MARINUS PHARMACEUTICALS INC
Form 10-K
March 13, 2017
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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, 2016
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.)

170 N Radnor Chester Rd, Suite 250

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	Name of Each Exchange on Which Registered
Voting Common Stock, par value $0.001 per share	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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S K is not contained herein, and will not be contained, to the best of the registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10 K or any amendment to this Form 10 K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non accelerated filer or a smaller reporting company. (Check one):

Large accelerated filer Accelerated filer Non accelerated filer Smaller reporting company

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 for the last business day of the Registrant's most recent completed second fiscal quarter: $12,612,115

The number of shares of the issuer's Common Stock outstanding as of March 10, 2017, was 21,542,212.

Documents Incorporated by Reference

Certain portions, as expressly described in this report, of the registrant's proxy statement for the 2017 Annual Meeting of the Stockholders to be held May11, 2017 are incorporated by reference into Part III, Items 10 14.

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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,” “might,” “objective,” “ongoing,” “plan,” “predict,” “project,” “potential,” “should,” “will,” or “would,” and or the negative of t 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;
- status, timing and results of preclinical studies and clinical trials;
- the potential benefits of ganaxolone;
- the timing of seeking regulatory approval of ganaxolone;
- our ability to obtain and maintain regulatory 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 corporate collaborations, license agreements, and other collaborative efforts for the development and commercialization of products;

- 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 or reimbursement for ganaxolone;

- the success of other competing therapies that may become available;

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- 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; and

- the trends that may affect the industry or us.

You should refer to Part I, Item 1A “Risk Factors” of this Annual Report on this Form 10-K for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. As a result of these factors, we cannot assure you that the forward-looking statements in this Annual Report on Form 10-K will prove to be accurate. 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.

PART I

Item 1. Business.

Overview

We are a clinical stage biopharmaceutical company focused on developing and commercializing innovative therapeutics to treat epilepsy and neuropsychiatric disorders. Our clinical stage product candidate, ganaxolone, is a positive allosteric modulator of $GABA_A$ being developed in three different dose forms: intravenous (IV), capsule and liquid. The multiple dose forms are intended to maximize the therapeutic range of ganaxolone for both adult and pediatric patient populations, in both acute and chronic care, and both in-patient and self-administered settings. Ganaxolone exhibits anti-seizure and anti-anxiety actions via its effects on synaptic and extrasynaptic $GABA_A$ receptors.

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Our Pipeline

We are developing ganaxolone to treat both adults and children suffering from acute and chronic epilepsy and other neuropsychiatric conditions where there is a mechanistic rationale for ganaxolone to provide a benefit, including the following indications:

Postpartum Depression (PPD)

PPD is a mood disorder that affects about 15% of women within the first year following childbirth. Common symptoms include feelings of extreme sadness, hopelessness, suicidal ideation, anxiety, and fatigue. PPD is thought to be linked to the rapid fluctuations in the levels of reproductive hormones and allopregnanolone (allo) after childbirth. Allo has shown early clinical evidence in treating patients with severe PPD. PPD can affect a mother's ability to care for her child and may negatively affect a child's cognitive development. There are no approved treatments for PPD but the most common treatments are psychotherapy and antidepressants. We believe that treatment with ganaxolone may provide benefit to women suffering from PPD.

We are making preparations to initiate a Phase 2 double-blind, placebo-controlled, multi-center, dose-finding study to evaluate the safety, efficacy and pharmacokinetics of ganaxolone IV in women with PPD in the first half of 2017, and expect to announce data from the initial patient cohort(s) in the second half of 2017. Our development strategy in PPD is to evaluate use of both IV and oral dose forms based on disease severity and treatment setting.

Status Epilepticus (SE)

SE is a life-threatening occurrence of continuous or intermittent seizures lasting more than five minutes in

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duration without full recovery. If SE is not treated immediately, permanent neuronal damage may occur, which contributes to high rates of morbidity and mortality. In refractory status epilepticus (RSE), certain synaptic $GABA_A$ receptors are internalized, thereby unavailable to drugs that target these receptors, such as benzodiazepines. According to LexisNexis, there are approximately 45,000 cases of hospitalized RSE treated in the United States annually. RSE patients who do not respond to additional antiepileptic drugs (AEDs), referred to as having super refractory status epilepticus (SRSE), are generally placed under IV anesthesia as a last resort to attempt to stop the seizures and prevent further damage to the brain and death.

Allo has shown early clinical evidence in treating certain SRSE patients. Like allo, ganaxolone modulates both synaptic and extrasynaptic $GABA_A$ receptors, allowing a therapeutic pathway in situations where synaptic $GABA_A$ receptors are unavailable. Ganaxolone has shown activity comparable to allo in preclinical rat models of benzodiazepine-resistant SE. Another preclinical rat model of benzodiazepine refractory SE showed anti-epileptic synergy with the combination of ganaxolone and diazepam in blocking pilocarpine-induced seizures in rats. Ganaxolone and diazepam plasma levels were identical when measured both alone and in combination, indicating that neither drug affected the pharmacokinetic disposition of the other. These data may have clinical implications on the treatment and dosing of ganaxolone in patients with SE who are or have been treated with benzodiazepines.

We are making preparations to initiate a Phase 2 clinical trial with ganaxolone IV in patients with SE in the second half of 2017 and will disclose particulars on design, scope and timing when we initiate the study.

In April 2016, the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation to the IV formulation of ganaxolone for the treatment of SE. Orphan Drug Designation is granted by the FDA Office of Orphan Products Development (OOPD) to novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. The designation provides the drug developer with a seven-year period of U.S. marketing exclusivity, as well as tax credits for clinical research costs, the ability to apply for annual grant funding, clinical research trial design assistance and waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

Genetic Orphan Disorders

We are evaluating selected genetic indications which we believe could benefit from the seizure and/or behavioral effects of ganaxolone's $GABA_A$ modulatory mechanism. To that end, ganaxolone is currently being evaluated in an ongoing Phase 2 open-label exploratory study as a treatment for orphan, genetic epilepsies including CDKL5 disorder, Lennox Gastaut Syndrome (LGS) and PCDH19 pediatric epilepsy (PCDH19-PE). In addition, we recently completed a Phase 2 double-blind placebo-controlled crossover study for the treatment of anxiety and attention deficit in children with Fragile X Syndrome (FXS). Children with genetic epilepsies and children with FXS often suffer from the same co-morbidities including cognitive and developmental impairment, behavioral challenges, sleep disorder and seizures. Ganaxolone could be helpful to these patients across a range of these co-morbidities.

We expect to complete and announce top-line results from the ongoing Phase 2 CDKL5 disorder and LGS cohorts of the genetic epilepsy study in mid-2017. These results along with those from the completed PCDH19-PE cohort and FXS Phase 2 study will be evaluated and prioritized based upon clinical results, anticipated regulatory pathway, program risk assessments and commercial considerations for advancement to late-stage clinical trials.

CDKL5 Disorder

CDKL5 disorder 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 girls 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 CDKL5 cannot walk, talk, or care for themselves. Many also suffer from scoliosis, visual impairment, gastrointestinal difficulties, and sleeping disorders. Currently, there are no approved therapies for CDKL5 disorder. We believe that no previous formal clinical trials have been conducted in this patient population.

Positive preliminary data from the initial patients in the CDKL5 disorder cohort of our study showed a notable

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reduction in seizure frequency compared to baseline in three of the four initial patients enrolled. All responders continue to receive treatment, two of whom have completed six months of treatment and have elected to participate in the study extension. One patient discontinued the study after four months of treatment due to lack of efficacy. Safety data to date are consistent with earlier studies where ganaxolone has shown to be generally safe and well-tolerated. This study is actively recruiting CDKL5 disorder patients, and we expect to complete enrollment and report top-line results from this cohort in mid-2017.

LGS

LGS is a rare and often debilitating form of childhood-onset epilepsy. The syndrome is characterized by multiple seizure types, moderate to severe cognitive impairment, and an abnormal EEG with slow spike-wave complexes. LGS is also a physically dangerous epilepsy syndrome of childhood because of the frequent falls, injuries, and cognitive impairment that can severely limit quality of life.

We are actively recruiting LGS patients for our study and expect to complete enrollment and report top-line results from this cohort in mid-2017.

PCDH19-PE

PCDH19-PE is a serious and rare epileptic syndrome that predominantly affects females. The condition is caused by an inherited mutation of the protocadherin 19 (PCDH19) gene, located on the X chromosome, is characterized by early-onset and highly variable cluster seizures, cognitive and sensory impairment, and behavioral disturbances with autistic traits. Genetic testing is available to determine if a child has the PCDH19 mutation. The mean age of onset of this condition is approximately 10 months. Although formal epidemiologic data is not available, it is suspected that approximately 10% of girls who have seizure onset before five years of age have PCDH19 mutations. We estimate the PCDH19-PE population to be approximately 3,000 to 5,000 patients in the United States Currently, there are no approved therapies for PCDH19-PE. We believe that no previous formal clinical trials have been conducted in this population.

We have completed this cohort in the study, and top-line results showed that ganaxolone reduced seizure frequency from baseline in 64% of patients enrolled, and was generally safe and well tolerated. In 2015, the U.S. Food and Drug Administration granted Orphan Drug Designation to ganaxolone for the treatment of PCDH19-PE.

Fragile X Syndrome (FXS)

FXS is the most common genetic cause of autism and is caused by a mutation in the FMR1 gene. FXS is characterized by a range of developmental problems and symptoms, including cognitive impairment, learning disabilities and behavioral challenges. Approximately one million individuals in the United States have, or are at risk for developing, a Fragile X associated disorder, with approximately 100,000 people having FXS. According to the Centers for Disease Control and Prevention, FXS affects 1 in 3,600 to 4,000 males and 1 in 4,000 to 6,000 females of all races and ethnic groups. Patients with FXS exhibit autism-like symptoms including anxiety and mood swings, attention deficit and heightened response to stimuli. Approximately 7% of women and 18% of men with FXS have seizures.

Treatment approaches focus primarily on supportive care and medications addressing development delays, learning disabilities, and social and behavioral problems caused by the disease. Various classes of medications are used to treat behavioral and mental health conditions associated with FXS. Currently, there are no known cures or approved therapies for FXS. Special education and symptomatic treatments are employed to lessen the burden of illness in FXS patients.

In a mouse model of the FMR1 gene mutation, certain brain regions show lower levels of the extrasynaptic GABAA receptors and reduction of proteins and enzymes responsible for GABA function. The result of fewer GABAA receptors in these mice include over-sensitivity to noise, anxiety, and seizures. Ganaxolone and other agents that have been shown to improve GABA function have also been shown to improve FXS symptoms in this mouse model. We believe that ganaxolone, with its high-affinity for extrasynaptic GABAA receptors, may increase signaling at existing

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receptors to normalize GABA function thereby reducing anxiety, attention, hyperactivity and other disabilities associated with this inherited disorder.

In 2016, we reported results from a Phase 2 exploratory, investigator-sponsored study to evaluate the safety, tolerability and efficacy of ganaxolone for the treatment of anxiety and attention in children with FXS. The study was conducted at the Medical Investigation of Neurodevelopmental Disorder (MIND) Institute at the University of California, Davis and at a site in Belgium. The Phase 2 study was a randomized, placebo-controlled, cross-over proof-of-concept clinical trial. Ganaxolone was generally safe and well tolerated with no serious adverse events (AEs) reported. Consistent with other pediatric studies conducted with ganaxolone, the most common AEs considered related to ganaxolone treatment and reported at greater rates than placebo were fatigue, somnolence, diarrhea, decreased appetite and rash. Most AEs were mild to moderate in severity and resolved. Five patients discontinued the study due to AEs: four during treatment with ganaxolone and one during placebo. The investigator selected Clinical Global Impression of Improvement (CGI-I), which is a broad scale to assess the overall improvement across the entire FXS, as the primary endpoint of the study. While this primary endpoint was not met, consistent with our expectations and ganaxolone's mechanism of action, treatment with ganaxolone improved anxiety and hyperactivity across multiple measures in FXS patients with high baseline anxiety. We believe that the results from this study support the anxiolytic effect of ganaxolone and provide a strong rationale to advance the clinical development of ganaxolone in anxious FXS patients.

In December 2016, the U.S. Food and Drug Administration granted Orphan Drug Designation to ganaxolone for the treatment of FXS.

Adult Focal Onset Seizures

In June 2016, we announced top-line results from our Phase 3 clinical trial in adults with drug-resistant focal onset seizures. In this trial, ganaxolone was generally safe, well tolerated and effective in reducing seizures, however, it did not meet the primary endpoint of demonstrating a statistically significant difference from placebo. A post hoc analysis was conducted and showed a greater reduction in median seizure frequency in patients who were the most drug-resistant. We have discontinued our program in adult focal onset seizures and will focus our efforts on advancing ganaxolone in PPD, SE, and orphan, genetic disorders. The majority of our research and development expenditures to date have been in support of our adult focal onset seizure program.

Ganaxolone Mechanism of Action

Ganaxolone is a synthetic analog of a naturally occurring neurosteroid, allopregnanolone, which exhibits potent anxiolytic, antidepressant, antiepileptic and sedative activity by virtue of its $GABA_A$ receptor modulating properties. Unlike ganxolone, allopregnanolone has the potential to convert back to its metabolic precursor progesterone, which

could lead to hormonal side effects. Ganaxolone has been designed with an added methyl group that prevents back conversion to an active steroid which we believe unlocks ganaxolone's potential for chronic use. In preclinical studies, ganaxolone has exhibited potency and efficacy comparable to allopregnanolone.

GABA (gamma-aminobutyric acid) is the chief inhibitory neurotransmitter in the brain. One of the subclasses of receptors that respond to GABA is the $GABA_A$ receptor. When activated, these receptors selectively conduct chloride ions through a pore that results in the inhibitory effect of hyperpolarization of the neuron. Synaptic $GABA_A$ receptors respond quickly to inhibit neurotransmission, while extrasynaptic $GABA_A$ receptors provide ambient tonic inhibition.

Ganaxolone and allopregnanolone interact with both synaptic and extrasynaptic $GABA_A$ receptors and at binding sites distinct from the benzodiazepines. Activity with extrasynaptic $GABA_A$ receptors may be of particular importance for treating patients who developed tolerance to benzodiazepines and barbiturates. Ganaxolone binds to the $GABA_A$ receptors, which opens the pore to allow chloride ions to move into the postsynaptic neuron, leading to the inhibition of neurotransmission.

