

MARINUS PHARMACEUTICALS INC

Form 10-Q

October 29, 2015

Table of Contents

s

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

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

FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2015

OR

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

COMMISSION FILE NUMBER 001-36576

MARINUS PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

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

Three Radnor Corporate Center

100 Matsonford Rd., Suite 304

Radnor, PA 19087

(Address of registrant's principal executive offices)

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

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No.

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) 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 whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer or a smaller reporting company. See definition of "large accelerated filer", "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Edgar Filing: MARINUS PHARMACEUTICALS INC - Form 10-Q

Large accelerated filer

Accelerated filer

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

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

The number of outstanding shares of the registrant's common stock, par value \$0.001 per share, as of October 29, 2015 was: 14,334,852.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

INDEX TO FORM 10-Q

FOR THE QUARTER ENDED SEPTEMBER 30, 2015

PART I – FINANCIAL INFORMATION

<u>Item 1.</u>	<u>Financial Statements (unaudited)</u>	
	<u>Balance Sheets as of September 30, 2015 and December 31, 2014</u>	3
	<u>Statements of Operations for the three and nine months ended September 30, 2015 and 2014</u>	4
	<u>Statements of Cash Flows for the nine months ended September 30, 2015 and 2014</u>	5
	<u>Notes to Financial Statements</u>	6
<u>Item 2.</u>	<u>Management’s Discussion and Analysis of Financial Condition and Results of Operations</u>	11
<u>Item 3.</u>	<u>Quantitative and Qualitative Disclosure About Market Risk</u>	18
<u>Item 4.</u>	<u>Controls and Procedures</u>	19

PART II – OTHER INFORMATION

<u>Item 1.</u>	<u>Legal Proceedings</u>	20
<u>Item 1A.</u>	<u>Risk Factors</u>	20
<u>Item 2.</u>	<u>Unregistered Sales of Equity Securities and Use of Proceeds</u>	51
<u>Item 3.</u>	<u>Defaults Upon Senior Securities</u>	51
<u>Item 4.</u>	<u>Mine Safety Disclosures</u>	51
<u>Item 5.</u>	<u>Other Information</u>	51
<u>Item 6.</u>	<u>Exhibits</u>	52
	<u>Signatures</u>	53

Table of Contents

PART I

FINANCIAL INFORMATION

Item 1. Financial Statements

MARINUS PHARMACEUTICALS, INC.

BALANCE SHEETS

(in thousands, except share and per share amounts)

(unaudited)

	September 30, 2015	December 31, 2014
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 28,027	\$ 49,720
Short-term investments	4,974	—
Prepaid expenses and other current assets	1,859	428
Total current assets	34,860	50,148
Property and equipment, net of accumulated depreciation of \$364 and \$356	36	44
Investments	1,489	—
Other assets	368	21
Total assets	\$ 36,753	\$ 50,213
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Current portion of notes payable	\$ 3,127	\$ —
Accounts payable	2,300	536
Accrued expenses	1,236	1,503
Total current liabilities	6,663	2,039
Notes payable	4,375	7,000
Other long-term liabilities	57	20
Total liabilities	11,095	9,059
Stockholders' equity:		

Edgar Filing: MARINUS PHARMACEUTICALS INC - Form 10-Q

Preferred stock, \$0.001 par value; 25,000,000 shares authorized, 0 shares issued and outstanding	—	—
Common stock, \$0.001 par value; 100,000,000 shares authorized, 14,364,083 issued and 14,334,852 outstanding at September 30, 2015 and 14,036,985 issued and 14,007,754 outstanding at December 31, 2014	14	14
Additional paid-in capital	115,217	113,476
Treasury stock at cost, 29,231 shares at September 30, 2015 and December 31, 2014	—	—
Accumulated deficit	(89,573)	(72,336)
Total stockholders' equity	25,658	41,154
Total liabilities and stockholders' equity	\$ 36,753	\$ 50,213

See accompanying notes to financial statements.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF OPERATIONS

(in thousands, except share and per share amounts)

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Expenses:				
Research and development	\$ 3,472	\$ 1,569	\$ 12,856	\$ 6,538
General and administrative	1,378	868	4,074	1,827
Loss from operations	(4,850)	(2,437)	(16,930)	(8,365)
Change in fair value of warrant liability	—	794	—	1,192
Interest income	15	4	44	6
Interest expense	(121)	(29)	(353)	(59)
Other income (expense)	(7)	—	2	—
Net loss	(4,963)	(1,668)	(17,237)	(7,226)
Cumulative preferred stock dividends	—	(372)	—	(2,545)
Net loss applicable to common stockholders	\$ (4,963)	\$ (2,040)	\$ (17,237)	\$ (9,771)
Per share information:				
Net loss per share of common stock—basic and diluted	\$ (0.35)	\$ (0.22)	\$ (1.21)	\$ (2.79)
Basic and diluted weighted average shares outstanding	14,289,939	9,449,355	14,194,793	3,499,808

