

MARINUS PHARMACEUTICALS INC

Form 10-Q

May 14, 2015

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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

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FORM 10-Q

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

FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2015

OR

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

COMMISSION FILE NUMBER 001-36576

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MARINUS PHARMACEUTICALS, INC.

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(Exact name of registrant as specified in its charter)

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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

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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.

Large accelerated filer

Accelerated filer

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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 May 11, 2015 was: 14,229,666.

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MARINUS PHARMACEUTICALS, INC.

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FOR THE QUARTER ENDED MARCH 31, 2015

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## PART I

## FINANCIAL INFORMATION

## Item 1. Financial Statements

## MARINUS PHARMACEUTICALS, INC.

## BALANCE SHEETS

(in thousands, except share and per share amounts)

(unaudited)

	March 31, 2015	December 31, 2014
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 44,477	\$ 49,720
Short-term investments	995	—
Prepaid expenses and other current assets	322	428
Total current assets	45,794	50,148
Property and equipment, net of accumulated depreciation of \$358 and \$356	42	44
Investments	496	—
Other assets	19	21
Total assets	\$ 46,351	\$ 50,213
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Current portion of long term debt	\$ 875	\$ —
Accounts payable	3,055	536
Accrued expenses	1,516	1,503
Total current liabilities	5,446	2,039
Notes payable	6,125	7,000
Other long term liabilities	50	20
Total liabilities	11,621	9,059
Stockholders' equity:		

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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, 14,228,897 issued and 14,199,666 outstanding at March 31, 2015 and 14,036,985 issued and 14,007,754 outstanding at December 31, 2014	14	14
Additional paid-in capital	114,059	113,476
Treasury stock at cost, 29,231 shares at March 31, 2015 and December 31, 2014	—	—
Accumulated deficit	(79,343)	(72,336)
Total stockholders' equity	34,730	41,154
Total liabilities and stockholders' equity	\$ 46,351	\$ 50,213

See accompanying notes to financial statements.

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MARINUS PHARMACEUTICALS, INC.

## STATEMENTS OF OPERATIONS

(in thousands, except share and per share amounts)

(unaudited)

	Three Months Ended March 31,	
	2015	2014
Expenses:		
Research and development	\$ 5,468	\$ 2,149
General and administrative	1,447	516
Loss from operations	(6,915)	(2,665)
Change in fair value of warrant liability	—	428
Interest income	14	4
Interest expense	(116)	—
Other income (expense)	10	(2)
Net loss	(7,007)	(2,235)
Cumulative preferred stock dividends	—	(1,071)
Net loss applicable to common stockholders	\$ (7,007)	\$ (3,306)
Per share information:		
Net loss per share of common stock—basic and diluted	\$ (0.50)	\$ (7.09)
Basic and diluted weighted average shares outstanding	14,068,238	466,531

See accompanying notes to financial statements.

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MARINUS PHARMACEUTICALS, INC.

## STATEMENTS OF CASH FLOWS

(in thousands)

(unaudited)

	Three Months Ended March 31,	
	2015	2014
Cash flows from operating activities		
Net loss	\$ (7,007)	\$ (2,235)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	2	3
Stock-based compensation expense	383	23
Change in fair value of warrant liability	—	(428)
Amortization of debt issuance costs	2	—
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	107	568
Accounts payable and accrued expenses	2,561	944
Net cash used in operating activities	(3,952)	(1,125)
Cash flows from investing activities		
Purchases of investments	(1,491)	—
Net cash used in investing activities	(1,491)	—
Cash flows from financing activities		
Proceeds from exercise of stock options	200	28
Proceeds from issuance of convertible preferred stock, net of issuance costs	—	(110)
Net cash provided by (used in) financing activities	200	(82)
Net decrease in cash and cash equivalents	(5,243)	(1,207)
Cash and cash equivalents—beginning of period	49,720	10,037
Cash and cash equivalents—end of period	\$ 44,477	\$ 8,830
Supplemental disclosure of cash flow information		
Cash paid for interest	\$ 114	\$ —

See accompanying notes to financial statements.





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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<sub>A</sub> type receptors in the brain, which has been identified as playing an important role in certain seizure, psychiatric and developmental disorders. Our primary focus to date since our inception has been directed towards developing business strategies, raising capital, 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 March 31, 2015 was \$79.3 million and we expect to incur substantial losses in future periods. We plan to finance our future operations with a combination of proceeds from the issuance of equity securities, the issuance of additional debt, potential collaborations and revenues from potential future product sales, if any. We have not generated positive cash flows from operations, and there are no assurances that we will be successful in obtaining an adequate level of financing for the development and commercialization of our planned product candidates.

In connection with the closing 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 March 31, 2015 are adequate to fund our operations into the second half 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 (the "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.

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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 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.

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.
- 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.

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MARINUS PHARMACEUTICALS, INC.

## NOTES TO INTERIM FINANCIAL STATEMENTS

(unaudited)

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
March 31, 2015				
Assets				
Money market funds (cash equivalents)	\$ 43,764	\$ —	\$ —	\$ 43,764
Short-term investments	995	—	—	995
Investments	496	—	—	496
Total assets	\$ 45,255	\$ —	\$ —	\$ 45,255
December 31, 2014				
Assets				
Money market funds (cash equivalents)	\$ 48,960	\$ —	\$ —	\$ 48,960
Short-term investments	—	—	—	—
Investments	—	—	—	—
Total assets	\$ 48,960	\$ —	\$ —	\$ 48,960

## 4. Accrued Expenses

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

	March 31, 2015	December 31, 2014
Payroll and related costs	286	419
Clinical trials and drug development	894	777
Professional fees	143	186
Other	193	121
Total accrued expenses	\$ 1,516	\$ 1,503

## 5. Notes Payable

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

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MARINUS PHARMACEUTICALS, INC.

## NOTES TO INTERIM FINANCIAL STATEMENTS

(unaudited)

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

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

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

In connection with the Amended LSA, we borrowed \$5.0 million available to us under Tranche B 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 3.5% or (b) 6.5% until December 2015. Commencing in January 2016 and continuing through December 2017, we are required to make monthly payments of payments of 1/24th of our principal borrowings plus interest. If we achieve certain clinical trial milestones by August 2015, both the interest-only period and principal maturity date will be extended by six months.

As of March 31, 2015, of our outstanding term loan balance of \$7.0 million, \$0.9 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 for the three months ended March 31, 2015. As of March 31, 2015, we had accrued interest of \$39 thousand. There are no financial covenants associated with these term loans. As of March 31, 2015, we were in compliance with all non-financial covenants.

## 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.

The following potentially dilutive securities outstanding as of March 31, 2015 and 2014 have been excluded from the computation of diluted weighted average shares outstanding, as they would be antidilutive:

	March 31,	
	2015	2014
Convertible preferred stock	—	7,596,927
Warrants	—	470,026
Stock options	1,478,662	1,066,173
	1,478,662	9,133,126

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MARINUS PHARMACEUTICALS, INC.

NOTES TO INTERIM FINANCIAL STATEMENTS

(unaudited)

7. Investments

As of March 31, 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 March 31, 2015, 778,662 options to purchase 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 March 31, 2015, 700,000 options to purchase shares of common stock were outstanding pursuant to grants in connection with the 2014 Plan, and 560,310 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 1,478,662 stock options outstanding as of March 31, 2015 at a weighted-average exercise price of \$4.80 per share. During the three-month period ended March 31, 2015, no options were granted and 191,912 options were exercised at a weighted-average exercise price of \$1.04.

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

	Three Months Ended March 31,	
	2015	2014
Research and development	\$ 127	\$ 2
General and administrative	256	21
Total stock based compensation expense	\$ 383	\$ 23

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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 f 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 or reimbursement for ganaxolone;

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- 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 small molecule that is a synthetic analog of allopregnanolone, an endogenous neurosteroid produced in the central nervous system and known for its anticonvulsive and antianxiety activity. By targeting the same spectrum of GABAA receptors as endogenous allopregnanolone, ganaxolone delivers its therapeutic benefit through a natural mechanism that we believe may offer safety and efficacy advantages compared to other marketed antiepileptic medications. Ganaxolone was rationally designed to unlock the potential of GABAA receptor modulation through chronic neurosteroid therapy. We have a dual strategy of 1) evaluating ganaxolone for treating seizure disorders, and 2) treating targeted orphan diseases for which there are no approved therapies and for which the development timelines may be abbreviated, where there is a strong mechanistic rationale for ganaxolone to offer therapeutic benefit to patients with very high unmet needs. Our orally administered solid and liquid suspension dose forms are being evaluated in our ongoing clinical trials and we are conducting the requisite preclinical experiments to ready our intravenous, or IV, dose form for clinical use.