Safety Overview

IV Safety

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In 2016, we completed a Phase 1 dose-escalation study in ganaxolone IV. In this study, we achieved dose levels targeted for efficacy in patients with postpartum depression (PPD), status epilepticus (SE) and other indications. The Phase 1 clinical study enrolled 36 subjects and was designed to determine the pharmacokinetics (PK), pharmacodynamics (PD), and safety of ganaxolone IV administered as an ascending bolus dose (Stage 1) or continuous infusion (Stage 2). Four cohorts of subjects were enrolled in Stage 1 and received escalating doses of ganaxolone, and one cohort of subjects was enrolled in Stage 2. The primary study objective was to evaluate the safety and PK of ganaxolone IV. The secondary study objectives included the PD effects of ganaxolone IV on electroencephalogram (EEG) parameters, as well as the effect on clinical sedation scores.

In the study, every dose regimen of ganaxolone IV administered, either bolus or continuous infusion, was generally safe and well tolerated, and reached targeted dose levels in a short period of time. Following treatment, six treatment-emergent adverse events were reported, all of which were mild in severity and resolved without intervention. Only headache was considered possibly related to treatment with ganaxolone IV. No subject discontinued due to an adverse event and no serious adverse events were reported. Ganaxolone IV plasma concentrations were generally proportional to the administered dose. Additionally, the continuous infusion of ganaxolone IV achieved the targeted exposure levels. Plasma exposures associated with anticonvulsant and anti-anxiety activity were reached in this study.

Oral Safety

More than 1,500 subjects have received oral treatment with ganaxolone ranging in duration from one day to more than two years using doses from 50 to 2,000 mg/day. Ganaxolone was administered in Phase 2 studies to pediatric subjects at doses up to 54 mg/kg and to adult subjects 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 were not medically serious and resolved upon discontinuation of therapy. The most common side effects are related to sedation. In the 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.

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 primarily metabolized by the CYP3A family of liver enzymes, a common route of drug metabolism. All in vitro studies have shown 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 studies. We have not detected potential for

ganaxolone to cause cellular mutations or carcinogenicity in studies to date.

In reproductive toxicology studies, ganaxolone did not cause any 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 all currently marketed AEDs have shown developmental toxicities in animal studies such as fetal death or skeletal abnormalities that indicates a finding of developmental toxicities in animal studies. Valproate, carbamazepine, phenytoin and topiramate have been linked with birth defects in humans (e.g. head and facial malformations and lowered birth weight) at a rate higher than observed in women who did not take these drugs. This association has resulted in labeling for these drugs indicating positive evidence of human fetal risk based on scientific data. Based on ganaxolone's mechanism and preclinical and clinical findings to date, we intend to seek differentiated labeling for ganaxolone, indicating that animal reproduction studies have failed to demonstrate a risk to the fetus, which we believe would be an important safety differentiator for women of childbearing age.

Our Strategy

Our goal is to maximize the value of ganaxolone as a first in class innovative neuropsychiatric therapy with a portfolio of diversified indications. The key elements of our strategy to achieve this goal include the following:

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•Broadening dose forms to acute care setting. To date, our clinical trials in patients have utilized our patented nanoparticulate composition administered in oral capsule and liquid suspension dose forms. As a complement to these orally administered dose forms, we have developed an IV dose form for the acute care setting and in-patient populations that may benefit from both inpatient ganaxolone IV before transitioning to an outpatient oral dose form.

•Pursuing orphan, genetic epilepsy indications for ganaxolone. Within epilepsy, there are several smaller patient populations where a genetic marker associated with the syndrome has been linked to deficits in GABAergic signaling. We believe that increasing GABAergic tone with ganaxolone could provide benefit and that treatments for these small populations have the potential for more efficient paths to regulatory approval and commercialization. Our proof of concept open label Phase 2 clinical trial is presently enrolling patient cohorts with CDKL5 and LGS. In addition, we have completed enrolling the PCDH19-PE cohort and Phase 2 FXS trial. We may also explore development of ganaxolone in other rare genetic epilepsy indications.

•Expanding non epilepsy indications for ganaxolone. Due to its mechanism of action, we believe ganaxolone has potential for therapeutic benefit in a variety of neuropsychiatric disorders in addition to epilepsy. Evidence from preclinical and clinical studies demonstrates that treatment with an agent similar to naturally occurring allopregnanolone could be of benefit in patients with anxiety, mood, sleep and other neuropsychiatric disorders. We believe our top-line results from the Phase 2 proof of concept clinical trials in FXS patients and anecdotal reports from investigators who treated PCDH19-PE patients support this hypothesis. We plan to commence a clinical trial in PPD in 2017 and may also explore development of ganaxolone in other neuropsychiatric disorders and rare disease neurology indications.

•Build on our intellectual property. We believe that our intellectual property around nanotechnology and other formulation know how creates significant barriers to competition. We have developed most of our technology internally which provides us with greater control and flexibility and reduces expense. We intend to further expand our intellectual property portfolio through internal development and opportunistic licensing or acquisition of complementary technologies.

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 ganaxolone synthetic methods and ganaxolone nanoparticles, which are used in oral solid, oral liquid, and intravenous dose formulations, other injectable ganaxolone formulations, and methods of treatment using ganaxolone formulations. 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 basis of our intellectual property for ganaxolone nanoparticle formulations was the discovery of a novel composition of ganaxolone nanoparticles and complexing agents that deliver consistent exposure and improved stability of ganaxolone. This discovery resulted in the issuance of our United States and foreign patents, which cover ganaxolone nanoparticle formulations and the use of these formulations for treating seizure disorders. Our patent portfolio for ganaxolone nanoparticle formulations contains eight United States patents, one pending United States patent application, and corresponding foreign patents and patent applications directed to solid and liquid ganaxolone formulations and methods for the making and use thereof. These patents expire in 2026, excluding accounting for possible patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, or for possible pediatric exclusivity. Corresponding foreign patents have been granted in Australia, Canada, Eurasia, Israel, Japan, Mexico, South Africa, New Zealand, Singapore and South Korea.

Corresponding foreign patent applications are pending in China, Europe, India, Israel, Japan, and Mexico. We have not licensed any rights to practice these patents in any of these territories. Pursuant to our agreement with Domain Russia Investments Limited, or DRI, we assigned DRI patent rights, which rights were subsequently assigned to NovaMedica LLC, along with the rights to develop and commercialize ganaxolone in Russia and certain other eastern European nations.

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Our patent portfolio also contains patents issued in Australia, United States, Europe, Japan, Mexico, New Zealand, China, Hong Kong and Israel covering our novel and cost effective ganaxolone synthesis process, which expire in 2030, excluding accounting for possible patent term extension under the Hatch-Waxman Act, or for possible pediatric exclusivity. Corresponding foreign patent applications are pending in Brazil, Canada, India, and South Korea.

We filed two provisional applications in 2015 directed to intravenous ganaxolone formulations and methods of using these formulations to treat refractory epileptic seizures and other disorders. Both of these patents have been converted to US non-provisional applications with corresponding Patent Cooperation Treaty (PCT) applications. If granted, these patents will expire in 2036, excluding accounting for possible patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984 or the Hatch-Waxman Act. We filed two provisional applications in 2016 directed to additional methods of treatment using our nanoparticulate and IV formulations. If converted to non-provisional applications and subsequently granted, the patents from these applications will expire in 2037.

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 a 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 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 on patents covering those products.

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.

Collaborations

NovaMedica

In connection with our Series C convertible preferred stock financing, in December 2012 we entered into a

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Technology Transfer Agreement, or the Transfer Agreement, with DRI, a significant stockholder of our company. 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, or 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, or 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, or the Field. Immediately thereafter, we, together with DRI, executed an Assignment and Assumption Agreement, pursuant to which all of DRI's rights and obligations under the Transfer Agreement were transferred to NovaMedica, LLC, or NovaMedica. We agreed to take all action required to register or record the patent transfers to DRI in each country in the Covered Territory and to ensure the assignment of DRI's rights under the Transfer Agreement to NovaMedica. NovaMedica is jointly owned by Rusnano Medinvest LLC, or Rusnano Medinvest, and DRI. RMI Investments, S.á.r.l, a stockholder of ours, is a wholly-owned subsidiary of Rusnano Medinvest.

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 as are reasonably available to us and as are reasonable and necessary to conduct clinical trials of Covered Product in the Covered Territory, provided that any such purchase does not reasonably interfere with our having sufficient supplies of Covered Products on hand for use in development (including the conduct of clinical trials) or commercialization outside of the Covered Territory. Such purchases will be made on a cost-plus basis. The Transfer Agreement provides that the parties shall enter into the Supply Agreement to supply ganaxolone and/or Covered Product for development in the Covered Territory within 60 calendar days from NovaMedica's request, which we have not yet received.

In accordance with the terms of the Transfer Agreement, on June 25, 2013 we entered into a Clinical Development and Collaboration Agreement, or 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. All decisions of these committees must be made by unanimous vote, subject to a dispute resolution process. Under the terms of the Collaboration Agreement, the joint committees will determine a development plan for ganaxolone in clinical trials and a plan for commercialization of ganaxolone. NovaMedica will have sole responsibility for the costs and expenses of obtaining regulatory approval for Covered Products and for commercializing any approved products in the Covered Territory, and NovaMedica will have the right to conduct its own clinical studies in the Covered Territory at its sole expense. NovaMedica also has the right to file applications for approval of Covered Products in the Covered Territory, subject to committee oversight. We have agreed, among other things, to provide NovaMedica with data and regulatory files necessary for it to obtain necessary approvals in the Covered Territory, information relating to applications for regulatory approval of Covered Products, certain commercialization information and to assist NovaMedica in conducting any clinical trials necessary for regulatory approval of Covered Products in the Covered Territory. We also have agreed to provide NovaMedica with certain development know-how and support, including making our clinical development personnel available to provide scientific and technical explanations, consultation and support that may be reasonably requested by NovaMedica.

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. In the event the

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parties are unable to agree on pricing under the supply agreement, they have agreed to engage an internationally recognized consulting firm reasonably acceptable to both parties to perform an analysis to determine final pricing under the supply agreement, which decision will be binding upon the parties. In the event that the parties are unable to reach a reasonably acceptable supply agreement or we are unable to supply Covered Products to NovaMedica under such supply agreement for a period of at least 60 calendar days after the specified delivery date and we thereafter fail to cure such failure within 60 days after written notice from NovaMedica, we have agreed to cooperate with NovaMedica to identify a mutually acceptable alternative source of supply and will provide the necessary consents to allow such alternative source of supply to provide the needed quantities of the Covered Products to NovaMedica. The terms of the alternative source of supply would be negotiated directly by NovaMedica with the supplier.

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 most recently 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 our 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 epilepsy and neuropsychiatric disorders.

There are a variety of available therapies marketed for epilepsy and neuropsychiatric 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 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.

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

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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.

Competitive Landscape

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

PPD

Approximately 500,000-750,000 mothers suffer from postpartum depression annually in the US. The majority of women suffering from depression do not seek treatment. There are no approved treatments for PPD, however the most common treatments are psychotherapy and prescription antidepressants. Many women who take antidepressants discontinue them prior to and after parturition due to concern for the child. Sage Therapeutics is developing an intravenous formulation of allopregnanolone and a new orally-administered chemical entity for PPD.

SE

SE patients generally are treated with benzodiazepine as first-line treatment. When benzodiazepines are not effective, several AEDs are used. When second-line AEDs are not effective, the patient is generally placed under IV anesthesia as a last resort to attempt to stop the seizures and prevent further damage to the brain and death. Morbidity and mortality rates increase for patients that progress to SRSE. Sage Therapeutics is developing an intravenous formulation of allopregnanolone for SRSE.

CDKL5 disorder

There are no drugs approved for the treatment of CDKL5 disorder. CDKL5 disorder patients are typically prescribed drugs approved for epileptic seizures, which often fail to control seizures in this patient population. To our knowledge, there is one other company currently conducting a Phase 2 clinical trial in CDKL5 disorder patients.

PCDH19-PE

There are no drugs approved for the treatment of PCDH19-PE. PCDH19-PE patients are typically prescribed drugs approved for epileptic seizures, which often times fail to control seizures in this patient population. To our knowledge, Marinus is the only company to ever conduct a formal clinical study in PCDH19-PE, although other companies are pursuing pediatric genetic epilepsies that may include PCDH19-PE.

FXS

There are no drugs approved for the treatment of behavioral and mental health conditions associated with FXS although various classes of medications are used off-label. Some patients with FXS benefit from medications that treat attention deficient disorders and other patients who experience general anxiety, social anxiety and other chronic conditions may benefit from different types of anti-anxiety medications.

We are aware of several drugs in development including a number of generic drugs used for other indications such as donepezil, memantine, sertraline, and minocycline.

Manufacturing

Manufacturing of drugs and product candidates, including ganaxolone, must comply with FDA current good manufacturing practice, or 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, or CMOs, to supply our clinical trials. We have an internal quality program and have qualified and signed quality agreements with our major

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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 as an IV, liquid suspension and as a capsule.

Commercial Operations

If we obtain FDA approval for ganaxolone, we intend to build a sales and marketing infrastructure 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 biopharmaceutical 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, or 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. Additionally, some significant aspects of regulation in Europe are addressed in a centralized way through the 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 and may not be successful.

United States Government Regulation

The FDA is the main regulatory body that controls 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 explicitly with any 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, or 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 non-clinical, or preclinical, studies, animal studies and formulation studies in compliance with the FDA’s good laboratory practice, or GLP, regulations;

- submission to the FDA of an IND to support human clinical testing;

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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, or GCPs, to establish the safety and efficacy of the investigational product candidate for each targeted indication;

submission of a new drug application, or 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 GCPs;

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.

Clinical Trials

An IND is a request for authorization from the FDA to administer an investigational product candidate to humans. This authorization is required before interstate shipping and administration of any new drug product to humans that is not the subject of an approved NDA. A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin. The FDA may submit questions after the 30-day period and after the trial was allowed to begin. Clinical trials involve the administration of the investigational product candidate to subjects under the supervision of qualified investigators following GCPs, requirements meant to protect the rights and health of subjects and to define the roles of clinical trial sponsors, administrators and monitors. Clinical trials are conducted under protocols that detail 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. 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 studies may be conducted in subjects with the target disease or condition or healthy volunteers. These studies are designed to evaluate the safety, metabolism, pharmacokinetic properties, or 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 studies, sufficient information about the investigational product candidate's PKs and pharmacological effects may be obtained to permit the design of Phase 2 studies. The total number of participants included in Phase 1 studies varies, but is generally in the range of 20 to 80.

Phase 2. Phase 2 includes the controlled clinical trials conducted to evaluate the effectiveness of the investigational product candidate for a particular indication(s) in subjects with the disease or condition under study, to determine dosage tolerance and optimal dosage, and to identify possible adverse side effects and safety risks associated with the product candidate. Phase 2 studies 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 studies 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

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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 studies usually involve several hundred to several thousand participants. In most cases, the FDA requires two adequate and well controlled Phase 3 studies to demonstrate the efficacy of the drug. A single Phase 3 study with other confirmatory evidence may be sufficient in rare instances where the study is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible.