See accompanying notes to financial statements.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

STATEMENTS OF CASH FLOWS

(in thousands)

(unaudited)

	Nine Months Ended September 30,	
	2015	2014
Cash flows from operating activities		
Net loss	\$ (17,237)	\$ (7,226)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	8	7
Stock-based compensation expense	1,368	263
Change in fair value of warrant liability	—	(1,192)
Amortization of debt issuance costs	5	3
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,260)	1,332
Accounts payable and accrued expenses	2,036	192
Net cash used in operating activities	(15,080)	(6,621)
Cash flows from investing activities		
Purchases of investments	(6,961)	—
Maturities of short-term investments	498	—
Deposit on equipment	(352)	—
Net cash used in investing activities	(6,815)	—
Cash flows from financing activities		
Proceeds from exercise of stock options	373	28
Repayment of notes payable	(82)	—
Proceeds from initial public offering, net of offering costs	—	41,672
Proceeds from notes payable, net of issuance costs	—	1,989
Payment of deferred offering costs	(89)	—
Net cash provided by financing activities	202	43,689
Net (decrease)/increase in cash and cash equivalents	(21,693)	37,068
Cash and cash equivalents—beginning of period	49,720	10,037
Cash and cash equivalents—end of period	\$ 28,027	\$ 47,105
Supplemental disclosure of cash flow information		
Conversion of preferred stock to common stock	\$ —	\$ 70,340

Edgar Filing: MARINUS PHARMACEUTICALS INC - Form 10-Q

Cash paid for interest	\$ 348	\$ 46
Financing arrangement with third-party vendor	\$ 584	\$ —
Issuance of Series C Preferred Stock	\$ —	\$ 500
Accrued initial public offering costs	\$ —	\$ 469

See accompanying notes to financial statements.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

NOTES TO INTERIM FINANCIAL STATEMENTS

(unaudited)

1. Description of the Business and Liquidity

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 naturally occurring neurosteroid in the human body. Allopregnanolone modulates the activity of gammaaminobutyric acid (GABA) at GABA_A type receptors in the brain, which have been identified as playing an important role in certain seizure, psychiatric and developmental disorders. Our primary focus to date has been directed towards developing business strategies, raising capital, conducting research and development activities, and conducting preclinical testing and human clinical trials of our product 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 September 30, 2015 was \$89.6 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 of 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, cash equivalents and investment balances as of September 30, 2015 are adequate to fund our operations into the fourth quarter of 2016.

2. Summary of Significant Accounting Policies

Basis of Presentation

The unaudited interim financial statements included herein have been prepared pursuant to the rules and regulations of the Securities and Exchange Commission (SEC). Accordingly, they do not include all information and disclosures necessary for a presentation of our financial position, results of operations and cash flows in conformity with generally accepted accounting principles in the United States of America (GAAP). In the opinion of management, these unaudited interim financial statements reflect all adjustments, consisting primarily of normal recurring accruals, necessary for a fair presentation of our financial position and results of operations and cash flows for the periods presented. The results of operations for interim periods are not necessarily indicative of the results for the full year. These unaudited interim financial statements should be read in conjunction with the audited financial statements for the year ended December 31, 2014 and accompanying notes thereto included in our annual report on Form 10-K filed with the SEC on March 12, 2015.

Use of Estimates

The preparation of financial statements in conformity with 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 income and expenses during the reporting period. Actual results could differ from such estimates.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

NOTES TO INTERIM FINANCIAL STATEMENTS

(unaudited)

Investments

Investments purchased with a maturity of more than three months and less than twelve months are classified as short-term investments. Investments purchased with a remaining maturity greater than twelve months are classified as long-term investments. We plan to hold these investments to maturity and have classified these investments as such as defined by GAAP.

Prepaid expenses and other current assets

Prepaid expenses and other current assets generally represent payments made for goods or services to be received within one year, and are expensed as the related benefit is received. As of September 30, 2015, this balance included a prepayment of \$1.0 million for clinical manufacturing supplies to be used in connection with our clinical trials.

Recent Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (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.

In April 2015, the FASB issued ASU No. 2015-03, Simplifying the Presentation of Debt Issuance Costs, which changes the presentation of debt issuance costs in financial statements. Under the ASU, an entity presents such costs in the balance sheet as a direct deduction from the related debt liability rather than as an asset. Amortization of the costs is reported as interest expense. For public business entities, the guidance in the ASU is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2015. Early adoption is allowed for all entities for financial statements that have not been previously issued. Entities would apply the new guidance retrospectively to all prior periods (i.e., the balance sheet for each period is adjusted). We do not expect the adoption of this ASU to have a material effect on our interim or annual financial statements.