Our lead indication for ganaxolone is as an adjunctive, or add-on, therapy for the treatment of partial, also known as focal, onset seizures in adults with epilepsy. We have completed a Phase 2 clinical trial in 147 patients with focal onset seizures demonstrating that patients who added ganaxolone to their medication regimen experienced a statistically significant reduction in seizures as compared to patients who added placebo. We are currently enrolling patients in a multinational, randomized, placebo-controlled, Phase 3 clinical trial to evaluate ganaxolone as adjunctive

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treatment of partial-onset seizures in adult subjects. We believe ganaxolone also has potential in a broad range of neuropsychiatric disorders, including orphan indications. We have generated proof-of-concept data for ganaxolone in the treatment of refractory pediatric seizures and as monotherapy for adult refractory focal onset seizures. We currently have a Phase 2 proof-of-concept clinical study on-going with ganaxolone for the treatment of PCDH19 female pediatric epilepsy (PCDH19) and a Phase 2 proof-of-concept investigator-sponsored clinical trial evaluating ganaxolone as a treatment for behaviors in Fragile X Syndrome (FXS). We believe that PCDH19 and FXS are orphan disorders that have been related to mutations affecting GABA signaling at extrasynaptic GABAA receptors.

Our operations to date have consisted primarily of organizing and staffing our company, developing ganaxolone, including conducting preclinical testing and clinical trials, and raising capital. We have funded our operations primarily through sales of equity and debt securities. From inception through March 31, 2015, we have received net proceeds of \$110.4 million from the issuance of preferred stock, common stock and convertible notes payable. At March 31, 2015, we had cash and cash equivalents of \$44.5 million and investment balances of \$1.5 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 net losses of \$7.0 million for the three months ended March 31, 2015. Our accumulated deficit as of March 31, 2015 was \$79.3 million, and we expect to continue to incur substantial losses in future periods. We anticipate that our operating expenses will increase substantially as we continue to advance our clinical-stage product candidate, ganaxolone.

We anticipate that our expenses will increase substantially as we:

increase the targeted enrollment and add enrollment sites and geographies for our ongoing Phase 3 clinical trial for adjunctive treatment of ganaxolone in adult patients with refractory partial onset epileptic seizures;

conduct clinical proof-of-concept clinical trials in targeted pediatric rare disease indications, including PCDH19 and FXS;

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

maintain, expand and protect our global intellectual property portfolio;

hire additional clinical, manufacturing, and scientific personnel; and

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

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

We believe that our cash, cash equivalents and investments as of March 31, 2015 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2016. However, we will need to secure additional funding in the future, from one or more equity or debt financings, collaborations, or other sources, in order to carry out all of our planned research and development activities with respect to ganaxolone.



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Financial Overview

Research and Development Expenses

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

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

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

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

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

costs associated with preclinical activities and regulatory operations.

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

We will incur substantial costs beyond our present and planned clinical trials in order to file a New Drug Application (NDA) and Supplemental New Drug Applications (sNDAs) for ganaxolone in patients with focal onset seizures, PCDH19, FXS and other target indications, and in each case, the nature, design, size and cost of further studies and trials will depend in large part on the outcome of preceding studies and trials and discussions with regulators. It is difficult to determine with certainty the costs and duration of our current or future clinical trials and preclinical studies, or if, when or to what extent we will generate revenue from the commercialization and sale of ganaxolone if we obtain regulatory approval. We may never succeed in achieving regulatory approval for ganaxolone. The duration, costs and timing of clinical trials and development of ganaxolone will depend on a variety of factors, including the uncertainties of future clinical trials and preclinical studies, uncertainties in clinical trial enrollment rate and significant and changing government regulation.

In addition, the probability of success for ganaxolone will depend on numerous factors, including competition, manufacturing capability and commercial viability. See “Risk Factors.” Our commercial success depends upon attaining significant market acceptance of ganaxolone, if approved, among physicians, patients, healthcare payors and the medical community. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success of ganaxolone, as well as an assessment of ganaxolone’s commercial potential.

#### General and Administrative Expenses

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

We expect that our general and administrative expenses will increase in the future as a result of new management and employee hiring and our scaling operations commensurate with supporting more advanced clinical trials and public company infrastructure. These increases will likely include increased costs for insurance, hiring of additional personnel, outside consultants, investor relations, legal counsel and accountants, among other expenses.

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### Change in Fair Value of Warrant Liability

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

### Results of Operations

#### Research and Development Expenses

Research and development expenses increased to \$5.5 million for the three months ended March 31, 2015, as compared to \$2.1 million same period in the prior year. The increase resulted primarily from an increase in clinical costs related to our ongoing clinical trial of ganaxolone in patients with focal onset seizures, as well as hiring additional clinical resources including our Chief Medical Officer. Substantially all research and development expenses relate to our clinical trial in focal onset epileptic seizures.

#### General and Administrative Expenses

General and administrative expenses increased to \$1.4 million three months ended March 31, 2015, as compared to \$0.5 million for the same period in the prior year. The increase in general and administrative expenses was primarily due to the hiring of new management and the upward scaling of our operations in connection with both our public company status as of July 31, 2014 and our ongoing clinical trial of ganaxolone in patients with focal onset 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 \$0.4 million for the three months ended March 31, 2014. We reduced the value of the liability to zero in connection with the closing of our initial public offering in the third quarter of 2014 as the warrants expired unexercised.

### Cumulative Preferred Stock Dividends

Cumulative preferred stock dividends were \$1.1 million for the three months ended March 31, 2014. Upon conversion of all outstanding convertible preferred stock in connection with our initial public offering during the third quarter of 2014, all cumulative preferred stock dividends were canceled.

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## Liquidity and Capital Resources

Since inception, we have incurred net losses and negative cash flows from our operations. We incurred net losses of \$7.0 million and \$2.2 million for the three months ended March 31, 2015 and 2014, respectively. Our cash used in operating activities was \$4.0 million for the three months ended March 31, 2015 compared to \$1.1 million for the same period a year ago. Historically, we have financed our operations principally through the sale of common stock, preferred stock and convertible debt. From inception through March 31, 2015, we have received net proceeds of \$110.4 million from the issuance of preferred stock, common stock and convertible notes payable. At March 31, 2015, we had cash and cash equivalents of \$44.5 million and investment balances of \$1.5 million.

## Square 1 Credit Facility

In April 2014, we borrowed \$2.0 million in connection with a term loan pursuant to a Loan and Security Agreement (LSA) we entered into with Square 1 Bank (Square 1). Pursuant to the terms of the LSA, we made monthly interest-only payments for outstanding borrowings at an interest rate equal to the greater of (a) prime plus 2.25% or (b) 5.5% until the LSA was amended in December 2014.

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

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

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

As of March 31, 2015, of our outstanding term loan balance of \$7.0 million, \$0.9 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 for the three months ended March 31, 2015. As of March 31, 2015, we had accrued interest of \$39 thousand. There are no financial covenants associated with these term loans. As of March 31,

2015, we were in compliance with all non-financial covenants.

#### Cash Flows

**Operating Activities.** Cash used in operating activities increased to \$4.0 million for the three months ended March 31, 2015 compared to \$1.1 million for the same period a year ago. The increase was driven primarily by an increase in our net loss of \$4.8 million, partially offset by net increases in operating liabilities of \$1.6 million and an increase in stock-based compensation expense of \$0.4 million. The increase in operating liabilities was primarily driven by increases in our trade accounts payable due to the upward scaling of our operations related to our ongoing clinical trial of ganaxolone in patients with focal onset seizures.

**Investing Activities.** Cash used in investing activities represents the purchase of \$1.5 million investments during the three months ended March 31, 2015. There were no investing activities during the three months ended March 31, 2014.

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Financing Activities. Cash provided by and used in financing activities was \$0.2 million or less for the three months ended March 31, 2015 and 2014.

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 March 31, 2015, will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2016. However, we will need to raise substantial additional financing in the future to fund our operations. In order to meet these additional cash requirements, we may seek to sell additional equity or convertible debt securities that may result in dilution to our stockholders. If we raise additional funds through the issuance of convertible debt securities, these securities could have rights senior to those of our common stock and could contain covenants that restrict our operations. There can be no assurance that we will be able to obtain additional equity or debt financing on terms acceptable to us, if at all. Our failure to obtain sufficient funds on acceptable terms when needed could have a negative impact on our business, results of operations, and financial condition. Our future capital requirements will depend on many factors, including:

the results of our preclinical studies and clinical trials;

the development, formulation and commercialization activities related to ganaxolone;

the scope, progress, results and costs of researching and developing ganaxolone or any other future product candidates, and conducting preclinical studies and clinical trials;

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

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

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

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

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

the expenses needed to attract and retain skilled personnel;

the costs associated with being a public company;

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

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



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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 three months ended March 31, 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 and cash equivalents of \$44.5 million and investment balances of \$1.5 million at March 31, 2015, consisting primarily of funds in cash and money market accounts and certificates of deposit. The primary objective of our investment activities is to preserve principal and liquidity while maximizing income without significantly increasing risk. We do not enter into investments for trading or speculative purposes. Due to the conservative nature of our investment portfolio, we do not believe an immediate 1.0% increase in interest rates would have a material effect on the fair market value of our portfolio, and accordingly we do not expect a sudden change in market interest rates to affect materially our operating results or cash flows.