The decision to terminate development of an investigational product candidate may be made by either a health authority body, 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 subjects. In some cases, clinical trials are overseen by an independent group of qualified experts organized by the trial sponsor, or the clinical monitoring board or data safety monitoring board. This group provides authorization for whether or not a trial may move forward at designated check points. These decisions are based on the 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 information pertaining to the trials as well as clinical trial results after completion.

A sponsor may be able to request a special protocol assessment, or SPA, the purpose of which is to reach agreement with the FDA on the Phase 3 study protocol design and analysis that will form the primary basis of an efficacy claim. A sponsor meeting the regulatory criteria 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 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 study will ultimately be adequate to support an approval even if the study is subject to an SPA.

Although the goal of an SPA is to reach concurrence on the adequacy of protocol elements intended to support a statutory finding of safety and efficacy, an SPA agreement with FDA does not imply that FDA has reviewed or concurs with each detail of the protocol. Absence of an FDA comment on a particular aspect of the trial does not necessarily indicate agreement on that aspect if the sponsor did not specifically ask about it, especially if the context of a certain protocol element has not been highlighted or explained.

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

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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 twelve 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 this review by three months to consider certain late-submitted information or information intended to clarify information already provided in the 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 typically held for New Chemical Entities (NCEs), novel indications, or for applications in which there is a specific safety/efficacy risk that the FDA is looking for advice on. However, the FDA can decide to hold advisory committee meeting for any application. An advisory committee meeting includes a panel of FDA members and clinicians and other 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. Additionally, 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, or 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, or 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 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.

Advertising and Promotion

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. Broadly speaking, 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

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subject a company to adverse publicity and enforcement action by the FDA, the United States Department of Justice, or DOJ, or the Office of the Inspector General of the United States Department of Health and Human Services, or HHS, as well as state authorities. This 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.

Post-Approval Regulations

After regulatory approval of a drug is obtained, a company 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 company would be required to report adverse reactions and production problems to the FDA, to provide updated safety and efficacy information, and to comply with requirements concerning advertising and promotional labeling for any of its products. Also, quality control and manufacturing procedures must continue to conform to cGMP after approval to assure and preserve the long term stability of the drug product. The FDA periodically inspects manufacturing facilities to assess compliance with cGMP, which imposes extensive procedural and substantive record keeping requirements. 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 documentation requirements upon a company and any third-party manufacturers that a company may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

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, manufacturer or holder of an approved NDA, 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.

Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

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 cited by potential generic competitors in support of approval of an abbreviated new drug application, or 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 the listed drug and has been shown through PK testing to be bioequivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are generally not required to conduct, or submit results of, preclinical studies or clinical tests 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 listed drug and contains full safety and effectiveness data as an NDA, but at least some of this information comes from studies not conducted by or for the applicant. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

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The ANDA or 505(b)(2) applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA 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 rather than certify to such listed method of use patent. 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, and 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 referenced 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 marketing exclusivity expires if a Paragraph IV certification is filed. In this case, the 30 months stay, if applicable, runs from the end of the five years marketing exclusivity period. If there is no listed patent in the Orange Book, there may not be a Paragraph IV certification, and, thus, no ANDA may be filed before the expiration of the exclusivity period.

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 Europe, it may be possible to obtain a supplementary patent certificate that would effectively extend patent protection for up to five years.

The Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act, or 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

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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, or CTA, much like the IND prior to the commencement of human clinical trials. In Europe, 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.

To obtain regulatory approval to commercialize a new drug under European Union regulatory systems, we must submit a marketing authorization application, or MAA. The MAA is similar to the NDA, with the exception of, among other things, country-specific document requirements.

For other countries outside of the European Union, 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.

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.

Other Special Regulatory Procedures

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 European Union, the EMA's Committee for Orphan Medicinal Products grants Orphan Drug Designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the European Union community. Additionally, 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 European Union would be sufficient to justify the necessary investment in developing the drug.

In the United States, Orphan Drug Designation entitles a party to financial incentives, such as opportunities for grant funding towards clinical trial costs, tax credits for certain research and user fee waivers under certain circumstances. 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

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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 European Union, Orphan Drug Designation also entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity is granted following drug approval. 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.

Orphan drug designation must be requested before submission of an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of the regulatory review and approval process.

Priority Review (United States) and Accelerated Review (European Union)

Based on results of the Phase 3 study(ies) 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 filing. Priority review is given where preliminary estimates indicate that a product, if approved, has the potential to provide a safe and effective therapy where no satisfactory alternative therapy exists, or a significant improvement compared to marketed products is possible. If criteria are not met for priority review, the standard FDA review period is ten months from FDA filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the Centralized Procedure in the European Union, 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, or 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, defined by three cumulative criteria: the seriousness of the disease (e.g., heavy disabling or life-threatening diseases) to be treated; the absence or insufficiency of an appropriate alternative therapeutic approach; and anticipation of high therapeutic benefit. In this circumstance, EMA ensures that the opinion of the CHMP is given within 150 days.

Healthcare Reform

In March 2010, President Obama signed one of the most significant healthcare reform measures in decades. The Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act of 2010, or Affordable Care Act, substantially changes the way healthcare will be financed by both governmental and private

insurers, and significantly impacts the pharmaceutical industry. The Affordable Care Act will impact existing government healthcare programs and will result in the development of new programs. For example, the Affordable Care Act provides for Medicare payment for performance initiatives and improvements to the 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 23.0% and 13.0% of the average manufacturer price, or AMP, for most branded and generic drugs, respectively;

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

- a new partial prescription drug benefit for Medicare recipients, or Medicare Part D, coverage gap discount program, in which manufacturers must agree to offer 50.0% point-of-sale discounts off negotiated prices of

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applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D;

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

expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals 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 entities eligible for discounts under the Public Health Service pharmaceutical 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 and Medicaid Services, or 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 2015 (pursuant to relief enacted by the Treasury Department).

The Affordable Care Act also establishes an Independent Payment Advisory Board, or IPAB, to reduce the per capita rate of growth in Medicare spending. Beginning in 2014, IPAB is mandated to propose changes in Medicare payments if it determines that the rate of growth of Medicare expenditures exceeds target growth rates. The IPAB has broad

discretion to propose policies to reduce expenditures, which may have a negative impact on payment rates for pharmaceutical products. A proposal made by the IPAB is required to be implemented by CMS unless Congress adopts a proposal with savings greater than those proposed by the IPAB. IPAB proposals may impact payments for physician and free-standing services beginning in 2015 and for hospital services beginning in 2020.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. In August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend 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 up to 2.0% per fiscal year, starting in 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, 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 new 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

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implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products. In addition, it is possible that there will be further legislation or regulation that could harm our business, financial condition and results of operations.

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 2003, the United States Congress enacted legislation providing Medicare Part D, which became effective at the beginning of 2006. Government payment for some of the costs of prescription drugs may increase demand for any products for which we receive marketing approval. However, 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. Future legislation could limit payments for pharmaceuticals such as the product candidates that we are developing.

Different pricing and reimbursement schemes exist in other countries. In the European Union, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national 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 European Union 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.

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Other Healthcare Laws and Compliance Requirements

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted 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 exemptions and regulatory safe harbors protecting some business arrangements from prosecution, the exemptions 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 qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from federal Anti-Kickback Statute liability. The reach of the Anti-Kickback Statute was broadened by the Affordable Care Act, which, among other things, amends the intent requirement of the federal Anti-Kickback Statute. Pursuant to the statutory amendment, a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the Affordable Care Act provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act (discussed below) or the civil monetary penalties statute, which imposes penalties against any person who is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal False Claims Act prohibits any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. As a result of a modification made by the Fraud Enforcement and Recovery Act of 2009, a claim includes “any request or demand” for money or property presented to the United States government. 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. The Health Insurance Portability and Accountability Act of 1996, or HIPAA, created new federal criminal statutes that prohibit knowingly and willfully executing a scheme to defraud any healthcare benefit program, including private third-party payers and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services. Also, many states have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer.

In addition, we may be subject to data privacy and security regulation by both the federal government and the states in which we conduct our business. HIPAA, as amended by The Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, imposes requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH makes HIPAA’s privacy and security standards directly applicable to “business associates”—independent contractors or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also increased the civil and criminal penalties that may be imposed against covered entities,

business associates and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

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 Office of Inspector General), the DOJ and individual United States Attorney offices within the DOJ, and state and local governments. If a drug product is reimbursed by Medicare or Medicaid, pricing and rebate programs must comply with, as applicable, The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, as well as the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990, or the OBRA, and the Veterans Health Care Act of 1992, or the VHCA, each as amended. Among other things, the OBRA requires drug manufacturers

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to pay rebates on prescription drugs to state Medicaid programs and empowers states to negotiate rebates on pharmaceutical prices, which may result in prices for our future products that will likely be lower than the prices we might otherwise obtain. If products are made available to authorized users of the Federal Supply Schedule of the General Services Administration, additional laws and requirements apply. Under the VHCA, drug companies are required to offer some drugs at a reduced price to a number of federal agencies including the United States Department of Veterans Affairs and the DoD, the Public Health Service and some private Public Health Service designated entities in order to participate in other federal funding programs including Medicaid. Recent legislative changes require that discounted prices be offered for specified DoD purchases for its TRICARE program via a rebate system. Participation under the VHCA requires submission of pricing data and calculation of discounts and rebates pursuant to complex statutory formulas, as well as the entry into government procurement contracts governed by the Federal Acquisition Regulation.

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, private “qui tam” actions brought by individual whistleblowers in the name of the government 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. Some states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. In addition, in November 2013, the Drug Quality and Security Act became law and establishes requirements to facilitate the tracing of prescription drug products through the pharmaceutical supply distribution chain. This law includes a number of new requirements that will be implemented over time and will require us to devote additional resources to satisfy these requirements. 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.

Research and Development

Conducting research and development is central to our business model. We have invested and expect to continue to invest significant time and capital in our research and development operations. Our research and development expenses were $22.0 million, $18.9 million and $8.7 million in 2016, 2015 and 2014, respectively.

Employees

As of December 31, 2016, we had 14 full-time employees and one part time employee. 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.

Corporate Information

We were incorporated in Delaware in August 2003. Our principal executive offices are located at 170 N Radnor

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Chester Rd, Suite 250, 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.

Item 1A. Risk Factors.

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

We commenced operations in 2003 and our operations to date have been limited to conducting product development activities for ganaxolone and performing research and development with respect to our clinical and preclinical programs. In addition, as a clinical stage biopharmaceutical 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 biopharmaceutical area. Nor have we demonstrated an ability to obtain regulatory approval to commercialize any of our product candidates. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a history of successfully developing and commercializing biopharmaceutical products.

We have incurred significant operating losses since our inception, including a net loss of $28.6 million for the year ended December 31, 2016. As of December 31, 2016, we had an accumulated deficit of $125.8 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, and incur the additional costs of operating as a public company. 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 biopharmaceutical products, we are unable to predict the extent of any future losses or whether or when we will become profitable, if ever.

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.

To date, 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 from ganaxolone or any other future product candidates also depends on a number of additional factors, including our ability to:

- successfully complete development activities, including enrollment of study participants and completion of the necessary clinical trials;
- complete and submit NDAs to the FDA and obtain regulatory approval for indications for which there is a commercial market;
- complete and submit applications to, and obtain regulatory approval from, foreign regulatory authorities;
- 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;

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find suitable partners to help us market, sell and distribute our approved products in other markets; and

obtain adequate pricing, coverage and reimbursement from third parties, including government and private payers.

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 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. 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 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, pay our debt obligations on a timely basis and otherwise enhance our liquidity.

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, if approved, and commercialize ganaxolone. We will require additional capital for the further development 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, 2016, will enable us to fund our operating expenses, debt obligations and capital expenditure requirements into the second half of 2018. 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 licensing agreements;

number and characteristics of product candidates that we discover or in-license and develop;

outcome, timing and cost of regulatory review by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies 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;

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costs and timing of the implementation of commercial-scale manufacturing activities;

costs and timing of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval; and

obligations to pay our debts, including principal and interest on our bank loan, on a timely basis and the ability to otherwise pay our expenses and enhance our liquidity position.

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 or pay our debts on a timely basis 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.

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, strategic collaborations and alliances and licensing arrangements. 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, 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 strategic collaborations and alliances or licensing arrangements 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 through equity or debt financing 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 indications, 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 in this indication. 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 royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to ganaxolone.

Risks Related to Our Business and Development of Our Product

Our future success is dependent on the successful clinical development, regulatory approval and commercialization of ganaxolone, which is currently undergoing two 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. Currently, our only clinical stage product candidate is ganaxolone. As a result, our business is dependent on our ability to successfully complete clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize ganaxolone in a timely

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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, generally including two adequate and well-controlled 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. 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 study 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 approval for 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 preclinical studies or clinical trials or receive regulatory approval.

Success in preclinical studies and early clinical trials does not ensure that later 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 preclinical studies and clinical trials, even after seeing promising results in earlier studies and trials. For example, while ganaxolone showed statistical separation from placebo in a Phase 2 study in adjunctive treatment of adults with focal onset seizures, ganaxolone failed to show a similar statistical separation in a Phase 3 study for the same indication. As a result, we are discontinued our program in adult focal onset seizures and to focus our efforts on advancing ganaxolone in postpartum depression, status epilepticus, and pediatric orphan 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.

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 heavily 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 testing is expensive, can take many years to complete, and is inherently uncertain as to outcome. Failure can occur at any time during the clinical trial process.

We may experience delays in our ongoing or future clinical trials and we do not know whether planned clinical

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trials will begin or enroll subjects 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;
- delay or failure in obtaining institutional review board (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 study subjects to participate in a trial;
- delay or failure in study subjects 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;

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, IRBs, data safety monitoring boards, or a comparable foreign regulatory authority, 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, an IRB, a comparable foreign regulatory authority, or us, or recommendation by a data safety monitoring board or comparable foreign regulatory authority, 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 adverse events;

failure of a product candidate to demonstrate any benefit;

difficulties in manufacturing or obtaining from third parties sufficient quantities of a product candidate for use in clinical trials;

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- 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 license rights to develop and obtain approval for ganaxolone, in a foreign country; or

- changes in governmental regulations or administrative actions.

Study subject enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the subject population, the proximity of subjects to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, ability to obtain and maintain subject consents, risk that enrolled subjects will drop out before completion, competing clinical trials and clinicians' and subjects' 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 studies could be particularly challenging. In the past, we have experienced delays in enrolling patients in studies 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.

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.

