

MARINUS PHARMACEUTICALS INC

Form 10-K

March 12, 2015

[Table of Contents](#)

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

x Annual Report Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934.

For the Fiscal Year Ended December 31, 2014

o 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: 000-36576

MARINUS PHARMACEUTICALS, INC.

(Exact name of Registrant as specified in its charter)

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Delaware
(State or other jurisdiction of
incorporation or organization)

20-0198082
(I.R.S. Employer
Identification No.)

Three Radnor Corporate Center

100 Matsonford Rd., Suite 304

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 |
|---|---|
| Common Stock, par value \$0.001 per share | The 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 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 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 requirement for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, 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 Registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendments to this Form 10-K. Yes No

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Indicate by check mark whether the Registrant is a large accelerated filer, an accelerated filer, or a non-accelerated filer, or a smaller reporting company. See the definitions of large accelerated filer, accelerated filer, and smaller reporting company in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer Accelerated filer
Non-accelerated filer Smaller reporting company
(Do not check if a smaller reporting company)

Indicate by check mark whether the Registrant is a shell company (as defined in Rule 12b-2 of the 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 recently completed second fiscal quarter: The aggregate market value of the voting stock held by non-affiliates of the registrant, as of July 31, 2014, was approximately \$51.5 million. Such aggregate market value was computed by reference to the closing price of the common stock as reported on the NASDAQ Global Market on July 31, 2014. The registrant has elected to use July 31, 2014 as the calculation date, as on June 30, 2014 (the last business day of the most recently completed second fiscal quarter) the registrant was a privately-held concern.

The number of shares of the issuer's Common Stock outstanding as of March 12, 2015 was 14,199,666.

DOCUMENTS INCORPORATED BY REFERENCE

The registrant's definitive proxy statement to be filed in connection with solicitation of proxies for its 2015 Annual Meeting of Stockholders to be held within 120 days of the end of its fiscal year, is incorporated by reference into Part III of this Annual Report on Form 10-K.

Part IV.

| | | |
|---------------------------------------|---|-----|
| <u>Item 15.</u> | <u>Exhibits, Financial Statement Schedules.</u> | 69 |
| <u>Signatures.</u> | | 71 |
| <u>Index to Financial Statements.</u> | | F-1 |

Table of Contents

Cautionary Note Regarding Forward-Looking Statements.

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

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

- our ability to develop and commercialize ganaxolone;
- 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;

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- 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 of reimbursement for ganaxolone;
- the success of other competing therapies that may become available;
- the manufacturing capacity for ganaxolone;
- our intellectual property position;
- our ability to maintain and protect our intellectual property rights;

Table of Contents

- 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.

Table of Contents

PART I

Item 1. Business.

Overview

We are a clinical stage biopharmaceutical company focused on developing and commercializing innovative neuropsychiatric therapeutics. Our clinical stage product candidate, ganaxolone, is a small molecule that is a synthetic analog of allopregnanolone, an endogenous neurosteroid known for its anticonvulsive and antianxiety activity. By targeting the same spectrum of GABAA receptors as allopregnanolone, ganaxolone delivers its therapeutic benefit through a mechanism that we believe may offer safety and efficacy advantages compared to other marketed antiepileptic drugs, or AEDs. Ganaxolone was rationally designed to unlock the potential for chronic neurosteroid therapy through modulation of the GABAA receptor. Our orally administered solid and liquid suspension dose forms are being evaluated in our ongoing clinical trials and we are conducting the requisite preclinical experiments to ready our intravenous, or IV, dose form for clinical use. We have a dual strategy of evaluating ganaxolone for treating seizure disorders, and treating targeted orphan diseases for which there are no approved therapies, the development timelines may be abbreviated, and for which there is a strong mechanistic rationale for ganaxolone to offer therapeutic benefit to patients with very high unmet needs.

Our Pipeline

We are developing ganaxolone for multiple epilepsy and other neuropsychiatric indications, including the following:

Our most advanced indication for ganaxolone is as an adjunctive, or add-on, therapy for the treatment of partial, also known as focal, onset seizures in adults with epilepsy. Phase 3 randomized multinational clinical trial utilizing our oral capsules is underway for this indication. We plan to release top line data by the first quarter of 2016. We have completed a Phase 2 clinical trial in 147 patients with focal onset seizures where we demonstrated that patients who added ganaxolone liquid suspension to their medication regimen experienced a statistically significant reduction in seizures as compared to patients who added placebo.

Based upon both proof-of-concept data for ganaxolone in the treatment of refractory pediatric seizures and a mechanistic rationale for providing a therapeutic benefit through increased GABAergic signaling, we have initiated an open-label Phase 2 clinical trial evaluating ganaxolone for treatment of the orphan disease, PCDH19 female pediatric epilepsy. We expect to release initial data from this study during 2015.

Table of Contents

We are developing an IV formulation for use in the hospital setting to control acute seizures. We are conducting the requisite preclinical experiments to ready our IV formulation for clinical use.

In addition to anticonvulsive activity, we believe ganaxolone's mechanism may improve anxiety and behaviors related to anxiety, such as irritability or social withdrawal. Ganaxolone is being evaluated in an investigator-sponsored, grant funded, randomized double-blind placebo-controlled Phase 2 crossover study as potential proof-of-concept in treating behaviors in Fragile X Syndrome, or FXS, which we believe to be an orphan indication. We plan to complete this study during 2015.

Ganaxolone Mechanism of Action

The effects of allopregnanolone have been studied for over two decades and its role in controlling seizures and improving anxiety, mood and sleep through positive modulation of GABAA type receptors is well documented. Despite these positive characteristics, we believe allopregnanolone is not suitable for chronic use due to potential undesired hormonal side effects. Ganaxolone was designed to have the same GABA modulation effects as allopregnanolone without these hormonal side effects. Ganaxolone and allopregnanolone differ from other GABA agents by interacting with unique binding sites on the GABAA receptor that are located both within, or synaptic, and outside, or extrasynaptic, the GABA synapse. Ganaxolone's activation of the extrasynaptic receptor is a unique mechanism that provides stabilizing effects that we believe differentiates it from other drugs that increase GABA signaling. Preclinical studies provide evidence that the GABA modulatory activity of ganaxolone is responsible for its anticonvulsive activity in epileptic seizures and its antianxiety effects in FXS and other neuropsychiatric disorders.

Ganaxolone in Epilepsy

Overview and Treatment of Epilepsy

Epilepsy is characterized by seizures that arise from abnormal electrical discharge in the brain, resulting in alterations of consciousness, involuntary movement, or altered sensations. Seizures in epilepsy may be related to a brain injury or heredity, but often the cause is unknown. Epileptic seizures are generally described in two major groups, primary generalized seizures and focal onset seizures. Primary generalized seizures begin with a widespread electrical discharge that involves both sides of the brain at once. Focal onset seizures begin with an electrical discharge in one limited area of the brain. Generally, a person is diagnosed as having epilepsy when they have had at least two seizures that do not have a self-limiting cause such as a high fever.

In 2012, Decision Resources reported that approximately five million people were under treatment for epilepsy in the United States, Europe and Japan. IMS Health reported that global sales of AEDs were approximately \$14 billion in 2011. It is estimated that approximately 3.8% of people will develop epilepsy during their lifetime,

Table of Contents

with a higher incidence in men than women. New cases of epilepsy are most common among children, especially during the first year of life. The rate of new cases gradually declines until about age 10, and then becomes stable. Remission is common for children as they age. After age 55 to 60, the rate of new epilepsy cases starts to increase, as people develop strokes, brain tumors, or Alzheimer's disease.

Newly diagnosed epilepsy patients are treated with daily administration of an AED. Approximately 60% of patients will achieve an adequate level of seizure control with a single AED, and the remainder will resort to polypharmacy. Approximately 30 to 35% of all patients do not reach an acceptable level of seizure control even with polypharmacy, and are considered to be refractory cases. We estimate that the market opportunity for this refractory patient population, which will be ganaxolone's initial target segment, will exceed \$4 billion in the United States, Europe and Japan. Despite the widespread availability of generic drugs for the treatment of epilepsy, in many countries, including the United States, health payors permit these epilepsy patients to switch to costlier medications or polypharmacy in order to gain seizure control or reduce side effects. Our market research suggests that many physicians attempt to add a medication with a new mechanism of action while also trying to minimize side effect burden when selecting a substitute or add-on AED. A subset of focal onset seizure patients who cannot gain acceptable seizure control through pharmacologic treatment options resort to implantable devices or removal of the part of the brain causing the seizures.

Market Opportunity

Epileptic seizures require chronic treatment, often over a lifetime. Available AEDs are efficacious for many patients, but chronic treatment is complicated by side effects, including cardiovascular risks, liver enzyme induction, kidney stones, behavioral changes, sedation and adverse effects on cognitive function, drug tolerance, and reproductive risk.

Women have the added complication that several currently available AEDs increase the risk to the fetus, including birth defects, lowered IQ and low birth weight. Most of these drugs are categorized by the U.S. Food and Drug Administration (FDA) as Pregnancy Category C, indicating a potential risk to the fetus, and three of these drugs (valproate, carbamazepine, phenytoin) have been classified as Pregnancy Category D, indicating that use is only justified if there is a serious condition where the need outweighs risk to the fetus based on registry data.

Despite the many available AEDs, approximately 30 to 35% of patients do not attain acceptable seizure control either with single drug or multiple drug therapy. Furthermore, medications with significant side effects or dosing regimens that undermine compliance make it difficult for patients to achieve and maintain seizure free status. For these reasons there is a need for new AEDs with novel mechanisms of action and improved side effect profiles that can maintain seizure control with chronic administration for people with refractory epilepsy. The recent successful introduction of Vimpat by UCB is an example of market acceptance of a new AED with a novel mechanism. Vimpat was approved in the United States and European Union in 2008, and achieved global sales of approximately 471 million in 2014. Several other successfully marketed AEDs experienced similar sales levels with similar duration of time on the market. UCB has stated that it expects Vimpat to achieve over 1.2 billion in peak sales globally.

Our Solution

We believe ganaxolone to be a first-in-class therapy with potential to provide meaningful treatment advantages for adults with focal onset seizures who do not achieve adequate seizure control from, have developed tolerance to, or have safety concerns with currently available medications. We believe ganaxolone, if approved, may provide the following benefits for patients:

- ***Efficacy for refractory patients with focal onset seizures.*** Our completed Phase 2 clinical trial in patients with refractory focal onset seizures demonstrated that patients who added ganaxolone to their medication regimen experienced a statistically significant reduction in seizures as compared to patients who added placebo.

- ***Improved safety and tolerability profile.*** Ganaxolone was engineered to be a synthetic analog of a natural molecule, allopregnanolone. Completed preclinical safety studies and Phase 1 and 2 clinical trials involving approximately 1,000 subjects show ganaxolone to be generally safe and well-tolerated, without evidence of toxicity to heart, liver, blood or other body systems and without many of the side effects common to other AEDs. We believe this safety profile may make ganaxolone a treatment of choice in antiepileptic polypharmacy regimens.

Table of Contents

- ***Improved reproductive toxicity profile.*** Based on ganaxolone's mechanism, and preclinical and clinical findings to date, we believe ganaxolone will offer a lower risk for reproductive toxicity than many currently available AEDs, which we believe would be an important safety differentiator for women of childbearing age.

Our market research with physicians indicates neurologists and epilepsy specialists would expect to use ganaxolone in 16 to 29% of their focal onset seizure patients.

Table of Contents***Clinical Trials for Epilepsy in Adults******Controlled Phase 2 Clinical Trial for Adjunctive Treatment of Drug-Resistant Focal Onset Seizures (Study 600)***

We successfully completed a double-blind, randomized, placebo-controlled, Phase 2 clinical trial in the United States of ganaxolone as an adjunctive treatment in patients with refractory focal onset seizures. In this trial, ganaxolone satisfied the primary efficacy endpoint and was considered to be generally safe and well tolerated. The study enrolled 147 patients who had been diagnosed with epilepsy on average 25 years prior and 75% of which were taking two or three AEDs to control seizures before they entered the study. Subjects were treated for ten weeks with placebo or ganaxolone as adjunctive treatment to existing therapy and recorded their seizures daily in a diary. Mean baseline seizure frequency was 6.5 and 9.2 seizures per seven days in the ganaxolone and placebo groups, respectively. Subjects gradually increased their daily dose up to 1,500 milligrams per day, or mg/day, over the first two weeks, known as titration or the titration period, followed by maintenance dosing at 1,500 mg/day for eight weeks, known as the maintenance period. The primary efficacy endpoint was change from baseline in weekly seizure frequency. Results of the analysis, using a statistical test (Kruskal-Wallis) that is employed when data have non-normal distribution, are presented in the table below:

% Seizure Reduction From Baseline

| | Ganaxolone (n=98) | Placebo (n=49) | Difference |
|---------------------------|--------------------------|-----------------------|-------------------|
| Mean (standard deviation) | -17.6% (48.9) | +2.0% (63.2) | 19.6% |
| Median | -26.0% | -10.2% | 15.7% |

In the intention-to-treat, or ITT, population, which included all study subjects who took at least one dose of study medication, there was a statistically significant reduction in the percent change in mean weekly seizure frequency in the ganaxolone group, which decreased 17.6% from baseline at week 10, whereas in the placebo group, mean weekly seizure frequency increased by 2% compared to baseline at week 10, a difference of 19.6% (p=0.014). The p-value represents the probability that the difference between the two groups is due to chance rather than drug effect, and when that probability is less than 5%, or p<0.05, the result is considered statistically significant. In the ganaxolone group median seizure frequency decreased by 26.0%, whereas in the placebo group median seizure frequency decreased by 10.2%, a difference of 15.7%. We believe this effect size for an adjunctive treatment of a highly refractory patient population is consistent with the Phase 2 results for other AEDs that ultimately received FDA approval.