3. Fair Value Measurements

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

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

- Level 1 — Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

NOTES TO INTERIM FINANCIAL STATEMENTS

(unaudited)

- 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
September 30, 2015				
Assets				
Money market funds (cash equivalents)	\$ 27,127	\$ —	\$ —	\$ 27,127
Certificates of deposit	6,463	—	—	6,463
December 31, 2014				
Assets				
Money market funds (cash equivalents)	\$ 48,960	\$ —	\$ —	\$ 48,960

4. Accrued Expenses

At September 30, 2015 and December 31, 2014 accrued expenses consisted of the following (in thousands):

	September 30, 2015	December 31, 2014
Payroll and related costs	599	419
Clinical trials and drug development	326	777
Professional fees	187	186
Other	124	121
Total accrued expenses	\$ 1,236	\$ 1,503

5. Notes Payable

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

In December 2014, we entered into a First Amendment to Loan and Security Agreement, and in February 2015 and October 2015 we entered into a Second and Third Amendment to Loan and Security Agreement (collectively, “the 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):

Table of Contents

MARINUS PHARMACEUTICALS, INC.

NOTES TO INTERIM FINANCIAL STATEMENTS

(unaudited)

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 is conditioned upon meeting certain clinical trial milestones, which must be met by March 31, 2016 and December 31, 2015 for Tranches C and D, respectively. The availability end date for Tranches C and D is March 31, 2016.

In connection with the Amended LSA, we borrowed \$5.0 million available to us under Tranche B in December 2014. 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 rate plus 3.25% or (b) 6.5% until March 2016. Commencing in April 2016 and continuing through March 2018, we are required to make monthly payments of 1/24th of our principal borrowings plus interest. If we achieve certain clinical trial milestones by March 3, 2016, both the interest-only period and principal maturity date will be extended by three months.

As of September 30, 2015, of our outstanding term loan balance of \$7.0 million, \$2.6 million will be due within the next twelve months, and is classified as the current portion of long-term debt on our balance sheet. Interest expense related to the term loans was \$116 thousand and \$346 thousand for the three and nine months ended September 30, 2015, respectively. As of September 30, 2015, we had accrued interest of \$38 thousand. There are no financial covenants associated with these term loans. As of September 30, 2015, we were in compliance with all non-financial covenants.

Vendor Debt

In August 2015, the Company entered into a short-term loan agreement with a third-party vendor to finance insurance premiums. The aggregate amount financed under this agreement was \$584 thousand. As of September 30, 2015, there was a balance of \$502 thousand, which will be repaid in monthly installments through September 2016.

6. Loss Per Share of Common Stock

Basic loss per share is computed by dividing net loss applicable to common stockholders by the weighted average number of shares of common stock outstanding during each period. Diluted loss per share includes the effect, if any, from the potential exercise or conversion of securities, such as convertible preferred stock, convertible notes payable, warrants, stock options, and unvested restricted stock, which would result in the issuance of incremental shares of common stock. In computing the basic and diluted net loss per share applicable to common stockholders, the weighted average number of shares remains the same for both calculations due to the fact that when a net loss exists, dilutive shares are not included in the calculation. These potentially dilutive securities are more fully described in Note 8.

Our outstanding stock options, which were 1,757,326 and 1,108,173 as of September 30, 2015 and 2014, respectively, have been excluded from the computation of diluted weighted average shares outstanding, as they would be antidilutive. As of September 30, 2015 and 2014, we had no other potentially dilutive securities.

Table of Contents

MARINUS PHARMACEUTICALS, INC.

NOTES TO INTERIM FINANCIAL STATEMENTS

(unaudited)

7. Investments

As of September 30, 2015, our investments consisted of certificates of deposit with various financial institutions, with original maturities ranging from three to 18 months. Certificates of deposit with remaining maturities less than 12 months are classified as short-term investments and maturities greater than 12 months are classified as long-term investments on our balance sheet. All investments are classified as held-to-maturity and are recorded at amortized cost. Fair value of our investments approximates the carrying value on our balance sheet.

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 September 30, 2015, 634,387 options to purchase shares of common stock were outstanding pursuant to grants in connection with the 2005 Plan. No additional shares are available for issuance under the 2005 Plan. The amount, terms of grants, and exercisability provisions are determined and set by our board of directors.