Our long-term debt carries a variable interest rate indexed to the prime rate, with a fixed minimum rate of 6.5%. The prime rate in the U.S. 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 March 31, 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 March 31, 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.

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(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 March 31, 2015 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

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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 for or to commercialize any of our product candidates. Consequently, any predictions about our future performance may not be as accurate as they would be if we had a history of successfully developing and commercializing biopharmaceutical products.

We have incurred significant operating losses since our inception, including net losses of \$7.0 million for three months ended March 31, 2015. As of March 31, 2015, we had an accumulated deficit of \$79.3 million. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital. Our losses have resulted principally from costs incurred in our research and development activities. We anticipate that our operating losses will substantially increase over the next several years as we execute our plan to expand our research, development and commercialization activities, including the clinical development and planned commercialization of our product candidate, ganaxolone, and incur the additional costs of operating as a public company. In addition, if we obtain regulatory approval of ganaxolone, we may incur significant sales and marketing expenses. Because of the numerous risks and uncertainties associated with developing biopharmaceutical products, we are unable to predict the extent of any future losses or whether or when we will become profitable, if ever.

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

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

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

complete and submit NDAs to the United States Food and Drug Administration (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;

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develop a commercial organization capable of manufacturing, selling, marketing and distributing any products we intend to sell ourselves in the markets in which we choose to commercialize on our own;

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

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

In addition, because of the numerous risks and uncertainties associated with product development, including that ganaxolone may not advance through development or achieve the endpoints of applicable clinical trials, we are unable to predict the timing or amount of increased expenses, or if or when we will be able to achieve or maintain profitability. Even if we are able to complete the development and regulatory process for ganaxolone, we anticipate incurring significant costs associated with commercializing ganaxolone.

Even if we are able to generate revenue from the sale of ganaxolone or any future commercial products, we may not become profitable and will need to obtain additional funding to continue operations. If we fail to become profitable or are unable to sustain profitability on a continuing basis, and we are not successful in obtaining additional funding, then we may be unable to continue our operations at planned levels, which would depress the market price of our common stock.

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

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

We believe that our cash, cash equivalents and investments as of March 31, 2015, will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2016. We have based this estimate on assumptions that may prove to be wrong, and we could deploy our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to the:

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

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

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

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

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

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

effects of competing technological and market developments;

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

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

If we are unable to expand our operations or otherwise capitalize on our business opportunities due to a lack of capital, our ability to become profitable will be compromised.

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

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

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

Because we have limited financial and managerial resources, we are focusing on research programs relating to ganaxolone for focal onset seizures, which concentrates the risk of product failure in the event ganaxolone proves to be ineffective or inadequate for clinical development or commercialization in this indication. As a result, we may forego or delay pursuit of opportunities for other indications or with other technologies or product candidates that later could prove to have greater commercial potential. We may be unable to capitalize on viable commercial products or profitable market opportunities as a result of our resource allocation decisions. Our spending on proprietary research and development programs relating to ganaxolone may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for ganaxolone, we may relinquish valuable rights to ganaxolone through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to ganaxolone.



Risks Related to Our Business and Development of Our Product

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

We do not have any products that have gained regulatory approval. Currently, our only clinical stage product candidate is ganaxolone. As a result, our business is dependent on our ability to successfully complete clinical development of, obtain regulatory approval for, and, if approved, successfully commercialize ganaxolone in a timely manner. We cannot commercialize ganaxolone in the United States without first obtaining regulatory approval from the FDA; similarly, we cannot commercialize ganaxolone outside of the United States without obtaining regulatory approval from comparable foreign regulatory authorities. Before obtaining regulatory approvals for the commercial sale of ganaxolone for a target indication, we must demonstrate with substantial evidence gathered in preclinical studies and clinical trials, generally including two adequate and well-controlled clinical trials, and, with respect to approval in the United States, to the satisfaction of the FDA, that ganaxolone is safe and effective for use for that target indication and that the manufacturing facilities, processes and controls are adequate. We have expanded our ongoing Phase 2b clinical trial so that it may serve as one of our adequate and well-controlled clinical trials for ganaxolone in epilepsy; however,

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we cannot be certain that the FDA will accept the trial as such. Even if ganaxolone were to successfully obtain approval from the FDA and comparable foreign regulatory authorities, any approval might contain significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval for ganaxolone in one or more jurisdictions, or any approval contains significant limitations, we may not be able to obtain sufficient funding or generate sufficient revenue to continue the development of any other product candidate that we may in-license, develop or acquire in the future. Furthermore, even if we obtain regulatory approval for ganaxolone, we will still need to develop a commercial organization, establish commercially viable pricing and obtain approval for adequate reimbursement from third-party and government payors. If we are unable to successfully commercialize ganaxolone, we may not be able to earn sufficient revenue to continue our business.

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

Success in preclinical studies and early clinical trials does not ensure that later trials will generate adequate data to demonstrate the efficacy and safety of ganaxolone. A number of companies in the pharmaceutical and biotechnology industries, including those with greater resources and experience, have suffered significant setbacks in preclinical studies and clinical trials, even after seeing promising results in earlier studies and trials. Despite the results reported in earlier clinical trials for ganaxolone, we do not know whether the clinical trials we may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market ganaxolone in any particular jurisdiction. If later stage clinical trials do not produce favorable results, our ability to achieve regulatory approval for ganaxolone may be adversely impacted.

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

Ganaxolone is a novel compound and its potential benefit as a therapeutic for focal onset seizures, PCDH19 and FXS is unproven. Our ability to generate revenue from ganaxolone, which we do not expect will occur for at least the next several years, if ever, will depend heavily on our successful development and commercialization after regulatory approval, which is subject to many potential risks and may not occur. Ganaxolone may interact with human biological systems in unforeseen, ineffective or harmful ways. If ganaxolone is associated with undesirable side effects or has characteristics that are unexpected, we may need to abandon its development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in early stage testing for treating the target indications for ganaxolone have later been found to cause side effects that prevented further development of the compound. As a result of these and other risks described herein that are inherent in the development of novel therapeutic agents, we may never successfully develop, enter into or maintain third-party licensing or collaboration transactions with respect to, or successfully commercialize, ganaxolone, in which case we will not achieve profitability and the value of our stock may decline.

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

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

We may experience delays in our ongoing or future clinical trials and we do not know whether planned clinical trials will begin or enroll subjects on time, need to be redesigned or be completed on schedule, if at all. There can be no assurance that the FDA or other foreign regulatory authorities will not put clinical trials of ganaxolone on clinical hold now or in the future. Clinical trials may be delayed, suspended or prematurely terminated for a variety of reasons, such as:

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

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

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

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

failure of a product candidate to demonstrate any benefit;

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

lack of adequate funding to continue the clinical trial, including the incurrence of unforeseen costs due to enrollment delays, requirements to conduct additional clinical trials or increased expenses associated with the services of our CROs and other third parties;

political developments that affect our ability to develop and obtain approval for ganaxolone, or license rights to develop and obtain approval for ganaxolone, in a foreign country; or

changes in governmental regulations or administrative actions.

Study subject enrollment, a significant factor in the timing of clinical trials, is affected by many factors including the size and nature of the subject population, the proximity of subjects to clinical sites, the eligibility criteria

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for the trial, the design of the clinical trial, ability to obtain and maintain subject consents, risk that enrolled subjects will drop out before completion, competing clinical trials and clinicians' and subjects' perceptions as to the potential advantages of the product candidate being studied in relation to other available therapies, including any new drugs that may be approved or product candidates that may be studied in competing clinical trials for the indications we are investigating. We rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials, and while we have agreements governing their committed activities, we have limited influence over their actual performance.

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

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

Undesirable side effects caused by ganaxolone could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign regulatory authority. Although ganaxolone has generally been well tolerated by subjects in our earlier-stage clinical trials, in some cases there were side effects, and some of the side effects were severe. Specifically, in our most recently completed clinical trial, where ganaxolone was administered as an adjunctive to standard therapy in adult subjects with focal onset seizures, the most frequent side effects (those reported in greater than 5% of ganaxolone subjects) were dizziness, fatigue and somnolence (or drowsiness). More side effects of the Central Nervous System (CNS) were categorized as severe as compared to side effects of other body systems, though no specific CNS side effect was reported as severe by more than one subject.