Undesirable side effects caused by ganaxolone could cause us 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 well tolerated by subjects in our

earlier-stage clinical trials, in some cases there were side effects, and some of the side effects were severe. Specifically, in our most recently completed clinical trial, where ganaxolone was administered as an adjunctive to standard therapy in adult subjects with focal onset seizures, the most frequent side effects (those reported in greater than 5% of ganaxolone subjects) were dizziness, fatigue and somnolence (or drowsiness). More side effects of the Central Nervous System (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, which could prevent us from ever generating revenue or achieving profitability. Furthermore, although we are currently developing ganaxolone for three 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 study subject recruitment or the ability of enrolled subjects to complete our future clinical trials and may result in potential product liability claims.

Additionally, 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:

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- 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 studies;
- we could be sued and held liable for harm caused to subjects or patients; and
- our reputation may suffer.

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

Even if ganaxolone receives regulatory approval, we may 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, 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 Risk Evaluation and Mitigation Strategy (REMS) or similar strategy, impose significant restrictions on ganaxolone's indicated uses or marketing, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. For example, the label ultimately approved for ganaxolone, if it achieves marketing approval, may include restrictions on use.

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 current good manufacturing practices (cGMP) and other regulations. If we or a regulatory authority discover previously unknown problems with a product, such as adverse events 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:

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;

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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 that could prevent, limit or delay regulatory approval of ganaxolone. 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 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 any product candidate that obtains approval in the United States will be heavily scrutinized by, among others, the FDA, the Department of Justice (DOJ), the Office of Inspector General of the Department of Health and Human Services (HHS), state attorneys general, members of Congress and the public. Violations, including promotion of our products for unapproved or off-label uses, are subject to enforcement letters, inquiries and investigations, and civil and criminal sanctions by the FDA or other government agencies. Additionally, advertising and promotion of any product candidate that obtains approval 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 federal False Claims Act, 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. Since 2004, these False Claims Act lawsuits against pharmaceutical companies have increased significantly in volume and breadth, leading to several substantial civil and criminal settlements based on certain sales practices promoting off-label drug uses. This increasing focus and scrutiny has increased the risk that a pharmaceutical company will have to defend a false claim action, pay settlement fines or restitution, agree to comply with burdensome reporting and compliance obligations, and be excluded from the Medicare, Medicaid and other federal and state healthcare

programs. 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 compromise our ability to become profitable.

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 European Union 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

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commercialize our products in any market. If we are unable to obtain approval of ganaxolone by regulatory authorities in the European Union or another country or jurisdiction, the commercial prospects of ganaxolone may be significantly diminished and our business prospects could decline.

We may not be able to obtain orphan drug exclusivity for ganaxolone or any other product candidates for which we seek it, which could limit the potential profitability of ganaxolone or such other product candidates.

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 applicable regulatory authority 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 for treating PCDH19-PE, FXS and SE 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 or any future product candidates. Even if we were to obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product from the competition of different drugs for the same condition, which could be approved during the exclusivity period. Additionally, 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 an orphan drug designation for any product candidates we may develop, the inability to maintain that designation for the duration of the applicable period, or the inability to obtain or maintain orphan drug exclusivity could reduce our ability to make sufficient sales of the applicable product candidate to balance our expenses incurred to develop it, which would have a negative impact on our operational results and financial condition.

Our business and operations would suffer in the event of computer system failures.

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. In addition, our systems safeguard important confidential personal data regarding subjects 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, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of ganaxolone could be delayed.

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

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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. 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.

Risks Related to the Commercialization of Our Product

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

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 the:

- 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;

product labeling or product insert requirements of the FDA or other 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; and

effectiveness of our sales and marketing efforts.

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.

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 any products that may be approved by the FDA and 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

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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.

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;
- 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.

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

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

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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.

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 primarily as a neuropsychiatric therapeutic. There are a variety of marketed therapies available for these patients.

Specifically, there are more than 15 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, and Sunovion Pharmaceuticals. Additionally, there are several drugs in development for the treatment of pediatric genetic epilepsies and behavioral and mental health conditions associated with FXS, including compounds being developed by GW Pharmaceuticals, Zogenix, Sunovion Pharmaceuticals, Zynerba, Alcobra and Neuren Pharmaceuticals. Sage Therapeutics is developing molecules with a similar mechanism of action as ganaxolone for treatment of SE and PPD.

Many of the approved drugs are well established therapies or products and are widely accepted by physicians,

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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 drugs 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.

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 study 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 or entire trial programs;

injury to our reputation and significant negative media attention;

withdrawal of clinical trial subjects;

significant costs to defend the related litigation;

substantial monetary awards to clinical trial subjects or 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 Office of Inspector General of the HHS, state attorneys general, members of Congress and the public.

We currently have $10.0 million in product liability insurance coverage in the aggregate, which may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to

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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 decrease our cash and adversely affect our business.

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 contractual duties or meet expected deadlines, 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 rely on these parties for execution of our preclinical studies and clinical trials, and we control only some aspects of their activities. Nevertheless, we are responsible for ensuring that each of our preclinical studies and clinical trials are conducted in accordance with the applicable protocol and legal, regulatory and scientific requirements and standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We also rely on third parties to assist in conducting our preclinical studies in accordance with Good Laboratory Practices (GLP) and the Animal Welfare Act requirements. We and our CROs are required to comply with federal regulations and Good Clinical Practices (GCP), which are international requirements meant to protect the rights and health of subjects that are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for ganaxolone and any future product candidates in clinical development. 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 preclinical studies and clinical trials, which would delay the regulatory approval process.

Our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing 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.

Because we have relied on third parties, our internal capacity to perform these functions is limited. Outsourcing these functions involves risk that third parties may not perform to our standards, may not produce results in a timely manner or may fail to perform at all. 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 identify and monitor our third-party providers. To the extent we are unable to identify and successfully manage the performance of third-party service providers in the future, 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.

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If we lose our relationships with CROs, our drug development efforts could be delayed.

We rely on third-party vendors and CROs for preclinical studies and clinical trials related to our drug development efforts. Switching or adding additional CROs would involve additional cost and requires management time and focus. Our CROs generally have the right to terminate their agreements with us in the event of an uncured material breach. In addition, some of our CROs have an ability to terminate their respective agreements with us, or research projects pursuant to such agreements, if, in the reasonable opinion of the relevant CRO, the safety of the subjects participating in our clinical trials warrants such termination. These agreements or research projects may also be terminated if we make a general assignment for the benefit of our creditors or if we are liquidated. 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 and the new CRO may not provide the same type or level of services as the original provider. 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.

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 contract manufacturing organizations (CMOs) for the chemical manufacture of active pharmaceutical ingredients for ganaxolone and other CMOs for the production of the ganaxolone nanoparticulate formulation into capsules, liquid suspension and IV. 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 or any products we may eventually commercialize in accordance with our specifications, and the possibility of termination or nonrenewal of the 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 would require that ganaxolone and any products that we may eventually commercialize 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 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 materials for an ongoing preclinical study or clinical trial could considerably delay completion of our preclinical study or clinical trial, product testing and potential regulatory approval of ganaxolone. If our manufacturers or we are unable to purchase these key 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.

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We may elect to enter into licensing 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 may seek to enter into 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 collaboration agreements will terminate the funding we may receive under the relevant 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 collaborators 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 additional collaborative licensing or other arrangements that are necessary for us to further develop and commercialize ganaxolone. Supporting diligence activities conducted by potential collaborators and negotiating the financial and other terms of a collaboration agreement are long and complex processes with uncertain results. Even if we are successful in entering into one or more collaboration agreements, collaborations may involve greater uncertainty for us, as we have less control over certain aspects of our collaborative programs than we do over our proprietary development and commercialization programs. We may determine that continuing a collaboration under the terms provided is not in our best interest, and we may terminate the collaboration. Our potential future collaborators could delay or terminate their agreements, and as a result ganaxolone may never be successfully commercialized.

Further, our potential future collaborators 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 collaborators 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 collaborators may adversely affect our business prospects and ability to earn revenue. In addition, we could have disputes with our potential future collaborators, 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.

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.

Our preclinical studies and clinical trials to evaluate ganaxolone in FXS patients have been conducted with the MIND Institute at the University of California, Davis which receives funding from the United States Department of Defense (DoD) for such studies and trials. In addition, our preclinical studies and clinical trials to evaluate ganaxolone in patients suffering from posttraumatic stress disorder (PTSD) have been primarily conducted by the United States Department of Veterans Affairs, which also receives funding from the DoD. Programs funded by the United States government and its agencies, including the DoD, 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;

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- 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.

Changes in government budgets and agendas may result in a decreased and de-prioritized emphasis on supporting the development of ganaxolone in patients suffering from certain FXS-associated behavioral symptoms. Although we intend to fund a portion of our development programs for ganaxolone in patients with FXS, any reduction or delay in DoD funding to our collaborators may force us to suspend or terminate these programs or seek alternative funding, which may not be available on non-dilutive terms, terms favorable to us or at all.

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,

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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, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly 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. The Centers for Medicare and Medicaid Services, the agency that runs the Medicare program, also has the authority to revise reimbursement rates and to implement coverage restrictions for some 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. While the Medicare Modernization Act and Medicare regulations apply only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from federal legislation or regulation may result in a similar reduction in payments from private payers.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act and the Health Care and Education Affordability Reconciliation Act of 2010 (Affordable Care Act) a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on pharmaceutical and medical device manufacturers and impose additional health policy reforms. 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 imposes a significant annual, nondeductible fee on companies that manufacture or import certain branded prescription drug products. Substantial new provisions affecting compliance have also been enacted, which may affect our business practices with healthcare practitioners, and a significant number of provisions

are not yet, or have only recently become, effective. Although it is too early to determine the effect of the Affordable Care Act, it appears likely to continue the pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. More recently, in August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, creates 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 up to 2.0% per fiscal year, starting in 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, 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 new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly,

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our financial operations. 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 European Union 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 European Union 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.

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.

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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 third-party payers will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payers and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may affect the business or financial arrangements and relationships through which we would market, sell and distribute our products. Even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payers, federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. Restrictions under applicable federal and state healthcare laws and regulations that may affect our operations (including our marketing, promotion, educational programs, pricing, and relationships with healthcare providers or other entities, among other things) and expose us to areas of risk including the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing 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, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

- federal civil and criminal false claims laws and civil monetary penalty laws impose criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA) imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also created federal criminal laws that prohibit 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;

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (HITECH) also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

the Affordable Care Act requires manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or Children's Health Insurance Program, to report annually to HHS information related to payments and other transfers of value to physicians and teaching hospitals, and ownership and investment interests held by physicians and their immediate family members and applicable group purchasing organizations; and

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

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 and 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.

Risks Related to Our 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, we do not know whether any of our granted or issued patents will effectively prevent others from commercializing competitive technologies and 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

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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 ganaxolone, 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 ganaxolone, 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 ganaxolone. 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 ganaxolone. 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 ganaxolone 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 ganaxolone is in preclinical studies and clinical trials, we believe that the use of ganaxolone 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. As ganaxolone progresses toward commercialization, the possibility of a patent infringement claim against us increases. While ganaxolone itself is off patent, we attempt to ensure that our solid and

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liquid nanoparticulate formulation of ganaxolone 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.

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

Filing, prosecuting and defending patents on ganaxolone 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 biopharmaceuticals, do not protect intellectual property rights to the same extent as federal and state laws in the United States. For example, novel formulations 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 European Union 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 ganaxolone, patents protecting such candidates might expire before or shortly after such candidates are commercialized. 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 a patent term extension of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension). However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent 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.

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Changes in patent laws, including 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.

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 September 2011, the Leahy-Smith America Invents Act (Leahy-Smith Act) was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the United States transitioned in March 2013 to a “first to file” system in which the first inventor to file a patent application will be 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 opposition, derivation, reexamination, inter-parties review or interference 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

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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.

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 ganaxolone formulations 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 Employee Matters and Managing Growth

Our future success depends on our ability to retain our executive officers and to attract, retain and motivate qualified personnel.

We are highly dependent upon Christopher M. Cashman, our Chief Executive Officer, and Edward F. Smith, our Chief Financial Officer. The employment agreements we have with the persons named above do not prevent such persons from terminating their employment with us at any time. We do not maintain “key person” insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

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

As of December 31, 2016, we had 14 full-time and one 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

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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.

If we are unable to attract and retain highly qualified employees, and other personnel, advisors and consultants with scientific, technical and managerial expertise, we may not be able to grow effectively.

Our future growth and success depend on our ability to recruit, retain, manage and motivate our employees, consultants and other third-parties. The loss of any member of our senior management team or the inability to hire or retain experienced management personnel could compromise our ability to execute our business plan and harm our operating results.

Because of the specialized scientific and managerial nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel, advisors and consultants. The competition for qualified personnel in the pharmaceutical field is significant and, as a result, we may be unable to continue to attract

and retain qualified personnel necessary for the development of our business.

We may acquire other assets or businesses, or form collaborations or make investments in other companies or technologies that could harm our operating results, dilute our stockholders' ownership, increase our debt or cause us to incur significant expense.

As part of our business strategy, we may pursue acquisitions of assets, including preclinical, clinical or commercial stage products or product candidates, or businesses, or strategic alliances and collaborations, to expand our existing technologies and operations. We may not identify or complete these transactions in a timely manner, on a cost-effective basis, or at all, and we may not realize the anticipated benefits of any such transaction, any of which could have a detrimental effect on our financial condition, results of operations and cash flows. We have no experience with acquiring other companies, products or product candidates, and limited experience with forming strategic alliances and collaborations. We may not be able to find suitable acquisition candidates, and if we make any acquisitions, we may not be able to integrate these acquisitions successfully into our existing business and we may incur additional debt or assume unknown or contingent liabilities in connection therewith. Integration of an acquired company or assets may also disrupt ongoing operations, require the hiring of additional personnel and the implementation of additional internal systems and infrastructure, especially the acquisition of commercial assets, and require management resources that would otherwise focus on developing our existing business. We may not be able to find suitable strategic alliance or collaboration partners or identify other investment opportunities, and we may experience losses related to any such investments.

To finance any acquisitions or collaborations, we may choose to issue debt or equity securities as consideration. Any such issuance of shares would dilute the ownership of our stockholders. If the price of our common stock is low or volatile, we may not be able to acquire other assets or companies or fund a transaction using our stock as consideration.

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Alternatively, it may be necessary for us to raise additional funds for acquisitions through public or private financings. Additional funds may not be available on terms that are favorable to us, or at all.

Our employees may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk of employee fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards we have established, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of conduct for our directors, officers and employees (Code of Conduct) but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

Risks Related to Ownership of Our Common Stock

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

The trading price of our common stock is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. In addition to the factors discussed in this “Risk Factors” section, these factors 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;

results of clinical trials of ganaxolone or product candidates of our competitors;

regulatory or legal developments in the United States and other countries;

developments or disputes concerning patent applications, issued patents or other proprietary rights;

the recruitment or departure of key personnel;

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;

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- 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;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; 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.

