compensation and travel expenses. Other general and administrative expenses include professional fees for legal, patent review, consulting and accounting services. General and administrative expenses are expensed when incurred. We expect that our general and administrative expenses will increase in the future as a result of employee hiring and our scaling-up of operations commensurate with supporting more advanced clinical trials and in preparation for commercial infrastructure. These increases will likely include increased costs for insurance, hiring of additional personnel, outside consultants, and legal counsel and accountants, among other expenses.

Interest Income

Interest income consists principally of interest income earned on cash and cash equivalent and investment balances.

Results of Operations

Federal contract revenue

We recognized $1.7 million in federal contract revenue in the year ended December 31, 2020 as a result of the BARDA contract we entered into in September 2020. No federal contract revenue was recognized in the year ending December 31, 2019.

Research and Development Expenses

We allocate 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 or purchasing clinical trial materials, to specific product development programs. We do not allocate 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 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 RSE, CDD, TSC and PCDH19-RE. We expect our research and development expenses for ganaxolone will continue to increase during subsequent periods. 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,	
	2020	2019
CDKL5 deficiency disorder (1)	$ 13,044	$ 10,108
Refractory status epilepticus (2)	9,767	3,996
PCDH19-related epilepsy (3)	6,011	7,417
Tuberous Sclerosis (4)	1,821	—
Postpartum depression (5)	—	6,809
Indirect research and development (6)	20,463	14,636
Total	$ 51,106	$ 42,966

Note: Certain prior year expenses have been reclassified to conform to current year presentation.

(1) The increase in the year ended December 31, 2020 was due to continued enrollment in the Marigold Study in the first half of 2020 and analysis of data results after completion of the clinical trial.

(2) The increase was due primarily to enhanced drug development activity, including preclinical studies and manufacturing activities in preparation for a Phase 3 clinical trial in RSE, for which enrollment commenced in January 2021.

(3) The decrease was due to reducing the scope of the clinical trial in 2020 from a Phase 3 trial to a Phase 2 proof-of-concept trial.

(4) We began making preparations for a Phase 2 clinical trial in TSC during the first quarter of 2020.

(5) We completed our clinical trials in postpartum depression in 2019, and have placed further development on hold.

(6) Indirect research and development expenses in support of all our programs have increased due to the overall increase in preclinical, clinical, and manufacturing activities.

General and Administrative Expenses

General and administrative expenses increased to $18.6 million compared to $11.5 million for the year ended December 31, 2020 compared to 2019. The primary drivers of the increase were increased legal and consulting fees of $2.7 million and increased headcount costs of $1.5 million as we scale up our operations and prepare for potential commercialization, and noncash stock-based compensation of $1.6 million.

Liquidity and Capital Resources

Sources of Liquidity

Since inception, we have incurred net losses and negative cash flows from our operations. We incurred a net loss of $67.5 million for the year ended December 31, 2020. Our cash used in operating activities was $60.9 million for year ended December 31, 2020 compared to $48.6 million for the same period a year ago. Historically, we have financed our operations principally through the sale of common stock, notes payable, preferred stock and convertible debt. At December 31, 2020, we had cash, cash equivalents and investment balances of $140.0 million.

In September 2020, we entered into the BARDA Contract. 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 the RAISE Trial, funding of pre-clinical studies to provide support that IV-administered ganaxolone could be an effective treatment for RSE due to chemical nerve gas agent exposure, and funding of certain manufacturing scale-up and regulatory activities.

The BARDA Contract consists of an approximately two-year base period-during which BARDA will provide 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 base period, the BARDA Contract provides for approximately $30 million of additional BARDA funding for three options in support of manufacturing, supply chain, clinical, regulatory and toxicology activities. 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 $51 million, if all development options are completed. The contract period-of-performance (base period plus option exercises) is up to approximately five years.

In connection with the closing of an equity financing in December 2020, we issued a total of 5,000,000 shares of common stock in an underwritten public offering resulting in aggregate net proceeds, after underwriting discounts and commissions in the public offering and other estimated offering expenses, of $64.9 million.

In connection with the closing of an equity financing in June 2020, we issued a total of 4,600,000 shares of common stock in an underwritten public offering resulting in aggregate net proceeds, after underwriting discounts and commissions in the public offering and other estimated offering expenses, of $42.9 million.

In connection with the closing of concurrent equity financings during the fourth quarter of 2019, we issued a total of 8,050,000 shares of common stock in an underwritten public offering and 30,000 shares of Series A convertible preferred stock in a private placement resulting in aggregate net proceeds, after underwriting discounts and commissions in the public offering and other estimated offering expenses, of $65.7 million.

In October 2017, we entered into an Equity Distribution Agreement (Prior EDA) with JMP Securities LLC (JMP), under which JMP, as our exclusive agent, at our discretion and at such times that we may determine from time to time, may sell over a three-year period from the execution of the agreement up to a maximum of $50 million of shares of our common stock. During the year ended December 31, 2020, we issued 78,807 shares of our common stock pursuant to the Prior EDA for aggregate net proceeds of $0.6 million. During the year ended December 31, 2019, we issued 423,072 shares of our common stock pursuant to the Prior EDA for aggregate net proceeds to us of $2.2 million. On July 9, 2020, we entered into a new Equity Distribution Agreement (New EDA) with 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 $60.0 million through or to JMP. Subject to the terms and conditions of the New EDA, JMP will use its commercially reasonable efforts to sell shares of our common stock from time to time, based upon our instructions. 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. The New EDA superseded and terminated the Prior EDA effective immediately upon effectiveness of our shelf registration statement on Form S-3 (File No. 333-239780) filed with the Securities and Exchange Commission on July 9, 2020 and declared effective by the Securities and Exchange Commission on July 27, 2020.

Cash Flows

Operating Activities. Cash used in operating activities increased to $60.9 million for the year ended December 31, 2020 compared to $48.6 million for the same period in 2019. The increase was driven primarily by a $13.4 million increase in net loss due to increased research and development activities as described above.

Investing Activities. Cash used in investing activities during the year ended December 31, 2020 represents the maturities of $8.2 million of short-term investments, offset by $8.9 million in in purchases of short-term investments. Cash provided by investing activities during the year ended December 31, 2019 represents $7.0 million in maturities of investments, offset by $2.7 million in short-term investment purchases and $0.4 million in capital expenditures.

Financing Activities. Cash provided by financing activities during the year ended December 31, 2020 includes $108.4 million in net proceeds from follow-on public offerings, the sale of common stock in connection with an equity distribution agreement and $1.0 million in proceeds from the exercise of stock options. Cash provided by financing activities during the year ended December 31, 2019 includes $67.9 million in net proceeds from a follow-on public offering and concurrent private placement, the sale of common stock in connection with an equity distribution agreement, and $0.1 million in proceeds from the exercise of stock options.

Funding Requirements

We have not achieved profitability since our inception, and we expect to continue to incur net losses for the foreseeable future. We expect our cash expenditures to increase in the near term as we fund our continuing and planned clinical trials for ganaxolone, as well as scale up our operations and prepare for the potential commercialization of ganaxolone.

We believe that our cash, cash equivalents and investments as of December 31, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2022. However, we will need to raise substantial additional financing in the future to fund our operations. 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. 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;
- the scope, progress, results and costs of researching and developing ganaxolone 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 or any other future product candidates;
- the cost of commercialization activities if ganaxolone 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;
- any product liability, infringement or other lawsuits related to our product candidates 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, future approved products, if any.

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

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements that have or are reasonably likely to have a current or future effect on our financial condition, changes in financial condition, revenues or expenses, results of operations, liquidity, capital expenditures or capital resources that are material to investors.

Discussion of Critical Accounting Policies and Significant Judgments 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 United States (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.

Recent Accounting Pronouncements

In June 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2016-13, Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments. The ASU replaces the incurred loss impairment methodology in current GAAP with a methodology that reflects expected credit losses and requires consideration of a broader range of reasonable and supportable information to inform credit loss estimates. We adopted the ASU effective as of January 1, 2020.

Our cash equivalents and short-term investments are accounted for as available-for-sale debt instruments and certificates of deposit, recorded at fair value. Interest income on these instruments is recorded as "Interest income" on the statements of operations and comprehensive loss. We have never experienced a credit loss on the principal or interest receivable of our cash equivalents or short-term investments. Our available-for-sale debt securities represent (U.S.) treasury securities, and our certificates of deposit are each individually and fully insured by the Federal Deposit Insurance Corporation (FDIC). Accordingly, we did not measure an allowance for credit losses on these securities and we did not record a cumulative-effect adjustment to accumulated deficit during the year ended December 31, 2020 upon adoption of ASU No. 2016-13.

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 the Company in the reports that it files or submits 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, 2020.

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 United States. 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, 2020. Based on the assessment, management has concluded that, as of December 31, 2020, 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, 2020 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Remediation of Material Weakness

As disclosed in Item 9A of our Annual Report on Form 10-K filed with the SEC on March 16, 2020 (2019 Form 10-K), management identified a material weakness in our information technology (IT) general controls (collectively, ITGCs) and related IT-dependent process level controls, which are part of our internal control over financial reporting. These deficiencies were the result of ineffective IT risk assessment which did not identify the risks associated with segregation of duties within these IT systems. We took measures to remediate the deficiency related to ineffective segregation of duties within these IT systems in December 2019 by transferring key administrative access to a third-party IT vendor. In the 2019 Form 10-K, we noted that this material weakness would not be considered remediated until this change in internal control with respect to segregation of duties within the IT systems operated for a sufficient period of time and could be tested and concluded by management to be designed and operating effectively. In September 2020, we completed this testing and management concluded that such material weakness has been remediated.

Item 9B. Other Information.

Departure of Edward F. Smith as Vice President, Chief Financial Officer and Treasurer

On March 9, 2021, we entered into a Separation and Consulting Agreement and General Release (Separation and Consulting Agreement) with Edward F. Smith, our Vice President, Chief Financial Officer and Treasurer, in connection with Mr. Smith's separation from us. Mr. Smith's last day of employment is March 9, 2021 (Separation Date). The Separation and Consulting Agreement sets forth Mr. Smith's separation benefits and the terms pursuant to which Mr. Smith will assist us in the transition of his roles and continue in a consulting capacity for a three-month period beginning as of the Separation Date (Consultancy Period).

During the Consultancy Period, Mr. Smith will make himself reasonably available to perform services as reasonably requested by our Chief Executive Officer. In exchange, Mr. Smith will be entitled to receive a monthly fee of $34,167 during the Consultancy Period. Mr. Smith may terminate the Consultancy Period at any time upon written notice to us. We may terminate the Consultancy Period at any time for "Cause" (as defined in the Separation and Consulting Agreement) upon notice to Mr. Smith.

In addition, the Separation and Consulting Agreement provides for the following separation benefits, subject to Mr. Smith agreeing to a release of claims and complying with certain other continuing obligations contained therein:

- we will pay Mr. Smith $307,500, which is equivalent to nine months of Mr. Smith's base salary as of the Separation Date, payable in nine equal monthly installments in accordance with the our usual compensation and payroll practices;

- all of Mr. Smith's unvested stock options will immediately vest as of the Separation Date and will become and remain exercisable pursuant to each stock option's terms until the earlier of (i) the one year anniversary of the Separation Date and (ii) the end of the term of such stock option, in each case, subject to the terms and conditions of the applicable equity incentive plan and award agreements; and

- if Mr. Smith timely elects to continue health care coverage under the Consolidated Omnibus Reconciliation Act of 1985 (COBRA) for himself and his eligible dependents, then we will pay that portion of Mr. Smith's premiums for COBRA coverage that we were paying prior to the Separation Date for a period of nine months following the Separation Date or, if earlier, until the date Mr. Smith becomes eligible to receive substantially equivalent coverage from another employer.

In addition, we will pay to Mr. Smith all accrued salary and all accrued and unused vacation earned by Mr. Smith through the Separation Date, in accordance with our usual compensation and payroll practices.

The description of the Separation and Consulting Agreement does not purport to be complete and is qualified in its entirety by reference to the complete text of the Separation and Consulting Agreement, which is filed as Exhibit 10.3 to this Annual Report on Form 10-K.

Appointment of Steven E. Pfanstiel, MBA, CMA, as Chief Financial Officer and Treasurer

On March 6, 2021, our board of directors appointed Steven Pfanstiel, MBA, CMA, age 48, as our Chief Financial Officer and Treasurer, effective as of April 12, 2021 (Pfanstiel Start Date).