Secondary analyses included the analysis of the percentage of subjects with greater than or equal to 50% improvement from baseline, or responder analysis, mean and percent change in seizure frequency from baseline, number of seizure-free days and seizure-free subjects, change in seizure frequency by week, and change from baseline in types of seizures. In general, the results of secondary efficacy analyses supported the primary outcome that subjects treated with ganaxolone showed improved seizure control compared to those treated with placebo. The responder analysis is considered by the EMA to be the primary analysis of a registration trial. In this study, the percent of responders in the ganaxolone group compared to the placebo group in the ITT population were 23.5% and 14.6% (p=0.192) for the titration plus maintenance period, and 26.3% v. 13.0% (p=0.057) for the maintenance period. No gender effect or effect of concomitant medication was observed.

Open Label Extension of Controlled Phase 2 Clinical Trial for Adjunctive Treatment of Drug-Resistant Focal Onset Seizures (Study 601)

Of the treatment-resistant subjects in our Phase 2 clinical trial, 95% of eligible subjects continued in a long-term open-label extension where the mean duration of treatment was 39 weeks. The objective of the open-label extension study was to evaluate the long-term safety, tolerability and efficacy of ganaxolone at a target dose of 1,500 mg/day. The primary endpoint was change in seizure frequency at endpoint compared to baseline of the double-blind study, presented as mean and median change. Secondary assessments were similar to those evaluated in the blinded portion of the study.

The mean and median percent reductions in weekly seizure frequency were 14.2% and 23.2% from baseline to endpoint, respectively. In total, 70% of subjects had a reduction in seizure frequency during the study. Importantly, subjects previously randomized to placebo in the double-blind study (Study 600) that were switched to ganaxolone in the open-label study showed mean and median reduction in seizure frequency comparable to patients randomized to ganaxolone in the double-blind study.

Table of Contents

Secondary analyses included assessment of responders and seizure free status. Twenty-four percent of subjects met responder criteria at endpoint, defined as a reduction in seizures of 50% or more from baseline, while 43% of those who remained in the study for 52 weeks or more met 50% responder criteria. Subjects in the study reported a mean increase in number of seizure-free days per week of 17.4%, an increase that we believe to be meaningful in the context of the severity and persistence of epilepsy in this drug-resistant population.

Phase 2 Clinical Trial of Monotherapy in Drug Resistant Focal Onset Seizures (Study 104)

A controlled clinical trial was also conducted to evaluate ganaxolone as monotherapy for focal onset seizures in a drug-resistant population. This double-blind, randomized, placebo-controlled, Phase 2 clinical trial enrolled 52 subjects who were withdrawn from their antiepileptic medications prior to evaluation for surgical treatment of their seizures. The subjects were treated with ganaxolone monotherapy 625 mg three times per day for eight days. The primary efficacy measure was duration of treatment prior to study withdrawal, due to emergence of seizures, as measured from day 2 of the study.

As depicted in the plot above showing the Kaplan-Meier survival function of subjects remaining in the study, 62% of subjects in the placebo group left the study due to emergence of seizures by day 8, compared with 39% of ganaxolone subjects ($p=0.08$). Statistical testing of completion rates between the two groups found a statistically significant difference between completion rates in the two groups, with 50% of ganaxolone subjects completing the study compared to 25% of placebo subjects ($p=0.04$).

Ganaxolone Safety Overview

More than 1,000 subjects have received treatment with ganaxolone ranging in duration from one day to more than two years using doses from 50 to 2,000 mg/day. A total of 289 healthy subjects received ganaxolone doses of 50 to 2,000 mg/day in Phase 1 studies, for periods of up to two weeks. In the completed Phase 2 clinical studies, 697 unique subjects have received ganaxolone including 135 pediatric subjects with epilepsy, 169 adult subjects with epilepsy, and 393 adult subjects with migraine. 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. 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.

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. We plan to initiate the final two-year carcinogenicity studies in rats and mice in 2015.

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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, generating a rating of Pregnancy Category C that indicates a finding of developmental toxicities in animal studies. Valproate, carbamazepine, phenytoin and topiramate have been linked with birth defects in humans, for example, 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 a categorization of Pregnancy Category D 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 including a designation of FDA Pregnancy Category B for ganaxolone, which indicates 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.

Table of Contents*Clinical Safety Results in Epilepsy*

Ganaxolone was considered to be generally safe and well tolerated in the Phase 2 adjunctive treatment trials in adults with focal onset seizures. The majority of adverse events associated with ganaxolone treatment were related to known CNS effects of GABA, were not medically serious and resolved upon discontinuation of therapy. The data did not show any trends of medically important changes in blood chemistry, vital signs, liver function, renal function or cardiovascular parameters related to ganaxolone treatment.

The most frequent treatment-emergent adverse events, or TEAEs, observed in Study 600 are presented in the table below.

**Summary of Most Frequently Reported ($\geq 5\%$ of Subjects) TEAEs by
System Organ Class and Preferred Term (ITT Population)**

| Preferred Term | Ganaxolone (n=98) % | Placebo (n=49) % |
|--|---------------------------|------------------------|
| Dizziness | 16.3 | 8.2 |
| Fatigue | 16.3 | 8.2 |
| Somnolence | 13.3 | 2.0 |
| Injury, poisoning and procedural complications | 17.3 | 22.4 |
| Headache | 8.2 | 12.2 |
| Coordination abnormal | 6.1 | 6.1 |
| Convulsion | 5.1 | 8.2 |
| Nasopharyngitis | 5.1 | 10.2 |
| Fall | 5.1 | 12.2 |

The two treatment groups had similar rates of discontinuation due to adverse events (7% ganaxolone, 6% placebo) and similar rates of serious adverse events, or SAEs (5% ganaxolone, 8% placebo), mostly related to the underlying epilepsy. The majority of TEAEs resolved with continued treatment or dose reduction. In contrast to some marketed AEDs, the incidence of behavioral TEAEs (reported as depression, insomnia, affective disorder, confusional state, affect lability, aggression, anxiety) was similar in the ganaxolone and placebo treatment groups.

Ganaxolone continued to be considered generally safe and well tolerated in the long-term open-label extension, Study 601, in which 120 subjects received ganaxolone for a mean duration of 39 weeks. The most common adverse events considered related to ganaxolone treatment were fatigue (14%), dizziness (9%) and somnolence, also known as sleepiness (7%). Eleven percent of the subjects discontinued due to one or more adverse events. One SAE out of 17 reported was considered related to ganaxolone treatment, a 59 year old female on 900 mg/day whose liver enzymes were elevated after 57 days of treatment. The enzyme levels returned to normal with a reduction in dose to 600 mg/day. In this long-term open-label study of ganaxolone for adjunctive treatment of focal onset seizures, no new safety concerns were identified during extended treatment with doses up to 1,500 mg/day.

In Study 104, the eight-day monotherapy study of ganaxolone and placebo in presurgical patients, ganaxolone was generally well-tolerated and the profile of adverse events between the two groups was similar. Dizziness, which was reported in four ganaxolone and three placebo subjects,

was the most frequent adverse event. One SAE was reported in each group; the ganaxolone subject experienced severe agitation and depression while the placebo subject experienced postictal psychosis. As in the other studies, no clinically meaningful differences between treatment groups were noted in laboratory, vital sign, electrocardiogram, or physical/neurological exam results.

Ongoing and Planned Clinical Trials in Epilepsy

In October 2013 we initiated an international, randomized, placebo-controlled, Phase 2b clinical trial in adult subjects for adjunctive treatment of focal onset seizures (Study 603). In Cohort 1, approximately 50 subjects were randomized to receive either placebo or ganaxolone capsules in a step titration of 1,200 mg/day for four weeks followed by 1,800 mg/day for four weeks. Blood levels of ganaxolone will be assessed at steady state for each dose level. Subjects from both cohorts enter a one year open label period after the double-blind treatment period.

The study protocol was then amended to meet the requirements for a phase 3 study and Cohort 2 was added to the protocol. In Cohort 2, an additional 300 subjects are being randomized to receive either placebo or

Table of Contents

1,800 mg/day of ganaxolone for 12 weeks. Cohort 2 is intended to meet the Phase 3 design and statistical power requirements and precedents for the FDA and other regulatory bodies for consideration as one of our adequate and well-controlled studies as part of an FDA or EMA filing package for registration. The primary endpoint of this trial is change in seizure frequency per month compared to baseline of Cohort 2 subjects after 14 weeks of treatment, at the end of the blinded phase of the study. We are capturing adverse events and other measures of safety as well as responder rate, seizure-free status and changes in seizure subtypes. We plan to release top line data in the first quarter of 2016.

We are planning to conduct a second Phase 3, double-blind, randomized fixed-dose study to confirm the efficacy, tolerability and safety of ganaxolone for adjunctive treatment of focal onset seizures in adults. This study, per European guidelines and United States precedents, will enroll similar patients as those in our ongoing trial: adult outpatients with drug-resistant focal onset seizures who require add-on therapy in addition to their current AEDs. The study will contain two or three fixed dose arms of ganaxolone versus placebo for 12 weeks of maintenance therapy. Change in seizure frequency compared to baseline will be the primary outcome measure. Patients who complete the study would be eligible to enroll in a one year open-label extension.

Ganaxolone is currently formulated as an oral suspension and as a capsule. Study 600 and 601 were conducted with an oral suspension formulation and Study 603 is being conducted with a capsule formulation. Pharmacokinetic studies are being planned to establish relative potency between the capsule and the suspension. Separate pharmacokinetic studies suggest that the capsule is approximately 20% more potent than the suspension.

Ganaxolone in PCDH19 Female Pediatric Epilepsy - An Orphan Disease

Overview of PCDH19 Female Pediatric Epilepsy

PCDH19 female pediatric epilepsy (PCDH19-FPE) is a rare and serious epileptic syndrome characterized by early-onset cluster seizures, cognitive and sensory impairment of varying degrees, and psychiatric and behavioral disturbances. The epileptic disorder is an X-linked condition caused by a missense mutation in the PCDH19 gene, which encodes for a calcium dependent cell-cell adhesion molecule that is expressed in the central nervous system (hippocampus, cerebral cortex, thalamus, amygdala) and which appears to be related to synaptic transmission and formation of synaptic connections during brain development. The mean age of onset of this condition is approximately 10 months (range of three-38 months). There is a genetic test available to determine whether or not a child has the PCDH19 mutation. Although formal epidemiologic data is not available, results from diagnostic screenings indicate that approximately 10% of girls who have seizure onset before five years of age have PCDH19 mutations. We estimate the PCDH19 population to be approximately 15,000 to 30,000 patients in the United States. These patients typically experience multiple types of seizures, including focal and generalized, as well as clusters of seizures that can last from one day to weeks. Many of these patients will experience developmental delay, intellectual disability and behavioral problems. Seizures often improve in adolescence, although developmental complications persist. Currently, there are no approved therapies for PCDH19-FPE.

Mechanism of Action

PCDH19 female pediatric epilepsy is caused by a mutation in the PCDH19 gene. This mutation results in impairment of GABAergic signaling both at the agonist and receptor levels. There is also indirect evidence linking progesterone/allopregnanolone to the onset and offset of seizures

in girls with PCDH19-FPE. It has been hypothesized that disturbances in certain neurosteroid hormones, such as allopregnanolone, may be implicated in the molecular pathogenesis of PCDH19-FPE. We believe that ganaxolone may be useful in the treatment of PCDH19 female pediatric epilepsy because it is a synthetic analog of allopregnanolone that can be used to increase GABAergic signaling in these patients. We also believe that data from our previously conducted studies of pediatric seizure disorders, which demonstrated ganaxolone's ability to treat multiple seizure types and showed a safety profile similar to that seen in adults, supports our rationale to pursue this rare disease as PCDH19 female pediatric patients may experience varying types of seizures.

Ongoing Clinical Trial in Patients with PCDH19 Female Pediatric Epilepsy

Based upon both proof-of-concept data for ganaxolone in the treatment of refractory pediatric seizures and a mechanistic rationale for providing a therapeutic benefit through increased GABAergic signaling we have initiated an expanded access protocol under our epilepsy investigational new drug application (IND) for an open label trial. This proof-of-concept Phase 2 trial is designed to enroll approximately 10 female pediatric patients between the ages of 2 and 10 years old, with a confirmed PCDH19 genetic mutation. After establishing baseline seizure frequency, patients will be treated with

Table of Contents

ganaxolone administered as either oral liquid suspension or capsules for up to 26 weeks. The primary endpoint of the study is percent change in seizure frequency per 28 days relative to baseline. We plan to release initial data from this study during 2015.

Clinical Trial in Refractory Pediatric Epilepsy

We also conducted an open-label clinical trial to evaluate ganaxolone as add-on therapy in children with refractory epilepsy of multiple seizure types including focal, absence, epileptic spasms, tonic and tonic-clonic. Forty-five subjects aged 1-13 years were treated at doses of up to 12 mg/kg, three times per day. Of the 29 subjects in the study at Week 8, the primary endpoint, twelve (41%) met responder criteria of having 50% or greater improvement from baseline.