If these side effects are reported in future clinical trials, or if other safety or toxicity issues are reported in our future clinical trials, we may not receive approval to market ganaxolone, which could prevent us from ever generating revenue or achieving profitability. Furthermore, although we are currently developing ganaxolone for three indications, negative safety findings in any one indication could force us to delay or discontinue development in other indications. Results of our clinical trials could reveal an unacceptably high severity and prevalence of side effects. In such an event, our clinical trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development, or deny approval, of ganaxolone for any or all targeted indications. Drug-related side effects could affect study subject recruitment or the ability of enrolled subjects to complete our future clinical trials and may result in potential product liability claims.

Additionally, if ganaxolone receives marketing approval, and we or others later identify undesirable side effects caused by ganaxolone, a number of potentially significant negative consequences could result, including:

we may be forced to suspend marketing of ganaxolone;

regulatory authorities may withdraw their approvals of ganaxolone;

regulatory authorities may require additional warnings on the label that could diminish the usage or otherwise limit the commercial success of ganaxolone;

we may be required to conduct post-marketing studies;

we could be sued and held liable for harm caused to subjects or patients; and

our reputation may suffer.

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

Even if ganaxolone receives regulatory approval, we may still face future development and regulatory difficulties.

Even if we obtain regulatory approval for ganaxolone, it would be subject to ongoing requirements by the FDA and comparable foreign regulatory authorities governing the manufacture, quality control, further development, labeling, packaging, storage, distribution, safety surveillance, import, export, advertising, promotion, recordkeeping and reporting of safety and other post-market information. The safety profile of ganaxolone will continue to be closely monitored by the FDA and comparable foreign regulatory authorities after approval. If new safety information becomes available after approval of ganaxolone, the FDA or comparable foreign regulatory authorities may require labeling changes or establishment of a Risk Evaluation and Mitigation Strategy (REMS) or similar strategy, impose significant restrictions on ganaxolone's indicated uses or marketing, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. For example, the label ultimately approved for ganaxolone, if it achieves marketing approval, may include restrictions on use.

In addition, manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current good manufacturing practices (cGMP) and other regulations. If we or a regulatory authority discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory authority may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. If we, ganaxolone or the manufacturing facilities for ganaxolone fail to comply with applicable regulatory requirements, a regulatory authority may:

issue warning letters or untitled letters;

mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;

require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;

seek an injunction or impose civil or criminal penalties or monetary fines;



suspend or withdraw regulatory approval;

suspend any ongoing clinical trials;

refuse to approve pending applications or supplements to applications filed by us;

suspend or impose restrictions on operations, including costly new manufacturing requirements; or

seize or detain products, refuse to permit the import or export of products, or require us to initiate a product recall.

The occurrence of any event or penalty described above may inhibit or preclude our ability to commercialize ganaxolone and generate revenue.

The FDA's and other regulatory authorities' policies may change, and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of ganaxolone. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval

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that we may have obtained, and we may not achieve or sustain profitability, which would adversely affect our business, prospects, financial condition and results of operations.

Advertising and promotion of any product candidate that obtains approval in the United States will be heavily scrutinized by, among others, the FDA, the Department of Justice (DOJ), the Office of Inspector General of the Department of Health and Human Services (HHS), state attorneys general, members of Congress and the public. Violations, including promotion of our products for unapproved or off-label uses, are subject to enforcement letters, inquiries and investigations, and civil and criminal sanctions by the FDA or other government agencies. Additionally, advertising and promotion of any product candidate that obtains approval outside of the United States will be heavily scrutinized by comparable foreign regulatory authorities.

In the United States, engaging in impermissible promotion of ganaxolone for off-label uses can also subject us to false claims litigation under federal and state statutes, and other litigation and/or investigation, which can lead to civil and criminal penalties and fines and agreements that materially restrict the manner in which we promote or distribute our drug products. These false claims statutes include the federal False Claims Act, which allows any individual to bring a lawsuit against a pharmaceutical company on behalf of the federal government alleging submission of false or fraudulent claims, or causing to present such false or fraudulent claims, for payment by a federal program such as Medicare or Medicaid. If the government prevails in the lawsuit, the individual will share in any fines or settlement funds. Since 2004, these False Claims Act lawsuits against pharmaceutical companies have increased significantly in volume and breadth, leading to several substantial civil and criminal settlements based on certain sales practices promoting off-label drug uses. This increasing focus and scrutiny has increased the risk that a pharmaceutical company will have to defend a false claim action, pay settlement fines or restitution, agree to comply with burdensome reporting and compliance obligations, and be excluded from the Medicare, Medicaid and other federal and state healthcare programs. If we do not lawfully promote our approved products, we may become subject to such litigation and/or investigation and, if we are not successful in defending against such actions, those actions could compromise our ability to become profitable.

Failure to obtain regulatory approval in international jurisdictions would prevent ganaxolone from being marketed in these jurisdictions.

In order to market and sell our products in the European Union and many other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. If we are unable to obtain approval of

ganaxolone by regulatory authorities in the European Union or another country or jurisdiction, the commercial prospects of ganaxolone may be significantly diminished and our business prospects could decline.

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

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States. Generally, if a product with an orphan drug designation subsequently receives the first marketing approval for an indication for which it receives the designation, then the product is entitled to a period of marketing exclusivity that precludes the applicable regulatory authority from approving another marketing application for the same drug for the same indication for the exclusivity period except in limited situations. For purposes of small molecule drugs, the FDA defines “same drug” as a drug that contains the same active moiety and is intended for the same use as the drug in question. A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation.

We have received orphan drug designation for the treatment of PCDH19 and expect that we may in the future pursue orphan drug designations for ganaxolone for one or more additional indications, including behaviors in FXS, as well as certain of our future product candidates. However, obtaining an orphan drug designation can be difficult and we may not be successful in doing so for ganaxolone or any of our future product candidates. Even if we were to obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product from the competition of different drugs for the same condition, which could be approved during the exclusivity period. Additionally, after an orphan drug is approved, the FDA could subsequently approve another application for the same drug for the same indication if the FDA concludes that the later drug is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusive marketing rights in the United States also may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition. The failure to obtain an orphan drug designation for any product candidates we may develop, the inability to maintain that designation for the duration of the applicable period, or the inability to obtain or maintain orphan drug exclusivity could reduce our ability to make sufficient sales of the applicable product candidate to balance our expenses incurred to develop it, which would have a negative impact on our operational results and financial condition.

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

Despite the implementation of security measures, our internal computer systems, and those of our CROs and other third parties on which we rely, are vulnerable to damage from computer viruses, unauthorized access, natural disasters, fire, terrorism, war and telecommunication and electrical failures. In addition, our systems safeguard important confidential personal data regarding subjects enrolled in our clinical trials. If a disruption event were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. For example, the loss of clinical trial data from completed, ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate

disclosure of confidential or proprietary information, we could incur liability and the further development of ganaxolone could be delayed.

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

Our operations could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce ganaxolone. Our ability to obtain clinical supplies of ganaxolone could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption. The ultimate impact on us, our significant suppliers and our general infrastructure of being in certain geographical areas is unknown, but our operations and financial condition could suffer in the event of a major earthquake, fire or other natural disaster.

Risks Related to the Commercialization of Our Product

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

Even if ganaxolone receives regulatory approval, it may not gain market acceptance among physicians, patients, government and private payors, or others in the medical community. Market acceptance of ganaxolone, if we receive approval, depends on a number of factors, including the:

efficacy and safety of ganaxolone, or ganaxolone administered with other drugs, each as demonstrated in clinical trials and post-marketing experience;

clinical indications for which ganaxolone is approved;

acceptance by physicians and patients of ganaxolone as a safe and effective treatment;

potential and perceived advantages of ganaxolone over alternative treatments;

safety of ganaxolone seen in a broader patient group, including its use outside the approved indications should physicians choose to prescribe for such uses;

prevalence and severity of any side effects;

product labeling or product insert requirements of the FDA or other regulatory authorities;

timing of market introduction of ganaxolone as well as competitive products;

cost of treatment in relation to alternative treatments;

availability of coverage and adequate reimbursement and pricing by government and private payors;

relative convenience and ease of administration; and

effectiveness of our sales and marketing efforts.

If ganaxolone is approved but fails to achieve market acceptance among physicians, patients, government or private payors or others in the medical community, or the products or product candidates that are being administered with ganaxolone are restricted, withdrawn or recalled, or fail to be approved, as the case may be, we may not be able to generate significant revenues, which would compromise our ability to become profitable.