Mr. Pfanstiel previously served as Vice President, Finance, of LifeScan, Inc. (LifeScan), a diagnostic systems manufacturer with products focusing on the diabetes market, from January 2020 to March 2021, where he was responsible for supporting LifeScan's global commercial and development organizations, as well as its financial planning analysis function and treasury. Before LifeScan, Mr. Pfanstiel served as Senior Director – FP&A of OptiNose, Inc. (Optinose), a publicly-traded specialty pharmaceutical company focused on creating and bringing to market innovative products for patients with diseases treated by ear, nose, and throat and allergy specialists, from February 2018 to January 2020. During his time at Optinose, Mr. Pfanstiel served as finance leader for the supply chain and R&D/clinical organizations and was responsible for the broader strategic finance analysis across the organization. From July 2016 to February 2018, Mr. Pfanstiel served as Senior Director – Global Strategic Marketing of DePuy Synthes Companies, a franchise of orthopedic and neurosurgery companies owned by Johnson & Johnson, where he provided financial leadership to the Global Orthopedics franchise and served as a member of the Orthopedics Global Management Board. From November 2013 to July 2016, Mr. Pfanstiel service as Senior Director – North America Commercial, Worldwide Financial Reporting, Strategic Marketing and R&D, for Animas Corporation and LifeScan, members of the Johnson & Johnson Family of Diabetes Companies, where he led the finance team transformation and implementation of a new business strategy with the diabetes leadership team. Earlier in his career, Mr. Pfanstiel held various finance positions for Johnson & Johnson, Janssen R&D and Ethicon Endo-Surgery. Mr. Pfanstiel received his B.A. in Physics from Wabash College, his M.S. in Environmental Systems Engineering from Clemson University and his MBA from Indiana University, Kelley School of Business.

In connection with Mr. Pfanstiel's appointment, the Company entered into an employment agreement, effective as of the Pfanstiel Start Date, with Mr. Pfanstiel (Pfanstiel Employment Agreement), which provides that Mr. Pfanstiel's employment will continue until either we or Mr. Pfanstiel terminates Mr. Pfanstiel's employment in accordance with the terms of the Pfanstiel Employment Agreement.

Pursuant to the Pfanstiel Employment Agreement, Mr. Pfanstiel is entitled to receive an annual base salary of $380,000, which will be reviewed at least annually and will be subject to increase (but not decrease) from time to time, as determined by our board of directors. In addition, pursuant to the Pfanstiel Employment Agreement, Mr. Pfanstiel is eligible to receive an annual cash bonus, which is based on the achievement of certain performance objectives and other criteria as determined by our board of directors or the compensation committee of our board of directors. Mr. Pfanstiel's initial target annual bonus is 40% of his annual base salary. The exact amount of the bonus payable to Mr. Pfanstiel for any calendar year during his employment with us will be determined by our board of directors or the compensation committee of our board of directors. Pursuant to the Pfanstiel Employment Agreement, Mr. Pfanstiel is also entitled to participate in or receive benefits consistent with other key employees under our employee benefit plans as they may be adopted and amended from time to time, subject to the terms and conditions of those employee benefit plans.

In addition, pursuant to the Pfanstiel Employment Agreement, we will grant Mr. Pfanstiel a stock option on the Pfanstiel Start Date (the "Sign-on Option") to purchase 220,000 shares of our common stock at an exercise price equal to the last reported sale price of our common stock on the Nasdaq Global Market on the Pfanstiel Start Date. The Sign-on Option has a ten-year term and will vest as to 25% of shares of our common stock subject to such Sign-on Option on the one-year anniversary of the Pfanstiel Start Date and monthly thereafter in 36 substantially equal installments, subject to Mr. Pfanstiel's continued employment with us through the applicable vesting dates. The grant of the Sign-on Option to

Mr. Pfanstiel will be made outside our 2014 Equity Incentive Plan, and any other equity incentive plan, as an inducement material to Mr. Pfanstiel's entering into employment with us pursuant to Nasdaq Stock Market LLC Listing Rule 5635(c)(4).

Upon a termination of Mr. Pfanstiel's employment by us without cause or a resignation by Mr. Pfanstiel for good reason, Mr. Pfanstiel is eligible to receive a continuation of his base salary for nine months, with an accelerated payment of any balance upon a change in control (as defined in the Pfanstiel Employment Agreement), subject to his execution and delivery of a general release of claims. If such termination occurs within three months before or within twelve months after a change in control, (i) the severance payable increases to an amount equal to his base salary for a period of eighteen months, payable in a lump sum, and (ii) Mr. Pfanstiel is also eligible to receive payment of a pro-rated target bonus for one year plus the target bonus for the year of his termination. Upon any termination described in this paragraph, Mr. Pfanstiel is also eligible to receive payment or reimbursement of his medical insurance premiums at the same level as was in effect on the termination date for a period of nine months, which period increases to eighteen months if the termination of employment occurs three months before or twelve months after a change in control.

Termination for "cause" under the Pfanstiel Employment Agreement generally means termination of Mr. Pfanstiel by us for: (i) his misuse of alcoholic beverages, controlled substances or other narcotics, which misuse has had or is reasonably likely to have a material adverse effect on our business or financial affairs or our reputation; (ii) failure to cooperate with us in any investigation or formal proceeding; (iii) the commission of, or a plea of guilty or nolo contendere with respect to, or conviction 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 Mr. Pfanstiel of any material term of the Pfanstiel Employment Agreement, including the his failure to faithfully, diligently and adequately perform his duties under the Pfanstiel Employment Agreement, that is not corrected within ten days after written notice from us, which notice shall set forth the nature of the breach; (vi) violation in any material respect of any of our rules, regulations or policies; (vii) gross insubordination by Mr. Pfanstiel in the performance of his duties under the Pfanstiel Employment Agreement; (viii) engaging in any conduct, action or behavior that, in our reasonable opinion, has had a material adverse effect on our reputation or Mr. Pfanstiel; (ix) any continued or repeated absences, unless the absence is approved or excused by our Chief Executive Officer or the result of his illness, disability or incapacity; or (x) misappropriation of any of our funds or property, theft, embezzlement or fraud.

Termination for "good reason" under the Pfanstiel Employment Agreement generally means termination by Mr. Pfanstiel for (i) a reassignment of Ms. Pfanstiel to a location outside the greater Philadelphia area; (ii) any material failure by us to comply with any material term of the Pfanstiel Employment Agreement; (iii) the demotion of Mr. Pfanstiel to a lesser position or a substantial diminution of Mr. Pfanstiel's authority, duties or responsibilities; or (iv) a material diminution of his base salary and benefits, in the aggregate, except under certain limited circumstances.

Mr. Pfanstiel's right to receive the severance payments and benefits described above under the Pfanstiel Employment Agreement is conditioned upon his execution and non-revocation of a general release of claims. Mr. Pfanstiel is entitled to participate in all of our group welfare plans, subject to the terms and conditions applicable to such plans as approved from time to time by our board of directors. Mr. Pfanstiel's employment agreement contains customary non-solicitation and non-competition covenants, which covenants remain in effect for twelve months following any cessation of employment with respect to Mr. Pfanstiel.

Other than with respect to the Pfanstiel Employment Agreement and the Sign-on Option, there are no arrangements or understandings between Mr. Pfanstiel and any other persons pursuant to which Mr. Pfanstiel was appointed as our Chief Financial Officer and Treasurer. There are also no family relationships between Mr. Pfanstiel and any of our directors or executive officers and Mr. Pfanstiel has no direct or indirect interest in any transaction or proposed transaction required to be disclosed pursuant to Item 404(a) of Regulation S-K.

The description of the Pfanstiel Employment Agreement does not purport to be complete and is qualified in its entirety by reference to the complete text of the Pfanstiel Employment Agreement, which is filed as Exhibit 10.4 to this Annual Report on Form 10-K.

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 2021 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 2021 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 2021 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 2021 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 2021 annual meeting of shareholders, to be filed with the SEC.

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.

(b) Exhibits:

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.)
4.1	Specimen Certificate evidencing shares of the Company's common stock. (Incorporated by reference to Exhibit 4.1 to Form S-1/A registration statement filed on July 18, 2014.)
4.2	Form of Third Amended and Restated Investors' Rights Agreement by and among the Company and the parties listed therein. (Incorporated by reference to Exhibit 4.2 to Form S-1/A registration statement filed on July 9, 2014.)
4.3	Description of the Registrant's Securities. (Filed herewith.)
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+	Separation and Consulting Agreement and General Release, dated as of March 9, 2021, between the Company and Edward F. Smith. (Filed herewith.)
10.4+	Employment Agreement, effective as of April 12, 2021, between the Company and Steven Pfanstiel (Filed herewith.)
10.5*	Technology Transfer Agreement dated December 4, 2012 between Domain Russia Investments Limited and the Company. (Incorporated by reference to Exhibit 10.6 to Form S-1 registration statement filed on May 12, 2014.)
10.6	Assignment and Assumption Agreement dated as of December 4, 2012 among Domain Russia Investments Limited, the Company and NovaMedica, LLC. (Incorporated by reference to Exhibit 10.7 to Form S-1 registration statement filed on May 12, 2014.)
10.7	Clinical Development and Collaboration Agreement dated as of June 25, 2013 between NovaMedica, LLC and the Company. (Incorporated by reference to Exhibit 10.8 to Form S-1 registration statement filed on May 12, 2014.)
10.8	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.)
10.9	Form of Amended and Restated Indemnification Agreement (Non-VC Directors). (Filed herewith.)
10.10*	Amended and Restated Agreement dated as of May 23, 2008 between the Company and Purdue Neuroscience Company. (Incorporated by reference to Exhibit 10.12 to Form S-1 registration statement filed on May 12, 2014.)
10.11+	Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on November 9, 2020.)
10.12+	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.)
10.13+	Form of Incentive Stock Option Agreement for Officers Under 2014 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.16 to Form 10-K annual report filed on March 12, 2015.)

Exhibit No.	Description of Exhibit
10.14+	Form of Incentive Stock Option Agreement for Employees Under 2014 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.17 to Form 10-K annual report filed on March 12, 2015.)
10.15+	Form of Nonqualified Stock Option Agreement Under 2014 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.18 to Form 10-K annual report filed on March 12, 2015.)
10.16	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.)
10.17	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.)
10.18	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.)
10.19	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.)
10.20+	Amended and Restated Employment Agreement dated as of August 6, 2019, between the Company and Scott Braunstein, M.D. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on August 8, 2019).
10.21+	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).
10.22+	Employment Agreement dated as of June 15, 2020 between the Company and Martha Manning. (Filed herewith.)
10.23	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).
10.24	Equity Distribution Agreement, dated July 9, 2020, by and between the Company and JMP Securities LLC. (Incorporated by reference to Exhibit 1.1 to Form S-3 registration statement filed on July 9, 2020.)
10.25	Form of Nonqualified Stock Option Inducement Award Agreement. (Incorporated by reference to Exhibit 10.2 to Form S-8 Registration Statement filed on July 10, 2020.)
10.26	Underwriting Agreement, dated May 28, 2020, by and among the Company, Cowen and Company, LLC and Cantor Fitzgerald & Co. (Incorporated by reference to Exhibit 1.1 to Form 8-K current report filed on May 29, 2020.)
10.27	Underwriting Agreement, dated December 8, 2020, by and between the Company and Cantor Fitzgerald & Co. (Incorporated by reference to Exhibit 1.1 to Form 8-K current report filed on December 9, 2020.)
10.28*	Contract, dated September 8, 2020, by and between the Company 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.)
16.1	Letter of KPMG LLC dated as of June 17, 2020 to the Securities and Exchange Commission. (Incorporated by reference to Exhibit 16.1 to Form 8-K current report filed on June 17, 2020.)
21	Subsidiaries of the Registrant. (Filed herewith.)
23.1	Consent of Ernst & Young LLP. (Filed herewith.)
23.2	Consent of KPMG 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. (Filed herewith.)
101.INS	XBRL Instance Taxonomy

Exhibit No.	Description of Exhibit
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 – The cover page interactive data file does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document

+ 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.

(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, 2021

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 Scott Braunstein	President, Chief Executive Officer (Principal Executive Officer) and Director	March 9, 2021
/s/ Edward F. Smith Edward F. Smith	Vice President, Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)	March 9, 2021
/s/ Nicole Vitullo Nicole Vitullo	Chairman of the Board and Director	March 9, 2021
/s/ Charles Austin Charles Austin	Director	March 9, 2021
/s/ Enrique J. Carrazana Enrique J. Carrazana, M.D.	Director	March 9, 2021
/s/ Michael R. Dougherty Michael R. Dougherty	Director	March 9, 2021
/s/ Elan Ezickson Elan Ezickson	Director	March 9, 2021
/s/ Seth H.Z. Fischer Seth H.Z. Fischer	Director	March 9, 2021
/s/ Tim M. Mayleben Tim M. Mayleben	Director	March 9, 2021

FINANCIAL STATEMENTS

MARINUS PHARMACEUTICALS, INC.

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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 balance sheet of Marinus Pharmaceuticals, Inc. (the Company) as of December 31, 2020, the related statements of operations and comprehensive loss, stockholders' equity and cash flows for the year ended December 31, 2020 and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2020, and the results of its operations and its cash flows for the year ended December 31, 2020, 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 audit. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the 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 audit 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 audit 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 audit 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 audit provides a reasonable basis for our opinion.