Ganaxolone in Fragile X Syndrome, or FXS - An Orphan Disease

Overview and Treatment of FXS

FXS is a genetic condition that causes intellectual disability, behavioral and learning challenges and various physical characteristics. The impairment can range from learning disabilities to more severe cognitive or intellectual disabilities. 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. Approximately 1 in 151 women carry the Fragile X gene and could pass it to their children. Approximately 1 in 468 men carry the Fragile X gene and their daughters will also be carriers. Patients with FXS exhibit autism-like symptoms including cognitive impairment, anxiety and mood swings, attention deficit and heightened stimuli. Approximately 7% of women and 18% of men with FXS have seizures. People with FXS are affected throughout their lives. Currently, there are no known cures or approved therapies for FXS. Special education and symptomatic treatments are employed to lessen the burden of illness.

Market Opportunity

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. 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. Some patients with FXS benefit from medications that treat attention deficient disorders. Other patients who experience general anxiety, social anxiety and other chronic conditions may benefit from different types of anti-anxiety medications and other neuropsychiatric treatments.

Mechanism of Action

FXS arises from a mutation of a gene known as the *fmr1* gene in the coding for the Fragile X mental retardation protein. In a mouse model of this 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. As FXS symptoms may be diagnosed as early as infancy, it would be beneficial for a drug approved to treat FXS to have a safety profile acceptable for use in children as well as adults.

We believe that ganaxolone, with its high-affinity for extrasynaptic GABAA receptors, may increase signaling at existing receptors to normalize GABA function thereby reducing anxiety, hyperactivity and other disabilities associated with this inherited disorder.

Ongoing Clinical Trial (Study 800)

The MIND Institute at the University of California, Davis has been awarded a medical research grant from the DoD to study ganaxolone for treatment of behaviors in FXS in children and adolescents. The MIND Institute, in collaboration with Marinus, is conducting a randomized, placebo-controlled, Phase 2 proof-of-concept clinical trial at UC Davis and a site in Belgium. Approximately 60 subjects will be enrolled and titrated up to a maximum dose of 1,800 mg/day of ganaxolone or placebo over a two-week period followed by four weeks of treatment. At the end of the first treatment period and following a washout period, subjects are crossed over to the other treatment for a similar two-week titration period followed by four weeks of treatment.

Table of Contents

The primary outcome measure of the study is Clinical Global Impression Rating Scale for Improvement. Secondary outcome measures include the Aberrant Behaviors Checklist and ratings scales for specific behaviors associated with childhood FXS. We plan to complete this study during 2015.

Other Potential Future Development Programs for Ganaxolone

We believe that due to its mechanism of action, there is a rationale for ganaxolone therapy to provide a clinical benefit in a broad array of indications beyond our current trials. Such additional indications may include generalized anxiety disorder, PTSD, addictive disorders, catamenial epilepsy, perinatal depression, ADHD, and other neurodegenerative disorders. Additionally ganaxolone might be useful in several rare genetic disorders related to impaired GABA function such as the orphan indications Neimann Pick Disease, Type C, or status epilepticus. We would need to conduct additional clinical trials in order to obtain labeled indications for any of these programs.

The Injury and Traumatic Stress Consortium, or INTRuST, a group of PTSD treatment centers in the United States, has received government funding from the US Department of Defense to evaluate new treatments for PTSD. In collaboration with Marinus, INTRuST conducted a proof-of-concept study of ganaxolone versus placebo in adults with PTSD. The Phase 2 clinical trial was conducted at seven Veterans Administration centers in the United States. The study enrolled 114 adults with PTSD who were treated for six weeks with ganaxolone at 400-1200 mg per day or placebo in ascending doses followed by six weeks on open label ganaxolone.

The primary efficacy measure is the Clinician-Administered PTSD Scale, or CAPS, the standard rating assessment in treatment studies for PTSD, measured at the end of week 6. The CAPS measures levels of repetitive thoughts, startle, vigilance, avoidance and depression that are the main characteristics of PTSD. Additional efficacy ratings include measures of resilience, sleep, mood and overall global improvement. Treatment has been completed in this study. While the primary efficacy endpoint was not met in this study, some evidence of efficacy was seen in patients at the highest dose. Periodic reviews of the safety data from the study showed ganaxolone was safe in this population. While PTSD is not an indication that is part of our core development strategy, we may continue to work with third parties to fund and conduct future clinical trials.

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:

- ***Executing our registration studies and pursue regulatory approval for ganaxolone for adjunctive treatment of focal onset seizures and other epilepsy indications.*** Building on efficacy established in our two completed Phase 2 clinical trials, and a differentiated safety profile as demonstrated in extensive preclinical studies and trials in more than 1,000 subjects, we are executing a clinical program to support a registration filing for ganaxolone for adjunctive treatment of focal onset seizures in adults in the United States, Europe and other major markets. Additionally, if the results from our adjunctive focal onset seizure trials are positive, we plan to develop ganaxolone in other segments of the epilepsy market including for monotherapy and pediatric epilepsy. As a result of its efficacy and safety profile, we believe ganaxolone could be a meaningful treatment for epilepsy patients who do not achieve adequate seizure control from, have developed tolerance to, or have safety concerns with currently available therapies including concerns around reproductive toxicity.

- ***Pursuing orphan disease epilepsy indications for ganaxolone.*** Within epilepsy, there are several smaller patient populations where a genetic marker associated with the syndrome has been identified that is linked to deficits in GABAergic signaling. Increasing GABAergic tone with ganaxolone, a positive allosteric modulator of GABA receptors, might provide benefit. These small populations have the potential for more efficient paths to regulatory approval and commercialization. A proof-of-concept open label Phase 2 clinical trial is ongoing for ganaxolone in patients with PCDH19-FPE. We may explore development of ganaxolone in other rare disease epilepsy indications.

- ***Broadening dose forms to acute care setting.*** Our present ongoing clinical trials utilize our patented nanoparticulate composition administered in oral capsule and liquid suspension dose forms. As a complement to these orally administered dose forms, we are conducting the requisite preclinical experiments to ready our intravenous dose form for clinical use. We plan to evaluate ganaxolone IV for the acute care setting for in-hospital use and in patient populations that may benefit from both inpatient ganaxolone IV and an outpatient oral form for chronic administration.

Table of Contents

- ***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 developmental disorders. A proof-of-concept clinical trial is ongoing for ganaxolone in patients with FXS. We may explore development of ganaxolone in other neuropsychiatric disorders and rare disease neurology indications.
- ***Commercializing ganaxolone in the United States either alone or in collaboration with others.*** We intend to build sales and marketing infrastructure to reach high-prescribing neurologists and epilepsy specialists in the United States. We may seek co-promotion partners for our sales efforts to reach other United States physician groups, such as primary care physicians. We believe a focused sales and marketing organization could be leveraged to market ganaxolone in other neurology or psychiatry indications if we are able to obtain regulatory approval for those other indications.
- ***Establishing collaborations to develop and commercialize ganaxolone in territories outside the United States.*** We believe that there is significant market opportunity for ganaxolone in epilepsy and other neurological and psychiatric conditions outside of the United States. In order to capitalize on this opportunity, we may seek collaborations with pharmaceutical companies that have greater reach and resources by virtue of their size and experience in markets outside the United States.

Intellectual Property

The proprietary nature of, and protection for our product candidates and discovery programs and know-how are important to our business. We have sought patent protection in the United States and internationally for ganaxolone and use of ganaxolone nanoparticles in oral solid and liquid dose 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 was the discovery of a novel composition of nanoparticles and complexing agents that deliver consistent exposure and improved stability of ganaxolone. This discovery resulted in the issuance of United States and foreign patents, which cover use of these complexed ganaxolone nanoparticles in oral solid and liquid dose formulations. Our patent portfolio contains seven United States patents, two pending United States patent applications, 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, Japan, Mexico, New Zealand, Singapore and South Korea. Corresponding foreign patent applications are pending in China, Europe, India, Israel, Japan, Mexico, and South Africa. 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 have assigned patent rights, which rights were subsequently assigned to NovaMedica LLC, whereby we licensed our patents, along with the rights to develop and commercialize ganaxolone, in Russia and certain other eastern European nations.

Our patent portfolio also contains patents issued in Australia, United States, Europe and New Zealand 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, China, Eurasia, Hong Kong, Israel, India, Japan, Mexico, New Zealand and South Korea. We continue to prosecute applications in additional geographies.

We filed a provisional application in February 2015 directed to intravenous ganaxolone formulations and methods of using these formulations to treat refractory epileptic seizures and other disorders. If granted, this patent 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.

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

Table of Contents

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 ganaxolone 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 would 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 when a third-party patent is identified, 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 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

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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

Table of Contents

outside of the Covered Territory. Such purchases will be made on a cost-plus basis. 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 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 and terminates upon 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

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. 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,

Table of Contents

ten years from the first commercial sale of a licensed product in each country. We believe that we will not be obligated to pay royalties under the agreement because the underlying patents have either expired or are not applicable to ganaxolone. 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 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 with companies that treat neuropsychiatric disorders.

There are a variety of available therapies marketed for 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 payors. Insurers and other third-party payors 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 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.

Epileptic Seizures

Currently available AEDs control seizures through a variety of mechanisms, including modulation of voltage-activated sodium channels, voltage-activated calcium channels, increasing GABA signaling, and interactions with α -2 μ protein or synaptic vesicle protein SV2A. There are more than 15 approved AEDs available in the United States and worldwide. The top prescribed AEDs include the generic products levetiracetam, lamotrigine, carbamazepine, oxcarbazepine, valproic acid and topiramate. Decision Resources reports that these AEDs are used to treat a substantial percentage of epilepsy patients. Recent market entrants include Vimpat (UCB), Potiga (GlaxoSmithKline) and Fycompa (Eisai), and Aptiom (Sunovion Pharmaceuticals). In addition to ganaxolone there are two new chemical entities in late stage development that we are aware of, brivaracetam (UCB) and carisbamate (SK Life Science), while Sage Therapeutics is developing SAGE-547, an intravenous formulation of allopregnanolone for super-refractory status epilepticus.

Table of Contents

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. Companies developing compounds include Alcobra, Sunovion Pharmaceuticals, Afraxis and Neuren Pharmaceuticals.

PCDH19-FPE

There are no drugs approved for the treatment of PCDH19-FPE. PCDH19-FPE patients are typically prescribed drugs approved for epileptic seizures, which often times fail to control seizures in this patients population.

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 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 the discovery of our patented nanoparticulate formulation and novel manufacturing process for ganaxolone address the pharmacokinetic and cost of manufacturing challenges that previously encumbered the clinical and commercial feasibility of ganaxolone.

Ganaxolone is currently formulated as an oral suspension and as a capsule. We are planning a pharmacokinetic study to establish relative potency between the oral suspension to the capsule. Additionally, we have developed an intravenous formulation and a prototype for a tablet. We are presently conducting the requisite preclinical experiments to ready our intravenous dose form for clinical use.

Commercial Operations

If we obtain FDA approval for ganaxolone as an adjunctive treatment for patients with focal onset seizures we intend to build a sales and marketing infrastructure to reach high prescribing neurologist and epilepsy specialists in the United States. We believe a focused sales and marketing organization for epilepsy could be leveraged to market ganaxolone in other 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 is significant market opportunity for ganaxolone in epilepsy and other neurological and psychiatric conditions outside of the United States. In order to capitalize on this opportunity, 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 products 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,

Table of Contents

but country-specific regulation remains essential in many respects. The process of obtaining regulatory marketing approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations 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;
- 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 I.* 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

Table of Contents

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 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.

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

Table of Contents

the product's chemistry, manufacturing, controls and proposed labeling, among other things. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational product candidate to the satisfaction of the FDA.

In most cases, the NDA must be accompanied by a substantial user fee; there may be some instances in which the user fee is waived. The FDA will initially review the NDA for completeness before it accepts the NDA for filing. The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. After the NDA submission is accepted for filing, the FDA begins an in-depth review. The FDA has agreed to certain performance goals in the review of NDAs. Most such applications for standard review product candidates are reviewed within ten to twelve months. 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. The FDA may refer applications for novel product candidates which present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

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.

Table of Contents

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 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.

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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.

Table of Contents

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.

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

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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

Table of Contents

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 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

Table of Contents

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 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

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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;

Table of Contents

- 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 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

Table of Contents

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

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 payors. Third-party payors include government health administrative authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the drug product. Third-party payors 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 payors 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 medically necessary or cost-effective. A payor'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 payors 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.

Table of Contents

Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Other Healthcare Laws and Compliance Requirements

The federal Anti-Kickback Statute 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 payors 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 payor.

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.

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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,

Table of Contents

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 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 \$8.7 million and \$4.2 million in 2014 and 2013, respectively.

Employees

As of December 31, 2014, we had nine full-time employees. In addition to our full-time employees, we contract with third-parties for the conduct of certain clinical development, manufacturing, accounting and administrative activities. We anticipate increasing our head count. We have no collective bargaining agreements with our employees and none are represented by labor unions.

Table of Contents

Corporate Information

We were incorporated in Delaware in August 2003. Our principal executive offices are located at 3 Radnor Corporate Center, 100 Matsonford Rd, Suite 304, 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.