Effective August 2014, we adopted our 2014 Equity Incentive Plan (2014 Plan) that authorizes us to grant options, restricted stock, and other equity-based awards, subject to adjustment in accordance with the 2014 Plan. As of September 30, 2015, 1,122,939 options to purchase shares of common stock were outstanding pursuant to grants in connection with the 2014 Plan, and 131,371 shares of common stock were available for future issuance. The amount, terms of grants, and exercisability provisions are determined and set by our board of directors.

There were 1,757,326 stock options outstanding as of September 30, 2015 at a weighted-average exercise price of \$7.49 per share. During the nine-month period ended September 30, 2015, 458,800 options were granted to employees, directors and consultants at a weighted-average exercise price of \$14.18, 327,098 options were exercised

Edgar Filing: MARINUS PHARMACEUTICALS INC - Form 10-Q

at a weighted-average exercise price of \$1.14, and 44,950 options were forfeited at a weighted-average exercise price of \$6.13.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Research and development	\$ 179	\$ 35	\$ 436	\$ 39
General and administrative	418	181	932	224
Total stock-based compensation expense	\$ 597	\$ 216	\$ 1,368	\$ 263

9. Commitments

In June 2015, we entered into a contract with a third party for up to \$3.8 million in clinical manufacturing supplies to support our ongoing clinical trials for ganaxolone. Delivery is expected to begin in December 2015 and continue into 2016. In July 2015, we paid \$1.0 million of this commitment, which is recorded in prepaid expenses and other current assets as of September 30, 2015. The balance is due in 2016..

Table of Contents

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

Cautionary Note Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q contains forward-looking statements, within the meaning of the U.S. Private Securities Litigation Reform Act of 1995, that involve substantial risks and uncertainties. In some cases, you can identify forward-looking statements by the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," "will," or "would," and or the negative of "not" 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 Quarterly Report on Form 10-Q, 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 Quarterly Report on Form 10-Q include, among other things, statements about:

- our ability to develop and commercialize ganaxolone;
- status, timing and results of preclinical studies and clinical trials;
- the potential benefits of ganaxolone;
- the timing of seeking regulatory approval of ganaxolone;
- our ability to obtain and maintain regulatory approval;
- our estimates of expenses and future revenue and profitability;
- our estimates regarding our capital requirements and our needs for additional financing;
- our plans to develop and market ganaxolone and the timing of our development programs;
- our estimates of the size of the potential markets for ganaxolone;
- our selection and licensing of ganaxolone;
- our ability to attract collaborators with acceptable development, regulatory and commercial expertise;
- the benefits to be derived from corporate collaborations, license agreements, and other collaborative or acquisition efforts, including those relating to the development and commercialization of ganaxolone;
- sources of revenue, including contributions from corporate collaborations, license agreements, and other collaborative efforts for the development and commercialization of products;
- our ability to create an effective sales and marketing infrastructure if we elect to market and sell ganaxolone directly;
- the rate and degree of market acceptance of ganaxolone;
- the timing and amount of reimbursement for ganaxolone;

Table of Contents

- the success of other competing therapies that may become available;
- the manufacturing capacity for ganaxolone;
- our intellectual property position;
- our ability to maintain and protect our intellectual property rights;
- our results of operations, financial condition, liquidity, prospects, and growth strategies;
- the industry in which we operate; and
- the trends that may affect the industry or us.

You should refer to Part II Item 1A. “Risk Factors” of this Quarterly Report on this Form 10-Q 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 Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q and the documents that we reference in this Quarterly Report on Form 10-Q and have filed as exhibits to this Quarterly Report on Form 10-Q 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.

The following Management’s Discussion and Analysis of Financial Condition and Results of Operations should be read in conjunction with: (i) the Financial Statements and related notes thereto which are included in this Quarterly Report on Form 10-Q; and (ii) our annual financial statements for the year ended December 31, 2014 which are included in our Annual Report on Form 10-K filed with the SEC on March 12, 2015.

Overview

We are a clinical stage biopharmaceutical company focused on developing and commercializing innovative neuropsychiatric therapeutics. Our clinical stage product candidate, ganaxolone, is a CNS-selective GABAA modulator that is a small molecule 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 deliver neuro-specific GABAA receptor activation without off-target hormonal side-effects associated with allopregnanolone. Our strategy is to bring convenient ganaxolone dose forms to target underserved patient populations in both the acute and chronic care settings where ganaxolone has the potential to provide valuable differentiated therapeutic benefit to patients suffering from seizures and other neuropsychiatric disorders. Our orally administered solid and liquid suspension dose forms are being evaluated in our ongoing clinical trials and our intravenous, or IV, dose form is ready for clinical use.