Critical Audit Matter

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

Clinical Trial Prepaid and Accrued Expenses

Description of the Matter	As disclosed in Note 2 to the financial statements, the Company expenses clinical trial expenditures as incurred, which include costs relating to contracts with vendors, clinical research organizations and consultants and 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 at December 31, 2020 is included in prepaid expenses and other current assets of $4.6 million on the balance sheet, the Company's clinical trial accrued expenses at December 31, 2020 of $2.5 million is included in accrued expenses on the balance sheet, and the Company's related 2020 clinical trial expenses are included in research and development expenses of $51.1 million on the statement of operations and comprehensive loss for the year ended December 31, 2020. 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 provided by certain service providers about the service providers' estimate of costs that had been incurred through December 31, 2020. Additionally, we assessed the historical accuracy of management's estimates when evaluating the current period estimates. 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.

/s/ Ernst & Young LLP

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

Philadelphia, Pennsylvania
March 9, 2021

Report of Independent Registered Public Accounting Firm

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

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheet of Marinus Pharmaceuticals, Inc. and subsidiary (the Company) as of December 31, 2019, the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for the year ended December 31, 2019, and the related notes (collectively, the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2019, and the results of its operations and its cash flows for the year ended December 31, 2019, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

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

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

/s/ KPMG LLP

We served as the Company's auditor from 2014 to 2020.

Philadelphia, Pennsylvania
March 16, 2020, except for the reverse stock split described in Note 1, as to which the date is March 9, 2021

MARINUS PHARMACEUTICALS, INC.

BALANCE SHEETS

(In thousands, except share and per share amounts)

	December 31,	
	2020	**2019**
ASSETS		
Current assets:		
Cash and cash equivalents	$ 138,509	$ 90,943
Short-term investments	1,474	739
Federal contract revenue receivable	1,646	—
Prepaid expenses and other current assets	4,638	2,452
Total current assets	146,267	94,134
Property and equipment, net	1,945	2,265
Other assets	2,250	2,443
Total assets	$ 150,462	$ 98,842
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 2,211	$ 2,763
Accrued expenses	8,518	5,268
Total current liabilities	10,729	8,031
Other long-term liabilities	2,534	3,042
Total liabilities	13,263	11,073
Commitments and contingencies (Note 10)	—	—
Series A convertible preferred stock, $0.001 par value; 25,000,000 shares authorized, 30,000 shares issued and outstanding at December 31, 2019	—	28,200
Stockholders' equity:		
Series A convertible preferred stock, $0.001 par value; 25,000,000 shares authorized, 4,753 shares issued and outstanding at December 31, 2020	4,469	—
Common stock, $0.001 par value; 150,000,000 shares authorized, 36,585,767 issued and 36,578,460 outstanding at December 31, 2020 and 21,625,088 issued and 21,617,781 outstanding at December 31, 2019	37	22
Additional paid-in capital	444,622	295,121
Treasury stock at cost, 7,307 shares at December 31, 2020 and December 31, 2019	—	—
Accumulated deficit	(311,929)	(235,574)
Total stockholders' equity	137,199	59,569
Total liabilities and stockholders' equity	$ 150,462	$ 98,842

See accompanying notes to financial statements.

MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

	Year Ended December 31,	
	2020	2019
Federal contract revenue	$ 1,718	$ —
Expenses:		
Research and development	$ 51,106	$ 42,966
General and administrative	18,549	11,456
Loss from operations	(67,937)	(54,422)
Interest income	499	354
Other expense, net	(37)	(53)
Net loss and comprehensive loss	$ (67,475)	$ (54,121)
Deemed dividends on convertible preferred stock	(8,880)	—
Net loss and comprehensive loss applicable to common shareholders	$ (76,355)	$ (54,121)
Per share information:		
Net loss per share of common stock—basic and diluted	$ (2.80)	$ (3.97)
Basic and diluted weighted average shares outstanding	27,270,055	13,628,194

See accompanying notes to financial statements.

MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF STOCKHOLDERS' EQUITY

(In thousands, except share and per share amounts)

	Series A Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Treasury Stock		Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount		Shares	Amount			
Balance, December 31, 2018	—	$ —	13,137,061	$ 13	$ 249,767	7,307	$ —	$ (2)	$ (181,453)	$ 68,325
Stock-based compensation expense	—	—	—	—	5,672	—	—	—	—	5,672
Exercise of stock options	—	—	20,005	—	97	—	—	—	—	97
Issuance of common stock in connection with follow-on public offering ($5.00 per share), net of expenses of $2,786			8,050,000	8	37,456	—	—	—	—	37,464
Issuance of common stock under equity distribution agreement, net of expenses of $95			423,072	1	2,129	—	—	—	—	2,130
Forfeiture of restricted stock	—	—	(5,050)	—	—	—	—	—	—	—
Unrealized gain on investments	—	—	—	—	—	—	—	2	—	2
Net loss	—	—	—	—	—	—	—	—	(54,121)	(54,121)
Balance, December 31, 2019	—	$ —	21,625,088	$ 22	$ 295,121	7,307	$ —	$ —	$ (235,574)	$ 59,569
Stock-based compensation expense	—	—	—	—	7,642	—	—	—	—	7,642
Exercise of stock options	—	—	198,475	—	1,020	—	—	—	—	1,020
Issuance of restricted stock	—	—	33,997	—	—	—	—	—	—	—
Deemed dividend on beneficial conversion feature - Series A convertible preferred stock	—	—	—	—	8,880	—	—	—	(8,880)	—
Issuance of common stock under equity distribution agreement, net of expenses of $161	—	—	78,807	—	489	—	—	—	—	489
Issuance of common stock in connection with follow-on public offering ($10 per share), net of expenses of $3,025	—	—	4,600,000	5	42,956	—	—	—	—	42,961
Transfer of convertible preferred stock into equity	9,303	8,745	—	—	—	—	—	—	—	8,745
Conversion of convertible preferred stock into common	(4,550)	(4,276)	5,049,400	5	23,727	—	—	—	—	19,456
Issuance of common stock in connection with follow-on public offering ($14 per share), net of expenses of $5,208	—	—	5,000,000	5	64,787	—	—	—	—	64,792
Net loss	—	—	—	—	—	—	—	—	(67,475)	(67,475)
Balance, December 31, 2020	4,753	$ 4,469	36,585,767	$ 37	$ 444,622	7,307	$ —	$ —	$ (311,929)	$ 137,199

See accompanying notes to financial statements.

MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF CASH FLOWS

(In thousands)

	Year Ended December 31,	
	2020	2019
Cash flows from operating activities		
Net loss	$ (67,475)	$ (54,121)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	334	278
Stock-based compensation expense	7,642	5,672
Loss on disposal of fixed assets	—	42
Noncash lease expense	264	225
Noncash lease liability	362	(131)
Amortization of discount on investments	3	(8)
Changes in operating assets and liabilities:		
Prepaid expenses and other current and non-current assets and federal contract revenue receivables	(3,919)	(1,480)
Accounts payable, accrued expenses and other long term-liabilities	1,877	890
Net cash used in operating activities	(60,912)	(48,633)
Cash flows from investing activities		
Maturities of short-term investments	8,193	6,994
Purchases of short-term investments	(8,931)	(2,725)
Purchases of property and equipment	—	(388)
Net cash (used in) provided by investing activities	(738)	3,881
Cash flows from financing activities		
Proceeds from exercise of stock options	1,020	97
Proceeds from equity offerings, net of offering costs	108,196	67,871
Net cash provided by financing activities	109,216	67,968
Net increase in cash and cash equivalents	47,566	23,216
Cash and cash equivalents—beginning of year	90,943	67,727
Cash and cash equivalents—end of year	$ 138,509	$ 90,943
Supplemental disclosure of cash flow information		
Financing in accounts payable and accrued expenses	$ 148	$ 195
Operating lease liability	$ —	$ 3,357
Operating right-of-use asset	$ —	$ 2,458

See accompanying notes to financial statements.

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

1. Organization and Description of the Business

We are a clinical stage pharmaceutical company focused on developing and commercializing innovative therapeutics to treat patients suffering from rare seizure disorders. Our clinical stage product candidate, ganaxolone, is a positive allosteric modulator of $GABA_A$ that is being developed in formulations for two different routes of administration: intravenous (IV) and oral. Ganaxolone is a synthetic analog of allopregnanolone, an endogenous neurosteroid. 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. Ganaxolone acts at both synaptic and extrasynaptic $GABA_A$ receptors, a target known for its anti-seizure, antidepressant and anxiolytic potential.

In December 2019, an outbreak of a novel strain of coronavirus (COVID-19) was identified in Wuhan, China. This virus was declared a pandemic by the World Health Organization in March 2020 and has spread to nearly every country in the world, including the United States (U.S.). Efforts to contain the spread of COVID-19 have intensified and many countries, including the U.S., have implemented severe travel restrictions, business shutdowns and social distancing measures that have impacted clinical development through supply chain shortages and clinical trial enrollment difficulties as hospitals reduce and redeploy staff, divert resources to patients suffering from COVID-19 and limit hospital access for non-patients. The pandemic poses the risk that we, our employees, contractors, suppliers, or other partners may be prevented from conducting normal business activities for an indefinite period of time, including those due to shutdowns that may be requested or mandated by governmental authorities.

The continued global spread of COVID-19 has impacted our operations but did not have a material impact on our business, operating results, financial condition or cash flows as of and for the year ended December 31, 2020. For example, several of our Phase 1 trials of oral ganaxolone to support the CDD indication have experienced delays in enrollment due to COVID-19, however we do not expect these trials to delay our ability to file an NDA. Further, in response to COVID-19, for our ongoing clinical trials, we have implemented multiple measures consistent with the U.S. Food and Drug Administration's guidance on the conduct of clinical trials of medical products during the COVID-19 pandemic, including implementing remote site monitoring and remote visits using telemedicine where needed. However, COVID-19 may still adversely impact our clinical trials. For example, our Phase 3 clinical trial in RSE is conducted in the hospital and resources related to the COVID-19 outbreak may divert staffing in the hospital taking resources away from our clinical trial. Our ganaxolone clinical trials in the outpatient setting may be negatively impacted if patients and their caregivers do not want to participate in a clinical trial while COVID-19 outbreaks continue. Although operations have not been materially affected by the COVID-19 pandemic as of and for the year ended December 31, 2020, we are unable to predict the impact that COVID-19 will have in the future on our business, financial position, operating results and cash flows due to numerous uncertainties. The duration and severity of the pandemic and its long-term impact on our business are uncertain at this time, and our ability to raise sufficient additional financing depends on many factors beyond our control, including the current volatility in the capital markets as a result of the COVID-19 pandemic.

Liquidity

We have not generated any product revenues and have incurred operating losses since inception, including losses of $67.5 million for the year ended December 31, 2020. There is no assurance that profitable operations will ever be achieved, and if achieved, could be sustained on a continuing basis. In addition, development activities, clinical and preclinical testing, if approved, and commercialization of our product candidates will require significant additional financing. Our accumulated deficit as of December 31, 2020 was $311.9 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 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 development and, if approved, commercialization of our product candidates.

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

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 refractory status epilepticus (RSE). The BARDA Contract provides for funding to include support, on a cost-sharing basis, the completion of a Phase 3 clinical trial of IV-administered ganaxolone in patients with RSE (our Phase 3 clinical trial evaluating IV ganaxolone for the treatment of RSE (RAISE Trial)) , funding of pre-clinical studies to provide support that IV-administered ganaxolone could be an effective treatment for RSE due to chemical nerve gas agent exposure, and funding of certain manufacturing scale-up and regulatory activities.

The BARDA Contract consists of an approximately two-year base period-during which BARDA will provide 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 base period, the BARDA Contract provides for approximately $30 million of additional BARDA funding for three options in support of manufacturing, supply chain, clinical, regulatory and toxicology activities. 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 $51 million, if all development options are completed. The contract period-of-performance (base period plus option exercises) is up to approximately five years.

In connection with the closing of an equity financing in December 2020, we issued a total of 5,000,000 shares of common stock in an underwritten public offering resulting in aggregate net proceeds, after underwriting discounts and commissions in the public offering and other estimated offering expenses, of $64.9 million.

In connection with the closing of an equity financing in June 2020, we issued a total of 4,600,000 shares of common stock in an underwritten public offering resulting in aggregate net proceeds, after underwriting discounts and commissions in the public offering and other estimated offering expenses, of $42.9 million.

In connection with the closing of concurrent equity financings during the fourth quarter of 2019, we issued a total of 8,050,000 shares of common stock in an underwritten public offering (2019 Public Offering) and 30,000 shares of Series A convertible preferred stock in a private placement resulting in aggregate net proceeds, after underwriting discounts and commissions in the public offering and other estimated offering expenses, of $65.7 million. We also raised, during the fourth quarter of 2019, net proceeds of $2.1 million in connection with the sale of 423,072 shares of common stock under our equity distribution agreement.

Reverse stock split

On September 23, 2020, we effected a 1-for-4 reverse split of shares of our common stock (Reverse Split), as approved by our board of directors and stockholders. The par value per share of our common stock was not adjusted as a result of the Reverse Split, and our authorized shares of common stock was reduced to 150,000,000. All of the share and per share amounts included in the accompanying financial statements and these notes have been adjusted to reflect the Reverse Split.