Risks Related to Our Financial Position and Capital Needs

We have a limited operating history, 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 we have only a limited operating history upon which you can evaluate our business and prospects. 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 for or 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 net losses of \$10.8 million for the year ended December 31, 2014. As of December 31, 2014, we had an accumulated deficit of \$72.3 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

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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 are able to successfully achieve 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 new drug applications, or NDAs, to the United States Food and Drug Administration, or 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;

Table of Contents

- develop a commercial organization capable of manufacturing, selling, marketing and distributing any products we intend to sell ourselves in the markets in which we choose to commercialize on our own;
- find suitable partners to help us market, sell and distribute our approved products in other markets; and
- obtain adequate pricing, coverage and reimbursement from third parties, including government and private payors.

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, which would depress the market price of our common stock.

We will require additional capital to fund our operations and if we fail to obtain necessary financing, we may be unable to complete the development and commercialization of ganaxolone.

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to advance the clinical development of ganaxolone and launch and commercialize ganaxolone, if we receive regulatory approval. We will require additional capital for the further development and potential commercialization of ganaxolone and may also need to raise additional funds sooner to pursue a more accelerated 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 and cash equivalents as of December 31, 2014, will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2016. We have based this estimate on assumptions that may prove to be wrong, and we could deploy 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;

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- 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;
- costs and timing of the implementation of commercial-scale manufacturing activities; and
- costs and timing of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval.

Table of Contents

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.

Raising additional capital may cause dilution to 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 other 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, for seizure disorders 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 for focal onset seizures, 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 indications or with 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 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. We have expanded our ongoing Phase 2b clinical trial so that it may serve as one of our adequate and well-controlled clinical trials for ganaxolone in epilepsy; however, we cannot be certain that the FDA will accept the trial as such. Even if ganaxolone were to successfully obtain approval from the FDA and comparable foreign regulatory authorities, any approval might contain significant limitations related to use restrictions for 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,

Table of Contents

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 payors. 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. Despite the results reported in earlier clinical trials for ganaxolone, 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. If later stage clinical trials 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 benefit as a therapeutic for focal onset seizures, PCDH19 female pediatric epilepsy, or PCDH19, and Fragile X Syndrome, or FXS, 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 its outcome is inherently uncertain. 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 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 clinical research organizations, or 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, or IRB, approval or the approval of other reviewing entities, including comparable foreign regulatory authorities, to conduct a clinical trial at each site;

Table of Contents

- 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, the IRB, 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, the 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;

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- 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;
- 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. 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

Table of Contents

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, or CNS, were categorized as severe as compared to side effects of other body systems, though no specific CNS side effect was reported as severe by more than one subject.

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:

- 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 future development and 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, or 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.

Table of Contents

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, or 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;

- 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.

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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, or the DOJ, the Office of Inspector General of the Department of Health and Human Services, or 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, engaging in impermissible promotion of ganaxolone for off-label uses 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

Table of Contents

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, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. If we are unable to obtain approval of ganaxolone by regulatory authorities in the 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 expect that we may in the future pursue orphan drug designations for ganaxolone for one or more indications, including behaviors in FXS and the treatment of PCDH19 female pediatric epilepsy, as well as certain of our future product candidates. However, obtaining an orphan drug designation can be difficult and we may not be successful in doing so for ganaxolone or any of our 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.

Table of Contents

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 clinical trial data from completed, ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our 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 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 payors and others in the medical community.

Even if ganaxolone receives regulatory approval, it may not gain market acceptance among physicians, patients, government and private payors, 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;

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- 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 payors;
- relative convenience and ease of administration; and
- effectiveness of our sales and marketing efforts.

Table of Contents

If ganaxolone is approved but fails to achieve market acceptance among physicians, patients, government or private payors 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 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;

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- 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.

Table of Contents

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 payors, 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 payors, such as private health insurers and health maintenance organizations, determine which medications they will cover and establish reimbursement levels. A primary trend in the United States healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for drugs. Third-party payors 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 payors 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 payors 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 payors 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 gamma-aminobutyric acid, or GABAA, 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 available marketed therapies available for these patients. Some of these other 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.

Specifically, there are more than 15 approved antiepileptic drugs, or AEDs, available in the United States and worldwide, including the generic products levetiracetam, lamotrigine, carbamazepine, oxcarbazepine, valproic

Table of Contents

acid and topiramate. Recent market entrants include branded products developed by UCB, GlaxoSmithKline, Eisai, and Sunovion Pharmaceuticals. Additionally, there are several drugs in development for the treatment of behavioral and mental health conditions associated with FXS, including compounds being developed by Roche, Sunovion Pharmaceuticals, Afraxis, Alcobra and Neuren Pharmaceuticals.

Many of the approved drugs are well established therapies or products and are widely accepted by physicians, patients and third-party payors. Insurers and other third-party payors 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 commercially sell ganaxolone after obtaining such regulatory approval. 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;

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- 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.

Table of Contents

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 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, or GLP, and the Animal Welfare Act requirements. We and our CROs are required to comply with federal regulations and Good Clinical Practices, or 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.

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

Table of Contents

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, or CMOs, for the chemical manufacture of active pharmaceutical ingredients for ganaxolone and another CMO for the production of the ganaxolone nanoparticulate formulation into capsules. 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.

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

Table of Contents

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, or the DoD, for such studies and trials. In addition, our preclinical studies and clinical trials to evaluate ganaxolone in patients suffering from posttraumatic stress disorder, or 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 reflect the government's 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;

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- audit contract-related costs and fees, including allocated indirect costs;
- suspend the contractor from receiving new contracts pending resolution of alleged violations of procurement laws or regulations;
- impose United States manufacturing requirements for products that embody inventions conceived or first reduced to practice under such agreements;
- suspend or debar the contractor from doing future business with the government; and
- control and potentially prohibit the export of products.

We may not have the right to prohibit the United States government from using or allowing others to use certain technologies developed by us, and we may not be able to prohibit third party companies, including our competitors, from using those technologies in providing products and services to the United States government. The

Table of Contents

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. Although we believe that our manufacturers' procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical, radioactive or hazardous materials. As a result of any such contamination or injury we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical radioactive or hazardous materials. Compliance with applicable

environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

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

Table of Contents

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 payors 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 payors.

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, or the 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, or 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, 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 payors 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.

Table of Contents

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, or FCPA, prohibits any United States individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring such companies to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the DOJ. The SEC is involved with enforcement of the books and records provisions of the FCPA.

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

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

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

Our relationships with customers and third-party payors 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 payors 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 payors 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 payors, 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:

Table of Contents

- 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, or 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, or 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
- 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 payors, 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 proprietary 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

Table of Contents

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 proprietary 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 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.

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 proprietary technologies. We may become party to, or threatened with, future 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, or 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

Table of Contents

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 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.

If we breach our license agreement with Purdue Neuroscience Company, it could have a material adverse effect on our commercialization efforts for ganaxolone or such other compounds in the United States.

In September 2004, we entered into a license agreement with Purdue Neuroscience Company, or Purdue, which was most recently amended and restated in May 2008, granting us exclusive rights to certain know-how and technology relating thereto, 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. If we materially breach or fail to perform any provision under this license agreement (including failure to make payments to Purdue when due for royalties and other sub-license revenue, failure to cure a breach for failure to use commercially reasonable efforts to develop and commercialize at least one licensed product, and commencement of bankruptcy or insolvency proceedings against us) Purdue has the right to terminate our license, and upon the effective date of such termination, we must cease all activities licensed all rights, data, information, know-how, and material licensed or transferred to us under this license agreement will revert to Purdue and all rights, data, information, know-how, material, records and registrations developed or made by us that relate in whole or in part to the activities contemplated by our amended and restated license agreement with Purdue will be transferred to Purdue. To the extent such a breach relates to ganaxolone, we would expect to exercise all rights and remedies available to us, including attempting to cure any breach by us, and otherwise seek to preserve our rights, but we may not be able to do so in a timely manner, at an acceptable cost to us or at all. Any uncured, material breach under the license agreement could result in our loss of rights to practice our patent rights and could have a material adverse effect on our commercialization efforts for ganaxolone.

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 do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, 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. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our or our licensors' patents or marketing of competing products in violation of our proprietary rights generally in those countries. 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.

The laws of certain foreign countries may not protect our rights to the same extent as the laws of the United States, and these foreign laws may also be subject to change. 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

Table of Contents

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.

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.

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, or the 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.

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The USPTO is currently developing regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, did not become effective until March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

Table of Contents

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 become involved in lawsuits to protect or enforce our intellectual property, which could be expensive, time consuming and unsuccessful and have a material adverse effect on the success of our business.

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.

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

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

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:

Table of Contents

- 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, Managing Growth and Becoming a Public Company

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

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We are highly dependent upon Christopher M. Cashman, our Chief Executive Officer, Edward F. Smith, our Chief Financial Officer, Albena I. Patroneva, M.D., our Chief Medical Officer and Gail M. Farfel, Ph.D., our Chief Clinical Development and Regulatory 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, 2014, we had nine full-time employees. As our development and commercialization plans and strategies develop, or as a result of any future acquisitions, we will need additional managerial, operational, sales, marketing, financial and other resources. In addition, it may become more cost effective to bring in house certain resources currently outsourced to consultants and other third-parties. Our management, personnel and systems currently in place may not be adequate to support our future growth. Future growth would impose significant added responsibilities on members of management, including:

- managing our clinical trials effectively;

- identifying, recruiting, maintaining, motivating and integrating additional employees;

- managing our internal development efforts effectively while complying with our contractual obligations to licensors, licensees, contractors and other third parties;

- improving our managerial, development, operational and finance systems; and

- expanding our facilities.

As our operations expand, we will need to manage additional relationships with various strategic partners, suppliers and other third parties. Our future financial performance and our ability to commercialize ganaxolone, if

Table of Contents

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. 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.

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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, or the 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.

Table of Contents

Risks Related to Ownership of Our Common Stock

We do not know whether an active, liquid and orderly trading market will develop for our common stock and as a result it may be difficult for you to sell your shares of our common stock.

Although we are listed on The NASDAQ Global Market, an active trading market for our shares may never develop or be sustained. The market value of our common stock may decrease. The lack of an active market may impair your ability to sell your shares at the time you wish to sell them or at a price that you consider reasonable. The lack of an active market may also reduce the fair market value of your shares. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into collaborations or acquire companies or products by using our shares of common stock as consideration.

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;

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- 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;
- 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.

Table of Contents

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 82% 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.

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We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or 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

Table of Contents

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.

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

Now that we are a public company, we are incurring significant legal, accounting and other expenses that we did not incur as a private company, and these expenses may increase even more after we are no longer an emerging growth company. We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Protection Act, as well as rules adopted, and to be adopted, by the SEC and NASDAQ Stock Market. Our management and other personnel need to devote a substantial amount of time to these compliance initiatives. Moreover, we expect these rules and regulations to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. We estimate that we will incur approximately \$1.0 million to \$2.0 million in incremental costs per year associated with being a publicly traded company, although it is possible that our actual incremental costs will be higher than we currently estimate. The increased costs will increase our net loss. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain sufficient coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

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, entered into in April 2014 and amended in December 2014, 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 after the lock-up and other legal restrictions on resale lapse, the trading price of our common stock could decline. As of December 31, 2014, we had outstanding a total of 14,007,754 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, the lock-up agreements and 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, 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. The number of

Table of Contents

shares of our common stock available for future grant under our 2005 Stock Option and Incentive Plan, as amended, and our 2014 Equity Incentive Plan was zero as of December 31, 2014. In accordance with our 2014 Equity Incentive Plan, on January 1, 2015, 560,310 shares of common stock became 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 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.

Table of Contents

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

Our principal offices occupy approximately 4,000 square feet of leased office space in Radnor, Pennsylvania pursuant to a lease agreement that expires in 2020. 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.

Table of Contents**PART II****Item 5. Market for Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.***Market Information*

Our common stock has been listed on the NASDAQ Global Market under the symbol MRNS since July 31, 2014. Prior to that time, there was no public market for our stock. 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, 2014 | | | |
|------------------------------------|------------------------------|-------|----|------|
| | | High | | Low |
| Third Quarter (from July 31, 2014) | \$ | 10.58 | \$ | 5.49 |
| Fourth Quarter | \$ | 11.18 | \$ | 4.00 |

Holder of Record

As of March 12, 2015 there were approximately 100 holders of record of shares of our common stock.

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.

Recent Sales of Unregistered Securities

None.

Use of Proceeds

Our initial public offering 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 was exercised in September 2014) at a public offering price of \$8.00 per share for an aggregate public offering price of \$46.1 million. Stifel, Nicolaus & Company, Incorporated and JMP Securities LLC acted as joint book-running managers for the initial public offering. Oppenheimer & Co. acted as lead manager and Janney Montgomery Scott acted as co-manager for the initial public offering.

As a result of the initial public offering, we received net proceeds of approximately \$41.2 million during the third 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. None of such payments were direct or indirect payments to any of (i) our directors or officers or their associates, (ii) persons owning 10 percent or more of our common stock, or (iii) our affiliates.

There has been no material change in the planned 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, 2014, we have used approximately \$2.5 million of the funds received from our initial public offering (IPO) for clinical trials and payments to research and development consultants

Item 6. Selected Financial Data

Not applicable for smaller reporting company.