Our most advanced indication for ganaxolone is as an adjunctive, or add-on, therapy for the treatment of focal, (also known as partial), 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

Table of Contents

treatment of focal-onset seizures in adults. We believe ganaxolone also has potential in a broad range of neuropsychiatric disorders. We currently have an exploratory Phase 2 proof-of-concept clinical study on-going with ganaxolone for the treatment of pediatric PCDH19 female epilepsy (PCDH19) and an exploratory Phase 2 proof-of-concept investigator-sponsored clinical trial evaluating ganaxolone as a treatment for behaviors in Fragile X Syndrome (FXS). We have received orphan designation from the United States Food and Drug Administration (FDA) for the treatment of PCDH19 with ganaxolone and, while we have not yet applied for orphan status, we believe FXS to be an orphan disorder for which ganaxolone may be eligible for orphan status. Both PCDH19 and FXS have been related to mutations affecting GABA signaling at 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 September 30, 2015, we have received net proceeds of \$110.4 million from the issuance of preferred stock, common stock and convertible notes payable. At September 30, 2015, we had cash, cash equivalents and investment balances of \$34.5 million. 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 a net loss of \$17.2 million for the nine months ended September 30, 2015. Our accumulated deficit as of September 30, 2015 was \$89.6 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 focal onset epileptic seizures;

- conduct clinical proof-of-concept clinical trials in targeted rare disease indications, including PCDH19, FXS and indications to be targeted using our intravenous dose form;

- complete regulatory and manufacturing activities and commence clinical trials for the intravenous form of ganaxolone;

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

conduct other preclinical and clinical studies to support the filing of a New Drug Application (NDA) with the FDA;

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.

Table of Contents

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 (Exchange Act), the Sarbanes-Oxley Act of 2002 (Sarbanes-Oxley Act) and the rules and regulations of The NASDAQ Global Market. Pursuant to Section 404 of the Sarbanes-Oxley Act, we will be required to furnish a report by our management on our internal control over financial reporting with respect to our fiscal year ending December 31, 2015. We are performing 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, cash equivalents and investments as of September 30, 2015 will enable us to fund our operating expenses and capital expenditure requirements into the fourth quarter 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:

employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;

expenses incurred under agreements with Clinical Research Organizations (CROs) and investigative sites that conduct our clinical trials and preclinical studies;

the cost of acquiring, developing and manufacturing clinical trial materials;

facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies; and

costs associated with preclinical activities and regulatory operations.

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

We will incur substantial costs beyond our present and planned clinical trials in order to file an NDA and Supplemental New Drug Applications (sNDAs) for ganaxolone for various clinical indications, and in each case, the nature, design, size and cost of further studies and trials will depend in large part on the outcome of preceding studies and trials and discussions with regulators. It is difficult to determine with certainty the costs and duration of our current or future clinical trials and preclinical studies, or if, when or to what extent we will generate revenue from the commercialization and sale of ganaxolone if we obtain regulatory approval. We may never succeed in achieving regulatory approval for ganaxolone. The duration, costs and timing of clinical trials and development of ganaxolone will depend on a variety of factors, including the uncertainties of future clinical trials and preclinical studies, uncertainties in clinical trial enrollment rate and significant and changing government regulation.

In addition, the probability of success for ganaxolone will depend on numerous factors, including competition, manufacturing capability and commercial viability. See “Risk Factors.” Our commercial success depends upon attaining significant market acceptance of ganaxolone, if approved, among physicians, patients, healthcare 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.

Table of Contents

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

Interest Income

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

Interest Expense

Interest expense is attributable to interest expense associated with our credit facility entered into in April 2014, and amended 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 and the resulting conversion of all outstanding shares of preferred stock into common stock, which occurred during the third quarter of 2014.

Results of Operations

Research and Development Expenses

Research and development expenses increased to \$3.5 million and \$12.9 million for the three and nine months ended September 30, 2015, respectively, as compared to \$1.6 million and \$6.5 million for the same periods in the prior year. The increases in both periods resulted primarily from an increase in clinical, drug development and consulting costs related to our ongoing clinical trials for ganaxolone, as well as increases in compensation-related costs due to hiring additional clinical resources, including our Chief Medical Officer, who was hired in December 2014. Most of our

research and development expenses relate to our ongoing Phase 3 clinical trial of ganaxolone in adults with refractory focal onset epileptic seizures.

General and Administrative Expenses

General and administrative expenses increased to \$1.4 million and \$4.1 million for the three and nine months ended September 30, 2015, respectively, as compared to \$0.9 million and \$1.8 million for the same periods in the prior year. The increases in general and administrative expenses were primarily due to the hiring of new management and the

Table of Contents

upward scaling of our operations in connection with both our public company status as of July 31, 2014 and our ongoing Phase 3 clinical trial of ganaxolone in adults with refractory focal onset epileptic seizures.