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, 2019. During the year ended December 31, 2020, the wholly-owned subsidiary was liquidated. In February 2021, a new wholly-owned subsidiary was established. All intercompany accounts and transactions have been eliminated.

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

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.

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 federal contract revenue receivable on our balance sheets. This revenue is not within the scope of Accounting Standards Codification (ASC) 606 – Revenue from contracts with customers.

Fair Value of Financial Instruments and Credit Risk

At December 31, 2020 and 2019, our financial instruments included cash equivalents, short-term investments, accounts payable and accrued expenses. The carrying amount of cash equivalents, accounts payable and accrued expenses approximated fair value, given their short-term nature. The carrying amounts of short-term investments are recorded at amortized cost, which for U.S. Treasury securities is based on the current market price of each security at the measurement date.

Cash equivalents and certificates of deposit 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.

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, 2020 and 2019, we invested a portion of our cash balances in money market investments, which we have included as cash equivalents on our balance sheets.

Investments

As of December 31, 2020 and 2019, our investments consisted of certificates of deposit with various financial institutions, with original maturities ranging from six to nine months. All investments were classified as held-to-maturity and were recorded at amortized cost. Interest income includes interest and dividends, realized gains and losses on sales of securities, if any.

Federal Contract Receivable

Federal contract 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.

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.

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

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 six years 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.

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.

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, 2020 and 2019, 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.

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, convertible notes payable, warrants, 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 8.

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

The following table sets forth the computation of basic and diluted earnings per share for the years ended December 31, 2020 and 2019 (in thousands, except share and per share amounts):

	Year Ended December 31,	
	2020	2019
Basic and diluted net loss per share of common stock:		
Net loss	$ (67,475)	$ (54,121)
Deemed Dividends	(8,880)	—
Net loss applicable to common stockholders	$ (76,355)	$ (54,121)
Weighted average shares of common stock outstanding	27,270,055	13,628,194
Net loss per share of common stock—basic and diluted	$ (2.80)	$ (3.97)

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

	December 31,	
	2020	2019
Convertible preferred stock	950,600	6,000,000
Restricted stock	24,625	8,100
Stock options	3,507,638	2,135,070
	4,482,863	8,143,170

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, 2020 and 2019, we had no other potentially dilutive securities.

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.

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.

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

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, 2020 and 2019 there were no material adjustments to our prior period estimates of accrued expenses for clinical trials.

Recent Accounting Pronouncements

In June 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2016-13, Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments. The ASU replaces the incurred loss impairment methodology in current GAAP with a methodology that reflects expected credit losses and requires consideration of a broader range of reasonable and supportable information to inform credit loss estimates. We adopted the ASU effective as of January 1, 2020.

Our cash equivalents and short-term investments are accounted for as held-to-maturity debt instruments as certificates of deposit, recorded at amortized cost. Interest income on these instruments is recorded as “Interest income” on the statements of operations and comprehensive loss. We have never experienced a credit loss on the principal or interest receivable of our cash equivalents or short-term investments. Our certificates of deposit are each individually and fully insured by the Federal Deposit Insurance Corporation (FDIC). Accordingly, we did not measure an allowance for credit losses on these securities and we did not record a cumulative-effect adjustment to accumulated deficit during the year ended December 31, 2020 upon adoption of ASU No. 2016-13.

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 (the 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, 2020 and 2019, all of our financial assets and liabilities were classified as Level 1 valuations.

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, 2020				
Assets				
Money market funds (cash equivalents)	$ 138,509	$ —	$ —	$ 138,509
Certificates of deposit	1,474	—	—	1,474
Total assets	$ 139,983	$ —	$ —	$ 139,983
December 31, 2019				
Assets				
Money market funds (cash equivalents)	$ 85,395	$ —	$ —	$ 85,395
Certificates of deposit	739	—	—	739
Total assets	$ 86,134	$ —	$ —	$ 86,134

4. Property and Equipment

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

	December 31, 2020	December 31, 2019
Laboratory equipment	$ 1,777	$ 1,777
Leasehold improvements	899	899
Office furniture and equipment	401	401
Total property and equipment	3,077	3,077
Less: accumulated depreciation	(1,132)	(812)
Total property and equipment, net	$ 1,945	$ 2,265

Depreciation expense was $0.3 million and $0.2 million for the years ended December 31, 2020 and 2019, respectively.

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	December 31, 2020	December 31, 2019
Payroll and related costs	$ 4,097	$ 2,514
Clinical trials and drug development	2,452	1,849
Professional fees	927	396
Short-term lease liabilities	510	446
Other	532	63
Total accrued expenses	$ 8,518	$ 5,268

6. Leases

We have entered into operating leases for real estate. These leases have terms which range from 36 to 78 months, and include renewal terms which can extend the lease terms by 24 to 60 months, which are included in the lease term when it is reasonably certain that we will exercise the option. As of December 31, 2020, our operating leases had a weighted average remaining lease term of 56 months. These right-of-use (ROU) assets are included in "Other assets" on our balance sheets as of December 31, 2020 and 2019, and represent 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, 2020 and 2019. The ROU assets were 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 assets are 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. Our ROU assets as of January 1, 2019 have been adjusted for $0.9 million in lease incentives.

Based on the present value of the lease payments for the remaining lease term of our existing leases, we initially recognized ROU assets of $2.5 million and lease liabilities for operating leases of $3.4 million during the first quarter of 2019. As of December 31, 2020 and 2019, ROU assets were $2.0 million and $2.2 million, respectively, and operating lease liabilities were $3.0 million and $3.5 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 the years ended December 31, 2020 and 2019, we recognized $0.6 million and $0.7 million, respectively, 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 each 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 ROU assets and lease liabilities as of January 1, 2019 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, 2020 and 2019, we have not recognized any impairment losses for our ROU assets.

We monitor for events or changes in circumstances that require a reassessment of one of our leases. When a reassessment results in the remeasurement of a lease liability, a corresponding adjustment is made to the carrying amount

of the corresponding ROU asset unless doing so would reduce the carrying amount of the ROU asset to an amount less than zero. In that case, the amount of the adjustment that would result in a negative ROU asset balance is recorded in our statements of operations and comprehensive loss.

Maturities of operating lease liabilities as of December 31, 2020 were as follows (in thousands):

2021	818
2022	807
2023	823
2024	840
Thereafter	642
	3,930
Less: imputed interest	(886)
Total lease liabilities	$ 3,044
Current operating lease liabilities	$ 510
Non-current operating lease liabilities	2,534
Total lease liabilities	$ 3,044

7. Investments

As of December 31, 2020 and 2019, our investments consisted of certificates of deposit with various financial institutions with original maturities of six to nine months. Investments are classified as short- or long-term investments on our balance sheets based on original maturity. Certificates of deposits were classified as held-to-maturity and were recorded at amortized cost, which approximated fair value. We have never experienced a credit loss on the principal or interest receivable of our cash equivalents or short-term investments. Our certificates of deposit are each individually and fully insured by the FDIC. Accordingly, we did not record any allowance for potential credit losses as of December 31, 2020.

8. 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, 2020, 42,492 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, 2020, 2,613,122 options to purchase shares of common stock and 24,625 restricted shares of common stock were outstanding pursuant to grants in connection with the 2014 Plan, and 167,254 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, 2021, the shares of common stock available for future grants under the 2014 Plan was increased to 1,630,393.

In addition, during the years ended December 31, 2020 and 2019, we granted 852,024 and 230,000 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,

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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,	
	2020	2019
Research and development	$ 2,938	$ 2,563
General and administrative	4,521	3,070
Total	$ 7,459	$ 5,633

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 occurs over a period of not greater than four years. A summary of activity for the years ended December 31, 2020 and 2019 is presented below (in thousands, except share and per share amounts):

	Shares	Weighted-Average Exercise Price Per Share	Aggregate Intrinsic Value
Outstanding—December 31, 2018	1,237,852	$ 22.48	
Granted	1,175,750	9.24	
Exercised	(20,005)	4.88	
Forfeited	(229,051)	16.84	
Expired	(29,476)	32.92	
Outstanding—December 31, 2019	2,135,070	15.80	
Granted	1,905,850	9.17	
Exercised	(198,475)	5.16	
Forfeited	(180,107)	11.04	
Expired	(154,700)	25.16	
Outstanding—December 31, 2020	3,507,638	$ 12.64	$ 11,600
Exercisable—December 31, 2020	1,632,823	$ 16.41	$ 4,625
Exercisable and expected to vest—December 31, 2020	3,507,638	$ 12.64	$ 11,600

The weighted average remaining contractual term of options outstanding and exercisable as of December 31, 2020 is 8.3 years.

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 2020 of $12.20 per share and the exercise price, multiplied by the number of in-the-money options.

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The weighted-average grant date fair value of options granted was $11.64 and $7.60 per share in 2020 and 2019, respectively, and was estimated at the date of grant using the Black-Scholes option-pricing model with the following ranges of weighted-average assumptions:

	2020	2019
Expected stock price volatility	116 - 121.71 %	104.7 - 118.87 %
Expected term of options	5.26 - 6.1 years	5.16 - 6.1 years
Risk-free interest rate	0.31 - 1.73 %	1.51 - 2.59 %
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.
- 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, 2020, there was $14.2 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:

2021	$ 6,478
2022	4,910
2023	1,837
2024	934
	$ 14,159

Restricted Stock

All issued and outstanding restricted shares of common stock are time-based and become vested one year 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 the Company's common stock on the date of the grant.

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A summary of activity for the years ended December 31, 2020 and 2019 is presented below:

	Shares	Weighted-average Grant Date Fair Value per Share
Outstanding—December 31, 2018	26,300	$ 4.84
Vested	(13,150)	4.84
Forfeited	(5,050)	4.84
Outstanding—December 31, 2019	8,100	4.84
Granted	34,000	12.93
Vested	(17,475)	10.04
Forfeited	—	—
Outstanding—December 31, 2020	24,625	$ 11.41
Expected to vest—December 31, 2020	24,625	$ 11.41

As of December 31, 2020, there was $0.2 million in unrecognized compensation cost related to unvested restricted stock.

Total compensation cost recognized for all restricted stock awards in the statements of operations for the years ended December 31, 2020 and 2019 is as follows (in thousands):

	Year Ended December 31,	
	2020	2019
Research and development	$ —	$ 19
General and administrative	183	20
Total	$ 183	$ 39

Equity Distribution Agreement

In October 2017, we entered into an Equity Distribution Agreement (Prior EDA) with JMP Securities LLC (JMP), under which JMP, as our exclusive agent, at our discretion and at such times that we may determine from time to time, may sell over a three-year period from the execution of the agreement up to a maximum of $50 million of shares of our common stock. During the year ended December 31, 2020, we issued 78,807 shares of our common stock pursuant to the Prior EDA for aggregate net proceeds of $0.5 million. During the year ended December 31, 2019, we issued 423,072 shares of our common stock pursuant to the Prior EDA for aggregate net proceeds to us of $2.1 million. On July 9, 2020, we entered into a new Equity Distribution Agreement (New EDA) with 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 $60.0 million through or to JMP. Subject to the terms and conditions of the New EDA, JMP will use its commercially reasonable efforts to sell shares of our common stock from time to time, based upon our instructions. 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. The New EDA superseded and terminated the Prior EDA effective immediately upon effectiveness of our shelf registration statement on Form S-3 (File No. 333-239780) filed with the Securities and Exchange Commission on July 9, 2020 and declared effective by the Securities and Exchange Commission on July 27, 2020.

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Public Offerings

In connection with the closing of an equity financing in December 2020, we issued a total of 5,000,000 shares of common stock in an underwritten public offering resulting in aggregate net proceeds, after underwriting discounts and commissions in the public offering and other estimated offering expenses, of $64.7 million.

In connection with the closing of an equity financing in June 2020, we issued a total of 4,600,000 shares of common stock in an underwritten public offering resulting in aggregate net proceeds, after underwriting discounts and commissions in the public offering and other estimated offering expenses, of $42.9 million.

On December 11, 2019, the Company entered into an underwriting agreement with Oppenheimer & Co., Inc., as representative of the underwriters (Underwriting Agreement), in connection with the underwritten public offering of 7,000,000 shares of the Company's common stock, par value $0.001 per share, at a price to the public of $5.00 per share (the "Public Offering"). Pursuant to the terms of the Underwriting Agreement, on December 13, 2019, the Company sold 8,050,000 shares of common stock, including the exercise of the option granted to the underwriters for 1,050,000 shares of common stock, and received net proceeds of $37.4 million, after deducting underwriting discounts and commissions and other transaction costs of $2.8 million.

9. Convertible Preferred Stock

Concurrent with the 2019 Public Offering, we entered into a Securities Purchase Agreement (the Purchase Agreement), by and among the Company and the investors listed therein. Pursuant to the terms of the Purchase Agreement, the Company sold to the investors an aggregate of 30,000 shares of Series A Participating Convertible Preferred Stock, par value $0.001 per share (the Series A Preferred Stock), at a per share price of $1,000 in a private placement (the 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 will be convertible into 200 shares of common stock, reflecting a conversion price equal to $5.00 per share, subject to customary anti-dilution adjustments. The shares of Series A Preferred Stock will be mandatorily convertible into shares of common stock, subject to a beneficial ownership limitation (described below), in partial or in full, thereof from and after filing the certificate of amendment to the Company's charter with the Secretary of State of the State of Delaware to increase the Company's authorized shares of common stock (Exercise Contingency).