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

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

A variety of risks associated with marketing ganaxolone internationally could materially adversely affect our business.

We plan to seek regulatory approval for ganaxolone outside of the United States, and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

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differing regulatory requirements in foreign countries;

the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;

unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;

economic weakness, including inflation, or political instability in particular foreign economies and markets;

compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;

foreign taxes, including withholding of payroll taxes;

foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;

difficulties staffing and managing foreign operations;

workforce uncertainty in countries where labor unrest is more common than in the United States;

challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;

production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;  
and



business interruptions resulting from geo-political actions, including war and terrorism.

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

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

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

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution.

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Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may only be temporary. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to obtain coverage and profitable reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to ganaxolone and will face competition with respect to any other product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing ganaxolone. Some of these competitive products and therapies are based on scientific approaches that are the same as, or similar to, our approach, and others are based on entirely different approaches. For example, there are several companies developing product candidates that target the same gamma-aminobutyric acid, or GABAA, neuroreceptor that we are targeting or that are testing product candidates in the same indications that we are testing. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Ganaxolone is presently being developed primarily as a neuropsychiatric therapeutic. There are a variety of available marketed therapies available for these patients. Some of these other drugs are branded and subject to patent protection, some are in clinical development and not yet approved, and others are available on a generic basis.

Specifically, there are more than 15 approved antiepileptic drugs (AEDs) available in the United States and worldwide, including the generic products levetiracetam, lamotrigine, carbamazepine, oxcarbazepine, valproic acid and topiramate. Recent market entrants include branded products developed by UCB, GlaxoSmithKline, Eisai, and Sunovion Pharmaceuticals. Additionally, there are several drugs in development for the treatment of behavioral and mental health conditions associated with FXS, including compounds being developed by Roche, Sunovion Pharmaceuticals, Afraxis, Alcobra and Neuren Pharmaceuticals.

Many of the approved drugs are well established therapies or products and are widely accepted by physicians, patients and third-party payors. Insurers and other third-party payors may also encourage the use of generic products. These factors may make it difficult for us to achieve market acceptance at desired levels or in a timely manner to ensure viability of our business.

More established companies may have a competitive advantage over us due to their greater size, cash flows and institutional experience. Compared to us, many of our competitors may have significantly greater financial, technical and human resources.

As a result of these factors, our competitors may obtain regulatory approval of their products before we are able to, which may limit our ability to develop or commercialize ganaxolone. Our competitors may also develop drugs that are safer, more effective, more widely used and cheaper than ours, and may also be more successful than us in manufacturing and marketing their products. These appreciable advantages could render ganaxolone obsolete or non-competitive before we can recover the expenses of ganaxolone's development and commercialization.

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Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific, management and commercial personnel, establishing clinical trial sites and subject registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of ganaxolone or other product candidates that we may develop.

We face an inherent risk of product liability exposure related to the testing of ganaxolone by us or our investigators in human clinical trials and will face an even greater risk if ganaxolone receives regulatory approval and we commercially sell ganaxolone after obtaining such regulatory approval. Product liability claims may be brought against us by study subjects enrolled in our clinical trials, patients, healthcare providers or others using, administering or selling ganaxolone. If we cannot successfully defend ourselves against claims that ganaxolone caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in, for example:

decreased demand for ganaxolone;

termination of clinical trial sites or entire trial programs;

injury to our reputation and significant negative media attention;

withdrawal of clinical trial subjects;

significant costs to defend the related litigation;

substantial monetary awards to clinical trial subjects or patients;

loss of revenue;

diversion of management and scientific resources from our business operations;

the inability to commercialize ganaxolone; and

increased scrutiny and potential investigation by, among others, the FDA, the DOJ, the Office of Inspector General of the HHS, state attorneys general, members of Congress and the public.

We currently have \$10.0 million in product liability insurance coverage in the aggregate, which may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. We intend to expand our product liability insurance coverage to include the sale of commercial products if we obtain marketing approval for ganaxolone, but we may be unable to obtain commercially reasonable product liability insurance for ganaxolone, if approved for marketing. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. A successful product liability claim or series of claims brought against us, particularly if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

#### Risks Related to Our Dependence on Third Parties

We rely on third parties to conduct our preclinical studies and clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize ganaxolone.

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We rely on third-party CROs to monitor and manage data for our ongoing preclinical and clinical programs. We rely on these parties for execution of our preclinical studies and clinical trials, and we control only some aspects of their activities. Nevertheless, we are responsible for ensuring that each of our preclinical studies and clinical trials are conducted in accordance with the applicable protocol and legal, regulatory and scientific requirements and standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We also rely on third parties to assist in conducting our preclinical studies in accordance with Good Laboratory Practices (GLP) and the Animal Welfare Act requirements. We and our CROs are required to comply with federal regulations and Good Clinical Practices (GCP) which are international requirements meant to protect the rights and health of subjects that are enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area and comparable foreign regulatory authorities for ganaxolone and any future product candidates in clinical development. Regulatory authorities enforce GCP through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable GCP, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP requirements. In addition, our clinical trials must be conducted with product produced under cGMP requirements. Failure to comply with these regulations may require us to repeat preclinical studies and clinical trials, which would delay the regulatory approval process.

Our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing clinical, nonclinical and preclinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements or for other reasons, our preclinical studies and clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize ganaxolone. As a result, our results of operations and the commercial prospects for ganaxolone would be harmed, our costs could increase and our ability to generate revenue could be delayed.

Because we have relied on third parties, our internal capacity to perform these functions is limited. Outsourcing these functions involves risk that third parties may not perform to our standards, may not produce results in a timely manner or may fail to perform at all. In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated. We currently have a small number of employees, which limits the internal resources we have available to identify and monitor our third-party providers. To the extent we are unable to identify and successfully manage the performance of third-party service providers in the future, our business may be adversely affected. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

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

We rely on third-party vendors and CROs for preclinical studies and clinical trials related to our drug development efforts. Switching or adding additional CROs would involve additional cost and requires management time and focus. Our CROs generally have the right to terminate their agreements with us in the event of an uncured material breach. In addition, some of our CROs have an ability to terminate their respective agreements with us, or research projects pursuant to such agreements, if, in the reasonable opinion of the relevant CRO, the safety of the subjects participating in our clinical trials warrants such termination. These agreements or research projects may also be terminated if we make a general assignment for the benefit of our creditors or if we are liquidated. Identifying, qualifying and managing performance of third-party service providers can be difficult, time consuming and cause delays in our development programs. In addition, there is a natural transition period when a new CRO commences work and the new CRO may not provide the same type or level of services as the original provider. If any of our relationships with our third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or to do so on commercially reasonable terms.

Our experience manufacturing ganaxolone is limited to the needs of our preclinical studies and clinical trials. We have no experience manufacturing ganaxolone on a commercial scale and have no manufacturing facility. We are

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dependent on third-party manufacturers for the manufacture of ganaxolone as well as on third parties for our supply chain, and if we experience problems with any such third parties, the manufacturing of ganaxolone could be delayed.

We do not own or operate facilities for the manufacture of ganaxolone. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. We currently rely on contract manufacturing organizations (CMOs) for the chemical manufacture of active pharmaceutical ingredients for ganaxolone and another CMO for the production of the ganaxolone nanoparticulate formulation into capsules. To meet our projected needs for preclinical and clinical supplies to support our activities through regulatory approval and commercial manufacturing, the CMOs with whom we currently work will need to increase the scale of production. We may need to identify additional CMOs for continued production of supply for ganaxolone. Although alternative third-party suppliers with the necessary manufacturing and regulatory expertise and facilities exist, it could be expensive and take a significant amount of time to arrange for alternative suppliers. If we are unable to arrange for alternative third-party manufacturing sources on commercially reasonable terms, in a timely manner or at all, we may not be able to complete development of ganaxolone, or market or distribute ganaxolone.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured ganaxolone ourselves, including reliance on the third party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control, including a failure to synthesize and manufacture ganaxolone or any products we may eventually commercialize in accordance with our specifications, and the possibility of termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or damaging to us. In addition, the FDA and other regulatory authorities would require that ganaxolone and any products that we may eventually commercialize be manufactured according to cGMP and similar foreign standards. Any failure by our third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of ganaxolone in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of ganaxolone. In addition, such failure could be the basis for the FDA to issue a warning letter, withdraw approvals for ganaxolone previously granted to us, or take other regulatory or legal action, including recall or seizure of outside supplies of ganaxolone, total or partial suspension of production, suspension of ongoing clinical trials, refusal to approve pending applications or supplemental applications, detention of product, refusal to permit the import or export of products, injunction, or imposing civil and criminal penalties.

