MARINUS PHARMACEUTICALS INC
Form 10-Q
August 03, 2018
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 JUNE 30, 2018

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.)

170 N. Radnor Chester Rd, Suite 250

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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer Accelerated filer

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

Emerging growth company

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

Indicate by check mark whether the registrant 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 August 1, 2018 was: 40,525,013.

Table of Contents

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

INDEX TO FORM 10-Q

FOR THE QUARTER ENDED JUNE 30, 2018

PART I – FINANCIAL INFORMATION

Table of Contents

PART I

FINANCIAL INFORMATION

Item 1. Financial Statements

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED BALANCE SHEETS

(in thousands, except share and per share amounts)

(unaudited)

	June 30, 2018	December 31, 2017
ASSETS		
Current assets:		
Cash and cash equivalents	$ 21,467	$ 33,531
Short-term investments	24,902	19,861
Prepaid expenses and other current assets	1,038	978
Total current assets	47,407	54,370
Property and equipment, net	1,280	1,338
Investments	—	4,964
Total assets	$ 48,687	$ 60,672
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,294	$ 927
Accrued expenses	2,266	1,617
Total current liabilities	3,560	2,544
Other long-term liabilities	107	120
Total liabilities	3,667	2,664
Stockholders' equity:		
Preferred stock, $0.001 par value; 25,000,000 shares authorized, no shares issued and outstanding	—	—
	41	41

Common stock, $0.001 par value; 100,000,000 shares authorized, 40,554,244 issued and 40,525,013 outstanding at June 30, 2018 and December 31, 2017		
Additional paid-in capital	205,279	202,790
Treasury stock at cost, 29,231 shares at June 30, 2018 and December 31, 2017	—	—
Accumulated other comprehensive loss	(69)	(96)
Accumulated deficit	(160,231)	(144,727)
Total stockholders' equity	45,020	58,008
Total liabilities and stockholders' equity	$ 48,687	$ 60,672

See accompanying notes to consolidated financial statements.

Table of Contents

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except share and per share amounts)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Expenses:				
Research and development	$ 7,232	$ 2,817	$ 11,159	$ 6,390
General and administrative	2,338	1,691	4,526	3,503
Loss from operations	(9,570)	(4,508)	(15,685)	(9,893)
Interest income	65	31	181	71
Interest expense	—	(72)	—	(156)
Other income (expense)	1	(3)	—	(12)
Net loss	$ (9,504)	$ (4,552)	$ (15,504)	$ (9,990)
Per share information:				
Net loss per share of common stock—basic and diluted	$ (0.24)	$ (0.21)	$ (0.38)	$ (0.47)
Basic and diluted weighted average shares outstanding	40,395,650	21,985,213	40,384,429	21,288,545
Net loss	$ (9,504)	$ —	$ (15,504)	$ —
Other comprehensive loss:				
Unrealized gain on available-for-sale securities	38	—	27	—
Total comprehensive loss	$ (9,466)	$ —	$ (15,477)	$ —

See accompanying notes to consolidated financial statements.

Table of Contents

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

(unaudited)

	Six Months Ended June 30,	
	2018	2017
Cash flows from operating activities		
Net loss	$ (15,504)	$ (9,990)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	65	22
Stock-based compensation expense	2,475	1,519
Amortization of debt issuance costs	—	4
Amortization of discount on investments	(51)	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(60)	(484)
Accounts payable and accrued expenses	1,198	(2,284)
Net cash used in operating activities	(11,877)	(11,213)
Cash flows from investing activities		
Maturities of short-term investments	—	3,178
Purchases of property and equipment	(8)	(143)
Net cash (used in) provided by investing activities	(8)	3,035
Cash flows from financing activities		
Proceeds from exercise of stock options	14	—
Principal payments of notes payable	—	(1,750)
Proceeds from equity offerings, net of offering costs	—	3,281
Repayments of short-term bank borrowings	(193)	—
Net cash (used in) provided by financing activities	(179)	1,531
Net decrease in cash and cash equivalents	(12,064)	(6,647)
Cash and cash equivalents—beginning of period	33,531	26,178
Cash and cash equivalents—end of period	$ 21,467	$ 19,531
Supplemental disclosure of cash flow information		
Cash paid for interest	$ —	$ 162

See accompanying notes to consolidated financial statements.