The holders of the Series A Preferred Stock had a feature that allowed the holders to have a liquidation preference to the Company's common stockholders. Because such a potential redemption-triggering event was not solely within the control of the Company, the Series A Preferred Stock was presented as "Convertible Preferred Stock" on our December 31, 2019 balance sheet in a manner consistent with temporary equity under applicable accounting standards.

During the year ended December 31, 2020, 25,247 shares of our Series A Preferred Stock converted into 5,049,400 shares of our common stock, pursuant to the terms of the Purchase Agreement. As of December 31, 2020, 4,753 shares of our Series A Preferred Stock remained outstanding, convertible into 950,600 shares of our common stock.

In May 2020, a registration statement covering the resale of shares of our common stock underlying our Series A Preferred Stock was declared effective by the Securities and Exchange Commission (SEC). In accordance with the securities purchase agreements underlying the Series A Preferred Stock, the liquidation preference was terminated at that time, and we reclassified the Series A Preferred Stock into permanent equity. The holders of the Series A Preferred Stock also 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

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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%.

The difference between the conversion price and the fair value of the Company's common stock on the commitment date (transaction date) resulted in a beneficial conversion feature the amount of $8.9 million.

10. Commitments and Contingencies

Employee Benefit Plan

We maintain a Section 401(k) retirement plan for all employees. Employees can contribute up to 50% of their eligible pay, subject to maximum amounts allowed under law. We may make discretionary profit sharing contributions, which 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.

In March 2017, the Company 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. As of December 31, 2020, we had not met any additional milestones under the License Agreement and have not made any additional payments to CyDex other than the upfront fee; however, we achieved a milestone in the first quarter of 2021 with a payment now due. 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.

Severance Agreements

In March 2021, we entered into a Severance Agreement and General Release (Severance Agreement) with Edward F. Smith, our Chief Financial Officer. In connection with this Severance Agreement, we agreed to pay certain severance benefits for nine months to Mr. Smith, including salary and benefits continuation and ongoing consulting

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services totaling approximately $0.5 million. In addition, certain of Mr. Smith's outstanding stock option agreements were modified to accelerate vesting and extend the exercise period, resulting in additional compensation cost of approximately $0.1 million. We did not record any severance charges related to Mr. Smith through the year ended December 31, 2020.

In March 2019, we entered into a Severance Agreement and General Release (Severance Agreement) with Christopher M. Cashman (Cashman), our former Chief Executive Officer. In connection with this Severance Agreement, we agreed to pay certain severance benefits for one year to Cashman, including salary and benefits continuation and a prorated bonus totaling $0.6 million. As of December 31, 2019, $0.1 million in severance benefits remained unpaid. In addition, certain of Cashman's outstanding stock option agreements were modified to accelerate vesting and extend the exercise period, resulting in additional compensation cost of $0.4 million. As of December 31, 2020, no amounts of severance remain unpaid to Cashman.

11. Income Taxes

On March 27, 2020, in response to COVID-19 and its detrimental impact to the global economy, then-President Trump signed The Coronavirus Aid, Relief, and Economic Security (CARES) Act into law, which provides a stimulus to the U.S. economy in the form of various individual and business assistance programs as well as temporary changes to existing tax law. The changes to the provision in business tax laws include a five-year net operating loss carryback for the 2018, 2019 and 2020 tax years, a deferral of the employer's portion of the social security tax, and an increase in the interest expense limitation under Section 163(j) from 30% to 50% for the 2019 and 2020 tax years, among other things. The CARES Act did not have a material impact on our income taxes, and we will continue to monitor for additional legislation related to COVID-19 and its impact on our results of operations.

On December 21, 2020, Congress approved the Consolidated Appropriations Act, 2021 (Appropriations Act), which was signed into law by then-President Trump on December 27, 2020. The Appropriations Act funds the federal government to the end of the 2021 fiscal year and provides further COVID-19 economic relief. Some of the business provisions included in the Appropriations Act are additional Paycheck Protection Program (PPP) loans, clarification of the deductibility of business expenses that were paid for with PPP funds, expansion of the employee retention credit, and temporary full deduction for business expenses for food and beverages provided by a restaurant. The Appropriations Act did not have a material impact on our income taxes, and we will continue to monitor for additional legislation related to COVID-19 and its impact on our results of operations.

Loss before income taxes is allocated as follows (in thousands):

	Year Ended December 31,	
	2020	2019
U.S. operations	$ 67,475	$ 18,544
Foreign operations	—	35,577
Loss before income taxes	$ 67,475	$ 54,121

As of December 31, 2020 and 2019, we had approximately $212.0 million and $143.7 million, respectively, of net operating loss (NOL) carry forwards available to offset future federal and state taxable income that will expire beginning in 2023. As of December 31, 2020, we also have federal research and development credit carryovers of approximately $11.5 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 may become subject to an annual limitation in the event of

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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 could limit the amount of NOLs that we can utilize annually to offset future taxable income or tax liabilities. The amount of the annual limitation, if any, will be 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 have not evaluated the ownership history of our company to determine if there were any ownership changes as defined under Section 382(g) of the Code and the effects any ownership change may have had.

The components of the net deferred tax asset are as follows (in thousands):

	December 31,	
	2020	2019
Gross deferred tax assets:		
Net operating loss carryforwards	$ 59,819	$ 40,120
Accrued expenses	218	200
Contributions	4	4
Depreciation	58	42
Stock-based compensation	4,270	2,967
Research and development and other credits and other carryforwards	11,906	9,118
Capitalized research and development expenses	15,891	—
Unrealized income	12	—
Total gross deferred tax assets	$ 92,178	$ 52,451
Gross deferred tax liabilities:		
Depreciation	—	—
Total gross deferred tax liabilities	—	—
Net deferred tax assets	92,178	52,451
Less: valuation allowance	(92,178)	(52,451)
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, 2020 and 2019. The valuation allowance increased by $39.7 million and $7.8 million during the years ended December 31, 2020 and 2019, respectively. The increase for the year ended December 31, 2020 was due primarily to our increase in net operating loss carryovers and an increase in tax attributes. The increase for the year ended December 31, 2019 was due primarily to our increase in net operating loss carryovers.

We did not have unrecognized tax benefits as of December 31, 2020 and 2019, 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, 2020 and 2019. As of December 31, 2020 and 2019, we had not accrued interest or penalties related to any uncertain tax positions.

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,	
	2020	2019
Federal income tax expense at statutory rate	21.0 %	21.0 %
Permanent items	(0.7)	(1.0)
State income tax, net of federal benefit	7.6	2.3
R&D tax credits	4.7	4.4
Change in state apportionment	—	1.1
Foreign income tax effect	—	(13.8)
Capitalized research and development expenses	26.2	—
Other	—	0.5
Change in valuation allowance	(58.8)	(14.5)
Effective income tax rate	0.0 %	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 United States, the State of Connecticut, and the Commonwealth of Pennsylvania. The federal and state income tax returns are generally subject to tax examinations for the tax years ended December 31, 2017 through December 31, 2019. 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 4.3

DESCRIPTION OF THE REGISTRANT'S SECURITIES REGISTERED PURSUANT TO SECTION 12 OF THE SECURITIES EXCHANGE ACT OF 1934

As of the date of the Annual Report on Form 10-K of which this exhibit forms a part, the only class of securities of Marinus Pharmaceuticals, Inc. ("we," "us" and "our") registered under Section 12 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), is our common stock, $0.001 par value per share.

CAPITAL STOCK

The following description of our capital stock summarizes provisions of our fourth amended and restated certificate of incorporation, as amended ("Certificate of Incorporation"), our amended and restated bylaws ("Bylaws") and the Delaware General Corporation Law (the "DGCL"). For a complete description, refer to our Certificate of Incorporation and our Bylaws, which are incorporated by reference as exhibits to the Annual Report on Form 10-K of which this exhibit is a part, and to the applicable provisions of the DGCL.

Classes of Stock

Our Certificate of Incorporation authorizes 175,000,000 shares of stock, of which 150,000,000 shares are common stock with a par value of $0.001 per share, and 25,000,000 shares are preferred stock with a par value of $0.001 per share.

Rights of Common Stock

Voting Rights.

Holders of our common stock are entitled to cast one vote for each share of common stock held of record on all matters submitted to a vote of the stockholders, including in all elections for directors. Stockholders are not entitled to cumulative voting in the election for directors. Our stockholders may vote either in person or by proxy. Certain matters identified in our Certificate of Incorporation and our Bylaws, including amending our charter, require the approval of a majority of our issued and outstanding shares of common stock. Our directors shall be elected by a plurality of votes cast. All other questions shall be decided by a majority of the shares present in person, by remote communication or represented by proxy.

Dividends.

Holders of our common stock are entitled to receive dividends ratably, as may be lawfully declared from time to time by our board of directors, subject to any preferential rights of holders of any outstanding shares of preferred stock.

Liquidation.

Holders of our common stock are entitled in the event of our liquidation, dissolution or winding up, whether voluntary or involuntary, after payment of our debts and other liabilities and making provision for the holders of outstanding shares of preferred stock, if any, to share ratably in the remainder of our assets.

Other Rights and Preferences.

Holders of our common stock do not have any preemptive, cumulative voting, subscription, conversion, redemption, or sinking fund rights. Our common stock is not subject to future calls or assessments by us.

Fully Paid and Nonassessable

All of our outstanding shares of common stock are fully paid and nonassessable.

Preferred Stock

Under our Certificate of Incorporation, our board of directors has the authority, without further action by our stockholders, to issue up to 25,000,000 shares of preferred stock, $0.001 par value per share, in one or more series and to fix the designations, powers, preferences, rights of the shares of each such series and to fix the qualifications, limitations, and restrictions of each series, including, but not limited to, dividend rights, terms of redemption, conversion rights, voting rights, and sinking fund terms, any or all of which may be greater than the rights of common stock, and the number of shares constituting such series.

On December 12, 2019, we filed a Certificate of Designations, Preferences and Rights of Series A Participating Convertible Preferred Stock (the "Certificate of Designations") with the Secretary of State of the State of Delaware to establish the terms, rights, obligations and preferences of our Series A Participating Convertible Preferred Stock, par value $0.001 per share ("Series A Preferred Stock"). The number of shares of Series A Preferred Stock designated is 30,000, and each share of Series A Preferred Stock has a stated value equal to $1,000.

Voting Rights.

Except as otherwise provided by the DGCL, other applicable law or as provided in the Certificate of Designations, the holders of the Series A Preferred Stock are not entitled to vote (or render written consents) on any matter submitted for a vote (or written consents in lieu of a vote as permitted by the DGCL, the Certificate of Incorporation and the Bylaws) of holders of common stock. The consent of the holders of at least a majority of the outstanding shares of Series A Preferred Stock will be required to, among other matters, alter or change adversely the terms of the Series A Preferred Stock. The express prior written consent of Oppenheimer & Co., Inc. or its respective designees will be required to directly or indirectly amend, alter, modify or repeal the Certificate of Designations in any manner adverse to the interests of the holders of our common stock (as so reasonably determined by such underwriters or their respective designees).

Dividends.

If our board of directors declares a dividend or other distribution payable upon the common stock, then the holders of the outstanding shares of Series A Preferred Stock will be entitled to the amount of dividends as would be payable in respect of the number of shares of common stock into which the shares of Series A Preferred Stock could be converted, such number to be determined as of the record date for the dividend or, if no such record date is established, as of the date of such dividend. Dividends are payable at the same time as and when dividends on the common stock are paid to the holders of common stock.

Liquidation Preference.

The liquidation preference applicable to the Series A Preferred Stock terminated in May 2020 upon the effectiveness of the registration statement covering the resale of the shares of common stock into which the shares of Series A Preferred Stock are convertible under the Securities Act of 1933, as amended (the "Securities Act").

Conversion.

The number of shares of common stock into which each share of Series A Preferred Stock is initially convertible is equal to the number obtained by dividing (i) the sum of $1,000, being the initial purchase price per share of the Series A Preferred Stock, and the amount of any accrued but unpaid dividends thereon by (ii) $5.00, being the conversion price per share of Series A Preferred Stock, subject to customary anti-dilution adjustments. ***[HL Note: MRNS to confirm all numbers reflect post-split amounts.]***

All shares of Series A Preferred Stock may be converted, at the option of the holder thereof, into the number of fully paid and nonassessable shares of common stock equal to the number obtained by dividing (i) the stated value of such Series A Preferred Stock, plus the amount of any accrued but unpaid dividends as of the conversion date by (ii) the conversion price in effect on the conversion date (determined as provided in the Certificate of Designations), provided that we may not effect, and the holder of Series A Preferred Stock does not have the right to, convert any portion of the Series A Preferred Stock to the extent that such conversion would result in the holder owning in excess of the Beneficial Ownership Limit (as described below). The Certificate of Designations contains certain mandatory conversion features, customary anti-dilution adjustments to the conversion price in the event of stock dividends, subdivisions or splits and upon stock combinations, as well as customary requirements regarding our obligation to effect conversions and deliver common stock shares certificates and for the payment by us of damages for our failure to comply with such requirements.