Table of Contents

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 neuropsychiatric therapeutics. Our clinical stage product candidate, ganaxolone, is a small molecule that is a synthetic analog of allopregnanolone, an endogenous neurosteroid produced in the central nervous system and known for its anticonvulsive and antianxiety activity. By targeting the same spectrum of GABAA receptors as endogenous allopregnanolone, ganaxolone delivers its therapeutic benefit through a natural mechanism that we believe may offer safety and efficacy advantages compared to other marketed antiepileptic medications. Ganaxolone was rationally designed to unlock the potential for chronic neurosteroid therapy through modulation of the GABAA receptor. We have a dual strategy of evaluating ganaxolone for treating seizure disorders, and treating targeted orphan diseases for which there are no approved therapies, the development timelines may be abbreviated, and for which there is a strong mechanistic rationale for ganaxolone to offer therapeutic benefit to patients with very high unmet needs. Our orally administered solid and liquid suspension dose forms are being evaluated in our ongoing clinical trials and we are conducting the requisite preclinical experiments to ready our intravenous, or IV, dose form for clinical use.

Our lead indication for ganaxolone is as an adjunctive, or add-on, therapy for the treatment of partial, also known as focal, onset seizures in adults with epilepsy. We have completed a Phase 2 clinical trial in 147 patients with focal onset seizures demonstrating that patients who added ganaxolone to their medication regimen experienced a statistically significant reduction in seizures as compared to patients who added placebo. We are currently enrolling patients in a multinational, randomized, placebo-controlled, Phase 3 clinical trial to evaluate ganaxolone as adjunctive treatment of partial-onset seizures in adult subjects. We believe ganaxolone also has potential in a broad range of neuropsychiatric disorders, including orphan indications. We have generated proof-of-concept data for ganaxolone in the treatment of refractory pediatric seizures and as monotherapy for adult refractory focal onset seizures. We currently have a Phase 2 proof-of-concept clinical study on-going with ganaxolone for the treatment of PCDH19 female pediatric epilepsy and a Phase 2 proof-of-concept investigator sponsored clinical trial evaluating ganaxolone as a treatment for behaviors in Fragile X Syndrome. Both PCDH19 female pediatric epilepsy and Fragile X Syndrome are potential orphan disorders that have been related to mutations affecting neurosteroid signaling at extrasynaptic GABAA receptors.

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. From inception through December 31, 2014, we have received net proceeds of \$110.4 million from the issuance of preferred stock, common stock and convertible notes payable. At December 31, 2014, we had cash and cash equivalents of \$49.7 million. In connection with our initial public offering, which closed during the third quarter of 2014, we received net proceeds of \$41.2 million from the sale of 5,758,000 shares of our common stock. We have no products currently available for sale and substantially all of our revenue to date has been derived from research grants. We have incurred operating losses since inception, have not generated any product sales revenue and have not achieved profitable operations. We incurred net losses of \$10.8 million for the year ended December 31, 2014. Our accumulated deficit as of December 31, 2014 was \$72.3 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:

- increase the targeted enrollment and add enrollment sites and geographies for our ongoing Phase 3 clinical trial for adjunctive treatment of ganaxolone in adult patients with refractory partial onset epileptic seizures;
- conduct clinical proof-of-concept clinical trials in targeted pediatric rare disease indications, including

Table of Contents

PCDH19 and FXS;

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

In addition, we have incurred and will continue to incur significant expenses as a result of becoming a public company, which subjects us to the reporting requirements of the Securities Exchange Act of 1934, as amended (the Exchange Act), the Sarbanes-Oxley Act of 2002 and the rules and regulations of The NASDAQ Global Market. Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we will be required to furnish a report by our management on our internal control over financial reporting. Commencing with our fiscal year ending December 31, 2015, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in our Form 10-K filing for that year, as required by Section 404.

We believe that our cash and cash equivalents as of December 31, 2014 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2016. 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.

Financial Overview

Research and Development Expenses

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

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- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;
- expenses incurred under agreements with Clinical Research Organizations, or 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, or sNDAs, for ganaxolone in patients with focal onset seizures, PCDH19, FXS and other target 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.

Table of Contents

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 payors 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 new management and employee hiring and our scaling operations commensurate with supporting more advanced clinical trials and public company infrastructure. These increases will likely include increased costs for insurance, hiring of additional personnel, outside consultants, investor relations, legal counsel and accountants, among other expenses.

Change in Fair Value of Warrant Liability

Our warrants to purchase our 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 as of December 31, 2014.

Interest Income

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

Interest Expense

Interest expense is primarily attributable to interest expense associated with our previously outstanding convertible notes and our credit facility entered into in April 2014, and amended in December 2014.

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. We are no longer recording preferred stock dividends effective upon the closing of our initial public offering, which occurred during the third quarter of 2014.

Results of Operations

Research and Development Expenses

Research and development expenses increased \$4.5 million, to \$8.7 million, for the year ended December 31, 2014, compared to the same period of 2013. The increase resulted primarily from an increase in clinical costs related to our ongoing clinical trial of ganaxolone in patients with focal onset seizures that commenced at the end of 2013. Substantially all research and development expenses relate to our clinical trial in focal onset epileptic seizures.

General and Administrative Expenses

General and administrative expenses increased \$2.0 million, to \$3.2 million, for the year ended December 31, 2014, compared to the same period of 2013. 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 new public company status and ongoing clinical trial of ganaxolone in patients with focal onset seizures that commenced during 2013.

Table of Contents

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 and \$0.2 million for the years ended December 31, 2014 and 2013, respectively. 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 decreased \$1.3 million, to \$2.5 million, for the year ended December 31, 2014, compared to the same period of 2013. Upon conversion of all outstanding convertible preferred stock in connection with our initial public offering, all cumulative preferred stock dividends were canceled.

Liquidity and Capital Resources

Since inception, we have incurred net losses and negative cash flows from our operations. We incurred net losses of \$10.8 million and \$5.3 million for the years ended December 31, 2014 and 2013, respectively. Our cash used in operating activities was \$8.6 million for the year ended December 31, 2014 compared to \$6.6 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. From inception through December 31, 2014, we have received net proceeds of \$110.4 million from the issuance of preferred stock, common stock and convertible notes payable. At December 31, 2014, we had cash and cash equivalents of \$49.7 million.

During the third quarter of 2014, we completed our initial public offering. In connection with the initial public offering, we sold a total of 5,758,000 shares of common stock and received aggregate net proceeds, after underwriting discounts and commissions and other estimated offering expenses, of approximately \$41.2 million, and all of our outstanding shares of convertible preferred stock converted into shares of common stock.

Square 1 Credit Facility

In April 2014, we borrowed \$2.0 million in connection with a term loan pursuant to a Loan and Security Agreement (LSA) we entered into with Square 1 Bank (Square 1). 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.

In December 2014, we entered into a First Amendment to Loan and Security Agreement (Amended LSA) with Square 1. The Amended LSA increased the total term loan availability from \$2.0 million to \$12.0 million, available in four tranches (in thousands):

| Tranche | Term Loan Available | Term Loan Borrowed | Borrowed Date |
|----------------|------------------------------------|-----------------------------------|----------------------|
| A | \$ 2,000 | \$ 2,000 | April 2014 |
| B | 5,000 | 5,000 | December 2014 |
| C | 2,500 | | * |
| D | 2,500 | | * |
| | \$ 12,000 | \$ 7,000 | |

* Our ability to borrow under the remaining tranches of \$2.5 million each depends upon meeting certain clinical trial milestones. The availability end dates of Tranches C and D are September 1, 2015 and March 31, 2016, respectively.

As of December 31, 2014, our outstanding term loan balance of \$7.0 million was classified as long-term debt on our balance sheet. In connection with the execution of both the LSA and Amended LSA, we paid debt costs to the financial institution of \$25 thousand, which are being amortized into interest expense over the remaining term of the loans. Interest expense related to the loans was \$117 thousand for the year ended December 31, 2014. As of December 31, 2014, we had accrued interest of \$39 thousand. There are no financial covenants associated with these term loans. As of December 31, 2014, we were in compliance with all non-financial covenants.

Table of Contents

Cash Flows

Operating Activities. Cash used in operating activities increased to \$8.6 million for the year ended December 31, 2014 compared to \$6.6 million for the same period a year ago. The increase was driven primarily by an increase in our net loss of \$5.6 million and an increase in our noncash gain on our warrant liability of \$1.0 million. This was partially offset by net decreases in operating assets and increases in operating liabilities of \$4.3 million, and increases in stock-based compensation expense of \$0.5 million. The net decreases in operating assets and increases in operating liabilities for the year ended December 31, 2014 compared to the same period a year ago are primarily due to \$1.7 million in prepaid clinical trial expenses from 2013 being expensed throughout 2014, as well as additional accrued clinical trial expenses of \$0.7 million as of December 31, 2014.

Investing Activities. Cash used in investing activities for the purchase of property and equipment was less than \$0.1 million for the years ended December 31, 2014 and 2013.

Financing Activities. Cash provided by financing activities increased to \$48.3 million for the year ended December 31, 2014 compared to \$8.0 million for the same period a year ago. The increase is primarily attributable to \$41.2 million of net proceeds received in 2014 from the closing of our initial public offering and \$7.0 million received in 2014 related to our credit facility with Square 1 Bank, offset by \$7.5 million received in 2013 related to the issuance of Series C Preferred Stock.

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 will incur significant legal, accounting and other expenses associated with being a public company that we were not required to incur as a private company. In addition, Section 404, as well as rules adopted by the SEC and The NASDAQ Stock Market, require public companies to implement specified corporate governance practices that were previously inapplicable to us as a private company. We expect these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

We believe that our cash and cash equivalents as of December 31, 2014, will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2016. However, we will need to raise substantial additional financing in the future to fund our operations. In order to meet these additional cash requirements, we may seek to sell additional equity or convertible debt securities that may result in dilution to our stockholders. If we raise additional funds through the issuance of convertible debt securities, these securities could have rights senior to those of our common stock and could contain covenants that restrict our operations. There can be no assurance that we will be able to obtain additional equity or debt financing on terms acceptable to us, if at all. 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;

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- 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;

Table of Contents

- 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, 2014 (in thousands):

| Contractual Obligations | Total | Payment due by period | | | |
|--------------------------------|-----------------|-----------------------|-----------------|---------------|----------------------|
| | | Less than 1 year | 1 - 3 years | 3 - 5 years | More than 5 years |
| Operating lease obligations(1) | \$ 681 | \$ 100 | \$ 397 | \$ 184 | \$ |
| Square 1 credit facility(2) | 7,943 | 462 | 7,481 | \$ | \$ |
| Total | \$ 8,624 | \$ 562 | \$ 7,878 | \$ 184 | \$ |

(1) Represents commitments for future minimum lease payments.

(2) Represents principal and interest payment obligations that will become due in connection with the 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

Table of Contents

life of the option, risk-free interest rates, and 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 through financial models 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 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 June 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-10, *Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation*, which eliminates all incremental financial reporting requirements for development stage entities by removing Accounting Standards Codification

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(ASC) Topic 915, *Development Stage Entities*, from the FASB Accounting Standards Codification. ASC Topic 915 is removed effective for annual periods beginning after December 15, 2014 and early adoption is permitted. The Company adopted the ASU effective with the issuance of the June 30, 2014 interim financial statements.

In August 2014, the FASB issued ASU No. 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 is effective for annual periods ending after December 15, 2016, and interim periods thereafter, with early adoption permitted. The company is currently evaluating the impact the adoption of this ASU will have on its financial statements.

Table of Contents

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 and cash equivalents of \$49.7 million at December 31, 2014, consisting primarily of funds in cash and money market accounts. 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 short-term 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. has remained at 3.25% since December of 2008. While we cannot predict when, if at all, this rate will be increased, 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 principal financial and accounting officer, to allow timely decisions regarding required disclosures.

Management's Report on Internal Control Over Financial Reporting

This annual report does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of the Company's registered public accounting firm due to a transition period established by rules of the SEC for newly public companies.

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

Table of Contents

December 31, 2014 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

On March 11, 2015, we entered into amended and restated employment agreements with Christopher M. Cashman and Edward F. Smith and an employment agreement with Albena Patroneva, each of which is described in this Item 9B.

Christopher M. Cashman

On March 11, 2015, we entered into an amended and restated employment agreement with Christopher M. Cashman, our Chairman, President and Chief Executive Officer. The principal terms of Mr. Cashman's employment agreement are as follows:

- base salary of \$470,000 per year;
- annual performance bonus in an amount up to 50.0% of base salary based on the achievement of certain performance goals established by our Board or the compensation committee; and
- stock options and awards as described below under the heading Outstanding Equity Awards at Fiscal Year-End.

Upon a termination of Mr. Cashman's employment by us without cause or a resignation by Mr. Cashman for good reason, Mr. Cashman is eligible to receive a continuation of his base salary for twelve months, with an accelerated payment of any balance upon a change in control as defined in the agreement, subject to his execution and delivery of a general release of claims. If such termination occurs within three months before or within twelve months after a change in control the severance payable increase to an amount equal to his base salary for a period of twenty-four months plus his prorated target bonus payable in a lump sum. Upon such termination, Mr. Cashman is also eligible to receive payment or reimbursement of his medical insurance premiums at the same level as was in effect on the termination date for a period of twelve months, which period increases to twenty-four months if the termination of employment occurs three-months before or twelve months after a change in control.