5

Table of Contents

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO INTERIM CONSOLIDATED FINANCIAL STATEMENTS

(unaudited)

1. Description of the Business and Liquidity

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

Liquidity

We have not generated any product revenues and have incurred operating losses since inception. There is no assurance that profitable operations will ever be achieved, and if achieved, could be sustained on a continuing basis. In addition, development activities, clinical and preclinical testing, and commercialization of our product candidates will require significant additional financing. Our accumulated deficit as of June 30, 2018 was $160.2 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 a secondary public offering during the third quarter of 2017, we issued a total of 10,733,334 shares of common stock resulting in aggregate net proceeds, after underwriting discounts and commissions and other estimated offering expenses, of $37.7 million. We believe that our cash, cash equivalents and investment balance as of June 30, 2018 is adequate to fund our operations for at least the next 12 months.

2. Summary of Significant Accounting Policies

Basis of Presentation

The unaudited interim consolidated 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) for annual financial statements. In the opinion of management, these unaudited interim consolidated financial statements reflect the elimination of all intercompany accounts and transactions and 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 consolidated financial statements should be read in conjunction with the audited consolidated financial statements for the year ended December 31, 2017 and accompanying notes thereto included in our annual report on Form 10-K filed with the SEC on March 6, 2018.

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

Table of Contents

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO INTERIM CONSOLIDATED FINANCIAL STATEMENTS

(unaudited)

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.

Recent Accounting Pronouncements

In January 2016, the Financial Accounting Standard Board (FASB) issued Accounting Standards Update (ASU) 2016-01, Financial Instruments - Overall (Subtopic 825-10), Recognition and Measurement of Financial Assets and Financial Liabilities, which addresses certain aspects of recognition, measurement, presentation, and disclosure of financial instruments. ASU 2016-01 will be effective for annual periods and interim periods within those annual periods beginning after December 15, 2017 and early adoption is not permitted. We adopted this ASU in the first quarter of 2018. The adoption of this ASU did not have a material impact on our interim consolidated financial statements.

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

In August 2016, the FASB issued ASU 2016-15, Statement of Cash Flows, which amends the guidance in Accounting Standards Codification (ASC) 230 on the classification of certain cash receipts and payments in the statement of cash flows. The primary purpose of the ASU is to reduce the diversity in practice that has resulted from the lack of consistent principles on this topic. The guidance in the ASU is effective for fiscal years beginning after December 15, 2017, including interim periods within those fiscal years. We adopted this ASU in the first quarter of 2018. The adoption of this ASU did not have a material impact on our interim consolidated financial statements.

In May 2017, the FASB issued ASU 2017-09, Scope of Modification Accounting, which amends the scope of modification accounting for share-based payment arrangements. The ASU provides guidance on the types of changes to the terms or conditions of share-based payment awards to which an entity would be required to apply modification accounting under ASC 718. Specifically, an entity would not apply modification accounting if the fair value, vesting

conditions, and classification of the awards are the same immediately before and after the modification. For all entities, the ASU is effective for annual reporting periods, including interim periods within those annual reporting periods, beginning after December 15, 2017, with early adoption permitted. We adopted this ASU in the first quarter of 2018. The adoption of this ASU did not have a material impact on our interim consolidated financial statements.

In June 2018, the FASB issued ASU 2018-07, Improvements to Nonemployee Share-Based Payment Accounting, which superseded ASC 505-50 and expands the scope of ASC 718 to include all share-based payment arrangements related to the acquisition of goods and services from both nonemployees and employees. As a result, most of the guidance in ASC 718 associated with employee share-based payments, including most of its requirements related to classification and measurement, applies to nonemployee share-base payment arrangements. We early-adopted this guidance effective April 1, 2018. The adoption of this guidance did not have a material impact on our interim 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.

Table of Contents

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO INTERIM CONSOLIDATED FINANCIAL STATEMENTS

(unaudited)

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

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

· Level 2 — Valuations based on observable inputs and quoted prices in active markets for similar assets and liabilities.