The "Beneficial Ownership Limitation" is 9.99% of the number of shares of our common stock outstanding immediately after giving effect to the issuance of shares of common stock issuable upon conversion of Series A Preferred Stock held by the applicable holder; provided that, subject to certain limitations, by written notice to us, a holder of Series A Preferred Stock may from time to time increase (but not decrease) the Beneficial Ownership Limitation to any other percentage not in excess of 19.99% specified in such notice.

In the event of (A) a capital reorganization of our common stock, (B) a reclassification of our common stock (other than a subdivision, split-up or combination of shares) or (C) a merger or consolidation of us with or into another corporation, or the sale of all or substantially all of our properties and assets to any other person, then, as a part of such reorganization, reclassification, merger, or consolidation or sale, provision will be made so that holders of Series A Preferred Stock, as the case may be, shall thereafter be entitled to receive upon conversion of the Series A Preferred Stock, the kind and amount of shares of stock or other securities or property of our company, or of the successor corporation resulting from such merger, consolidation or sale, to which such holder would have been entitled if such holder had converted its shares of Series A Preferred Stock immediately prior to such capital reorganization, reclassification, merger, consolidation or sale.

Ranking.

The Series A Preferred Stock ranks senior to our common stock with respect to distributions upon any Liquidation, on parity to any class or series of our capital stock hereafter created specifically ranking

by its terms on parity with the Series A Preferred Stock and junior to any class or series of our capital stock hereafter created specifically ranking by its terms senior to the Series A Preferred Stock.

Anti-Takeover Effect of Our Charter and Bylaw Provisions

Our Certificate of Incorporation and Bylaws contain provisions that could make it more difficult to complete an acquisition of us by means of a tender offer, a proxy contest or otherwise or the removal and replacement of our incumbent officers and directors.

Staggered Board; Removal of Directors; Board Vacancies; Board Size; No Cumulative Voting in Election of Directors. Our Certificate of Incorporation divides our board of directors into three classes with staggered three-year terms. Moreover, it provides for the removal of any of our directors only for cause and requires a stockholder vote of at least a majority of the voting power of the then outstanding voting stock. In addition, our Certificate of Incorporation provides that any vacancy occurring on our board of directors may be filled by a majority of directors then in office, even if less than a quorum, unless the board of directors determines that such vacancy shall be filled by the stockholders. Under our Bylaws, the authorized number of directors may be changed only by a resolution of adopted by a majority of the board of directors. Finally, our Certificate of Incorporation does not allow cumulative voting in the election of directors. This system of a staggered board, removing directors, filling vacancies, fixing the size of the board, and not allowing for cumulative voting makes it more difficult for stockholders to replace a majority of the directors.

Special Stockholder Meetings; No Written Consent Allowed. Our Bylaws provide that a special meeting of stockholders may be called only by the board of directors pursuant to a resolution adopted by a majority of our board of directors, by our chief executive officer, or by the chairperson of the board. All stockholder actions must be effected at a duly called annual or special meeting of stockholders and not by written consent.

Stockholder Advance Notice Procedure. Our Bylaws establish an advance notice procedure for stockholders to make nominations of candidates for election as directors or to bring other business before an annual meeting of our stockholders. The Bylaws provide that any stockholder wishing to nominate persons for election as directors at, or bring other business before, an annual meeting must deliver to our secretary a written notice of the stockholder's intention to do so. To be timely, the stockholder's notice must be delivered to or mailed and received by us not more than 120 days, and not less than 90 days before the anniversary date of the preceding annual meeting, except that if the annual meeting is set for a date that is not within 30 days before or 60 days after such anniversary date, we must receive the notice not earlier than the close of business on the 120th day prior to the annual meeting and not later than the close of business on the later of (i) the 90th day prior to the annual meeting or (ii) the tenth day following the day on which we first made public announcement of the date of meeting. The notice must include the following information:

- as to director nominations, all information relating to each director nominee that is required by the rules of the Securities and Exchange Commission to be disclosed in solicitations of proxies, or is otherwise required by Regulation 14 of the Exchange Act;

- as to any other business that the stockholder proposes to bring before the meeting, a brief description of the business to be proposed, the reasons for conducting such business at the meeting and, if any, the stockholder's material interest in the proposed business; and

- (A) the name and address of the stockholder proponent, (B) the class, series, and number of our shares beneficially owned of record, (C) a description of any agreement, arrangement or

understanding with respect to such nomination or proposal, (D) a representation that the proponent is a holder of record of our voting shares and intends to appear in person or by proxy at the stockholder meeting, (E) a representation as to whether the proponent intends to deliver a proxy statement and form of proxy, (F) to the extent known by the proponent, the name and address of any other stockholder supporting the proposal on the date of such stockholder's notice, and (G) a description of all derivative transactions by the proponent during the previous twelve-month period, including the date of the transactions and the class, series and number of securities involved in such transactions.

Undesignated Preferred Stock. The ability to authorize and issue undesignated preferred stock makes it possible for our board of directors to issue preferred stock, without stockholder approval, with voting or other rights or preferences that could have the effect of delaying, deferring, preventing, or otherwise impeding any attempt to change control of us.

Indemnification. Our Certificate of Incorporation and our Bylaws provide that we will indemnify officers and directors against losses as they incur them in investigations and legal proceedings resulting from their services to us, which may include service in connection with a takeover.

Delaware Anti-Takeover Statute. We are subject to Section 203 of the DGCL, which prohibits persons deemed "interested stockholders" from engaging in a "business combination" with a publicly traded Delaware corporation for three years following the date these persons become interested stockholders unless the business combination is, or the transaction in which the person became an interested stockholder was, approved in a prescribed manner or another prescribed exception applies. Generally, an "interested stockholder" is a person who, together with affiliates and associates, owns, or within three years prior to the determination of interested stockholder status did own, 15% or more of a corporation's voting stock. Generally, a "business combination" includes a merger, asset or stock sale, or other transaction resulting in a financial benefit to the interested stockholder. The existence of this provision may have an anti-takeover effect with respect to transactions not approved in advance by the board of directors, such as discouraging takeover attempts that might result in a premium over the market price of our common stock. We anticipate that the provisions of Section 203 might encourage companies interested in acquiring us to negotiate in advance with our board of directors since the stockholder approval requirement would be avoided if a majority of the directors then in office approve either the "business combination" or the transaction that resulted in the stockholder becoming an "interested stockholder."

Exclusive Forum. 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.

Listing

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

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC. Its address is 6201 15th Avenue, Brooklyn, NY 11219.

Exhibit 10.3

SEPARATION AND CONSULTING AGREEMENT AND GENERAL RELEASE

This Separation and Consulting Agreement and General Release (the "Agreement") is being entered into between Edward Smith ("Smith") and Marinus Pharmaceuticals, Inc. (the "Company") in connection with Smith's separation from the Company effective March 9, 2021 (the "Separation Date").

WHEREAS, Smith's employment with the Company is ending as of the Separation Date;

WHEREAS, the Company wishes to retain Smith as a consultant for a period of time after the Separation Date to assist with the orderly transition of Smith's duties;

WHEREAS, the parties wish to clarify and memorialize certain agreements made between them in regard to such employment, termination of employment and consultancy period;

NOW, THEREFORE, in consideration of the foregoing premises and the terms stated herein, it is mutually agreed between the parties as follows:

1. Accrued Salary and Vacation. On the next regular payroll date following the Separation Date, the Company will pay Smith all accrued salary and all accrued and unused vacation earned through the Separation Date, subject to standard payroll deductions and withholdings. Smith will receive these payments regardless of whether or not he signs this Agreement.

2. Wages and Benefits as of Separation Date. By signing this Agreement, Smith acknowledges and agrees that, upon payments of the amounts set forth in Section 1, Smith has been fully paid all outstanding, accrued compensation due and owing to Smith up to and including the Separation Date, including all wages, salary, commissions, bonuses, incentive payments, vacation, paid time off, profit-sharing payments, expense reimbursements, leave or other benefits. Smith acknowledges and agrees that, except as expressly provided in this Agreement, as of Smith's Separation Date, Smith is no longer eligible to participate in or accrue benefits under any of the Company's benefit plans, including, but not limited to, any dental or medical insurance, long term care plans, retirement or 401(k) plans, vacation leave, sick leave, long term disability insurance, life insurance, cash incentive plans, deferred compensation, pensions, profit sharing or personal accident insurance, or, except as specifically set forth herein, any plan of the Company relating to equity awards, such as stock options. Upon execution of the Agreement Smith hereby relinquishes the titles of Vice President, Chief Financial Officer and Treasurer.

3. Consideration for Signature. If Smith signs this Agreement on the Separation Date, does not revoke it under Section 12(b) hereof, and at all times abides by its terms, then:

a. The Company will pay to Smith the total gross amount of Three Hundred Seven Thousand Five Hundred Dollars ($307,500.00), the equivalent of nine (9) months of Smith's base salary as of the Separation Date ("Severance"), less appropriate federal, state and local taxes and other withholdings as determined by the Company, payable in equal installments, in accordance with the Company's regular payroll practices over a nine (9) month period, with the first installment being paid on the first payroll date after the Effective Date of this Agreement as defined in Section 12(b). Smith acknowledges and agrees that the Severance is not compensation for Smith's services rendered through the Separation Date, but rather constitute consideration for the promises contained in this Agreement, and is above and beyond any wages

or salary or other sums to which Smith is entitled from the Company under the terms of Smith's employment with the Company or under any other contract or law.

b. All of Smith's unvested stock options, as set forth on Schedule A hereto, will immediately vest as of the Separation Date and will become and remain exercisable pursuant to each stock option's terms until the earlier of (i) the one year anniversary of the Separation Date, and (ii) the end of the term of such stock option. Except as specifically set forth in this Section 3(b), all stock options held by Smith shall continue to be governed by the applicable incentive plan and award agreements (the "Option Award Agreements").

c. If Smith timely elects to participate in a healthcare continuation coverage program such as under Section 4980B of the Internal Revenue Code ("COBRA") or any similar state medical and dental insurance continuation coverage program for himself and his covered dependents, then the Company will pay, as and when due to the insurance carrier or COBRA administrator (as applicable), that portion of Smith's premiums for COBRA coverage that it was paying prior to the Separation Date for a period of nine (9) months following the Separation Date (collectively, "COBRA Premium Payments"); provided, however, that the COBRA Premium Payments shall immediately cease prior to the end of such nine-month period in the event, and upon the date, that Smith becomes eligible for and obtains substantially equivalent employer-offered health insurance coverage. Thereafter, Smith will be responsible for the full COBRA premium to continue coverage. Notwithstanding the foregoing and regardless of whether Smith signs this Agreement, Smith understands that he shall have the right to COBRA continuation coverage at his own expense under the normal COBRA health care continuation rules and applicable plan terms.

d. The Company shall retain Smith as a consultant for a three-month period, beginning on the Separation Date and ending on June 8, 2021 (the "Consultancy Period"), unless the Consultancy Period is terminated earlier as set forth below. The terms of the consultancy during the Consultancy Period are as follows:

i. During the Consultancy Period, and subject to Section 3(d)(ii), Smith shall make himself reasonably available to perform services as reasonably requested by Scott Braunstein, M.D., CEO at the Company (the "Services"). The Services shall include, but may not be limited to, Services related to assisting in the transition as requested.

ii. During the Consultancy Period, Smith may accept other full-time employment or engagements and may participate in any other activities; provided, however, that such other employment, engagements and activities do not unreasonably interfere with Smith's ability or obligation to provide the Services required hereunder, create a conflict of interest or violate the terms of this Agreement. In the event that the Company deems any such employment, activities or engagement to unreasonably interfere with Smith's duties hereunder, the Company will promptly provide written notice to Smith and provide Smith an opportunity to cure.

iii. Subject to the Company's quality specifications, Smith shall perform the Services at appropriate times and location(s) in the reasonable discretion of Smith, provided that the Company shall on occasion be entitled to reasonably request Smith to perform services at specific times or locations, so long as such specifications do not unreasonably conflict with Smith's obligations in connection with any new employment. Smith's contact person at the Company for purposes of performing the Services shall be Scott Braunstein, M.D. and/or his designee.

iv. The Company agrees to pay Smith during the Consultancy Period a monthly fee of $34,167 ("Monthly Consultant Fee"), payable in accordance with the Company's ordinary payroll procedures. Such payment(s) shall be subject to a Form 1099-MISC.

v. For the duration of the Consultancy Period, Smith (i) understands that he is an independent contractor and shall have sole control of the manner and means of performing the Services and shall complete such Services in accordance with his own means and methods of work, and according to his own schedule; (ii) shall be solely responsible for any federal, state or local income taxes or self-employment taxes arising with respect to the amounts payable under this Section 3(d); (iii) has no federal, state or local law workers' compensation rights with respect to the Services; (iv) shall not be entitled to disability insurance, Social Security or unemployment compensation coverage or any other statutory benefit generally granted to employees of the Company; (v) shall comply at his expense with all applicable provisions of workers' compensation laws, unemployment compensation laws, federal Social Security law, the Fair Labor Standards Act, OSHA regulations, federal, state and local income tax laws, and all other applicable federal, state and local laws, regulations and codes relating to terms and conditions of employment required to be fulfilled by employers or independent contractors; and (vi) shall not have the authority or ability to legally bind or commit the Company or any of its affiliates. Nothing contained in this Section 3(d) is intended to give rise to, or gives rise to, a partnership, joint venture, agency, fiduciary, employment, or other relationship between the parties or imposes upon the parties any of the duties or responsibilities of partners, joint venturers or employer-employee, beyond the relationship of independent parties to a commercial contract.

vi. Smith agrees to observe and comply with, and that as a consultant he is subject to, the policies and rules of the Company. Smith agrees to observe and comply with all such policies that by their operation survive termination of his consultancy hereunder.

vii. Smith may terminate the Consultancy Period at any time upon written notice to the Company, at which time the Company shall have no further obligations to Smith, except as stated in this Section 3(d)(vii). The Company may terminate the Consultancy Period at any time for "Cause" upon notice to Smith and subject to any cure period specified in this Section 3(d)(vii). For purposes of this Section 3(d), the term "Cause" shall be defined as Smith failing to provide Services as reasonably requested by the Company, which are agreed may be coordinated with any new full-time employment obtained by Smith, after the expiration of ten (10) days without cure after written notice of such failure. In the event that Smith terminates the Consultancy Period for any reason or the Company terminates the Consultancy Period for Cause, the Company shall pay Smith a pro-rata portion of the Monthly Consultant Fee for the month in which the Consultancy Period terminates. The Company may not terminate the Consultancy Period except for Cause.