Termination for cause under Mr. Cashman's employment agreement generally means termination of Mr. Cashman by us for: (i) his misuse of alcoholic beverages, controlled substances or other narcotics, which misuse has had or is reasonably likely to have a material adverse effect on our business or financial affairs or our reputation; (ii) failure to cooperate with us in any investigation or formal proceeding; (iii) the commission of, or a plea of guilty or nolo contendere with respect to, or conviction for, a felony (or any lesser included offense or crime in exchange for withdrawal of a felony indictment or charged crime that might result in a penalty of incarceration), a crime involving moral turpitude or any other offense that results in or could result in any prison sentence; (iv) adjudication as an incompetent; (v) a breach of any material term of the

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employment agreement; (vi) violation in any material respect of any of our rules, regulations or policies; (vii) gross insubordination; (viii) engaging in any conduct, action or behavior that, in the reasonable opinion of our Board, has had a material adverse effect on our reputation; (ix) any continued or repeated absence; or (x) misappropriation of any funds or property.

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

Table of Contents

Mr. Cashman is entitled to participate in all of our group welfare plans, subject to the terms and conditions applicable to such plans as approved from time to time by our Board. Mr. Cashman's employment agreement contains customary non-solicitation and non-competition covenants, which covenants remain in effect for one year following any cessation of employment with respect to Mr. Cashman. Further, Mr. Cashman has executed a Mutual Non-Disclosure Agreement.

Edward F. Smith

On March 11, 2015, we entered into an amended and restated employment agreement with Edward F. Smith, our Vice President, Chief Financial Officer, Treasurer and Secretary. The principal terms of Mr. Smith's employment agreement are as follows:

- base salary of \$350,000 per year;
- annual performance bonus in an amount up to 35.0% of base salary based on the achievement of certain performance goals established by our Board or the compensation committee; and
- stock options and awards as described below under the heading Outstanding Equity Awards at Fiscal Year-End.

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

Termination for cause under Mr. Smith's employment agreement generally means termination of Mr. Smith by us for: (i) his misuse of alcoholic beverages, controlled substances or other narcotics, which misuse has had or is reasonably likely to have a material adverse effect on our business or financial affairs or our reputation; (ii) failure to cooperate with us in any investigation or formal proceeding; (iii) the commission of, or a plea of guilty or nolo contendere with respect to, or conviction for, a felony (or any lesser included offense or crime in exchange for withdrawal of a felony indictment or charged crime that might result in a penalty of incarceration), a crime involving moral turpitude or any other offense that results in or could result in any prison sentence; (iv) adjudication as an incompetent; (v) a breach of any material term of the employment agreement; (vi) violation in any material respect of any of our rules, regulations or policies; (vii) gross insubordination; (viii) engaging in any conduct, action or behavior that, in the reasonable opinion of our Board, has had a material adverse effect on our reputation; (ix) any continued or repeated absence; or (x) misappropriation of any funds or property.

Termination for good reason under Mr. Smith's employment agreement generally means termination by Mr. Smith for (i) a reassignment to a location outside the greater Philadelphia area; (ii) any material failure by us to comply with any material term of the employment agreement;

(iii) the demotion of Mr. Smith to a lesser position or a substantial diminution of Mr. Smith's authority, duties or responsibilities or (iv) a material diminution of his base salary and benefits, except under certain limited circumstances.

Mr. Smith is entitled to participate in all of our group welfare plans, subject to the terms and conditions applicable to such plans as approved from time to time by our Board. Mr. Smith's employment agreement contains customary non-solicitation and non-competition covenants, which covenants remain in effect for six months following any cessation of employment with respect to Mr. Smith. Further, Mr. Smith has executed a Confidentiality Agreement which expires five years after the last disclosure of confidential information by the Company.

Table of Contents

Albena Patroneva, M.D.

On March 11, 2015, we entered into an employment agreement with Albena Patroneva, our Chief Medical Officer. The principal terms of Dr. Patroneva's employment agreement are as follows:

- base salary of \$335,000 per year;
- annual performance bonus in an amount up to 35.0% of base salary based on the achievement of certain performance goals established by our Board or the compensation committee; and
- stock options and awards as described below under the heading Outstanding Equity Awards at Fiscal Year-End.

Upon a termination of Dr. Patroneva's employment by us without cause or a resignation by Dr. Patroneva for good reason, Dr. Patroneva is eligible to receive a continuation of her base salary for nine months, with an accelerated payment of any balance upon a change in control as defined in the agreement, subject to her execution and delivery of a general release of claims. If such termination occurs within three months before or within twelve months after a change in control the severance payable increase to an amount equal to her base salary for a period of eighteen months payable in a lump sum. Upon such termination, Dr. Patroneva is also eligible to receive payment or reimbursement of the her medical insurance premiums at the same level as was in effect on the termination date for a period of nine months, which period increases to eighteen months if the termination of employment occurs three months before or twelve months after a change in control.

Termination for cause under Dr. Patroneva's employment agreement generally means termination of Dr. Patroneva by us for: (i) her misuse of alcoholic beverages, controlled substances or other narcotics, which misuse has had or is reasonably likely to have a material adverse effect on our business or financial affairs or our reputation; (ii) failure to cooperate with us in any investigation or formal proceeding; (iii) the commission of, or a plea of guilty or nolo contendere with respect to, or conviction for, a felony (or any lesser included offense or crime in exchange for withdrawal of a felony indictment or charged crime that might result in a penalty of incarceration), a crime involving moral turpitude or any other offense that results in or could result in any prison sentence; (iv) adjudication as an incompetent; (v) a breach of any material term of the employment agreement; (vi) violation in any material respect of any of our rules, regulations or policies; (vii) gross insubordination; (viii) engaging in any conduct, action or behavior that, in the reasonable opinion of our Board, has had a material adverse effect on our reputation; (ix) any continued or repeated absence; or (x) misappropriation of any funds or property.

Termination for good reason under Dr. Patroneva's employment agreement generally means termination by Dr. Patroneva for (i) a reassignment to a location outside the greater Philadelphia area; (ii) any material failure by us to comply with any material term of the employment agreement; (iii) the demotion of Dr. Patroneva to a lesser position or a substantial diminution of Dr. Patroneva's authority, duties or responsibilities or (iv) a material diminution of her base salary and benefits, except under certain limited circumstances.

Dr. Patroneva is entitled to participate in all of our group welfare plans, subject to the terms and conditions applicable to such plans as approved from time to time by our Board. Dr. Patroneva's employment agreement contains customary non-solicitation and non-competition covenants,

which covenants remain in effect for six months following any cessation of employment with respect to Dr. Patroneva. Further, Dr. Patroneva has executed a Confidentiality Agreement which expires five years after the last disclosure of confidential information by the Company.

Table of Contents

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 2015 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 2015 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 2015 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 2015 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 2015 annual meeting of shareholders, to be filed with the SEC.

Item 15. Exhibits and Financial Statement Schedules.

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(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 March 11, 2015 between the Company and Christopher M. Cashman. (Filed herewith.) |
| 10.4+ | Amended and Restated Employment Agreement dated as of March 11, 2015 between |
| 10.5+ | Employment Agreement dated as of November 2, 2012 between the Company and Gail M. Farfel. (Incorporated by reference to Exhibit 10.5 to Form S-1 registration statement filed on May 12, 2014.) |

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Table of Contents

| Exhibit No. | Description of Exhibit |
|--------------------|--|
| | the Company and Edward F. Smith. (Filed herewith.) |
| 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. (Filed herewith in corrected form.) |
| 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+ | Employment Agreement dated as of March 11, 2015 between the Company and Albenia Patroneva. (Filed herewith.) |
| 10.16+ | Form of Incentive Stock Option Agreement for Officers Under 2014 Equity Incentive Plan. (Filed herewith.) |
| 10.17+ | Form of Incentive Stock Option Agreement for Employees Under 2014 Equity Incentive Plan. (Filed herewith.) |
| 10.18+ | Form of Nonqualified Stock Option Agreement Under 2014 Equity Incentive Plan. (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 |

+ 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.

Table of Contents

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 12, 2015 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 12, 2015 |
| /s/ Edward F. Smith Edward F. Smith | Vice President, Chief Financial Officer, Secretary and Treasurer (Principal Finance and Accounting Officer) | March 12, 2015 |
| /s/ Stephen Bloch Stephen Bloch, M.D. | Director | March 12, 2015 |
| /s/ Enrique J. Carrazana Enrique J. Carrazana, M.D. | Director | March 12, 2015 |
| /s/ Anton Gopka Anton Gopka | Director | March 12, 2015 |
| /s/ Tim M. Mayleben Tim M. Mayleben | Director | March 12, 2015 |
| /s/ Anand Mehra Anand Mehra, M.D. | Director | March 12, 2015 |
| /s/ Jay P. Shepard Jay P. Shepard | Director | March 12, 2015 |
| /s/ Nicole Vitullo Nicole Vitullo | Director | March 12, 2015 |

Table of Contents

FINANCIAL STATEMENTS

MARINUS PHARMACEUTICALS, INC.

INDEX TO FINANCIAL STATEMENTS

CONTENTS

| | Page |
|--|-------------|
| <u>Report of Independent Registered Public Accounting Firm</u> | F-2 |
| <u>Balance Sheets as of December 31, 2014 and 2013</u> | F-3 |
| <u>Statements of Operations for the Years Ended December 31, 2014 and 2013</u> | F-4 |
| <u>Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit) for the Years Ended December 31, 2014 and 2013</u> | F-5 |
| <u>Statements of Cash Flows for the Years Ended December 31, 2014 and 2013</u> | F-6 |
| <u>Notes to Financial Statements</u> | F-7 |

Table of Contents

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, 2014 and 2013, and the related statements of operations, convertible preferred stock and stockholders' equity (deficit), and cash flows for the years then ended. 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 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, 2014 and 2013, and the results of its operations and its cash flows for the years then ended, in conformity with U.S. generally accepted accounting principles.

/s/ KPMG LLP

Philadelphia, Pennsylvania
March 12, 2015

Table of Contents**MARINUS PHARMACEUTICALS, INC.****BALANCE SHEETS****(In thousands, except share and per share amounts)**

| | 2014 | December 31, | 2013 |
|---|-----------|--------------|----------|
| ASSETS | | | |
| Current assets: | | | |
| Cash and cash equivalents | \$ 49,720 | \$ | 10,037 |
| Prepaid expenses and other current assets | 428 | | 1,762 |
| Total current assets | 50,148 | | 11,799 |
| Property and equipment, net | 44 | | 16 |
| Other assets | 21 | | 9 |
| Total assets | \$ 50,213 | \$ | 11,824 |
| LIABILITIES AND STOCKHOLDERS EQUITY (DEFICIT) | | | |
| Current liabilities: | | | |
| Accounts payable | \$ 536 | \$ | 78 |
| Accrued expenses | 1,503 | | 1,096 |
| Warrant liability | | | 1,192 |
| Total current liabilities | 2,039 | | 2,366 |
| Notes payable | 7,000 | | |
| Other long term liabilities | 20 | | |
| Total liabilities | 9,059 | | 2,366 |
| Commitments and contingencies (Note 9) | | | |
| Series A convertible preferred stock, \$0.001 par value; 18,777,860 shares authorized, issued and outstanding at December 31, 2013 | | | 30,596 |
| Series B convertible preferred stock, \$0.001 par value; 15,275,824 shares authorized, 12,220,661 shares issued and outstanding at December 31, 2013 | | | 17,929 |
| Series C convertible preferred stock, \$0.001 par value; 18,900,000 shares authorized, 18,381,463 shares issued and outstanding at December 31, 2013 | | | 21,314 |
| Stockholders' equity (deficit): | | | |
| 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, 14,036,985 issued and 14,007,754 outstanding at December 31, 2014 and 494,260 issued and 465,029 outstanding at December 31, 2013 | | | 1 |
| Additional paid-in capital | 113,476 | | 1,121 |
| Treasury stock at cost, 29,231 shares at December 31, 2014 and 2013 | | | |
| Accumulated deficit | (72,336) | | (61,503) |
| Total stockholders' equity (deficit) | 41,154 | | (60,381) |
| Total liabilities and stockholders' equity (deficit) | \$ 50,213 | \$ | 11,824 |

See accompanying notes to financial statements.