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

If the inputs used to measure fair value fall within different levels of the hierarchy, the category level is based on the lowest priority level input that is significant to the fair value measurement of the instrument.

Valuation Techniques - Level 2 Inputs

The Company estimates the fair values of its financial instruments categorized as level 2 in the fair value hierarchy, including U.S. Treasury securities, by taking into consideration valuations obtained from third-party pricing services. The pricing services use industry standard valuation models, including both income- and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, benchmark yields, issuer credit spreads, benchmark securities, and other observable inputs. The Company obtains a single price for each financial instrument and does not adjust the prices obtained from the pricing service. The Company validates the prices provided by its third-party pricing services by reviewing their pricing methods, obtaining market values from other pricing sources and comparing them to the share prices presented by the third-party pricing services. After completing its validation procedures, the Company did not adjust or override any fair value measurements provided by its third-party pricing services as of June 30, 2018.

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
June 30, 2018				
Assets				
Money market funds (cash equivalents)	$ 10,595	$ —	$ —	$ 10,595
U.S. Treasury securities	—	24,902	—	24,902
Total assets	$ 10,595	$ 24,902	$ —	$ 35,497
December 31, 2017				
Assets				
Money market funds (cash equivalents)	$ 33,531	$ —	$ —	$ 33,531
U.S. Treasury securities	—	24,825	—	24,825
Total assets	$ 33,531	$ 24,825	$ —	$ 58,356

Table of Contents

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO INTERIM CONSOLIDATED FINANCIAL STATEMENTS

(unaudited)

4. Accrued Expenses

At June 30, 2018 and December 31, 2017 accrued expenses consisted of the following (in thousands):

	June 30, 2018	December 31, 2017
Payroll and related costs	$ 946	$ 1,323
Clinical trials and drug development	1,035	156
Professional fees	224	113
Other	61	25
Total accrued expenses	$ 2,266	$ 1,617

5. Notes Payable

In 2014, we borrowed an aggregate of $7.0 million in connection with a Loan and Security Agreement, as amended, with an interest rate equal to the greater of (a) prime rate plus 3.25% or (b) 6.5%. In July 2017, we paid in full the entire outstanding term loans balance of $3.5 million and accrued interest, with no penalty for prepayment. Interest expense related to the term loans was $70 thousand and $152 thousand for the three and six months ended June 30, 2017, respectively.

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, and summarized in the table below:

	June 30,	
	2018	2017
Restricted stock	117,867	409,300
Stock options	4,741,109	2,908,836
	4,858,976	3,318,136

7. Investments

As of June 30, 2018, our investments consisted of U.S. Treasury securities, maturing at various dates through January 2019. These securities are classified as short-term investments on our consolidated balance sheets based on maturity and are classified as available-for-sale and are recorded at fair value.

As of June 30, 2018, all five of our U.S. Treasury securities were in an unrealized loss position, none of which had been in an unrealized loss position for 12 months or greater. Total amortized cost and fair value were $25.0 million and $24.9 million, respectively, as of June 30, 2018. Based on review of these securities, we believe that the cost basis of these available-for-sale securities is recoverable and that there were no other-than-temporary impairments on these securities as of June 30, 2018.

Table of Contents

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO INTERIM CONSOLIDATED FINANCIAL STATEMENTS

(unaudited)

8. Stockholders' Equity

In 2005, we adopted the 2005 Stock Option and Incentive Plan (2005 Plan) that authorizes us to grant options, restricted stock and other equity-based awards. As of June 30, 2018, 362,112 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.

In August 2014, we adopted our 2014 Equity Incentive Plan, amended in May 2017 (2014 Plan), that authorizes us to grant options, restricted stock, and other equity-based awards, subject to adjustment in accordance with the 2014 Plan. The amount, terms of grants, and exercisability provisions are determined and set by our board of directors. As of June 30, 2018, 4,378,997 options to purchase shares of common stock and 117,867 shares of restricted stock were outstanding pursuant to grants in connection with the 2014 Plan, and 1,286,003 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.