We may be subject to securities litigation, which is expensive and could divert our management’s attention.

The market price of our common stock may be volatile, and in the past companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management’s attention from other business concerns, which could seriously harm our business.

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

We estimate that our executive officers, directors, and holders of 5% or more of our capital stock collectively beneficially own approximately 39% of our voting stock. This concentration of ownership could harm the market price of our common stock by:

- delaying, deferring or preventing a change in control of our company;
- impeding a merger, consolidation, takeover or other business combination involving our company; or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of our company.

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.

We are an “emerging growth company” and we intend to take advantage of reduced disclosure and governance requirements applicable to emerging growth companies, which could result in our common stock being less attractive to investors.

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (JOBS Act). For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various

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reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years following the year in which we completed our initial public offering, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700.0 million as of any June 30 before that time or if we have total annual gross revenue of $1.0 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31. If we issue more than $1.0 billion in non-convertible debt during any three-year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a “smaller reporting company” which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, our ability to pay cash dividends is prohibited by our credit facility with Square 1 Bank, as amended, and the terms of any future debt agreements may also preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

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 December 31, 2016 we had outstanding a total of 19,705,120 shares of common stock. In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules and either the registration of such shares under the Securities Act or the application of exemptions from such registration with respect to any sales such as Rule 144 and Rule 701 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.

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

We expect that significant additional capital will be needed in the future to continue our planned operations. To raise capital, we may sell substantial amounts of common stock or securities convertible into or exchangeable for common stock. These future issuances of equity or equity-linked securities, together with the exercise of stock options, warrants outstanding or granted in the future and any additional shares issued in connection with acquisitions, if any,

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may result in material dilution to our investors. Such sales may also result in material dilution to our stockholders, and new investors could gain rights, preferences and privileges senior to those of holders of our common stock.

Pursuant to our equity incentive plans, our compensation committee is authorized to grant equity-based incentive awards to our directors, executive officers and other employees and service providers. As of December 31, 2016, there were no shares of our common stock available for future grant under our 2005 Stock Option and Incentive Plan, as amended. The number of shares of our common stock available for future grant under our 2014 Equity Incentive Plan was 31,922 as of December 31, 2016. In accordance with the 2014 Plan, on January 1, 2017, 820,127 shares of common stock were available for future grant under the plan. Future equity incentive grants and issuances of common stock under our equity incentive plans may have an adverse effect on the market price of our common stock.

We have broad discretion in the use of our cash reserves and may not use them effectively.

Our management has broad discretion over the management of our operations and cash resources and could deploy our resources in ways that do not improve our business, including our ganaxolone clinical development programs, or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could have a material adverse effect on our business, cause the market price of our common stock to decline and delay the development of ganaxolone.

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 amended and restated 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;

- 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

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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, which may discourage, delay or prevent someone from acquiring us or merging with us whether or not it is desired by or beneficial to our stockholders. Under Delaware law, a corporation may not, in general, engage in a business combination with any holder of 15.0% or more of its capital stock unless the holder has held the stock for three years or, among other things, the board of directors has approved the transaction. Any provision of our amended and restated certificate of incorporation or amended and restated bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

We are required to meet the NASDAQ Stock Market's continued listing requirements and other NASDAQ rules, or we may risk delisting. Delisting could negatively affect the price of our common stock, which could make it more difficult for us to sell securities in a future financing or for you to sell our common stock.

We are required to meet the continued listing requirements of the NASDAQ Stock Market and other NASDAQ rules, including those regarding director independence and independent committee requirements, minimum stockholders' equity, minimum share price and certain other corporate governance requirements. In particular, we are required to maintain a minimum bid price for our listed common stock of $1.00 per share. If we do not meet these continued listing requirements, our common stock could be delisted. Delisting from the NASDAQ Stock Market would cause us to pursue eligibility for trading of these securities on other markets or exchanges, or on the "pink sheets." In such case, our stockholders' ability to trade, or obtain quotations of the market value of our common stock would be severely limited because of lower trading volumes and transaction delays. These factors could contribute to lower prices and larger spreads in the bid and ask prices of these securities. There can be no assurance that our securities, if delisted from the NASDAQ Stock Market in the future, would be listed on a national securities exchange, a national quotation service, the over-the-counter markets or the pink sheets. Delisting from the NASDAQ Stock Market, or even the issuance of a notice of potential delisting, would also result in negative publicity, make it more difficult for us to raise additional capital, cause us to lose eligibility to register the sale or resale of our shares on Form S-3 and the automatic exemption from registration under state securities laws for exchange-listed securities, adversely affect the market liquidity of our securities, decrease securities analysts' coverage of us or diminish investor, supplier and employee confidence.

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

Our principal offices occupy approximately 8,500 square feet of leased office space in Radnor, Pennsylvania pursuant to a lease agreement that expires in 2021. We believe that our current 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.

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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. The following table sets forth for the indicated periods the high and low intra-day sales prices per share for our common stock on the NASDAQ Global Market.

	Year Ended December 31, 2016	
	High	Low
First Quarter	$ 7.56	$ 4.00
Second Quarter	$ 6.76	$ 1.19
Third Quarter	$ 2.73	$ 1.23
Fourth Quarter	$ 1.84	$ 0.82

	Year Ended December 31, 2015	
	High	Low
First Quarter	$ 16.60	$ 8.78
Second Quarter	$ 13.72	$ 7.00
Third Quarter	$ 20.72	$ 7.67
Fourth Quarter	$ 10.24	$ 4.52

Holders of Record

As of March 7, 2016 there were approximately 34 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.

Dividend Policy

We have never declared or paid any cash dividends on our common stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. We do not intend to pay cash dividends on our common stock for the foreseeable future. Additionally, our ability to pay cash dividends is prohibited by our credit facility with Square 1 Bank, as amended.

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Stock Performance Graph

The following graph compares the annual percentage change in the cumulative total shareholder return on the Company's common stock with the cumulative total return of (i) the NASDAQ Composite Total Return Index and (ii) the NASDAQ US Benchmark Pharmaceuticals Index. The comparison assumes $100 was invested on July 31, 2014 (the first day the Company's stock was traded on the NASDAQ Global Market) in the Company's common stock and in each of the following indices and assumes the reinvestment of dividends.

	7/31/2014	12/31/2014	12/31/2015	12/31/2016
Marinus Pharmaceuticals	$ 100	$ 132.13	$ 95.50	$ 12.63
NASDAQ Composite Total Return Index	$ 100	$ 108.96	$ 116.54	$ 126.88
NASDAQ US Benchmark Pharmaceuticals Index	$ 100	$ 111.80	$ 117.87	$ 116.59

This performance graph shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or incorporated by reference into any filing of ours under the Securities Act of 1933, as amended, or the Securities Exchange Act, except as shall be expressly set forth by specific reference to such filing.

Recent Sales of Unregistered Securities

None.

Use of Proceeds

Our initial public offering (IPO) of common stock was effected pursuant to a registration statement on Form S-1 (File No 333-195895) that was declared effective by the SEC on July 31, 2014, pursuant to which we registered the offering and sale of 6,468,750 shares of common stock, $0.001 par value per share (including 843,750 shares available to the underwriters' for exercise of an option to purchase additional shares, of which 133,000 shares were exercised in September 2014) at a public offering price of $8.00 per share for an aggregate public offering price of $46.1 million.

As a result of the initial public offering, we received net proceeds of approximately $41.2 million during the third

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quarter of 2014 from the sale of 5,758,000 shares of common stock, after deducting underwriting discounts, commissions and estimated offering expenses borne by us. Other than pursuant to standard compensation arrangements, none of such payments were direct or indirect payments to any of (i) our directors or officers or their associates, (ii) persons owning 10% or more of our common stock, or (iii) our affiliates.

There has been no material change in use of proceeds from our initial public offering from that described in the final prospectus related to the offering, which we filed with the SEC on August 1, 2014. As of December 31, 2016, we have used approximately $30.0 million of the funds received from our IPO in support of our clinical trials and payments to research and development consultants, including compensation of certain employees and officers, and approximately $11.2 million for general corporate purposes, including compensation of certain employees, officers, and directors.

Item 6. Selected Financial Data

The following selected financial data as of and for the years ended December 31, 2016, 2015 and 2014 are derived from our audited financial statements included elsewhere in this Annual Report on Form 10-K. The following selected financial data as of and for the years ended December 31, 2013 and 2012 have been derived from our audited financial statements not included in this report. The information set forth in the following table should be read in conjunction with our financial statements and related notes thereto included elsewhere in this Annual Report on Form 10-K.

	Year Ended December 31,				
	2016	2015	2014	2013	2012
	(in thousands, except share and per share amounts)				
Statement of Operations Data:					
Revenue	$ —	$ —	$ —	$ —	$ 100
Operating expenses:					
Research and development	22,005	18,916	8,690	4,150	846
General and administrative	6,237	5,516	3,230	1,229	685
Loss from operations	(28,242)	(24,432)	(11,920)	(5,379)	(1,431)
Other (expense) income, net	(401)	(418)	1,087	109	23
Net loss	$ (28,643)	$ (24,850)	$ (10,833)	$ (5,270)	$ (1,408)
Cumulative preferred stock dividends	—	—	(2,545)	(3,804)	(2,186)
Net loss available to common stockholders	$ (28,643)	$ (24,850)	$ (13,378)	$ (9,074)	$ (3,594)
Per share information:					
Net loss per share of common stock—basic and diluted	$ (1.47)	$ (1.67)	$ (2.17)	$ (19.60)	$ (8.00)
Basic and diluted weighted average shares outstanding	19,498,143	14,919,783	6,152,669	462,972	449,514

	As of December 31,				
	2016	2015	2014	2013	2012
Balance Sheet Data:	(in thousands)				
Cash, cash equivalents and investments	$ 30,100	$ 57,684	$ 49,720	$ 10,037	$ 8,634
Total assets	31,447	59,648	50,213	11,824	8,682
Current portion of notes payable	3,500	2,128	—	—	—
Notes payable	1,743	5,236	7,000	—	—
Total convertible preferred stock	—	—	—	69,840	59,549
Total stockholders' equity (deficit)	21,479	46,921	41,154	(60,382)	(55,367)

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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 biopharmaceutical company focused on developing and commercializing innovative therapeutics to treat epilepsy and neuropsychiatric disorders. Our clinical stage product candidate, ganaxolone, is a positive allosteric modulator of $GABA_A$ being developed in three different dose forms: intravenous (IV), capsule and liquid. The multiple dose forms are intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Ganaxolone acts on a well-characterized synaptic and extrasynaptic $GABA_A$ target known for both anti seizure and anti anxiety activity.

Our operations to date have consisted primarily of organizing and staffing our company, developing ganaxolone, including conducting preclinical testing and clinical trials, and raising capital. We have funded our operations primarily through sales of equity and debt securities. At December 31, 2016, we had cash, cash equivalents and investment balances of $30.1 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 $28.6 million for the year ended December 31, 2016. Our accumulated deficit as of December 31, 2016 was $125.8 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 post-partum depression (PPD), status epilepticus (SE), CDKL5 disorder (CDKL5), Lennox-Gastaut Syndrome (LGS), PCDH19 pediatric epilepsy (PCDH19-PE), and Fragile X Syndrome (FXS);

continue the research, development and scale-up manufacturing capabilities to optimize products and dose forms for which we may obtain regulatory approval;

conduct other preclinical and clinical studies to support the filing of New Drug Applications (NDAs) with the Food and Drug Administration (FDA) and other regulatory agencies in other countries;

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 and potential future commercialization efforts.

We believe that our cash, cash equivalents and investments as of December 31, 2016 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2018. However, we will need to secure additional funding in the future, from one or more equity or debt financings, collaborations, or other sources, in order to carry out all of our planned research and development activities with respect to ganaxolone.

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Financial Overview

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 subject enrollment, clinical site activations or 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) for ganaxolone for various clinical indications, and in each case, the nature, design, size and cost of further studies and trials 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 ganaxolone will depend on numerous factors, including competition, manufacturing capability and commercial viability. See “Risk Factors.” Our commercial success depends upon attaining significant market acceptance of ganaxolone, 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 of ganaxolone, as well as an assessment of ganaxolone’s 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 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, legal counsel and accountants, among other expenses.

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Change in Fair Value of Warrant Liability

Our previously outstanding warrants to purchase preferred stock were classified as warrant liability and recorded at fair value. This warrant liability was subject to re-measurement at each balance sheet date and we recognized any change in fair value in our statements of operations as a change in fair value of the derivative liability. These warrants expired upon our initial public offering and, as a result, the fair value of the warrants was reduced to zero during the third quarter of 2014.

Interest Income

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

Interest Expense

Interest expense is attributable to interest expense associated with our credit facility entered into in April 2014, and amended.

Cumulative Preferred Stock Dividends

Cumulative preferred stock dividends represented dividends payable upon a liquidation or deemed liquidation in connection with our Series B and C convertible preferred stock. Effective upon the closing of our initial public offering, all outstanding shares of preferred stock converted into common stock during the third quarter of 2014.

Results of Operations

Year Ended December 31, 2016 compared to year ended December 31, 2015

Research and Development Expenses

Research and development expenses increased $3.1 million, to $22.0 million, for the year ended December 31, 2016, compared to the same period of 2015. The increase was primarily due to an increase of $2.7 million associated with preclinical and clinical activities in our IV program, and $1.4 million associated with increases in salaries, benefits and noncash stock-based compensation, mostly attributable to increased headcount. This increase was partially offset by a decrease of $1.3 million in costs associated with our drug-resistant focal onset seizure program, which discontinued in June 2016.

The primary drivers of our research and development expenditures have been in our drug-resistant focal onset seizures and IV programs. We incurred $12.8 million and $3.3 million in research and development expenses related to our preclinical and clinical activities associated with our drug-resistant focal onset seizures and IV programs, respectively,

in the year ended December 31, 2016, compared to $14.1 million and $0.6 million for the year ended December 31, 2015.

General and Administrative Expenses

General and administrative expenses increased $0.7 million, to $6.2 million, for the year ended December 31, 2016, compared to the same period of 2015. The increase in general and administrative expenses was primarily due to an increase in noncash stock-based compensation expense.

Cash Flows

Operating Activities. Cash used in operating activities increased to $24.8 million for the year ended December 31, 2016 compared to $20.1 million for the same period a year ago. The increase was driven primarily by an increase in our net loss of $3.8 million and a net decrease in the change in operating assets and liabilities of $1.8 million,

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partially offset by an increase in stock-based compensation expense of $1.0 million. The net decrease in the change in operating assets and liabilities was due primarily to upfront payment obligations related to certain drug manufacturing contracts and deposits on laboratory equipment in 2015.