4. General Release. Except for any rights granted under this Agreement, by signing this Agreement, Smith, for himself, and, to the extent permitted by law, for Smith's heirs, assigns, executors and administrators, hereby releases, remises and forever discharges the Company, its parents, subsidiaries, affiliates, divisions, predecessors, successors, assigns, and each of their respective members, managers, directors, officers, partners, attorneys, shareholders, administrators, employees, agents, representatives, employment benefit plans, plan administrators, fiduciaries, trustees, insurers and re-insurers, and investors, and all of their predecessors, successors and assigns, and each of their respective members, managers, directors, officers, partners, attorneys, shareholders, administrators, employees, agents, representatives, employment benefit plans, plan administrators, fiduciaries, trustees, insurers and re-insurers, investors (collectively, the "Releasees") of and from all claims, causes of action, covenants, contracts, agreements, promises, damages, disputes, demands, and all other manner of actions whatsoever, in law or in equity, that Smith ever had, may have had, now has, or that Smith's heirs, assigns, executors or administrators hereinafter can, shall or may have, whether known or unknown, asserted or unasserted, suspected or unsuspected, as a result of or related to Smith's employment with the Company, including vacation pay, profit sharing plans, retirement plans or any other benefit plans of any type or nature other than as preserved hereby, the termination of Smith's employment, or under any contract relating to Smith's employment, including the Amended and Restated Employment Agreement entered into between the Company and

Smith, dated August 3, 2016 (the "Employment Agreement"), or any act or omission which has occurred at any time up to and including the date of the execution of this Agreement (collectively, the "Released Claims").

a. Released Claims. The Released Claims include, but are not limited to, claims for monetary damages; claims related to Smith's employment with the Company or the termination thereof; claims to severance or similar benefits as of the date of this Agreement, but not to include any claims for Smith's future eligibility under the Change in Control Severance Plan (the "Severance Plan"); claims to expenses, attorneys' fees or other indemnities; claims based on any actions or failures to act that occurred on or before the date of this Agreement; and claims for other personal remedies or damages sought in any legal proceeding or charge filed with any court or federal, state or local agency either by Smith or by any person claiming to act on Smith's behalf or in Smith's interest. Smith understands that the Released Claims may have arisen under different local, state and federal statutes, regulations, or common law doctrines. Smith hereby specifically, but without limitation, agrees to release all Releasees from any and all claims under each of the following:

i. Antidiscrimination laws, such as Title VII of the Civil Rights Act of 1964, as amended, and Executive Order 11246 (which prohibit discrimination based on race, color, national origin, religion, or sex); Section 1981 of the Civil Rights Act of 1866 (which prohibits discrimination based on race or color); the Americans with Disabilities Act and Sections 503 and 504 of the Rehabilitation Act of 1973 (which prohibit discrimination based upon disability); the Age Discrimination in Employment Act, as amended; 29 U.S.C. Section 621 et seq. (which prohibits discrimination on the basis of age); the Equal Pay Act (which prohibits paying men and women unequal pay for equal work); the Pennsylvania Human Relations Act; or any other local, state or federal statute, regulation, common law or decision concerning discrimination, harassment, or retaliation on these or any other grounds or otherwise governing the employment relationship.

ii. Other employment laws, such as the federal Worker Adjustment and Retraining Notification Act of 1988; the Executive Retirement Income Security Act of 1974 (which, among other things, protects employee benefits); the Fair Labor Standards Act of 1938 (which regulates wage and hour matters); the Family and Medical Leave Act of 1993 (which requires employers to provide leaves of absence under certain circumstances); the Pennsylvania Whistleblower Law; the Pennsylvania Public Employee Relations Act; the Pennsylvania Wage Payment and Collections Law as well as any amendments to such laws; the U.S. Patriot Act, the Sarbanes Oxley Act; the Dodd Frank Act; and any other federal, state, or local statute, regulation, common law or decision relating to employment, reemployment rights, leaves of absence or any other aspect of employment.

iii. Other laws of general application, such as federal, state, or local laws enforcing express or implied employment agreements or other contracts or covenants, or addressing breaches of such agreements, contracts or covenants; federal, state or local laws providing relief for alleged wrongful discharge or termination, physical or personal injury, emotional distress, fraud, intentional or negligent misrepresentation, defamation, invasion of privacy, violation of public policy or similar claims; common law claims under any tort, contract or other theory now or hereafter recognized, and any other federal, state, or local statute, regulation, common law doctrine, or decision regulating or regarding employment.

b. Participation in Agency Proceedings. Nothing in this Agreement shall prevent Smith from filing a charge (including a challenge to the validity of this Agreement) with the Equal Employment Opportunity Commission (the "EEOC"), the National Labor Relations Board (the "NLRB"), or other similar federal, state or local agency, or from participating in any investigation or proceeding conducted by the EEOC, the NLRB, or similar federal, state or local agencies. However, by entering into

this Agreement, Smith understands and agrees that Smith is waiving any and all rights to recover any monetary relief or other personal relief against the Releasees as a result of any such EEOC, NLRB, or similar federal, state or local agency proceeding, including any subsequent legal action.

c. Claims Not Released. The Released Claims do not include claims by Smith for: (1) unemployment insurance; (2) worker's compensation benefits; (3) state disability compensation; (4) previously vested benefits under any Company-sponsored benefits plan; (5) all rights under the Option Award Agreements and that certain Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, as Amended (the "Plan"); (6) events that occur after the date Smith signs this Agreement; and (7) any other rights that cannot by law be released by private agreement. Furthermore, Smith is not releasing any right of indemnification he may have for any liabilities arising from his actions within the course and scope of his employment with the Company or within the course and scope of his role as an officer of the Company.

d. No Existing Claims or Assignment of Claims. Smith represents and warrants that Smith has not previously filed or joined in any claims that are released in this Agreement and that Smith has not given or sold any portion of any claims released herein to anyone else, and that Smith will indemnify and hold harmless the Company and the Releasees from all liabilities, claims, demands, costs, expenses and/or attorneys' fees incurred as a result of any such prior assignment or transfer.

e. Acknowledgement of Legal Effect of Release. BY SIGNING THIS AGREEMENT, SMITH UNDERSTANDS THAT HE IS WAIVING ALL RIGHTS HE MAY HAVE HAD TO PURSUE OR BRING A LAWSUIT OR MAKE ANY LEGAL CLAIM AGAINST THE COMPANY OR ANY RELEASEES, INCLUDING, BUT NOT LIMITED TO, CLAIMS THAT IN ANY WAY ARISE FROM OR RELATE TO HIS EMPLOYMENT OR THE TERMINATION OF THAT EMPLOYMENT, FOR ALL OF TIME UP TO AND INCLUDING THE DATE OF THE EXECUTION OF THIS AGREEMENT. SMITH FURTHER UNDERSTANDS THAT BY SIGNING THIS AGREEMENT, HE IS PROMISING NOT TO PURSUE OR BRING ANY SUCH LAWSUIT OR LEGAL CLAIM SEEKING MONETARY OR OTHER RELIEF.

f. Restrictions. Notwithstanding anything to the contrary herein, Smith understands that nothing in this Agreement or any other agreement that Smith may have with the Company restricts or prohibits Smith from initiating communications directly with, responding to any inquiries from, providing testimony before, providing confidential information to, reporting possible violations of law or regulation to, or from filing a claim or assisting with an investigation directly with a self-regulatory authority or a government agency or entity (collectively, "Government Agencies"), or from making other disclosures that are protected under the whistleblower provisions of state or federal law or regulation, and Smith does not need the Company's prior authorization to engage in such conduct. Notwithstanding, in making any such disclosures or communications, Smith must take all reasonable precautions to prevent any unauthorized use or disclosure of any information that may constitute Company Confidential Information to any parties other than the Government Agencies. This Agreement does not limit Smith's right to receive an award for information provided to any Government Agencies.

5. Proprietary and/or Confidential Information. Smith agrees that any sensitive, proprietary, or confidential information or data relating to the Company or any of its affiliates or other Releasees (as defined in Section 4 above), including, without limitation, trade secrets, processes, practices, pricing information, billing histories, customer requirements, customer lists, customer contacts, employee lists, salary information, personnel matters, financial data, operating results, plans, contractual relationships, projections for new business opportunities, new or developing business for the Company, technological innovations in any stage of development, the Company's financial data, long range or short range plans, any confidential or proprietary information of others licensed to the Company, and all other data and information of a competition-sensitive nature, including but not limited to all other data and

information of a competitive-sensitive nature that Smith obtained while serving as a director, officer or employee of the Company or any of its affiliates or Releasees, together with any information received from any former affiliates of the Company or its affiliates or other Releasees (collectively, "Confidential Information"), and all notes, records, software, drawings, handbooks, manuals, policies, contracts, memoranda, sales files, or any other documents generated or compiled by any employee of the Company or Releasees reflecting such Confidential Information, that Smith acquired while an employee of the Company will not be disclosed or used for Smith's own purposes or in a manner detrimental to the Company's interests. Notwithstanding the foregoing, pursuant to 18 USC § 1833(b), an individual may not be held liable under any criminal or civil federal or state trade secret law for disclosure of a trade secret: (i) made in confidence to a government official, either directly or indirectly, or to an attorney, solely for the purpose of reporting or investigating a suspected violation of law, or (ii) in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal. Additionally, an individual suing an employer for retaliation based on the reporting of a suspected violation of law may disclose a trade secret to his or her attorney and use the trade secret information in the court proceeding, so long as any document containing the trade secret is filed under seal and the individual does not disclose the trade secret except pursuant to court order.

6. Return of Information and Property. Smith agrees to return to the Company on the Separation Date all property and equipment belonging to the Company and the Releasees, including without limitation all computers, hard drives, phones, and access cards, the originals and all copies (regardless of medium) of all information, files, materials, documents or other property relating to the business of the Company, the Releasees, or their affiliates. If Smith fails to timely return any such property, the Company shall be entitled to deduct from the Severance an amount equal to the value of non-returned property, and reserves all other rights and remedies.

7. Non-disparagement. Smith agrees not to make to any person or entity any false, disparaging, or derogatory comments about the Company, its business affairs, its employees, clients, contractors, agents, or any of the other Releasees in any manner likely to be harmful to them or their business, business reputation or personal reputation. The Company agrees to instruct its executive team not to make to any person or entity any false, disparaging, or derogatory comments about Smith that is likely to be harmful to Smith's personal or business reputation. Nothing in this Section 7 shall prevent the Company or Smith from responding truthfully to a valid subpoena, court order and/or similar process from a judicial, law enforcement, administrative or regulatory body of competent jurisdiction.