Stock Options

There were 4,741,109 stock options outstanding as of June 30, 2018 at a weighted-average exercise price of $5.50 per share. During the six months ended June 30, 2018, 1,121,000 options were granted to employees, directors and consultants at a weighted-average exercise price of $6.36 per share.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Research and development	$ 503	$ 175	$ 816	$ 370
General and administrative	828	489	1,607	955
Total	$ 1,331	$ 664	$ 2,423	$ 1,325

Restricted Stock

All issued and outstanding restricted shares of common stock are time-based, and become vested between one and three years after the grant date. Compensation expense is recorded ratably over the requisite service period. Compensation expense related to restricted stock is measured based on the fair value using the closing market price of our common stock on the date of the grant.

During the six months ended June 30, 2017, we issued 245,200 restricted shares of common stock to employees, directors and consultants. We did not issue any restricted shares of common stock during the six months ended June 30, 2018. As of June 30, 2018 there were 117,867 restricted shares of common stock outstanding, and 113,933 shares vested during the six months ended June 30, 2018.

Table of Contents

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO INTERIM CONSOLIDATED FINANCIAL STATEMENTS

(unaudited)

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Research and development	$ 5	$ 31	$ 12	$ 49
General and administrative	11	77	40	145
Total	$ 16	$ 108	$ 52	$ 194

9. Commitments

In March 2017, the Company and CyDex Pharmaceuticals, Inc. (CyDex) entered into a License Agreement and a Supply Agreement. Under the terms of the License Agreement, CyDex has granted us an exclusive license to use CyDex's Captisol drug formulation system and related intellectual property in connection with the development and commercialization of ganaxolone in any and all therapeutic uses in humans, with some exceptions.

As consideration for this license, we paid an upfront fee which was recorded as research and development expense in the three months ended March 31, 2017, and are required to make additional payments in the future upon achievement of various specified clinical and regulatory milestones. We will also be required to pay royalties to CyDex on sales of ganaxolone, if successfully developed, in the low-to-mid single digits based on levels of annual net sales.

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

certain limitations.

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 t or other comparable terminology intended to identify statements about the future. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement contained in this 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;
- enrollment in clinical trials, availability of data from ongoing clinical trials, expectations for regulatory approvals, or the attainment of clinical trial results that will be supportive of regulatory approvals;
- the potential benefits of ganaxolone;
- the timing of seeking 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;

Table of Contents

- the rate and degree of market acceptance of ganaxolone;
- the timing and amount or reimbursement for ganaxolone;
- the success of other competing therapies that may become available;
- 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 interim consolidated financial statements and related notes thereto which are included in this Quarterly Report on Form 10-Q; and (ii) our annual consolidated financial statements for the year ended December 31, 2017 which are included in our Annual Report on Form 10-K filed with the SEC on March 6, 2018.

Overview

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

CDKL5 Deficiency Disorder (CDD)

The Company is enrolling patients in its pivotal Phase 3 clinical trial (Marigold Study) evaluating the use of oral ganaxolone in children and young adults with CDD. The Marigold Study is a global, double-blind, placebo-controlled, trial that will enroll approximately 70 patients between the ages of 2 and 21 with a confirmed disease-related CDKL5 gene variant. Patients will undergo a baseline period before being randomized to receive either ganaxolone (up

Table of Contents

to 1,800 mg/day) or placebo for 17 weeks, in addition to their existing anti-seizure treatment. Following the treatment period, all patients that meet certain eligibility requirements will have the opportunity to receive ganaxolone in the open label phase of the study. The study's primary efficacy endpoint is percent reduction in seizures. Secondary outcome measures will include non-seizure-related endpoints to capture certain changes in behavioral and sleep disturbances that were reported in previous clinical studies with ganaxolone. There are currently no approved treatments for CDD.

Postpartum Depression (PPD)

The Company is in the process of completing enrollment in the IV only portion of the Magnolia study, a Phase 2 double-blind, placebo-controlled, dose-optimization clinical trial to evaluate ganaxolone in women diagnosed with severe PPD (Hamilton Depression Rating Scale (HAMD17) score ≥26). The efficacy endpoint is change from baseline in the HAMD17 score. The Company expects to provide top-line data in the fourth quarter and discuss next steps in our PPD program, which include evaluating an IV followed by oral formulations of ganaxolone.