Investing Activities. Cash provided by investing activities during the year ended December 31, 2016 represents the purchase of $2.4 million in investments and $0.6 million in laboratory equipment, offset by maturities of short-term investments of $4.4 million. Cash used in investing activities during the year ended December 31, 2015 represents the purchase of $7.7 million in investments and a deposit of $0.4 million on laboratory equipment, offset by maturities of short-term investments of $1.7 million.

Financing Activities. Cash used in financing activities during the year ended December 31, 2016 represents principal payments of notes payable of $2.1 million and payment of public offering costs of $0.2 million, offset by proceeds from the exercise of outstanding stock options of $0.1 million. Cash provided by financing activities during the year ended December 31, 2015 represents $28.3 million in net proceeds received from a secondary public offering, as well as $0.4 million from the exercise of outstanding stock options, offset by $0.2 million in installment payments made to a third-party vendor for financed insurance premiums.

Year ended December 31, 2015 compared to year ended December 31, 2014

Research and Development Expenses

Research and development expenses increased $10.2 million, to $18.9 million, for the year ended December 31, 2015, compared to the same period of 2014. The increase resulted primarily from an increase in clinical, drug development and consulting costs related to our ongoing clinical trials for ganaxolone, as well as increases in compensation-related costs due to hiring additional clinical resources, including our Chief Medical Officer, who was hired in December 2014. Most of our research and development expenses relate to our ongoing Phase 3 clinical trial of ganaxolone in adults with refractory focal onset epileptic seizures.

General and Administrative Expenses

General and administrative expenses increased $2.3 million, to $5.5 million, for the year ended December 31, 2015, compared to the same period of 2014. The increase in general and administrative expenses was primarily due to the hiring of new management and the upward scaling of our operations in connection with both our public company status as of July 31, 2014 and our ongoing Phase 3 clinical trial of ganaxolone in adults with refractory focal onset epileptic seizures.

Change in Fair Value of Warrant Liability

We recorded changes in the fair value of our warrant liability which resulted in a gain of $1.2 million for the year ended December 31, 2014. We reduced the value of the liability to zero in connection with the closing of our initial public offering in the third quarter of 2014 as the warrants expired unexercised.

Cumulative Preferred Stock Dividends

Cumulative preferred stock dividends were $2.5 million for the year ended December 31, 2014. Upon conversion of all outstanding convertible preferred stock in connection with our initial public offering during the third quarter of 2014, all cumulative preferred stock dividends were canceled.

Cash Flows

Operating Activities. Cash used in operating activities increased to $20.1 million for the year ended December 31, 2015 compared to $8.6 million for the same period a year ago. The increase was driven primarily by an increase in our net loss of $14.0 million, partially offset by an increase in stock-based compensation expense of $1.4 million and reduction in the change in the fair value of our warrant liability of $1.2 million. Additionally, we had an

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increase in net use of cash related to the changes in operating assets and liabilities of $0.1 million. This increase in net use of cash primarily was due to upfront payment obligations related to certain drug manufacturing contracts in 2015 and increases in our trade accounts payable due to increased operating expenses. The increase in net loss was primarily driven by increases in our operating expenses due to the upward scaling of our operations related to our ongoing clinical trials for ganaxolone.

Investing Activities. Cash used in investing activities represents the purchase of $7.7 million in investments and $0.4 million in deposits on clinical research equipment during the year ended December 31, 2015, partially offset by maturities of short-term investments of $1.7 million. During 2014, we had a minimal purchase of office furniture.

Financing Activities. Cash provided by financing activities was $28.4 million for the year ended December 31, 2015 due primarily to $28.3 million in net proceeds received from our secondary public offering, as well as from the exercise of outstanding stock options offset by installment payments made to a third-party vendor for financed insurance premiums. Cash provided by financing activities for the year ended December 31, 2014 of $48.3 million was primarily due to $41.2 million received in connection with our initial public offering, and $7.0 million received in connection with our credit facility.

Liquidity and Capital Resources

Since inception, we have incurred net losses and negative cash flows from our operations. We incurred a net loss of $28.6 million for the year ended December 31, 2016. Our cash used in operating activities was $24.8 million for year ended December 31, 2016 compared to $20.1 million for the same period a year ago. Historically, we have financed our operations principally through the sale of common stock, preferred stock and convertible debt. At December 31, 2016, we had cash, cash equivalents and investment balances of $30.1 million.

Credit Facility

In April 2014, we borrowed $2.0 million pursuant to a Loan and Security Agreement (LSA) we entered into with a financial institution. Pursuant to the terms of the LSA, we made monthly interest-only payments for outstanding borrowings at an interest rate equal to the greater of (a) prime plus 2.25% or (b) 5.5% until the LSA was amended. In December 2014, February 2015, October 2015 and April 2016 we entered into four amendments to the LSA (collectively, the “Amended LSA”) with the same financial institution.

In connection with the Amended LSA, we borrowed an additional $5.0 million in December 2014. Pursuant to the terms of the Amended LSA, we were required to make monthly interest-only payments for all outstanding borrowings at an interest rate equal to the greater of (a) prime rate plus 3.25% or (b) 6.5% until June 2016. Commencing in July 2016 and continuing through June 2018, we are required to make monthly payments of 1/24th of our principal

borrowings plus interest.

As of December 31, 2016, of our outstanding term loan balance of $5.3 million, $3.5 million will be due within the next twelve months, and is classified as the current portion of notes payable on our balance sheet. Interest expense related to the term loans was $459 thousand and $461 thousand for the years ended December 31, 2016 and 2015, respectively. As of December 31, 2016, we had accrued interest of $31 thousand. There are no financial covenants associated with these term loans. As of December 31, 2016, we were in compliance with all non-financial covenants.

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 planned clinical trials for ganaxolone.

We believe that our cash, cash equivalents and investments as of December 31, 2016, will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2018. However, we will need to raise substantial additional financing in the future to fund our operations. In order to meet these additional cash requirements,

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we may seek to sell additional equity or convertible debt securities, including securities from our Equity Distribution Agreement with JMP 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. 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 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 ganaxolone or any other future product candidates in preclinical studies, clinical trials and, if approved, in commercial sale;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;
- any product liability, infringement or other lawsuits related to our products;
- the expenses needed to attract and retain skilled personnel;
- the costs associated with being a public company;
- 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.

Significant Contractual Obligations and Commitments

The following summarizes our significant contractual obligations as of December 31, 2016 (in thousands):

	Payment due by period				
Contractual Obligations	Total	Less than 1 year	1 - 3 years	3 - 5 years	More than 5 years
Operating lease obligations(1)	$ 1,444	$ 333	$ 642	$ 469	$ —
Credit facilities(2)	5,534	3,749	1,785	—	—
Total	$ 6,978	$ 4,082	$ 2,427	$ 469	$ —

(1) Represents commitments for future minimum lease payments.

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(2) Represents principal and interest payment obligations that will become due in connection with our outstanding loan facility.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements, as defined by applicable SEC regulations.

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 warrant liabilities, stock-based compensation and 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.

Stock-Based Compensation

We recognize compensation expense related to the fair value of stock-based awards in our statements of operations. For stock options we issued to employees, consultants, 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 awards subject to time-based vesting, we recognize stock-based compensation expense, net of estimated forfeitures, on a straight-line basis over the requisite service period, which is generally the vesting term of the award. For awards subject to performance-based vesting conditions, we recognize stock-based compensation expense when it is probable

that the performance condition will be achieved. We are required to estimate forfeitures at the time of grant and to revise the estimates, if necessary, in subsequent periods if actual forfeitures differ from those estimates. We record stock-based awards issued to non-employees at their fair values, and periodically revalue them as the equity instruments vest and are recognized as expense over the related service period of the award.

Clinical Trial Expense Accruals

As part of the process of preparing our financial statements, we are required to estimate our accrued 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 as of each balance sheet date in our financial statements based on

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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.

JOBS Act

Section 107 of the JOBS Act also provides that an “emerging growth company” can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act for complying with new or revised accounting standards. In other words, an “emerging growth company” can delay the adoption of new or revised accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Recent Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2014-15, Disclosure of Uncertainties About an Entity’s Ability to Continue as a Going Concern, which provides guidance on determining when and how to disclose going-concern uncertainties in the financial statements. The new standard requires management to perform interim and annual assessments of an entity’s ability to continue as a going concern within one year of the date the financial statements are issued. An entity must provide certain disclosures if conditions or events raise substantial doubt about the entity’s ability to continue as a going concern. The ASU applies to all entities and was effective for annual periods ending after December 15, 2016, and interim periods thereafter, with early adoption permitted. We adopted this ASU for the year ended December 31, 2016, and the adoption of this ASU had no material effect on our annual financial statements.

In April 2015, the FASB issued ASU 2015-03, Simplifying the Presentation of Debt Issuance Costs, which changes the presentation of debt issuance costs in financial statements. Under the ASU, an entity presents such costs in the balance sheet as a direct deduction from the related debt liability rather than as an asset. Amortization of the costs is reported as interest expense. We adopted this ASU in the first fiscal quarter of 2016, which required retrospective adjustment to previously issued financial statements. As a result of the adoption of this ASU, we reclassified our capitalized debt issuance costs previously recorded within Other Assets to a contra-liability reducing Notes Payable on the balance sheets. The reclassification was $14 thousand as of December 31, 2015. This ASU had no effect on our results of operations or liquidity.

In February 2016, the FASB issued ASU 2016-02, Leases, which requires that lease arrangements longer than 12 months result in an entity recognizing an asset and liability. The updated guidance is effective for interim and annual periods beginning after December 15, 2018, and early adoption is permitted. We have not evaluated the impact of the

updated guidance on our financial statements.

In March 2016, the FASB issued ASU 2016-09, Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting. The ASU is the result of the FASB's simplification initiative intended to improve GAAP by reducing costs and complexity while maintaining or enhancing the usefulness of related financial statement information. The ASU simplifies several aspects of the accounting for employee share-based payment transactions, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. While the effective date of ASU 2016-09 is for fiscal years beginning after December 15, 2016, earlier adoption is permitted and we adopted the amendments in ASU 2016-09 during the fourth quarter of 2016. The adoption of this ASU did not have a material effect on our annual financial statements.

In August 2016, the FASB issued ASU 2016-15, Statement of Cash Flows, which amends the guidance in Accounting Standards Codification (ASC) 230 on the classification of certain cash receipts and payments in the statement of cash flows. The primary purpose of the ASU is to reduce the diversity in practice that has resulted from the lack of consistent principles on this topic. The guidance in the ASU is effective for fiscal years beginning after

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December 15, 2017, including interim periods within those fiscal years. We do not expect the adoption of this ASU to have a material effect on our interim or annual financial statements.

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

We are exposed to market risks in the ordinary course of our business. These market risks are principally limited to interest rate fluctuations.

We had cash, cash equivalents and investment balances of $30.1 million at December 31, 2016, consisting of funds in cash, money market accounts and certificates of deposit. The primary objective of our investment activities is to preserve principal and liquidity while maximizing income without significantly increasing risk. We do not enter into investments for trading or speculative purposes. Due to the conservative nature of our investment portfolio, we do not believe an immediate 1.0% increase in interest rates would have a material effect on the fair market value of our portfolio, and accordingly we do not expect a sudden change in market interest rates to affect materially our operating results or cash flows.

Our long-term debt carries a variable interest rate indexed to the prime rate, with a fixed minimum rate of 6.5%. The prime rate in the U.S. had remained at 3.25% since December of 2008, and increased to 3.50% in December 2015 and to 3.75% in December 2016. While we cannot predict when, if at all, this rate will be adjusted again, we believe the stability of the prime rate over the past six years sufficiently mitigates interest rate risk related to our debt. We do not believe an immediate 1.0% increase in the prime rate would have a material effect on the future cash flows related to our debt, and accordingly we do not expect a sudden change in the prime rate to affect materially our operating results or cash flows.

Item 8. Financial Statements and Supplementary Data.

Our financial statements, accompanying notes and Report 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. 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, as of the end of the period covered by this report, our disclosure controls and procedures were effective 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, as our principal financial and accounting officer, to allow timely decisions regarding required disclosures.

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Management's Report on Internal Control Over Financial Reporting

Management of Marinus Pharmaceuticals, Inc. 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. 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 conduct an assessment of the effectiveness of our internal control over financial reporting as of December 31, 2016. Based on the assessment, management has concluded that, as of December 31, 2016, 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, 2016 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

None.

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 2017 annual meeting of shareholders, to be filed with the SEC.

Item 11. Executive Compensation.

We incorporate the information required by this Item 11 by reference to the definitive proxy statement for our 2017 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 2017 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 2017 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 2017 annual meeting of shareholders, to be filed with the SEC.

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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	Amended and Restated By-laws. (Incorporated by reference to Exhibit 3.2 to Form 8-K current report filed on August 7, 2014.)
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.)
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+	Amended and Restated Employment Agreement dated as of August 3, 2016 between the Company and Christopher M. Cashman. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on August 9, 2016.)
10.4+	Amended and Restated Employment Agreement dated as of August 3, 2016 between the Company and Edward F. Smith. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on August 9, 2016.)
10.6*	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.)
10.7	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.)
10.8	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.)
10.9	Loan and Security Agreement dated as of April 2, 2014 between Square 1 Bank and the Company. (Incorporated by reference to Exhibit 10.9 to Form S-1 registration statement filed on May 12, 2014.)
10.10	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.)
10.11	Form of Amended and Restated Indemnification Agreement (Non-VC Directors). (Incorporated by reference to Exhibit 10.11 to Form S-1 registration statement filed on May 12, 2014.)
10.12*	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.)
10.13+	Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, effective as of August 5, 2014. (Incorporated by reference to Exhibit 10.13 to Form 10-K annual report filed on March 12, 2015.)

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Exhibit No.	Description of Exhibit
10.14	First Amendment to Loan and Security Agreement dated as of December 3, 2014 between Square 1 Bank and the Company. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on August 7, 2014.)
10.15+	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.)
10.16+	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.)
10.17+	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.)
10.18+	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.)
10.19	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.)
10.20	Second Amendment to Loan and Security Agreement dated as of February 2, 2015 between Square 1 Bank and Marinus Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on October 29, 2015.)
10.21	Third Amendment to Loan and Security Agreement dated as of October 29, 2015 between Pacific Western Bank (as successor in interest by merger to Square 1 Bank) and Marinus Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 10.2 to Form 10-Q quarterly report filed on October 29, 2015.)
10.22	Equity Distribution Agreement dated as of August 13, 2015 between the Company and JMP Securities LLC. (Incorporated by reference to Exhibit 1.1 to Form S-3 registration statement filed on August 13, 2015.)
10.23	Fourth Amendment to Loan and Security Agreement dated as of April 29, 2016 between Pacific Western Bank and the Company. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on May 2, 2016.)
21	Subsidiaries of the Registrant. (Filed herewith.)
23.1	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
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

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+Indicates management contract or compensatory plan.