Any significant disruption in our supplier relationships could harm our business. Any significant delay in the supply of ganaxolone or its key materials for an ongoing preclinical study or clinical trial could considerably delay completion of our preclinical study or clinical trial, product testing and potential regulatory approval of ganaxolone. If our manufacturers or we are unable to purchase these key materials after regulatory approval has been obtained for ganaxolone, the commercial launch of ganaxolone would be delayed or there would be a shortage in supply, which would impair our ability to generate revenues from the sale of ganaxolone.

We may elect to enter into licensing or collaboration agreements to partner ganaxolone in territories currently retained by us. Our dependence on such relationships may adversely affect our business.



Because we have limited resources, we may seek to enter into collaboration agreements with other pharmaceutical or biotechnology companies. Any failure by our partners to perform their obligations or any decision by our partners to terminate these agreements could negatively impact our ability to successfully develop, obtain regulatory approvals for and commercialize ganaxolone. In the event we grant exclusive rights to such partners, we would be precluded from potential commercialization of ganaxolone within the territories in which we have a partner. In addition, any termination of our collaboration agreements will terminate the funding we may receive under the relevant collaboration agreement and may impair our ability to fund further development efforts and our progress in our development programs.

Our commercialization strategy for ganaxolone may depend on our ability to enter into agreements with collaborators to obtain assistance and funding for the development and potential commercialization of ganaxolone in the territories in which we seek to partner. Despite our efforts, we may be unable to secure additional collaborative licensing or other arrangements that are necessary for us to further develop and commercialize ganaxolone. Supporting diligence

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

Further, our potential future collaborators may develop alternative products or pursue alternative technologies either on their own or in collaboration with others, including our competitors, and the priorities or focus of our collaborators may shift such that ganaxolone receives less attention or resources than we would like, or they may be terminated altogether. Any such actions by our potential future collaborators may adversely affect our business prospects and ability to earn revenue. In addition, we could have disputes with our potential future collaborators, such as the interpretation of terms in our agreements. Any such disagreements could lead to delays in the development or commercialization of ganaxolone or could result in time-consuming and expensive litigation or arbitration, which may not be resolved in our favor.

Government funding for certain of our programs adds uncertainty to our research efforts with respect to those programs and may impose requirements that increase the costs of commercialization and production of product candidates developed under those government-funded programs.

Our preclinical studies and clinical trials to evaluate ganaxolone in FXS patients have been conducted with the MIND Institute at the University of California, Davis which receives funding from the United States Department of Defense (DoD) for such studies and trials. In addition, our preclinical studies and clinical trials to evaluate ganaxolone in patients suffering from posttraumatic stress disorder (PTSD) have been primarily conducted by the United States Department of Veterans Affairs, which also receives funding from the DoD. Programs funded by the United States government and its agencies, including the DoD, include provisions that reflect the government's substantial rights and remedies, many of which are not typically found in commercial contracts, including powers of the government to:

terminate agreements, in whole or in part, for any reason or no reason;

reduce or modify the government's obligations under such agreements without the consent of the other party;

claim rights, including intellectual property rights, in products and data developed under such agreements;

audit contract-related costs and fees, including allocated indirect costs;

suspend the contractor from receiving new contracts pending resolution of alleged violations of procurement laws or regulations;

impose United States manufacturing requirements for products that embody inventions conceived or first reduced to practice under such agreements;

suspend or debar the contractor from doing future business with the government; and

control and potentially prohibit the export of products.

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

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In addition, government contracts normally contain additional requirements that may increase our costs of doing business, reduce our profits, and expose us to liability for failure to comply with these terms and conditions. These requirements include, for example:

specialized accounting systems unique to government contracts;

mandatory financial audits and potential liability for price adjustments or recoupment of government funds after such funds have been spent;

public disclosures of certain contract information, which may enable competitors to gain insights into our research program; and

mandatory socioeconomic compliance requirements, including labor standards, non-discrimination and affirmative action programs and environmental compliance requirements.

If we fail to maintain compliance with these requirements, we may be subject to potential contract liability and to termination of our contracts.

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

If our third-party manufacturers use hazardous and biological materials in a manner that causes injury or violates applicable law, we may be liable for damages.

Our research and development activities involve the controlled use of potentially hazardous substances, including chemical and biological materials by our third-party manufacturers. Our manufacturers are subject to federal, state and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical, radioactive and hazardous materials. Although we believe that our manufacturers' procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical, radioactive or hazardous materials. As a result of

any such contamination or injury we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical radioactive or hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

#### Risks Related to Regulatory Compliance

Recently enacted and future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain marketing approval of and commercialize ganaxolone and affect the prices we may obtain.

The regulations that govern, among other things, marketing approvals, coverage, pricing and reimbursement for new drug products vary widely from country to country. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of ganaxolone, restrict or regulate post-approval activities and affect our ability to successfully sell ganaxolone, if we obtain marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In recent years, Congress has considered further reductions in Medicare reimbursement for drugs administered by physicians. The Centers for Medicare and Medicaid Services, the agency that runs the Medicare program, also has the authority to revise reimbursement rates and to implement coverage

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restrictions for some drugs. Cost reduction initiatives and changes in coverage implemented through legislation or regulation could decrease utilization of and reimbursement for any approved products, which in turn would affect the price we can receive for those products. While the Medicare Modernization Act and Medicare regulations apply only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from federal legislation or regulation may result in a similar reduction in payments from private payors.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act and the Health Care and Education Affordability Reconciliation Act of 2010 (Affordable Care Act) a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on pharmaceutical and medical device manufacturers and impose additional health policy reforms. The Affordable Care Act expanded manufacturers' rebate liability to include covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations, increased the minimum rebate due for innovator drugs from 15.1% of average manufacturer price (AMP) to 23.1% of AMP and capped the total rebate amount for innovator drugs at 100.0% of AMP. The Affordable Care Act and subsequent legislation also changed the definition of AMP. Furthermore, the Affordable Care Act imposes a significant annual, nondeductible fee on companies that manufacture or import certain branded prescription drug products. Substantial new provisions affecting compliance have also been enacted, which may affect our business practices with healthcare practitioners, and a significant number of provisions are not yet, or have only recently become, effective. Although it is too early to determine the effect of the Affordable Care Act, it appears likely to continue the pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. More recently, in August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, creates the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee did not achieve a targeted deficit reduction of an amount greater than \$1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to healthcare providers of up to 2.0% per fiscal year, starting in 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several categories of healthcare providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. If we ever obtain regulatory approval and commercialization of ganaxolone, these new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and accordingly, our financial operations. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of ganaxolone may be.

In the United States, the European Union and other potentially significant markets for ganaxolone, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Furthermore, the increased emphasis on managed healthcare in the United States and on country and regional

pricing and reimbursement controls in the European Union will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for ganaxolone in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, which could negatively impact the revenue we are able to generate from the sale of the product in that particular country. Adverse

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pricing limitations may hinder our ability to recoup our investment in ganaxolone even if ganaxolone obtains marketing approval.

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

As we seek to expand our operations outside of the United States, we must comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The creation and implementation of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

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

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

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

The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the



United States government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on United States exchanges for violations of the FCPA's accounting provisions.

Our relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may affect the business or financial arrangements and relationships through which we would market, sell and distribute our products. Even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, federal and state healthcare laws and regulations pertaining to fraud and

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abuse and patients' rights are and will be applicable to our business. Restrictions under applicable federal and state healthcare laws and regulations that may affect our operations (including our marketing, promotion, educational programs, pricing, and relationships with healthcare providers or other entities, among other things) and expose us to areas of risk including the following:

the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

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

the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA) imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also created federal criminal laws that prohibit knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statements in connection with the delivery of or payment for healthcare benefits, items or services;

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

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

analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws govern the

privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties are compliant with applicable healthcare laws and regulations will involve the expenditure of appropriate, and possibly significant, resources. Nonetheless, it is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any physicians or other healthcare providers or entities with whom we expect to do business are found to not be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

#### Risks Related to Our Intellectual Property

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If we are unable to protect our intellectual property rights or if our intellectual property rights are inadequate for our technology and product candidates, our competitive position could be harmed.

Our commercial success will depend in large part on our ability to obtain and maintain patent and other intellectual property protection in the United States and other countries with respect to our proprietary technology and products. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. We seek to protect our proprietary position by filing and prosecuting patent applications in the United States and abroad related to our novel technologies and products that are important to our business.