Change in Fair Value of Warrant Liability

We recorded changes in the fair value of our warrant liability which resulted in a gain of \$0.8 million and \$1.2 million for the three and nine months ended September 30, 2014, 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 were \$0.4 million and \$2.5 million for the three and nine months ended September 30, 2014, respectively. Upon conversion of all outstanding convertible preferred stock in connection with our initial public offering during the third quarter of 2014, all cumulative preferred stock dividends were canceled.

Liquidity and Capital Resources

Since inception, we have incurred net losses and negative cash flows from our operations. We incurred net losses of \$5.0 million and \$17.2 million for the three and nine months ended September 30, 2015, respectively. Our cash used in operating activities was \$15.1 million for the nine months ended September 30, 2015 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 September 30, 2015, we have received net proceeds of \$110.4 million from the issuance of preferred stock, common stock and convertible notes payable. At September 30, 2015, we had cash, cash equivalents and investment balances of \$34.5 million.

Credit Facility

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

In December 2014, we entered into a First Amendment to Loan and Security Agreement, and in February 2015 and October 2015 we entered into a Second and Third Amendment to Loan and Security Agreement (collectively, "the 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):

Edgar Filing: MARINUS PHARMACEUTICALS INC - Form 10-Q

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 is conditioned upon meeting certain clinical trial milestones, which must be met by March 31, 2016 and December 31, 2015 for Tranches C and D, respectively. The availability end date for Tranches C and D is March 31, 2016.

In connection with the Amended LSA, we borrowed \$5.0 million available to us under Tranche B in December 2014. 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 rate plus 3.25% or (b) 6.5% until March 2016.

Table of Contents

Commencing in April 2016 and continuing through March 2018, we are required to make monthly payments of 1/24th of our principal borrowings plus interest. If we achieve certain clinical trial milestones by March 3, 2016 both the interest-only period and principal maturity date will be extended by three months.

Cash Flows

Operating Activities. Cash used in operating activities increased to \$15.1 million for the nine months ended September 30, 2015 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 \$10.0 million, partially offset by an increase in stock-based compensation expense of \$1.1 million and reduction in the change in the fair value of our warrant liability of \$1.2 million. Additionally, we had an increase in net use of cash related to the changes in operating assets and liabilities of \$0.7 million. This increase in net use of cash primarily was due to upfront payment obligations related to certain drug manufacturing contracts in 2015 and increases in our trade accounts payable due to increased operating expenses. The increase in net loss was primarily driven by increases in our operating expenses due to the upward scaling of our operations related to our ongoing clinical trials for ganaxolone.

Investing Activities. Cash used in investing activities represents the purchase of \$7.0 million in investments and \$0.4 million in deposits on clinical research equipment during the nine months ended September 30, 2015, partially offset by maturities of short-term investments of \$0.5 million. There were no investing activities during the nine months ended September 30, 2014.

Financing Activities. Cash provided by financing activities was \$0.2 million for the nine months ended September 30, 2015 due to proceeds received from the exercise of outstanding stock options offset by installment payments made to a third-party vendor for financed insurance premiums and payment of deferred offering costs. Cash provided by financing activities for the nine months ended September 30, 2014 of \$43.7 million was primarily due to \$41.7 million received in connection with our initial public offering, and \$2.0 million received in connection with our credit facility.

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, cash equivalents and investments as of September 30, 2015, will enable us to fund our operating expenses and capital expenditure requirements into the fourth quarter 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;

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;

17

Table of Contents

the timing of, and the costs involved in, obtaining regulatory approvals for ganaxolone or any other future product candidates;

the cost of commercialization activities if ganaxolone or any other future product candidates are approved for sale, including marketing, sales and distribution costs;

the cost of manufacturing ganaxolone or any other future product candidates in preclinical studies, clinical trials and, if approved, in commercial sale;

our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;

any product liability, infringement or other lawsuits related to our products;

the expenses needed to attract and retain skilled personnel;

the costs associated with being a public company;

the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation; and

the timing, receipt and amount of sales of, or royalties on, future approved products, if any.

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

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

The preparation of financial statements in conformity with GAAP requires us to use judgment in making certain estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities and the reported amounts of revenues and expenses in our financial statements and accompanying notes. Critical accounting policies are those that are most important to the portrayal of our financial condition and results of operations and require difficult, subjective and complex judgments by management in order to make estimates about the effect of matters that are inherently uncertain. During the nine months ended September 30, 2015, there were no significant changes to our critical accounting policies from those described in our annual financial statements for the year ended December 31, 2014, which we included in our Annual Report on Form 10-K and was filed with the SEC on March 12, 2015.