*Portions of this exhibit (indicated by asterisks) have been omitted pursuant to an order granting confidential treatment under the Securities Act of 1933.

(c)None.

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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 13, 2017 By: /s/ Christopher M. Cashman
Christopher M. Cashman
President and Chief Executive Officer

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/ Christopher M. Cashman Christopher M. Cashman	President, Chief Executive Officer (Principal Executive Officer) and Chairman	March 13, 2017
/s/ Edward F. Smith Edward F. Smith	Vice President, Chief Financial Officer, Secretary and Treasurer (Principal Finance and Accounting Officer)	March 13, 2017
/s/ Enrique J. Carrazana Enrique J. Carrazana, M.D.	Director	March 13, 2017
/s/ Michael R. Dougherty Michael R. Dougherty	Director	March 13, 2017
/s/ Seth H.Z. Fischer Seth H.Z. Fischer	Director	March 13, 2017
/s/ Tim M. Mayleben	Director	

		March 13, 2017
Tim M. Mayleben		
/s/ Jay P. Shepard	Director	March 13, 2017
Jay P. Shepard		
/s/ Nicole Vitullo	Director	March 13, 2017
Nicole Vitullo		

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FINANCIAL STATEMENTS

MARINUS PHARMACEUTICALS, INC.

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Stockholders
Marinus Pharmaceuticals, Inc.

We have audited the accompanying balance sheets of Marinus Pharmaceuticals, Inc. as of December 31, 2016 and 2015, and the related statements of operations, convertible preferred stock and stockholders' equity (deficit), and cash flows for each of the years in the three year period ended December 31, 2016. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Marinus Pharmaceuticals, Inc. as of December 31, 2016 and 2015, and the results of its operations and its cash flows for each of the years in the three year period ended December 31, 2016, in conformity with U.S. generally accepted accounting principles.

/s/ KPMG LLP

Philadelphia, Pennsylvania
March 13, 2017

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MARINUS PHARMACEUTICALS, INC.

BALANCE SHEETS

(In thousands, except share and per share amounts)

	December 31, 2016	2015
ASSETS		
Current assets:		
Cash and cash equivalents	$ 26,178	$ 51,722
Short-term investments	3,922	4,474
Prepaid expenses and other current assets	199	1,571
Total current assets	30,299	57,767
Property and equipment, net	1,148	31
Investments	—	1,488
Other assets	—	362
Total assets	$ 31,447	$ 59,648
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Current portion of notes payable	$ 3,500	$ 2,128
Accounts payable	2,809	3,146
Accrued expenses	1,775	2,161
Total current liabilities	8,084	7,435
Notes payable	1,743	5,236
Other long-term liabilities	141	56
Total liabilities	9,968	12,727
Commitments and contingencies (Note 8)		
Stockholders' equity:		
Preferred stock, $0.001 par value; 25,000,000 shares authorized, no shares issued and outstanding	—	—
Common stock, $0.001 par value; 100,000,000 shares authorized, 19,734,351 issued and 19,705,120 outstanding at December 31, 2016 and 19,420,086 issued and 19,390,855 outstanding at December 31, 2015	20	19
Additional paid-in capital	147,288	144,088
Treasury stock at cost, 29,231 shares at December 31, 2016 and 2015	—	—
Accumulated deficit	(125,829)	(97,186)
Total stockholders' equity	21,479	46,921
Total liabilities and stockholders' equity	$ 31,447	$ 59,648

See accompanying notes to financial statements.

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MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF OPERATIONS

(In thousands, except share and per share amounts)

	Year Ended December 31,		
	2016	2015	2014
Expenses:			
Research and development	$ 22,005	$ 18,916	$ 8,690
General and administrative	6,237	5,516	3,230
Loss from operations	(28,242)	(24,432)	(11,920)
Change in fair value of warrant liability	—	—	1,192
Interest income	128	64	12
Interest expense	(464)	(475)	(117)
Other expense	(65)	(7)	—
Net loss	$ (28,643)	$ (24,850)	$ (10,833)
Cumulative preferred stock dividends	—	—	(2,545)
Net loss applicable to common stockholders	$ (28,643)	$ (24,850)	$ (13,378)
Per share information:			
Net loss per share of common stock—basic and diluted	$ (1.47)	$ (1.67)	$ (2.17)
Basic and diluted weighted average shares outstanding	19,498,143	14,919,783	6,152,669

See accompanying notes to financial statements.

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MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)

(In thousands, except share and per share amounts)

	Convertible Preferred Stock						Stockholders' Equity (Def		
	Series A		Series B		Series C		Common Stock		Additi Paid-i
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Capita
Balance, December 31, 2013	18,777,860	$ 30,596	12,220,661	$ 17,929	18,381,463	$ 21,314	494,260	$ 1	$ 1,12
Stock-based compensation expense	—	—	—	—	—	—	—	—	698
Issuance of Series C Preferred Stock	—	—	—	—	422,119	500	—	—	—
Exercise of stock options	—	—	—	—	—	—	122,634	—	128
Conversion of convertible preferred stock into common	(18,777,860)	(30,596)	(12,220,661)	(17,929)	(18,803,582)	(21,814)	7,661,871	7	70,3
Exercise and conversion of convertible preferred stock warrants into common	—	—	—	—	—	—	220	—	—
Issuance of common stock in connection with initial public offering ($8.00 per share), net of expenses of $4,862	—	—	—	—	—	—	5,758,000	6	41,1

Net loss	—	—	—	—	—	—	—	—	—
Balance, December 31, 2014	—	—	—	—	—	—	14,036,985	14	113
Stock-based compensation expense	—	—	—	—	—	—	—	—	2,10
Exercise of stock options	—	—	—	—	—	—	327,098	—	373
Issuance of common stock in connection with secondary public offering ($6.00 per share), net of expenses of $2,200							5,056,003	5	28,1
Net loss	—	—	—	—	—	—	—	—	—
Balance, December 31, 2015	—	—	—	—	—	—	19,420,086	19	144
Stock-based compensation expense	—	—	—	—	—	—	—	—	3,07
Exercise of stock options	—	—	—	—	—	—	118,365	1	123
Issuance of restricted stock	—	—	—	—	—	—	195,900	—	—
Net loss	—	—	—	—	—	—	—	—	—
Balance, December 31, 2016	—	$ —	—	$ —	—	$ —	19,734,351	$ 20	$ 147

See accompanying notes to financial statements.

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MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF CASH FLOWS

(In thousands)

	Year Ended December 31,		
	2016	2015	2014
Cash flows from operating activities			
Net loss	$ (28,643)	$ (24,850)	$ (10,833)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	23	11	5
Stock-based compensation expense	3,077	2,108	698
Change in fair value of warrant liability	—	—	(1,192)
Amortization of debt issuance costs	7	7	4
Loss on disposal of fixed assets	—	2	—
Changes in operating assets and liabilities:			
Prepaid expenses and other assets	1,396	(947)	1,344
Accounts payable and accrued expenses	(629)	3,540	1,385
Net cash used in operating activities	(24,769)	(20,129)	(8,589)
Cash flows from investing activities			
Purchases of investments	(2,434)	(7,705)	—
Maturities of short-term investments	4,474	1,743	—
Purchases of property and equipment	(644)	(352)	(33)
Net cash provided by (used in) investing activities	1,396	(6,314)	(33)
Cash flows from financing activities			
Proceeds from exercise of stock options	124	373	128
Proceeds from public offerings, net of offering costs	(167)	28,278	41,202
Proceeds from notes payable, net of issuance costs	—	—	6,975
Principal payments of notes payable	(2,128)	(206)	—
Net cash (used in) provided by financing activities	(2,171)	28,445	48,305
Net (decrease) increase in cash and cash equivalents	(25,544)	2,002	39,683
Cash and cash equivalents—beginning of year	51,722	49,720	10,037
Cash and cash equivalents—end of year	$ 26,178	$ 51,722	$ 49,720
Supplemental disclosure of cash flow information			
Conversion of preferred stock to common stock	$ —	$ —	$ 70,340
Issuance of Series C Preferred Stock	$ —	$ —	$ 500
Cash paid for interest	$ 460	$ 468	$ 89
Financing arrangement with third-party vendor	$ —	$ 584	$ —
Purchase of property and equipment in accounts payable	$ 134	$ —	$ —
Accrued public offering costs	$ —	$ 142	$ —

See accompanying notes to financial statements.

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MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

1. Organization and Description of the Business

We are a clinical stage biopharmaceutical company focused on developing and commercializing innovative therapeutics to treat epilepsy and neuropsychiatric disorders. Our clinical stage product candidate, ganaxolone, is a positive allosteric modulator of $GABA_A$ being developed in three different dose forms (intravenous, capsule and liquid) intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Ganaxolone acts on the $GABA_A$ receptor, a well characterized target in the brain known for both anti seizure and anti anxiety effects. Our primary focus to date has been directed towards developing business strategies, raising capital, conducting research and development activities, and conducting preclinical testing and human clinical trials of our product candidate.

Liquidity

We have not generated any product revenues and have incurred operating losses since inception. 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, and commercialization of our product candidates will require significant additional financing. Our accumulated deficit as of December 31, 2016 was $125.8 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 additional debt, potential collaborations and revenues from potential 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 commercialization of our planned product candidates. We believe that our cash, cash equivalents and investment balance as of December 31, 2016 is adequate to fund our operations into the second half of 2018.

2. Summary of Significant Accounting Policies

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.

Fair Value of Financial Instruments and Credit Risk

At December 31, 2016 and 2015, our financial instruments included cash equivalents, certificates of deposit, accounts payable, accrued expenses, and notes payable. The carrying amount of cash equivalents, certificates of deposit, accounts payable and accrued expenses approximated fair value, given their short-term nature. The carrying amount of our notes payable approximate fair value because the interest rates on these instruments are reflective of rates that we could obtain on debt with similar terms and conditions.

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 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.

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MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

Cash and Cash Equivalents

We consider all highly liquid investments that have maturities of three months or less when acquired to be cash equivalents. As of December 31, 2016 and 2015, 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, 2016 and 2015, our investments consisted of certificates of deposit with various financial institutions, with original maturities ranging from three to 18 months. Certificates of deposit with maturities less than 12 months are classified as short-term investments and maturities greater than 12 months are classified as long-term investments on our balance sheet. All investments are classified as held-to-maturity and are recorded at amortized cost. Fair value of our investments approximates the carrying value on our balance sheet.

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. As of December 31, 2015, this balance included a deposit of $1.0 million for clinical manufacturing supplies to be used in connection with our clinical trials, all of which was expensed in 2016.

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, and five years for laboratory equipment, office equipment, and furniture. 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. Through December 31, 2016, no impairment has occurred.

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

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MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

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, 2016 and 2015, 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 7.

The following table sets forth the computation of basic and diluted earnings per share for the years ended December 31, 2016, 2015 and 2014 (in thousands, except share and per share amounts):

	Year Ended December 31,		
	2016	2015	2014
Basic and diluted net loss per share of common stock:			
Net loss	$ (28,643)	$ (24,850)	$ (10,833)
Dividends on Series B and C Preferred Stock	—	—	(2,545)
Net loss applicable to common stockholders	$ (28,643)	$ (24,850)	$ (13,378)
Weighted average shares of common stock outstanding	19,498,143	14,919,783	6,152,669
Net loss per share of common stock—basic and diluted	$ (1.47)	$ (1.67)	$ (2.17)

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

	December 31,		
	2016	2015	2014
Restricted stock	195,900	—	—

Stock options	2,239,044	1,799,226	1,670,574
	2,434,944	1,799,226	1,670,574

As of December 31, 2016, 2015 and 2014, we had no other potentially dilutive securities.

Comprehensive Loss

Comprehensive loss is defined as the change in equity of a business enterprise during a period from transactions and other events and circumstances from non owner sources. Comprehensive loss was equal to net loss for all periods presented.

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MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

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 neuropsychiatric therapeutics.

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 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, net of estimated forfeitures, 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. Stock based awards issued to non employees are recorded at their fair values, and are periodically revalued as the equity instruments vest and are recognized as expense over the related service period in accordance with the provisions of ASC 718 and ASC Topic 505, Equity.

Clinical Trial Expense Accruals

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, 2016, 2015

and 2014 there were no material adjustments to our prior period estimates of accrued expenses for clinical trials.

Recent Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2014-15, Disclosure of Uncertainties About an Entity's Ability to Continue as a Going Concern, which provides guidance on determining when and how to disclose going-concern uncertainties in the financial statements. The new standard requires management to perform interim and annual assessments of an entity's ability to continue as a going

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concern within one year of the date the financial statements are issued. An entity must provide certain disclosures if conditions or events raise substantial doubt about the entity's ability to continue as a going concern. The ASU applies to all entities and was effective for annual periods ending after December 15, 2016, and interim periods thereafter, with early adoption permitted. We adopted this ASU for the year ended December 31, 2016, and the adoption of this ASU had no material effect on our annual financial statements.

In April 2015, the FASB issued ASU 2015-03, Simplifying the Presentation of Debt Issuance Costs, which changes the presentation of debt issuance costs in financial statements. Under the ASU, an entity presents such costs in the balance sheet as a direct deduction from the related debt liability rather than as an asset. Amortization of the costs is reported as interest expense. We adopted this ASU in the first fiscal quarter of 2016, which required retrospective adjustment to previously issued financial statements. As a result of the adoption of this ASU, we reclassified our capitalized debt issuance costs previously recorded within Other Assets to a contra-liability reducing Notes Payable on the balance sheets. The reclassification was $14 thousand as of December 31, 2015. This ASU had no effect on our results of operations or liquidity.

In February 2016, the FASB issued ASU 2016-02, Leases, which requires that lease arrangements longer than 12 months result in an entity recognizing an asset and liability. The updated guidance is effective for interim and annual periods beginning after December 15, 2018, and early adoption is permitted. We have not evaluated the impact of the updated guidance on our financial statements.

In March 2016, the FASB issued ASU 2016-09, Compensation - Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting. The ASU is the result of the FASB's simplification initiative intended to improve GAAP by reducing costs and complexity while maintaining or enhancing the usefulness of related financial statement information. The ASU simplifies several aspects of the accounting for employee share-based payment transactions, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. While the effective date of ASU 2016-09 is for fiscal years beginning after December 15, 2016, earlier adoption is permitted and we adopted the amendments in ASU 2016-09 during the fourth quarter of 2016. The adoption of this ASU did not have a material effect on our annual financial statements.