Enrollment is on-going in the Company's Amaryllis study, a Phase 2 clinical trial to evaluate the safety, tolerability and efficacy of oral ganaxolone in women with moderate PPD (HAMD17 score of ≥ 20, but < 26). Patients enrolled in the initial open label phase of the study receive one of multiple treatment regimens with ganaxolone capsules which include once-daily dosing. The efficacy endpoint is change from baseline in the HAMD17 score. Data from the open label phase are expected in the fourth quarter of 2018 and will inform later stage development of novel treatment paradigms that may include an IV followed by oral ganaxolone pivotal program. Upon successful completion of the open label phase, the study will continue as a double-blind placebo-controlled trial.

Status Epilepticus (SE)

The Company is enrolling patients with refractory status epilepticus (RSE) in its Phase 2 study with ganaxolone IV. Initial data from this proof-of-concept study are expected in the fourth quarter of 2018.

Our operations to date have consisted primarily of organizing and staffing our company and developing ganaxolone, including conducting preclinical testing and clinical trials. We have funded our operations primarily through sales of equity and debt securities. At June 30, 2018, we had cash, cash equivalents and investment balances of $46.4 million. We have no products currently available for sale, have incurred operating losses since inception, have not generated any product sales revenue and have not achieved profitable operations. We incurred a net loss of $9.5 million and $15.5 million for the three and six months ended June 30, 2018. Our accumulated deficit as of June 30, 2018 was $160.2 million, and we expect to continue to incur substantial losses in future periods. We anticipate that our operating expenses will increase substantially as we continue to advance our clinical-stage product candidate, ganaxolone.

We anticipate that our expenses will increase substantially as we:

- conduct later stage clinical trials in targeted indications, which could include CDD, PPD, SE, PCDH19 pediatric epilepsy (PCDH19-PE), Lennox-Gastaut Syndrome, Fragile X Syndrome (FXS) and other indications;

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

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

- acquire the rights to other product candidates and fund its development;

- maintain, expand and protect our global intellectual property portfolio;

- hire additional clinical, manufacturing and scientific personnel; and

Table of Contents

add operational, financial and management information systems and personnel, including personnel to support our drug development and potential future commercialization efforts.

We believe that our cash, cash equivalents and investments as of June 30, 2018, will enable us to fund our operating expenses and capital expenditure requirements into 2020. 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), or equivalent Marketing Authorization Applications (MAA) outside the US, 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 our clinical programs will depend on numerous factors, including competition, manufacturing capability and commercial viability. See “Risk Factors.” Our commercial success depends upon attaining significant market acceptance, if approved, among physicians, patients, healthcare payers and the medical community. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success, as well as an assessment of commercial potential.

General and Administrative Expenses

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

Table of Contents

increase in the future as a result of employee hiring and scaling of our operations commensurate with supporting more advanced clinical trials and in preparation for commercial infrastructure. These increases will likely include increased costs for insurance, hiring of additional personnel, outside consultants, legal counsel and accountants, among other expenses.

Interest Income

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

Interest Expense

Interest expense is primarily attributable to interest expense associated with our credit facility entered into in April 2014, as amended, which was paid in full and closed in July 2017.

Results of Operations

Research and Development Expenses

Research and development expenses increased to $7.2 million and $11.2 million for the three and six months ended June 30, 2018, as compared to $2.8 million and $6.4 million for the same periods in the prior year. The primary drivers of our research and development expenditures are currently in our programs in PPD, refractory status epilepticus (RSE) and CDD. We have initiated Phase 2 trials each in PPD and RSE, and initiated a Phase 3 trial in CDD.

The following table shows our research and development expenses incurred with respect to each active program, in millions:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
CDKL5 disorder (1)	$ 1.2	$ 0.4	$ 1.7	$ 0.7
Postpartum depression (2)	2.5	0.6	3.5	0.9
Refractory status epilepticus (3)	1.1	0.3	1.5	1.1
Indirect research and development (4)	2.4	1.4	4.5	2.8
Focal onset seizures (5)				