Item 3. Quantitative and Qualitative Disclosure About Market Risk

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

We had cash, cash equivalents and investment balances of \$34.5 million at September 30, 2015, consisting primarily of funds in cash, money market accounts and certificates of deposit. The primary objective of our investment activities is to preserve principal and liquidity while maximizing income without significantly increasing risk. We do not enter into investments for trading or speculative purposes. Due to the conservative nature of our investment portfolio, we do not believe an immediate 1.0% increase in interest rates would have a material effect on the fair market value of our portfolio, and accordingly we do not expect a sudden change in market interest rates to affect materially our operating results or cash flows.

Table of Contents

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 4. Controls and Procedures

(a) 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 September 30, 2015. 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 September 30, 2015, our disclosure controls and procedures were effective to ensure that the information required to be disclosed in our reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in SEC rules and forms, and that information required to be disclosed in the reports that we file or submit under the Exchange Act is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, to allow timely decisions regarding required disclosure.

(b) 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(f) and 15d-15(f) of the Exchange Act that occurred during the quarter ended September 30, 2015 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

Table of Contents

PART II

OTHER INFORMATION

Item 1. 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 1A. Risk Factors

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 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 \$5.0 million and \$17.2 million for the three and nine months ended September 30, 2015, respectively. As of September 30, 2015, we had an accumulated deficit of \$89.6 million. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital. Our losses have resulted principally from costs incurred in our research and development activities. We anticipate that our operating losses will substantially increase over the next several years as we execute our plan to expand our research, development and commercialization activities, including the clinical development and planned commercialization of our product candidate, ganaxolone, and incur the additional costs of operating as a public company. In addition, if we obtain regulatory approval of ganaxolone, we may incur significant sales and marketing expenses. Because of the numerous risks and uncertainties associated with developing biopharmaceutical products, we are unable to predict the extent of any future losses or whether or when we will become profitable, if ever.

We have not generated any revenue to date from product sales. We may never achieve or sustain profitability, which could depress the market price of our common stock, and could cause you to lose all or a part of your investment.

To date, we have no products approved for commercial sale and have not generated any revenue from sales of any of our product candidates, and we do not know when, or if, we will generate revenues in the future. Our ability to generate revenue from product sales and achieve profitability will depend upon our ability to successfully gain regulatory approval and commercialize ganaxolone or other product candidates that we may develop, in-license or acquire in the future. Even if we obtain regulatory approval for ganaxolone, we do not know when we will generate revenue from product sales, if at all. Our ability to generate revenue from product sales from ganaxolone or any other future product candidates also depends on a number of additional factors, including our ability to:

successfully complete development activities, including enrollment of study participants and completion of the necessary clinical trials;

complete and submit NDAs to the FDA and obtain regulatory approval for indications for which there is a commercial market;

complete and submit applications to, and obtain regulatory approval from, foreign regulatory authorities;

make or have made commercial quantities of our products at acceptable cost levels;

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, or at all, which would likely materially and adversely affect the market price of our common stock.

We will require additional capital to fund our operations and if we fail to obtain necessary financing, we may be unable to complete the development and commercialization of ganaxolone, pay our debt obligations on a timely basis and otherwise enhance our liquidity.

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

We believe that our cash, cash equivalents and investments as of September 30, 2015, will enable us to fund our operating expenses and capital expenditure requirements into the fourth quarter of 2016. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to the:

initiation, progress, timing, costs and results of preclinical studies and clinical trials, including patient enrollment in such trials, for ganaxolone or any other future product candidates;

clinical development plans we establish for ganaxolone and any other future product candidates;

obligation to make royalty and non-royalty sublicense receipt payments to third-party licensors, if any, under our licensing agreements;

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

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

costs of filing, prosecuting, defending and enforcing any patent claims and maintaining and enforcing other intellectual property rights;

effects of competing technological and market developments;

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

Table of Contents

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

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

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

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

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

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

Because we have limited financial and managerial resources, we are focusing on research programs relating to ganaxolone, which concentrates the risk of product failure in the event ganaxolone proves to be ineffective or inadequate for clinical development or commercialization in this indication. As a result, we may forego or delay pursuit of opportunities for other technologies or product candidates that later could prove to have greater commercial potential. We may be unable to capitalize on viable commercial products or profitable market opportunities as a result

of our resource allocation decisions. Our spending on proprietary research and development programs relating to ganaxolone may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for ganaxolone, we may relinquish valuable rights to ganaxolone through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to ganaxolone.

Risks Related to Our Business and Development of Our Product

Our future success is dependent on the successful clinical development, regulatory approval and commercialization of ganaxolone, which is currently undergoing two clinical trials and will require significant capital resources and years of additional clinical development effort.