In August 2016, the FASB issued ASU 2016-15, Statement of Cash Flows, which amends the guidance in ASC 230 on the classification of certain cash receipts and payments in the statement of cash flows. The primary purpose of the ASU is to reduce the diversity in practice that has resulted from the lack of consistent principles on this topic. The guidance in the ASU is effective for fiscal years beginning after December 15, 2017, including interim periods within those fiscal years. We do not expect the adoption of this ASU to have a material effect on our interim or annual financial statements.

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.

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· Level 3—Valuations based on inputs that are unobservable and models that are significant to the overall fair value measurement.

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, 2016				
Assets				
Money market funds (cash equivalents)	$ 25,629	$ —	$ —	$ 25,629
Certificates of deposit	3,922	—	—	3,922
December 31, 2015				
Assets				
Money market funds (cash equivalents)	$ 50,682	$ —	$ —	$ 50,682
Certificates of deposit	5,962	—	—	5,962

4. Property and Equipment

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

	December 31,	
	2016	2015
Laboratory equipment	$ 1,367	$ 311
Office equipment	139	69
	1,506	380

Less: accumulated depreciation	(358)	(349)
	$ 1,148	$ 31

Depreciation expense was $23, $11 and $5 thousand for the years ended December 31, 2016, 2015 and 2014, respectively.

5. Accrued Expenses

At December 31, 2016 and 2015, accrued expenses consisted of the following (in thousands):

	December 31,	
	2016	2015
Payroll and related costs	880	631
Clinical trials and drug development	681	1,188
Professional fees	101	190
Other	113	152
Total accrued expenses	$ 1,775	$ 2,161

6. Notes Payable

In April 2014, we borrowed $2.0 million pursuant to a Loan and Security Agreement (LSA) we entered into

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MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

with a financial institution. Pursuant to the terms of the LSA, we made monthly interest-only payments for outstanding borrowings at an interest rate equal to the greater of (a) prime plus 2.25% or (b) 5.5% until the LSA was amended. In December 2014, February 2015, October 2015 and April 2016 we entered into four amendments to the LSA (collectively, the "Amended LSA") with the same financial institution.

In connection with the Amended LSA, we borrowed an additional $5.0 million in December 2014. We were required to make monthly interest-only payments for all outstanding borrowings at an interest rate equal to the greater of (a) prime rate plus 3.25% or (b) 6.5% until June 2016. Commencing in July 2016 and continuing through June 2018, we are required to make monthly payments of 1/24th of our principal borrowings plus interest.

As of December 31, 2016, of our outstanding term loan balance of $5.3 million, $3.5 million will be due within the next twelve months, and is classified as the current portion of notes payable on our balance sheet. Interest expense related to the term loans was $459 thousand, $461 thousand and $117 thousand for the years ended December 31, 2016, 2015 and 2014, respectively. As of December 31, 2016, we had accrued interest of $31 thousand. There are no financial covenants associated with these term loans. As of December 31, 2016, we were in compliance with all non-financial covenants.

Vendor Debt

In August 2015, the Company entered into a short-term loan agreement with a third-party vendor to finance insurance premiums. The aggregate amount financed under this agreement was $584 thousand, which was fully paid as of December 31, 2016.

Maturities of our debt obligations over the next five years are as follows (in thousands):

	Debt Maturities
2017	$ 3,500
2018	1,750
Total maturities	$ 5,250

7. Stockholders' Equity

In 2005, we adopted the 2005 Stock Option and Incentive Plan (2005 Plan) that authorizes us to grant options, restricted stock and other equity-based awards. As of December 31, 2016, 430,922 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 (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, 2016, 1,808,122 options to purchase shares of common stock and 195,900 restricted shares of common stock were outstanding pursuant to grants in connection with the 2014 Plan, and 31,922 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, 2017, shares of common stock available for future grants under the 2014 plan was increased to 820,127.

In August 2015, we entered into an Equity Distribution Agreement with JMP Securities LLC. Subsequent to December 31, 2016 we sold 1,669,092 shares under this agreement for aggregate net proceeds of approximately $2.0 million.

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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,		
	2016	2015	2014
Research and development	$ 980	$ 685	$ 137
General and administrative	1,975	1,423	561
Total	$ 2,955	$ 2,108	$ 698

Options issued under both the 2005 Plan and 2014 Plan may 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, 2016, 2015 and 2014 is presented below (in thousands, except share and per share amounts):

	Shares	Weighted-Average Exercise Price Per Share	Aggregate Intrinsic Value
Outstanding—December 31, 2013	1,093,208	$ 1.04	
Granted	700,000	8.99	
Exercised	(122,634)	1.04	
Outstanding—December 31, 2014	1,670,574	4.37	
Granted	585,800	12.56	
Exercised	(327,098)	1.14	
Forfeited	(44,950)	6.13	
Expired	(85,100)	1.04	
Outstanding—December 31, 2015	1,799,226	7.74	
Granted	603,975	2.42	
Exercised	(118,365)	1.04	
Forfeited	(45,792)	12.08	
Outstanding—December 31, 2016	2,239,044	$ 6.57	$ —
Exercisable—December 31, 2016	1,182,966	$ 6.96	$ —
Exercisable and expected to vest—December 31, 2016	2,239,044	$ 6.57	$ —

The weighted average remaining contractual term of options outstanding and exercisable as of December 31, 2016 is 8.1 and 7.5 years, respectively.

Intrinsic value in the table above was determined by calculating the difference between the market value of our common stock on the last trading day of 2016 of $1.01 per share and the exercise price, multiplied by the number of in-the-money options. The aggregate intrinsic value as of December 31, 2016 was zero, because all outstanding options have exercise prices in excess of market value. The aggregate intrinsic value of options exercised in 2016, 2015 and 2014 was $0.5 million, $3.4 million and $0.5 million, respectively.

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The weighted average grant date fair value of options granted was $1.60, $8.15, and $6.09 per share in 2016, 2015 and 2014, respectively, and was estimated at the date of grant using the Black Scholes option pricing model with the following weighted average assumptions:

	2016	2015	2014
Expected stock price volatility	74.69 - 87.67%	73.64 - 81.21%	77.66 - 86.08%
Expected term of options	5.2 - 6.1 years	5.2 - 6.1 years	5.5 - 6.06 years
Risk-free interest rate	1.07 - 1.86 %	1.44 - 1.92 %	1.75 - 1.98 %
Expected annual dividend yield	0 %	0 %	0 %

The weighted average valuation assumptions were determined as follows:

- Expected stock price volatility: The expected volatility is based on historical volatilities of similar entities within our industry which were commensurate with our expected term assumption as described in the SEC's Staff Accounting Bulletin, or SAB, No. 107.
- 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, 2016, there was $4.2 million of total unrecognized compensation expense related to unvested stock options granted under the 2005 Plan and 2014 Plan. That expense is expected to be recognized over the next four years as follows, in thousands:

2017	$ 2,473
2018	1,480
2019	242
2020	12
	$ 4,207

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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No restricted shares of common stock were issued prior to fiscal year 2016. A summary of activity for the year ended December 31, 2016 is presented below (in thousands, except share and per share amounts):

	Shares	Weighted-average Grant Date Fair Value per Share
Outstanding—December 31, 2015	—	$ —
Granted	196,275	1.51
Vested	—	—
Forfeited	(375)	1.50
Outstanding—December 31, 2016	195,900	$ 1.51
Expected to vest—December 31, 2016	195,900	$ 1.51

As of December 31, 2016, there was $0.2 million of total unrecognized compensation cost related to unvested restricted stock is expected to be recognized over a weighted average service period of 0.59 years.

Total compensation cost recognized for all restricted stock awards in the statements of operations for the year ended December 31, 2016 is as follows (in thousands):

Research and development	$ 48
General and administrative	74
Total	$ 122

8. Commitments and Contingencies

Leases

In October 2014, we entered into a five-year operating lease agreement for office space in Radnor, Pennsylvania. Rent payments under this lease commence May 1, 2015, with payment amounts escalating each May 1 thereafter through the end of the lease term. In December 2015, we entered into a First Amendment to this lease agreement (Amended Lease) to lease approximately 8,500 rentable square feet of office space in Radnor, Pennsylvania. This Lease amended an existing lease agreement to replace leased premises of approximately 4,000 rentable square feet of office space, and we commenced leasing the larger office space in April 2016. Rent payments under the Amended Lease are expected commenced June 1, 2016, with payment amounts escalating each June 1 thereafter through the end of the 62-month lease term.

In November 2015, we entered into a one-year operating lease agreement for approximately 1,000 square feet of office space in Madison, Connecticut, with two annual renewal options of one year each. In November 2016, we exercised the first renewal option. Rent payments under this lease commenced on November 1, 2015, and increase with each renewal period. Prior to that and through October 2015, we leased a facility in New Haven, Connecticut. Rent expense under these operating leases, in thousands, was $322, $143 and $50 for the years ended 2016, 2015 and 2014, respectively. All leases are non-cancelable.

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Our annual future minimum lease payments under these leases are as follows (in thousands):

	Operating Lease Payments
2017	$ 333
2018	318
2019	324
2020	330
2021	139
Thereafter	—
Total minimum lease payments	$ 1,444

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 Agreement

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.

9. Income Taxes

As of December 31, 2016 and 2015, we had approximately $121.8 million and $93.2 million, respectively, of net operating loss (NOL) carry forwards available to offset future federal and state taxable income that will expire beginning in 2023. We also have federal research and development credit carryovers of approximately $4.4 million and state credit carryovers of approximately $0.9 million which expire beginning in 2019.

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 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. Additionally, 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 are currently evaluating 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.

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

	December 31,	
	2016	2015
Gross deferred tax assets:		
Net operating loss carryforwards	$ 48,654	$ 37,051
Accrued expenses	55	29
Contributions	6	6
Deferred expenses	58	23
Stock-based compensation	1,523	826
Research and development and other credits	5,258	4,123
Total gross deferred tax assets	55,554	42,058
Gross deferred tax liabilities:		
Depreciation	(3)	(2)
Total gross deferred tax liabilities	(3)	(2)
Net deferred tax assets	55,551	42,056
Less: valuation allowance	(55,551)	(42,056)
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, 2016. The valuation allowance increased by $13.5 million and $12.3 million during the years ended December 31, 2016 and 2015, respectively, due primarily to the generation of NOLs during those periods.

We did not have unrecognized tax benefits as of December 31, 2016 and 2015, 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, 2016 and 2015. As of December 31, 2016 and 2015, we have not accrued interest or penalties related to any uncertain tax positions. Our tax

returns filed since inception are still subject to examination by major tax jurisdictions.

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MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

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,		
	2016	2015	2014
Federal income tax expense at statutory rate	34.0 %	34.0 %	34.0 %
Permanent items	(1.2)	(1.0)	(5.4)
State income tax, net of federal benefit	7.5	7.1	3.8
R&D tax credits	5.2	4.2	4.2
Other	1.6	5.0	—
Change in valuation allowance	(47.1)	(49.3)	(36.6)
Effective income tax rate	0.0 %	0.0 %	0.0 %

For all years through December 31, 2016, 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, 2013 through December 31, 2015. 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.

10. Quarterly Financial Information (unaudited)

	First Quarter	Second Quarter	Third Quarter	Fourth Quarter	Total Year
2016					
Research and development expenses	$ 5,494	$ 7,258	$ 4,840	$ 4,413	$ 22,005
General and administrative expenses	$ 1,604	$ 1,586	$ 1,529	$ 1,518	$ 6,237
Net loss	$ (7,216)	$ (8,949)	$ (6,464)	$ (6,014)	$ (28,643)
Net loss per share, basic and diluted	$ (0.37)	$ (0.46)	$ (0.33)	$ (0.31)	$ (1.47)

2015					
Research and development expenses	$ 5,468	$ 3,915	$ 3,472	$ 6,061	$ 18,916
General and administrative expenses	$ 1,447	$ 1,255	$ 1,378	$ 1,436	$ 5,516
Net loss	$ (7,007)	$ (5,273)	$ (4,963)	$ (7,607)	$ (24,850)
Net loss per share, basic and diluted	$ (0.50)	$ (0.37)	$ (0.35)	$ (0.45)	$ (1.67)

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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	Amended and Restated By-laws. (Incorporated by reference to Exhibit 3.2 to Form 8-K current report filed on August 7, 2014.)
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.)
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+	Amended and Restated Employment Agreement dated as of August 3, 2016 between the Company and Christopher M. Cashman. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on August 9, 2016.)
10.4+	Amended and Restated Employment Agreement dated as of August 3, 2016 between the Company and Edward F. Smith. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on August 9, 2016.)
10.6*	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.)
10.7	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.)
10.8	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.)
10.9	Loan and Security Agreement dated as of April 2, 2014 between Square 1 Bank and the Company. (Incorporated by reference to Exhibit 10.9 to Form S-1 registration statement filed on May 12, 2014.)
10.10	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.)
10.11	Form of Amended and Restated Indemnification Agreement (Non-VC Directors). (Incorporated by reference to Exhibit 10.11 to Form S-1 registration statement filed on May 12, 2014.)
10.12*	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.)
10.13+	Marinus Pharmaceuticals, Inc. 2014 Equity Incentive Plan, effective as of August 5, 2014. (Incorporated by reference to Exhibit 10.13 to Form 10-K annual report filed on March 12, 2015.)
10.14	First Amendment to Loan and Security Agreement dated as of December 3, 2014 between Square 1 Bank and the Company. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on August 7, 2014.)
10.15+	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.)
10.16+	

	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.)
10.17+	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.)
10.18+	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.)

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Exhibit No.	Description of Exhibit
10.19	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.)
10.20	Second Amendment to Loan and Security Agreement dated as of February 2, 2015 between Square 1 Bank and Marinus Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on October 29, 2015.)
10.21	Third Amendment to Loan and Security Agreement dated as of October 29, 2015 between Pacific Western Bank (as successor in interest by merger to Square 1 Bank) and Marinus Pharmaceuticals, Inc. (Incorporated by reference to Exhibit 10.2 to Form 10-Q quarterly report filed on October 29, 2015.)
10.22	Equity Distribution Agreement dated as of August 13, 2015 between the Company and JMP Securities LLC. (Incorporated by reference to Exhibit 1.1 to Form S-3 registration statement filed on August 13, 2015.)
10.23	Fourth Amendment to Loan and Security Agreement dated as of April 29, 2016 between Pacific Western Bank and the Company. (Incorporated by reference to Exhibit 10.1 to Form 10-Q quarterly report filed on May 2, 2016.)
21	Subsidiaries of the Registrant. (Filed herewith.)
23.1	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
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

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+Indicates management contract or compensatory plan.

* Portions of this exhibit (indicated by asterisks) have been omitted pursuant to an order granting confidential treatment under the Securities Act of 1933.