The patent positions of biotechnology and pharmaceutical companies generally are highly uncertain, involve complex legal and factual questions and have in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patents, including those patent rights licensed to us by third parties, are highly uncertain. The steps we or our licensors have taken to protect our proprietary rights may not be adequate to preclude misappropriation of our proprietary information or infringement of our intellectual property rights, both inside and outside the United States. Further, the examination process may require us or our licensors to narrow the claims for our pending patent applications, which may limit the scope of patent protection that may be obtained if these applications issue. The rights already granted under any of our currently issued patents or those licensed to us and those that may be granted under future issued patents may not provide us with the proprietary protection or competitive advantages we are seeking. If we or our licensors are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficient, our competitors could develop and commercialize technology and products similar or superior to ours, and our ability to successfully commercialize our technology and products may be adversely affected. It is also possible that we or our licensors will fail to identify patentable aspects of inventions made in the course of our development and commercialization activities before it is too late to obtain patent protection on them.

With respect to patent rights, we do not know whether any of our granted or issued patents will effectively prevent others from commercializing competitive technologies and products. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or in some cases not at all, until they are issued as a patent. Therefore we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

Our pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, issued patents that we own or have licensed from third parties may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in the loss of patent protection, the narrowing of claims in such patents or the invalidity or unenforceability of such patents,

which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection for our technology and products. Protecting against the unauthorized use of our or our licensors' patented technology, trademarks and other intellectual property rights is expensive, difficult and may in some cases not be possible. In some cases, it may be difficult or impossible to detect third-party infringement or misappropriation of our intellectual property rights, even in relation to issued patent claims, and proving any such infringement may be even more difficult.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability to develop, manufacture, market and sell ganaxolone, and to use our related proprietary technologies. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to ganaxolone, including interference or derivation proceedings before the United States Patent and Trademark Office (USPTO). Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue

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commercializing ganaxolone. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Under certain circumstances, we could be forced, including by court order, to cease commercializing ganaxolone. In addition, in any such proceeding or litigation, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing ganaxolone or force us to cease some of our business operations, which could materially harm our business. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar negative impact on our business.

While ganaxolone is in preclinical studies and clinical trials, we believe that the use of ganaxolone in these preclinical studies and clinical trials falls within the scope of the exemptions provided by 35 U.S.C. Section 271(e) in the United States, which exempts from patent infringement liability activities reasonably related to the development and submission of information to the FDA. As ganaxolone progresses toward commercialization, the possibility of a patent infringement claim against us increases. While ganaxolone itself is off patent, we attempt to ensure that our solid and liquid nanoparticulate formulation of ganaxolone and the methods we employ to manufacture ganaxolone do not infringe other parties' patents and other proprietary rights. There can be no assurance they do not, however, and competitors or other parties may assert that we infringe their proprietary rights in any event.

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

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

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

Filing, prosecuting and defending patents on ganaxolone and any future product candidates throughout the world would be prohibitively expensive, and our or our licensors' intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws and practices of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions into or within the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products, and may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing with us in these jurisdictions.

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Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our or our licensor's patents or marketing of competing products in violation of our proprietary rights generally in those countries. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful.

The laws of certain foreign countries may not protect our rights to the same extent as the laws of the United States, and these foreign laws may also be subject to change. For example, novel formulations and manufacturing processes may not be patentable in certain jurisdictions, and the requirements for patentability may differ in certain countries, particularly developing countries. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our patents, requiring us to engage in complex, lengthy and costly litigation or other proceedings. Generic drug manufacturers may develop, seek approval for, and launch generic versions of our products. Many countries, including European Union countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under certain circumstances to grant licenses to third parties. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of our patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from our intellectual property.

Patent terms may be inadequate to protect our competitive position on our products for an adequate amount of time.

Given the amount of time required for the development, testing and regulatory review of new product candidates, such as ganaxolone, patents protecting such candidates might expire before or shortly after such candidates are commercialized. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension). However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case.

Changes in patent laws, including recent patent reform legislation, could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.



As is the case with other pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the pharmaceutical industry involve technological and legal complexity, and obtaining and enforcing pharmaceutical patents is costly, time-consuming, and inherently uncertain. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. For example, the United States Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce existing patents and patents we may obtain in the future. Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

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In September 2011, the Leahy-Smith America Invents Act (Leahy-Smith Act) was signed into law. The Leahy-Smith Act includes a number of significant changes to United States patent law. These include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In particular, under the Leahy-Smith Act, the United States transitioned in March 2013 to a “first to file” system in which the first inventor to file a patent application will be entitled to the patent. Third parties are allowed to submit prior art before the issuance of a patent by the USPTO and may become involved in opposition, derivation, reexamination, inter-parties review or interference proceedings challenging our patent rights or the patent rights of our licensors. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate patent rights, which could adversely affect our competitive position.

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

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submissions, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

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

Competitors may infringe our patents or misappropriate or otherwise violate our intellectual property rights. To counter infringement or unauthorized use, litigation may be necessary in the future to enforce or defend our intellectual property rights, to protect our trade secrets or to determine the validity and scope of our own intellectual property rights or the proprietary rights of others. Also, third parties may initiate legal proceedings against us to

challenge the validity or scope of intellectual property rights we own or control. These proceedings can be expensive and time consuming. Many of our current and potential competitors have the ability to dedicate substantially greater resources to defend their intellectual property rights than we can. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating our intellectual property. Litigation could result in substantial costs and diversion of management resources, which could harm our business and financial results. In addition, in an infringement proceeding, a court may decide that a patent owned or controlled by us is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated, held unenforceable or interpreted narrowly. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock.

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

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

Intellectual property rights do not necessarily address all potential threats to our competitive advantage.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business, or permit us to maintain our competitive advantage. The following examples are illustrative:

others may be able to make compounds or ganaxolone formulations that are similar to our ganaxolone formulations but that are not covered by the claims of the patents that we own or control;

we or any strategic partners might not have been the first to make the inventions covered by the issued patents or pending patent applications that we own or control;

we might not have been the first to file patent applications covering certain of our inventions;

others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our intellectual property rights;

it is possible that our pending patent applications will not lead to issued patents;

issued patents that we own or control may not provide us with any competitive advantages, or may be held invalid or unenforceable as a result of legal challenges;

our competitors might conduct research and development activities in the United States and other countries that provide a safe harbor from patent infringement claims for certain research and development activities, as well as in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;

we may not develop additional proprietary technologies that are patentable; and

the patents of others may have an adverse effect on our business.

#### Risks Related to Employee Matters, Managing Growth and Becoming a Public Company

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

We are highly dependent upon Christopher M. Cashman, our Chief Executive Officer, Edward F. Smith, our Chief Financial Officer, Albena I. Patroneva, M.D., our Chief Medical Officer and Gail M. Farfel, Ph.D., our Chief

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Clinical Development and Regulatory Officer. The employment agreements we have with the persons named above do not prevent such persons from terminating their employment with us at any time. We do not maintain “key person” insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

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

As of March 31, 2015, we had nine full-time and one part-time employee. As our development and commercialization plans and strategies develop, or as a result of any future acquisitions, we will need additional managerial, operational, sales, marketing, financial and other resources. In addition, it may become more cost effective to bring in house certain resources currently outsourced to consultants and other third-parties. Our management, personnel and systems currently in place may not be adequate to support our future growth. Future growth would impose significant added responsibilities on members of management, including:

managing our clinical trials effectively;

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

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

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

expanding our facilities.

As our operations expand, we will need to manage additional relationships with various strategic partners, suppliers and other third parties. Our future financial performance and our ability to commercialize ganaxolone, if approved, and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to manage our development efforts and clinical trials effectively and hire, train and integrate additional management, administrative and sales and marketing personnel. Our failure to accomplish any of these tasks could prevent us from successfully growing our company.

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

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

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

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

As part of our business strategy, we may pursue acquisitions of assets, including preclinical, clinical or commercial stage products or product candidates, or businesses, or strategic alliances and collaborations, to expand our existing technologies and operations. We may not identify or complete these transactions in a timely manner, on a cost-effective basis, or at all, and we may not realize the anticipated benefits of any such transaction, any of which could have a detrimental effect on our financial condition, results of operations and cash flows. We have no experience with

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acquiring other companies, products or product candidates, and limited experience with forming strategic alliances and collaborations. We may not be able to find suitable acquisition candidates, and if we make any acquisitions, we may not be able to integrate these acquisitions successfully into our existing business and we may incur additional debt or assume unknown or contingent liabilities in connection therewith. Integration of an acquired company or assets may also disrupt ongoing operations, require the hiring of additional personnel and the implementation of additional internal systems and infrastructure, especially the acquisition of commercial assets, and require management resources that would otherwise focus on developing our existing business. We may not be able to find suitable strategic alliance or collaboration partners or identify other investment opportunities, and we may experience losses related to any such investments.