8. General Provisions. This Agreement, including Schedule A, contains the entire understanding and agreement between the parties relating to the subject matter of this Agreement, and supersedes any and all prior agreements or understandings between the parties pertaining to the subject matter hereof, except for Section 5 of the Employment Agreement, which is incorporated herein by reference, and the Option Award Agreements granted under and including the Plan. For the avoidance of doubt, this Agreement does not supersede or otherwise affect the enforceability of the Severance Plan, which remains in full force and effect, and the Company agrees that, under the circumstances of his separation from the Company, in the event of a Change in Control (as defined in the Severance Plan), Smith remains eligible to receive additional severance benefits under the Severance Plan according to its terms. This Agreement may not be altered or amended except by an instrument in writing signed by both parties. Smith has not relied upon any representation or statement outside this Agreement with regard to the subject matter, basis or effect of this Agreement. This Agreement will be governed by, and construed in accordance with, the laws of the Commonwealth of Pennsylvania, excluding the choice of law rules thereof. This Agreement will be binding upon and inure to the benefit of the parties and their respective representatives, successors and permitted assigns. No waiver of a party's rights will be effective unless such waiver is in writing signed by the waiving party. This Agreement and the rights and obligations of the parties hereunder may not be assigned by Smith without the prior written consent of the Company, but may be assigned by

the Company or its successors and assigns without Smith's permission or consent. If any one or more of the provisions of this Agreement, or any part thereof, will be held to be invalid, illegal or unenforceable, the validity, legality and enforceability of the remainder of this Agreement will not in any way be affected or impaired thereby. This Agreement may be signed in one or more counterparts, each of which will be deemed an original, and all of which together will constitute one instrument.

9. No Admission. The parties agree that nothing contained in this Agreement will constitute or be treated as an admission of liability or wrongdoing by either of them.

10. Cooperation. Smith agrees that Smith will cooperate fully with, and make himself reasonably available to, the Company with respect to transitioning Smith's duties and responsibilities and any matter in which Smith was in any way involved during Smith's employment with the Company by making himself reasonably available in conjunction with any new employment that Smith may obtain. Smith shall render such cooperation in a reasonable manner on reasonable notice from the Company.

11. Continuing Obligations and Injunctive Relief. Smith acknowledges and agrees that Section 5 of the Employment Agreement remains in full force and effect and Smith's obligations under Section 5 of the Employment Agreement survive Smith's termination of employment with the Company. Smith further acknowledges that Smith's compliance with Sections 5 and 7 of this Agreement and Section 5 of the Employment Agreement is necessary to protect the goodwill and other proprietary interests of the Company and Smith was one of the principal executives of the Company and conversant with its affairs, its trade secrets and other proprietary information. Smith acknowledges that a breach of Sections 5 or 7 of this Agreement or Section 5 of the Employment Agreement will result in irreparable and continuing damage to the Company for which there will be no adequate remedy at law; and Smith agrees that in the event of any such breach, the Company and its successors and assigns shall be entitled to injunctive relief and to such other and further relief as may be proper.

12. Waiver of Age Discrimination Claims and Claims under ADEA; Acknowledgment/Time Periods. With respect to the General Release in Section 4 of this Agreement, Smith agrees and understands that by signing this Agreement, Smith is specifically releasing all claims Smith may have against Releasees, including without limitation all claims for age discrimination under the Age Discrimination in Employment Act as amended, 29 U.S.C. Section 621 et seq. Smith acknowledges that he has carefully read and understands this Agreement in its entirety, and executes it voluntarily and without coercion.

a. Consideration Period; Deadline. Smith acknowledges that he has been given a period of at least twenty-one (21) days to consider and execute this Agreement before signing it, and that no material changes have been made to this Agreement during the course of discussions leading up to the execution of this Agreement following January 29, 2021, the date this Agreement was first presented to Smith. If Smith fails to sign this Agreement and deliver it to the Company on the Separation Date this Agreement shall be deemed null and void. Smith further acknowledges that he is hereby being advised in writing to consult with a competent, independent attorney of his choice, at his own expense, regarding the legal effect of this Agreement before signing it.

b. Revocation Deadline. Smith understands and acknowledges that Smith has seven (7) days following Smith's execution of this Agreement to revoke his release of ADEA claims in writing, and that should Smith exercise that right, the Company has the option in its sole discretion of voiding the Agreement in its entirety, in which case the Company shall be relieved of all obligations to provide any benefits set forth in Section 3, and to the extent that Smith already received benefits pursuant to those Sections he must immediately return them, except as otherwise stated in Section 3(d)(vii). This Agreement

shall be automatically effective and enforceable on the day following the expiration of the seven (7) day revocation period described in this Section 12(b) without Smith's revocation. For revocation to be effective, written notice must be delivered by email to the attention of Rose McKinley, Vice President Human Resources rmckinley@marinuspharma.com, no later than 11:59 p.m. ET on the seventh (7th) calendar day after Smith signs the Agreement.

13. Internal Revenue Code Section 409A. The parties intend to comply with the requirements of Section 409A of the Internal Revenue Code of 1986, as amended ("Section 409A"). All payments under this Agreement are intended to either be exempt from or comply with the requirements of Section 409A. All payments made under this Agreement shall be strictly paid in accordance with the terms of this Agreement. The parties expressly understand that the provisions of this Agreement shall be construed and interpreted to avoid the imputation of any additional tax, penalty or interest under Section 409A and to preserve (to the nearest extent reasonably possible) the intended benefits payable to Smith hereunder. The Severance paid under this Agreement shall be treated as a separate payment of compensation for purposes of Section 409A. Any reimbursements or in-kind benefits provided under this Agreement that are subject to Section 409A shall be made or provided in accordance with the requirements of Section 409A, including, where applicable, the requirement that (i) any reimbursement is for expenses incurred during the period of time specified in the Agreement, (ii) the amount of expenses eligible for reimbursement, or in-kind benefits provided, during a calendar year may not affect the expenses eligible for reimbursement, or in-kind benefits to be provided, in any other calendar year, (iii) the reimbursement of an eligible expense will be made no later than the last day of the calendar year following the year in which the expense is incurred, and (iv) the right to reimbursement or in-kind benefits is not subject to liquidation or exchange for another benefit. Smith's right to any deferred compensation, as defined under Section 409A, shall not be subject to borrowing, anticipation, alienation, sale, transfer, assignment, pledge, encumbrance, attachment, or garnishment by creditors, to the extent necessary to avoid additional tax, penalties and/or interest under Section 409A. Nothing herein, including the foregoing sentence, shall change the Company's rights and/or remedies under the Agreement and/or applicable law. In the exercise of any of its remedies, the Company will consider in good faith the impact of Section 409A on Smith and shall meaningfully consult with Smith before taking any action that might have an adverse impact on Smith under Section 409A. In no event shall the Company be liable for any penalties, costs, damages, levies or taxes imposed on Smith pursuant to Section 409A.

[Execution Page to Follow]

FOR EXECUTION ON, BUT NOT BEFORE, MARCH 9, 2021

IN WITNESS WHEREOF, the undersigned, intending to be bound hereby, have agreed to the terms and conditions of this Agreement as of the date first set forth below.

EDWARD SMITH

/s/ Edward Smith

Date: March 9, 2021

MARINUS PHARMACEUTICALS, INC.

By: /s/ Scott Braunsetin

Name: Scott Braunstein

Title: Chief Executive Officer

Date: March 9, 2021

Schedule A

Schedule of Outstanding Stock Options that shall become Vested Effective as of the Separation Date

Grant Date	Exercise Price	Number of Shares Subject to Option Award (Exercisable)	Number of Shares Subject to Option Award (Unexercisable)	Number of Shares Subject to Option Award that shall become Vested Effective as of the Separation Date*
11/25/2013	$4.16	35,927		
12/22/2014	$34.80	15,500		
7/20/2015	$57.20	17,500		
8/3/2016	$6.00	14,350		
1/7/2017	$4.84	14,700		
12/6/2017	$24.76	50,000		
2/26/2019	$15.84	37,500	12,500	12,500
8/21/2019	$4.48	7,917	7,083	7,083
1/8/2020	$8.28	74,375	116,875	116,875

*The accelerated vesting of stock options set forth in this column is subject to Smith's satisfaction of the terms and conditions set forth in Section 4 of the Agreement to which this Schedule A is attached.

EXHIBIT 10.4

EMPLOYMENT AGREEMENT

EMPLOYMENT AGREEMENT is effective on April 12, 2021 between Marinus Pharmaceuticals, Inc. (the "Company"), a Delaware corporation, and Steven Pfanstiel, MBA, CMA (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 Financial 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 Financial 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 $380,000 (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) 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 220,000 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.

(d) 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.

(e) 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 hereby does irrevocably assign 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.

MARINUS PHARMACEUTICALS, INC.

By: /s/ Scott Braunstein, MD
Scott Braunstein, MD
Chief Executive Officer

Date: March 7, 2021

/s/ Steven Pfanstiel

By: Steven Pfanstiel, MBA, CMA
Date: March 5, 2021

EXHIBIT 10.9

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 10.9

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 10.9 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 Director
Nicole Vitullo, Chairman of the Board
Chuck Austin, Director
Enrique Carrazana, Director
Michael R. Dougherty, Director
Elan Ezickson, Director
Seth H.Z. Fischer, Director
Timothy Mayleben, Director

EXHIBIT 10.22

EMPLOYMENT AGREEMENT

EMPLOYMENT AGREEMENT effective as of June 15, 2020 between Marinus Pharmaceuticals, Inc. (the "Company"), a Delaware corporation, and Martha Manning (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 Vice President, General Counsel & Secretary, 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 Vice President, General Counsel & Secretary 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 $375,000 (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) 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 300,000 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 thereafterin 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. All stock options and restricted stock awards granted to the Employee that vest over time shall, if the Employee's employment is terminated by the Company without Cause in accordance with Section 4(d) or the Employee resigns from the Company's employ for Good Reason in accordance with Section 4(e), in each case upon or during the twelve-month period that immediately follows a Change in Control (as defined in Section 4(h)), become fully vested upon the termination of the Employee's employment to the extent permitted by the terms of the applicable plan and subject to the satisfaction by the Employee of the requirements of Section 4(g) of this Agreement.

(d) 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.

(e) 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, except as set forth in Schedule A, attached hereto, during the Employee's employment by the Company hereunder and for an additional period of six (6) months after the termination of the Employee's employment hereunder (with respect to central nervous system disorders and pediatric epilepsies) and a period of twelve (12) months (with respect to status epilepticus and any other Competitive Business), neither the Employee nor any corporation or other entity in which the Employee may be interested as a partner, trustee, director, officer, employee, agent, shareholder, lender of money or guarantor, or for which the Employee performs services in any capacity (including as a consultant or independent contractor) shall at any time during such period (i) be engaged, directly or indirectly, in any Competitive Business (as that term is hereinafter defined) or (ii) solicit, hire, contract for services or otherwise employ, directly or indirectly, any of the employees of the Company. For purposes of this Section 5(b), the term "Competitive Business"

shall mean any firm or business organization that competes with the Company in the development and/or commercialization of drugs that prevent or treat partial complex seizures, post-traumatic stress disorder or fragile-x syndrome or any other Ganaxolone-related technology, product or service being developed, manufactured, marketed, distributed or planned in writing by the Company at the time of termination of the Employee's employment with 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 to continue the Employee's undertaking in the Prior Agreement and 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.

MARINUS PHARMACEUTICALS, INC.

By: /s/ Scott Braunstein, MD May 22, 2020
Scott Braunstein, MD Date
Chief Executive Officer

/s/ Martha Manning, Esq. May 22, 2020
Martha Manning, Esq Date

Schedule A

Activities not subject to Section Section 5(b)

Non-Competition

1) Continuing activities with pre-existing contracted consulting/employment commitments (entered into prior to the effective date of this Agreement) and/or minimal ownership interests (five percent (5%) or less) in the following companies and/or their current or future subsidiaries:

None

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. 333-239785) 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. 333-233131) 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. 333-219613) pertaining to the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan; and
6. Registration Statement (Form S-8 No. 333-200701) pertaining to the Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan;

of our report dated March 9, 2021, with respect to the financial statements of Marinus Pharmaceuticals, Inc. included in this Annual Report (Form 10-K) of Marinus Pharmaceuticals, Inc. for the year ended December 31, 2020.

/s/ Ernst & Young LLP
Philadelphia, PA
March 9, 2021

Exhibit 23.2

Consent of Independent Registered Public Accounting Firm

The Board of Directors
Marinus Pharmaceuticals, Inc.:

We consent to the incorporation by reference in the registration statements (Nos. 333-239785, 333-233131, 333-219613 and 333-200701) on Form S-8 and registration statements (Nos. 333-239780 and 333-237903) on Form S-3 of Marinus Pharmaceuticals, Inc. of our report dated March 16, 2020, except for the reverse stock split described in Note 1, as to which the date is March 9, 2021, with respect to the consolidated balance sheet of Marinus Pharmaceuticals, Inc. as of December 31, 2019, the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for the year ended December 31, 2019, and the related notes, which report appears in the December 31, 2020 annual report on Form 10-K of Marinus Pharmaceuticals, Inc.

/s/ KPMG LLP

Philadelphia, Pennsylvania
March 9, 2021

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 15(d)-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, 2021

/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, Edward F. Smith, 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 15(d)-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, 2021

/s/ Edward F. Smith
Edward F. Smith,
Chief Financial Officer and Treasurer
(Principal Financial 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, 2020 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, 2021

/s/ Scott Braunstein
Chief Executive Officer and Director
(Principal executive officer)

Date: March 9, 2021

/s/ Edward F. Smith
Chief Financial Officer and Treasurer
(Principal financial and accounting officer)