Table of Contents**MARINUS PHARMACEUTICALS, INC.****STATEMENTS OF OPERATIONS****(In thousands, except share and per share amounts)**

| | Year Ended December 31, | |
|---|--------------------------------|-------------|
| | 2014 | 2013 |
| Expenses: | | |
| Research and development | \$ 8,690 | \$ 4,150 |
| General and administrative | 3,230 | 1,229 |
| Loss from operations | (11,920) | (5,379) |
| Change in fair value of warrant liability | 1,192 | 153 |
| Interest income | 12 | 47 |
| Interest expense | (117) | (91) |
| Net loss | (10,833) | (5,270) |
| Cumulative preferred stock dividends | (2,545) | (3,804) |
| Net loss applicable to common stockholders | \$ (13,378) | \$ (9,074) |
| Per share information: | | |
| Net loss per share of common stock basic and diluted | \$ (2.17) | \$ (19.60) |
| Basic and diluted weighted average shares outstanding | 6,152,669 | 462,972 |

See accompanying notes to financial statements.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS EQUITY (DEFICIT)

(In thousands, except share amounts)

| | Convertible Preferred Stock | | | | | | Stockholders Equity (Deficit) | | | | Total Stockholders Equity (Deficit) | | |
|---|-----------------------------|-----------|--------------|-----------|--------------|-----------|-------------------------------|--------|----------------------------------|-------------------|--|------------------------|-----------|
| | Series A | | Series B | | Series C | | Common Stock | | Additional Paid-in Capital | Treasury Stock | | Accumulated Deficit | |
| | Shares | Amount | Shares | Amount | Shares | Amount | Shares | Amount | | Shares | Amount | Deficit | (Deficit) |
| Balance, December 31, 2012 | 18,777,860 | \$ 30,596 | 12,220,661 | \$ 17,929 | 9,654,443 | \$ 11,023 | 478,745 | \$ 1 | 867 | 29,231 | \$ (56,233) | \$ (55,365) | |
| Stock-based compensation expense | | | | | | | | | 236 | | | | 236 |
| Issuance of preferred stock and conversion of notes | | | | | 8,727,020 | 10,291 | | | | | | | |
| Exercise of stock options | | | | | | | 15,515 | | 18 | | | | 18 |
| Net loss | | | | | | | | | | | (5,270) | (5,270) | |
| Balance, December 31, 2013 | 18,777,860 | 30,596 | 12,220,661 | 17,929 | 18,381,463 | 21,314 | 494,260 | 1 | 1,121 | 29,231 | (61,503) | (60,381) | |
| Stock-based compensation expense | | | | | | | | | 698 | | | | 698 |
| Issuance of Series C Preferred Stock | | | | | 422,119 | 500 | | | | | | | |
| Exercise of stock options | | | | | | | 122,634 | | 128 | | | | 128 |
| Conversion of convertible preferred stock into common stock | (18,777,860) | (30,596) | (12,220,661) | (17,929) | (18,803,582) | (21,814) | 7,661,871 | 7 | 70,333 | | | | 70,340 |
| Exercise and conversion of convertible preferred stock warrants into common stock | | | | | | | | 220 | | | | | |

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| | | | | | | | | | | |
|--|----|----|----|------------|-------|------------|--------|----|-------------|-----------|
| Issuance of common stock in connection with initial public offering (\$8.00 per share), net of expenses of \$4,862 | | | | 5,758,000 | 6 | 41,196 | | | 41,202 | |
| Net loss | | | | | | | | | (10,833) | (10,833) |
| Balance, December 31, 2014 | \$ | \$ | \$ | 14,036,985 | \$ 14 | \$ 113,476 | 29,231 | \$ | \$ (72,336) | \$ 41,154 |

See accompanying notes to financial statements.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF CASH FLOWS

(In thousands)

| | Year Ended December 31, | |
|--|-------------------------|------------|
| | 2014 | 2013 |
| Cash flows from operating activities | | |
| Net loss | \$ (10,833) | \$ (5,270) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Depreciation | 5 | 10 |
| Stock-based compensation expense | 698 | 236 |
| Noncash interest on convertible notes payable | | 91 |
| Change in fair value of warrant liability | (1,192) | (153) |
| Amortization of debt issuance costs | 4 | |
| Changes in operating assets and liabilities: | | |
| Prepaid expenses and other assets | 1,344 | (1,731) |
| Accounts payable and accrued expenses | 1,385 | 189 |
| Net cash used in operating activities | (8,589) | (6,628) |
| Cash flows from investing activities | | |
| Purchases of property and equipment | (33) | (17) |
| Net cash used in investing activities | (33) | (17) |
| Cash flows from financing activities | | |
| Proceeds from exercise of stock options | 128 | 18 |
| Proceeds from initial public offering, net of offering costs | 41,202 | |
| Proceeds from notes payable, net of issuance costs | 6,975 | |
| Proceeds from investor deposit | | 500 |
| Proceeds from issuance of convertible preferred stock, net of issuance costs | | 7,530 |
| Net cash provided by financing activities | 48,305 | 8,048 |
| Net increase in cash and cash equivalents | 39,683 | 1,403 |
| Cash and cash equivalents beginning of year | 10,037 | 8,634 |
| Cash and cash equivalents end of year | \$ 49,720 | \$ 10,037 |
| Supplemental disclosure of cash flow information | | |
| Conversion of preferred stock to common stock | \$ 70,340 | \$ |
| Conversion of notes principal and accrued interest to preferred stock | \$ | \$ 2,761 |
| Cash paid for interest | \$ 89 | \$ |
| Issuance of Series C Preferred Stock | \$ 500 | \$ |

See accompanying notes to financial statements.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

1. Organization and Description of the Business

We are a biopharmaceutical company dedicated to the development of innovative neuropsychiatric therapeutics. Our clinical stage product candidate, ganaxolone, is a synthetic small molecule that is an analog of allopregnanolone, a natural occurring neurosteroid produced by the human body. Allopregnanolone modulates the activity of gammaaminobutyric acid (GABA) at GABAA type receptors in the brain, which has been identified as playing an important role in certain seizure, psychiatric and developmental disorders. Our primary focus to date since our inception has been directed towards developing business strategies, raising capital, research and development activities and conducting preclinical testing and human clinical trials of our product candidates.

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, 2014 was \$72.3 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.

In connection with the closing our initial public offering during the third quarter of 2014, we issued a total of 5,758,000 shares of common stock and received aggregate net proceeds, after underwriting discounts and commissions and other estimated offering expenses, of approximately \$41.2 million. Our cash and cash equivalents balance as of December 31, 2014 is adequate to fund our operations into the second half of 2016.

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.

Recapitalization

Our board of directors and stockholders approved a reverse stock split of our common stock at a ratio of one share for every six and one-half shares previously held. The reverse stock split was effected on July 16, 2014. All common stock share and per-share data included in these financial statements reflect the reverse stock split. In addition, the board of directors approved an increase in the authorized shares of our common stock to 100,000,000 shares.

Fair Value of Financial Instruments and Credit Risk

At December 31, 2014 and 2013, our financial instruments included cash and cash equivalents, accounts payable, accrued expenses, notes payable and derivative liabilities. The carrying amount of cash and cash equivalents, 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. The carrying value of the derivative liabilities was the estimated fair value of the liability as more fully described below.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

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

Cash and Cash Equivalents

We consider all highly liquid investments that have maturities of three months or less when acquired to be cash equivalents. As of December 31, 2014 and 2013, we invested a portion of our cash balances in money market investments, which we have included as cash equivalents on our balance sheets.

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, 2014, 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, clinical site activations, or information provided to us by our vendors with respect to their actual costs incurred. Payments for these activities are based on the terms of the individual arrangements, which may differ from the pattern of costs incurred, and are reflected in the financial statements as prepaid or accrued research and development expense, as the case may be.

Income Taxes

We recognize deferred tax assets and liabilities for temporary differences between the financial reporting basis and the tax basis of our assets and liabilities and the expected benefits of net operating loss carryforwards. The impact of changes in tax rates and laws on deferred taxes, if any, applied during the years in which temporary differences are expected to be settled, is reflected in the financial statements in the period of enactment. The measurement of deferred tax assets is reduced, if necessary, if, based on weight of the evidence, it is more likely than not that some, or all, of the deferred tax assets will not be realized. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the period that such tax rate changes are enacted. At December 31, 2014 and 2013, 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 consolidated 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

Table of Contents**MARINUS PHARMACEUTICALS, INC.****NOTES TO FINANCIAL STATEMENTS**

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, 2014 and 2013 (in thousands, except share and per share amounts):

| | Year Ended December 31, | |
|---|--------------------------------|-------------|
| | 2014 | 2013 |
| Basic and diluted net loss per share of common stock: | | |
| Net loss | \$ (10,833) | \$ (5,270) |
| Dividends on Series B and C Preferred Stock | (2,545) | (3,804) |
| Net loss applicable to common stockholders | \$ (13,378) | \$ (9,074) |
| Weighted average shares of common stock outstanding | 6,152,669 | 462,972 |
| Net loss per share of common stock basic and diluted | \$ (2.17) | \$ (19.60) |

The following potentially dilutive securities outstanding at December 31, 2014 and 2013 have been excluded from the computation of diluted weighted average shares outstanding, as they would be antidilutive:

| | December 31, | |
|-----------------------------|---------------------|-------------|
| | 2014 | 2013 |
| Convertible preferred stock | | 7,596,927 |
| Warrants | | 470,026 |
| Stock options | 1,670,574 | 1,093,208 |
| | 1,670,574 | 9,160,161 |

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.

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

Table of Contents

MARINUS PHARMACEUTICALS, INC.

NOTES TO FINANCIAL STATEMENTS

expected volatility of the common stock consistent with the expected life of the option, risk-free interest rates, and 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. 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 through financial models taking 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, 2014 and 2013, there were no material adjustments to our prior period estimates of accrued expenses for clinical trials.

Recent Accounting Pronouncements

In June 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-10, *Elimination of Certain Financial Reporting Requirements, Including an Amendment to Variable Interest Entities Guidance in Topic 810, Consolidation*, which eliminates all incremental financial reporting requirements for development stage entities by removing Accounting Standards Codification (ASC) Topic 915, *Development Stage Entities*, from the FASB Accounting Standards Codification. ASC Topic 915 is removed effective for annual periods beginning after December 15, 2014 and early adoption is permitted. The Company adopted the ASU effective with the issuance of the June 30, 2014 interim financial statements.

In August 2014, the FASB issued ASU No. 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 is effective for annual periods ending after December 15, 2016, and interim periods thereafter, with early adoption permitted. The Company is currently evaluating the impact the adoption of this ASU will have on its 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

Table of Contents**MARINUS PHARMACEUTICALS, INC.****NOTES TO FINANCIAL STATEMENTS**

related disclosures. In determining fair value, we use quoted prices and observable inputs. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from independent sources.

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

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

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, 2014 | | | | |
| Assets | | | | |
| Money market funds (cash equivalents) | \$ 48,960 | \$ | \$ | \$ 48,960 |
| Total assets | \$ 48,960 | \$ | \$ | \$ 48,960 |
| December 31, 2013 | | | | |
| Assets | | | | |
| Money market funds (cash equivalents) | \$ 9,251 | \$ | \$ | \$ 9,251 |
| Total assets | \$ 9,251 | \$ | \$ | \$ 9,251 |
| Liabilities | | | | |
| Warrant liability | \$ | \$ | \$ 1,192 | \$ 1,192 |
| Total liabilities | \$ | \$ | \$ 1,192 | \$ 1,192 |

We had outstanding warrants to purchase our Series B Preferred Stock, or the Series B Warrants, as of December 31, 2013. While the Series B Preferred Stock was outstanding, holders were able to redeem for cash upon an event that was not within our control, such as the liquidation of our preferred stock, as the preferred stockholders had voting control of our company and control of our board of directors, and therefore had the ability to trigger the liquidation of our preferred stock. As a result, the Series B Warrants were recorded as a warrant liability on our balance

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sheet with subsequent changes to fair value recorded on our statements of operations as change in fair value of warrant liability. In connection with our initial public offering during the third quarter of 2014, these warrants expired unexercised, and the Series B Preferred Stock underlying the Series B Warrants converted to common stock. As a result, the fair value of the Series B warrants recorded as a liability as of December 31, 2013 was reduced to zero in 2014. On the grant date and in subsequent periods, we estimated the fair value of the preferred stock warrant liability using an option-pricing model, which required inputs such as the expected volatility based on comparable public companies (75% - 80%), the estimated fair value of the Series B Preferred Stock (\$0.63 - \$1.51 per share), and the estimated time to liquidity (0.2 - 4 years). For this liability, we developed our own assumptions that do not have observable inputs or available market data to support the fair value.

The following tables set forth a summary of changes in the fair value of Level 3 preferred stock warrant liability for the years ended December 31, 2014 and 2013 (in thousands):

| | Beginning of Year | Issuances | Exercises | Change in Fair Value | End of Year |
|------------------------------|----------------------------------|------------------|------------------|---------------------------------|--------------------|
| Year ended December 31, 2014 | \$ 1,192 | \$ | \$ | \$ (1,192) | \$ |
| Year ended December 31, 2013 | \$ 1,345 | \$ | \$ | \$ (153) | \$ 1,192 |

F-11

Table of Contents**MARINUS PHARMACEUTICALS, INC.****NOTES TO FINANCIAL STATEMENTS****4. Property and Equipment**

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

| | December 31, | |
|--------------------------------|--------------|--------|
| | 2014 | 2013 |
| Laboratory equipment | \$ 326 | \$ 369 |
| Office equipment | 74 | 76 |
| | 400 | 445 |
| Less: accumulated depreciation | (356) | (429) |
| | \$ 44 | \$ 16 |

Depreciation expense, in thousands, was \$5 and \$10 for the years ended December 31, 2014 and 2013, respectively.

5. Accrued Expenses

At December 31, 2014 and 2013, accrued expenses consisted of the following (in thousands):

| | December 31, | |
|--------------------------------------|--------------|----------|
| | 2014 | 2013 |
| Investor deposit | \$ 500 | \$ 500 |
| Payroll and related costs | 419 | 204 |
| Clinical trials and drug development | 777 | 73 |
| Professional fees | 186 | 295 |
| Other | 121 | 24 |
| | \$ 1,503 | \$ 1,096 |

Investor deposit represents funds received from an investor for 422,119 shares of Series C Preferred Stock. The shares were issued in April 2014.

6. Notes Payable

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In April 2014, we borrowed \$2.0 million in connection with a term loan pursuant to a Loan and Security Agreement (LSA) we entered into with a financial institution. In connection with this term loan, we issued to the financial institution warrants to purchase 37,991 shares of our Series C Preferred Stock with a term of 8 years. The warrants were net exercised and converted into 220 shares of common stock in connection with the closing our initial public offering during the third quarter of 2014. 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.