We do not have any products that have gained regulatory approval. Currently, our only clinical stage product candidate is ganaxolone. As a result, our business is dependent on our ability to successfully complete clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize ganaxolone in a timely 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

Table of Contents

from comparable foreign regulatory authorities. Before obtaining regulatory approvals for the commercial sale of ganaxolone for a target indication, we must demonstrate with substantial evidence gathered in preclinical studies and clinical trials, generally including two adequate and well-controlled clinical trials, and, with respect to approval in the United States, to the satisfaction of the FDA, that ganaxolone is safe and effective for use for that target indication and that the manufacturing facilities, processes and controls are adequate. Even if ganaxolone were to obtain approval from the FDA and comparable foreign regulatory authorities, any approval might contain significant limitations, such as restrictions as to specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval for ganaxolone in one or more jurisdictions, or any approval contains significant limitations, we may not be able to obtain sufficient funding or generate sufficient revenue to continue the development of any other product candidate that we may in-license, develop or acquire in the future. Furthermore, even if we obtain regulatory approval for ganaxolone, we will still need to develop a commercial organization, establish commercially viable pricing and obtain approval for adequate reimbursement from third-party and government 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 therapeutic benefit is unproven. Our ability to generate revenue from ganaxolone, which we do not expect will occur for at least the next several years, if ever, will depend heavily on our successful development and commercialization after regulatory approval, which is subject to many potential risks and may not occur. Ganaxolone may interact with human biological systems in unforeseen, ineffective or harmful ways. If ganaxolone is associated with undesirable side effects or has characteristics that are unexpected, we may need to abandon its development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in early stage testing for treating the target indications for ganaxolone have later been found to cause side effects that prevented further development of the compound. As a result of these and other risks described herein that are inherent in the development of novel therapeutic agents, we may never successfully develop, enter into or maintain third-party licensing or collaboration transactions with respect to, or

successfully commercialize, ganaxolone, in which case we will not achieve profitability and the value of our stock may decline.

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

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

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

Table of Contents

delay or failure in reaching agreement with the FDA or a comparable foreign regulatory authority on a trial design that we are able to execute;

delay or failure in obtaining authorization to commence a trial or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical trial;

delay or failure in reaching agreement on acceptable terms with prospective CROs and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;

delay or failure in obtaining institutional review board (IRB) approval or the approval of other reviewing entities, including comparable foreign regulatory authorities, to conduct a clinical trial at each site;

withdrawal of clinical trial sites from our clinical trials as a result of changing standards of care or the ineligibility of a site to participate in our clinical trials;

delay or failure in recruiting and enrolling suitable study subjects to participate in a trial;

delay or failure in study subjects completing a trial or returning for post-treatment follow-up;

clinical sites and investigators deviating from a trial protocol, failing to conduct the trial in accordance with regulatory requirements, or dropping out of a trial;

inability to identify and maintain a sufficient number of trial sites, many of which may already be engaged in other clinical trial programs, including some that may be for competing product candidates with the same indication;

failure of our third-party clinical trial managers to satisfy their contractual duties or meet expected deadlines;

delay or failure in adding new clinical trial sites;

ambiguous or negative interim results or results that are inconsistent with earlier results;

feedback from the FDA, IRBs, data safety monitoring boards, or a comparable foreign regulatory authority, or results from earlier stage or concurrent preclinical studies and clinical trials, that might require modification to the protocol for the trial;

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

unacceptable risk-benefit profile, unforeseen safety issues or adverse side effects or adverse events;

failure of a product candidate to demonstrate any benefit;

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

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.

Table of Contents

Study subject enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the subject population, the proximity of subjects to clinical sites, the eligibility criteria for the trial, the design of the clinical trial, ability to obtain and maintain subject consents, risk that enrolled subjects will drop out before completion, competing clinical trials and clinicians' and subjects' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved or product candidates that may be studied in competing clinical trials for the indications we are investigating. Some of our clinical trials are directed at small patient populations. Patient enrollment in these studies could be particularly challenging. In the past, we have experienced delays in enrolling patients in studies directed at small patient populations. We rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and while we have agreements governing their committed activities, we have limited influence over their actual performance.

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

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

Undesirable side effects caused by ganaxolone could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign regulatory authority. Although ganaxolone has generally been well tolerated by subjects in our earlier-stage clinical trials, in some cases there were side effects, and some of the side effects were severe. Specifically, in our most recently completed clinical trial, where ganaxolone was administered as an adjunctive to standard therapy in adult subjects with focal onset seizures, the most frequent side effects (those reported in greater than 5% of ganaxolone subjects) were dizziness, fatigue and somnolence (or drowsiness).

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;

Table of Contents