In December 2014, we entered into a First Amendment to Loan and Security Agreement (Amended LSA) with the same financial institution. The Amended LSA increased the total term loan availability from \$2.0 million to \$12.0 million, available in four tranches (in thousands):

| Tranche | Term Loan Available | Term Loan Borrowed | Borrowed Date |
|---------|---------------------------|--------------------------|---------------|
| A | \$ 2,000 | \$ 2,000 | April 2014 |
| B | 5,000 | 5,000 | December 2014 |
| C | 2,500 | | * |
| D | 2,500 | | * |
| | \$ 12,000 | \$ 7,000 | |

F-12

Table of Contents**MARINUS PHARMACEUTICALS, INC.****NOTES TO FINANCIAL STATEMENTS**

* Our ability to borrow under the remaining tranches of \$2.5 million each depends upon meeting certain clinical trial milestones. The availability end dates of Tranches C and D are September 1, 2015 and March 31, 2016, respectively.

In connection with the Amended LSA, we borrowed \$5.0 million available to us under Tranche B. Pursuant to the terms of the Amended LSA, we are required to make monthly interest-only payments for all outstanding borrowings at an interest rate equal to the greater of (a) prime plus 3.25% or (b) 6.5% until December 2015. Commencing in January 2016 and continuing through December 2017, we are required to make monthly payments of 1/24th of our principal borrowings plus interest with the remaining principal balance due in December 2017. If we achieve certain clinical trial milestones by August 2015, both the interest-only period and principal maturity dates will be extended by six months.

As of December 31, 2014, our outstanding term loan balance of \$7.0 million was classified as long-term debt on our balance sheet. In connection with the execution of both the LSA and Amended LSA, we paid debt costs to the financial institution of \$25 thousand, which are being amortized into interest expense over the remaining term of the loans. Interest expense related to the loans was \$117 thousand for the year ended December 31, 2014. As of December 31, 2014, we had accrued interest of \$39 thousand. There are no financial covenants associated with these term loans. As of December 31, 2014, we were in compliance with all non-financial covenants.

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

| | Debt Maturities | |
|------------------------------|----------------------------|-------|
| 2015 | \$ | |
| 2016 | | 3,500 |
| 2017 | | 3,500 |
| Total minimum lease payments | \$ | 7,000 |

7. Convertible Preferred Stock

In June 2013, we issued 6,396,065 shares of Series C Preferred Stock at \$1.1845 per share for proceeds of \$7.5 million, net of issuance costs of \$46 thousand. In addition, we issued 2,330,955 shares of Series C Preferred Stock from the conversion of the remaining outstanding \$2.8 million in Series C Convertible Notes, including related accrued interest of \$461 thousand. In April 2014, we issued 422,119 shares of Series C Preferred Stock. Proceeds of \$500 thousand related to this issuance were received in 2013 and were reflected as an investor deposit in accrued expenses on the balance sheet as of December 31, 2013.

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In connection with the closing our initial public offering during the third quarter of 2014, all outstanding shares of convertible preferred stock were converted to 7,661,871 shares of common stock.

F-13

Table of Contents**MARINUS PHARMACEUTICALS, INC.****NOTES TO FINANCIAL STATEMENTS****8. Stock Option and Incentive Plans**

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, 2014, 970,574 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 in 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 plan. As of December 31, 2014, 700,000 options to purchase shares of common stock were outstanding pursuant to grants in connection with the 2014 Plan; and no shares of common stock were available for future issuance. In accordance with the 2014 Plan, on January 1, 2015, 560,310 shares of common stock became available for future grant under the plan.

There were 1,670,574 stock options outstanding as of December 31, 2014 at a weighted average exercise price of \$4.37 per share, and 54,000 options were granted to nonemployee directors and consultants during the year ended December 31, 2014. Total compensation cost recognized for all stock option awards in the statements of operations is as follows (in thousands):

| | | Year Ended December 31, | |
|--|-------------|------------------------------------|-------------|
| | 2014 | | 2013 |
| Research and development | \$ | 137 | \$ 15 |
| General and administrative | | 561 | 221 |
| Total stock-based compensation expense | \$ | 698 | \$ 236 |

Stock Options

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, 2014 and 2013 is presented below (in thousands, except share and per share amounts):

| Shares | Weighted- Average | Aggregate Intrinsic |
|--------|----------------------|------------------------|
|--------|----------------------|------------------------|

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| | | | Exercise Price Per Share | | Value |
|----------------------------------|-------------------|-----------|---|----|--------------|
| Outstanding | December 31, 2012 | 689,663 | | | |
| Granted | | 589,719 | | | |
| Exercised | | (15,515) | 1.04 | | |
| Forfeited | | (170,659) | | | |
| 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 | \$ | 10,357 |
| Exercisable | December 31, 2014 | 764,338 | \$ 1.75 | \$ | 6,740 |
| Exercisable and Expected to vest | December 31, 2014 | 1,670,574 | \$ 4.37 | \$ | 10,357 |

F-14

Table of Contents**MARINUS PHARMACEUTICALS, INC.****NOTES TO FINANCIAL STATEMENTS**

The weighted average remaining contractual term of options outstanding and exercisable as of December 31, 2014 is 8.6 and 7.4 years, respectively. The aggregate intrinsic value in the preceding tables represent the total intrinsic value that would have been received had all option holders exercised their options on December 31, 2014. Intrinsic value is determined by calculating the difference between the fair value of our common stock on the last day of the year and the exercise price, multiplied by the number of options.

The weighted-average grant date fair value of options granted was \$6.09 and \$0.85 per share in 2014 and 2013 and was estimated at the date of grant using the Black-Scholes option-pricing model with the following weighted-average assumptions:

| | 2014 | | 2013 | |
|---------------------------------|-------------|------------|-------------|------------|
| Expected stock price volatility | 77.66 | 86.08% | 103.9 | 118.0% |
| Expected term of options | 5.5 | 6.06 years | 5 | 6.25 years |
| Risk-free interest rate | 1.75 | 1.98% | 0.84 | 1.75% |
| Expected annual dividend yield | | 0% | | 0% |

The weighted-average valuation assumptions were determined as follows:

- **Expected stock price volatility:** The expected volatility is based on historical 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.

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As of December 31, 2014, there was \$4.1 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 in the years ended as follows, in thousands:

| | | |
|-------------------|----|-------|
| December 31, 2015 | \$ | 1,448 |
| December 31, 2016 | | 1,302 |
| December 31, 2017 | | 929 |
| December 31, 2018 | | 403 |
| | \$ | 4,082 |

9. 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 February 2013, we entered into a two-year operating lease agreement for office space in New Haven, Connecticut and pay a flat rent per month over the term of the lease which ends in June 2015. Prior to that and through April 2013, we leased a facility in Branford, Connecticut. Rent

Table of Contents**MARINUS PHARMACEUTICALS, INC.****NOTES TO FINANCIAL STATEMENTS**

expense under these operating leases, in thousands, was \$50 and \$34 for the years ended 2014 and 2013, respectively. All leases are non-cancelable.

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

| | Operating Lease Payments |
|------------------------------|---|
| 2015 | \$ 100 |
| 2016 | 130 |
| 2017 | 132 |
| 2018 | 135 |
| 2019 | 138 |
| Thereafter | 46 |
| Total minimum lease payments | \$ 681 |

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 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.

10. Income Taxes

As of December 31, 2014 and 2013, we had approximately \$68.0 million and \$59.0 million, respectively, of net operating loss, or 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 \$2.6 million and state credit carryovers of approximately \$0.4 million which expire beginning in 2019.

The State of Connecticut provides companies with the opportunity to exchange certain research and development credit carryforwards for cash in exchange for foregoing the carryforward. The program provides for such exchange of the research and development credits at a rate of 65% of the annual research and development credit, as defined. During 2014 and 2013, we recorded a net benefit, in thousands, of \$2 and \$28, respectively, primarily for the estimated proceeds from the exchange of the research and development credit.

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

Table of Contents**MARINUS PHARMACEUTICALS, INC.****NOTES TO FINANCIAL STATEMENTS**

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.

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

| | December 31, | |
|---|---------------------|-------------|
| | 2014 | 2013 |
| Gross deferred tax assets: | | |
| Net operating loss carryovers | \$ 26,505 | \$ 23,006 |
| Accrued expenses | | 18 |
| Contributions | 5 | 1 |
| Stock-based compensation | 211 | 143 |
| Research and development and other credits | 3,075 | 2,693 |
| Total gross deferred tax assets | 29,796 | 25,861 |
| Gross deferred tax liabilities: | | |
| Depreciation | (1) | (2) |
| Total gross deferred tax liabilities | (1) | (2) |
| Net deferred tax assets | 29,795 | 25,859 |
| Less: valuation allowance | (29,795) | (25,859) |
| Net deferred tax assets after valuation allowance | \$ | \$ |

In assessing the realizability of deferred tax assets, we consider whether it is more-likely-than-not that some portion or all of the deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which the temporary differences representing net future deductible amounts become deductible. After consideration of all the evidence, both positive and negative, we have recorded a full valuation allowance against our net deferred tax assets at December 31, 2014 and 2013, respectively, because our management has determined that it is more likely than not that these assets will not be fully realized. The valuation allowance increased by \$3.9 million and \$2.3 million during the years ended December 31, 2014 and 2013, respectively, due primarily to the generation of NOLs during those periods.

We did not have unrecognized tax benefits as of December 31, 2014 and 2013, and do not expect this to change significantly over the next twelve months. We recognize interest and penalties accrued on any unrecognized tax benefits as a component of income tax expense. As of December 31, 2014 and 2013, 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.

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, | |
|--|----------------------------|--------|
| | 2014 | 2013 |
| Federal income tax expense at statutory rate | 34.0% | 34.0% |
| Permanent items | (5.4) | (0.3) |
| State income tax, net of federal benefit | 3.8 | 5.0 |
| State refundable credit | 0.0 | 0.7 |
| R&D tax credits | 4.2 | (3.3) |
| Change in valuation allowance | (36.6) | (35.4) |
| Effective income tax rate | 0.0% | 0.7% |

For all years through December 31, 2014, we generated research 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

Table of Contents**MARINUS PHARMACEUTICALS, INC.****NOTES TO FINANCIAL STATEMENTS**

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, 2011 through December 31, 2013. 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.

11. Quarterly Financial Information (unaudited)

| | First Quarter | Second Quarter | Third Quarter | Fourth Quarter | Total Year |
|--|--------------------------|---------------------------|--------------------------|---------------------------|-------------------|
| 2014: | | | | | |
| Research and development expenses | \$ 2,149 | \$ 2,805 | \$ 1,569 | \$ 2,167 | \$ 8,690 |
| General and administrative expenses | \$ 517 | \$ 458 | \$ 868 | \$ 1,387 | \$ 3,230 |
| Net loss | \$ (2,235) | \$ (3,323) | \$ (1,668) | \$ (3,607) | \$ (10,833) |
| Cumulative preferred stock dividends | \$ (1,071) | \$ (1,102) | \$ (372) | | \$ (2,543) |
| Net loss applicable to common shareholders | \$ (3,306) | \$ (4,425) | \$ (2,040) | \$ (3,607) | \$ (13,378) |
| Net loss per share, basic and diluted | \$ (7.09) | \$ (7.98) | \$ (0.22) | \$ (0.26) | \$ (2.17) |
| 2013: | | | | | |
| Research and development expenses | \$ 747 | \$ 1,327 | \$ 1,008 | \$ 1,068 | \$ 4,150 |
| General and administrative expenses | \$ 180 | \$ 314 | \$ 313 | \$ 422 | \$ 1,229 |
| Net loss | \$ (970) | \$ (1,682) | \$ (1,224) | \$ (1,394) | \$ (5,270) |
| Cumulative preferred stock dividends | \$ (786) | \$ (940) | \$ (1,029) | \$ (1,049) | \$ (3,804) |
| Net loss applicable to common shareholders | \$ (1,756) | \$ (2,622) | \$ (2,253) | \$ (2,443) | \$ (9,074) |
| Net loss per share, basic and diluted | \$ (3.82) | \$ (5.66) | \$ (4.58) | \$ (5.54) | \$ (19.60) |

Table of Contents

| 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 March 11, 2015 between the Company and Christopher M. Cashman. (Filed herewith.) |
| 10.4+ | Amended and Restated Employment Agreement dated as of March 11, 2015 between the Company and Edward F. Smith. (Filed herewith.) |
| 10.5+ | Employment Agreement dated as of November 2, 2012 between the Company and Gail M. Farfel. (Incorporated by reference to Exhibit 10.5 to Form S-1 registration statement filed on May 12, 2014.) |
| 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. (Filed herewith in corrected form.) |
| 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+ | Employment Agreement dated as of March 11, 2015 between the Company and Albenia Patroneva. (Filed herewith.) |
| 10.16+ | Form of Incentive Stock Option Agreement for Officers Under 2014 Equity Incentive Plan. (Filed herewith.) |
| 10.17+ | Form of Incentive Stock Option Agreement for Employees Under 2014 Equity Incentive Plan. (Filed herewith.) |
| 10.18+ | Form of Nonqualified Stock Option Agreement Under 2014 Equity Incentive Plan. (Filed herewith.) |

Table of Contents

| Exhibit No. | Description of Exhibit |
|------------------------|--|
| 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 |

+ 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.