To finance any acquisitions or collaborations, we may choose to issue debt or equity securities as consideration. Any such issuance of shares would dilute the ownership of our stockholders. If the price of our common stock is low or volatile, we may not be able to acquire other assets or companies or fund a transaction using our stock as consideration. Alternatively, it may be necessary for us to raise additional funds for acquisitions through public or private financings. Additional funds may not be available on terms that are favorable to us, or at all.

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

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

## Risks Related to Ownership of Our Common Stock

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



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

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

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

the success of competitive products or technologies;

regulatory actions with respect to our products or our competitors’ products;

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actual or anticipated changes in our growth rate relative to our competitors;

announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;

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

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

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

the recruitment or departure of key personnel;

the level of expenses related to our clinical development programs;

the results of our efforts to in-license or acquire additional product candidates or products;

actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;

variations in our financial results or those of companies that are perceived to be similar to us;

fluctuations in the valuation of companies perceived by investors to be comparable to us;

share price and volume fluctuations attributable to inconsistent trading volume levels of our shares;

announcement or expectation of additional financing efforts;

sales of our common stock by us, our insiders or our other stockholders;

changes in the structure of healthcare payment systems;

market conditions in the pharmaceutical and biotechnology sectors;

general economic, industry and market conditions; and

other events or factors, many of which are beyond our control.

In addition, the stock market in general, The NASDAQ Global Market and pharmaceutical and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. The realization of any of these risks or any of a broad range of other risks, including those described in these “Risk Factors,” could have a dramatic and material adverse impact on the market price of our common stock.

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

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

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

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We estimate that our executive officers, directors, and holders of 5% or more of our capital stock collectively beneficially own approximately 82% of our voting stock. This concentration of ownership could harm the market price of our common stock by:

delaying, deferring or preventing a change in control of our company;

impeding a merger, consolidation, takeover or other business combination involving our company; or

discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of our company.

The interests of this group of stockholders may not always coincide with your interests or the interests of other stockholders and they may act in a manner that advances their best interests and not necessarily those of other stockholders, including seeking a premium value for their common stock, and might negatively affect the prevailing market price for our common stock.

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

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (JOBS Act). For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years following the year in which we completed our initial public offering, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds \$700.0 million as of any June 30 before that time or if we have total annual gross revenue of \$1.0 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31. If we issue more than \$1.0 billion in non-convertible debt during any three-year period before that time, we would cease to be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a “smaller reporting company” which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being

required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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

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

Now that we are a public company, we are incurring significant legal, accounting and other expenses that we did not incur as a private company, and these expenses may increase even more after we are no longer an “emerging

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growth company.” We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Protection Act, as well as rules adopted, and to be adopted, by the SEC and NASDAQ Stock Market. Our management and other personnel need to devote a substantial amount of time to these compliance initiatives. Moreover, we expect these rules and regulations to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. We estimate that we will incur approximately \$1.0 million to \$2.0 million in incremental costs per year associated with being a publicly traded company, although it is possible that our actual incremental costs will be higher than we currently estimate. The increased costs will increase our net loss. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain sufficient coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

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

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, our ability to pay cash dividends is prohibited by our credit facility with Square 1 Bank, entered into in April 2014 and amended in December 2014, and the terms of any future debt agreements may also preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

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

If our stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after legal restrictions on resale lapse, the trading price of our common stock could decline. As of March 31, 2015 we had outstanding a total of 14,199,166 shares of common stock. In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

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

We expect that significant additional capital will be needed in the future to continue our planned operations. To raise capital, we may sell substantial amounts of common stock or securities convertible into or exchangeable for common stock. These future issuances of equity or equity-linked securities, together with the exercise of stock options, warrants outstanding or granted in the future and any additional shares issued in connection with acquisitions, if any, may result in material dilution to our investors. Such sales may also result in material dilution to our stockholders, and new investors could gain rights, preferences and privileges senior to those of holders of our common stock.

Pursuant to our equity incentive plans, our compensation committee is authorized to grant equity-based incentive awards to our directors, executive officers and other employees and service providers. There were no shares of our common stock available for future grant under our 2005 Stock Option and Incentive Plan, as amended as of March 31, 2015. The number of shares of our common stock available for future grant under our 2014 Equity Incentive Plan was 560,310 as of March 31, 2015. Future equity incentive grants and issuances of common stock under our equity incentive plans may have an adverse effect on the market price of our common stock.

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

Our management has broad discretion over the management of our operations and cash resources and could deploy our resources in ways that do not improve our business, including our ganaxolone clinical development programs, or enhance the value of our common stock. The failure by our management to apply these funds effectively

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could result in financial losses that could have a material adverse effect on our business, cause the market price of our common stock to decline and delay the development of ganaxolone.

Some provisions of our charter documents and Delaware law may have anti-takeover effects that could discourage an acquisition of us by others, even if an acquisition would be beneficial to our stockholders and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our amended and restated certificate of incorporation and amended and restated bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders, or remove our current management. These include provisions that:

permit our board of directors to issue up to 25,000,000 shares of preferred stock, with any rights, preferences and privileges as it may designate;

provide that all vacancies on our board of directors, including as a result of newly created directorships, may, except as otherwise required by law, be filled by the affirmative vote of a majority of directors then in office, even if less than a quorum;

establish a classified board of directors such that only one of three classes of directors is elected each year;

provide that directors can only be removed for cause;

require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent;

provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide advance notice in writing, and also specify requirements as to the form and content of a stockholder's notice;



not provide for cumulative voting rights, thereby allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election; and

provide that special meetings of our stockholders may be called only by the chairperson of the board of directors, the chief executive officer or the board of directors.

These provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors, who are responsible for appointing the members of our management. Because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may discourage, delay or prevent someone from acquiring us or merging with us whether or not it is desired by or beneficial to our stockholders. Under Delaware law, a corporation may not, in general, engage in a business combination with any holder of 15.0% or more of its capital stock unless the holder has held the stock for three years or, among other things, the board of directors has approved the transaction. Any provision of our amended and restated certificate of incorporation or amended and restated bylaws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

## Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

### Unregistered Sales of Equity Securities

None.

### Use of Initial Public Offering Proceeds

Our initial public offering of common stock was effected pursuant to a registration statement on Form S-1 (File No 333-195895) that was declared effective by the SEC on July 31, 2014, pursuant to which we registered the offering and sale of 6,468,750 shares of common stock, \$0.001 par value per share (including 843,750 shares available to the

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underwriters' for exercise of an option to purchase additional shares, of which 133,000 was exercised in September 2014) at a public offering price of \$8.00 per share for an aggregate public offering price of \$46.1 million.

As a result of the initial public offering, we received net proceeds of approximately \$41.2 million during the third quarter of 2014 from the sale of 5,758,000 shares of common stock, after deducting underwriting discounts, commissions and estimated offering expenses borne by us. Other than pursuant to standard compensation arrangements, none of such payments were direct or indirect payments to any of (i) our directors or officers or their associates, (ii) persons owning 10% or more of our common stock, or (iii) our affiliates.

There has been no material change in the planned use of proceeds from our initial public offering from that described in the final prospectus related to the offering, which we filed with the SEC on August 1, 2014. As of March 31, 2015, we have used approximately \$5.4 million of the funds received from our initial public offering (IPO) in support of our clinical trials and payments to research and development consultants, including compensation of certain employees and officers, and approximately \$4.2 million for general corporate purposes, including compensation of certain employees, officers, and directors.

Item 3. Defaults upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

Item 2.02 of Form 8-K Disclosure

The Company issued a press release on May 11, 2015 announcing its financial results for the first quarter ended March 31, 2015. A copy of the press release is being furnished to the Securities and Exchange Commission as Exhibit 99.1 to this Quarterly Report on Form 10-Q and is incorporated by reference to this Item 5.

## Edgar Filing: MARINUS PHARMACEUTICALS INC - Form 10-Q

The information furnished pursuant to this Item 5 shall not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934 (the “Exchange Act”) or otherwise subject to the liabilities of that section, nor shall it be deemed to be incorporated by reference into any of the Company’s filings with the Securities and Exchange Commission under the Exchange Act or the Securities Act of 1933, whether made before or after the date hereof, regardless of any general incorporation language in such a filing, except as expressly set forth by specific reference in such a filing. Except as required by law, we undertake no duty or obligation to publicly update or revise the information so furnished.

### Item 5.02 of Form 8-K Disclosure

On May 8, 2015, Gail Farfel, Ph.D., the Company’s Chief Development and Regulatory Officer, resigned from the Company effective June 30, 2015. As previously reported on January 7, 2015, the Company hired Albena Patroneva, MD as Chief Medical Officer. Dr. Patroneva will assume all responsibilities formerly held by Dr. Farfel. Dr. Farfel will remain available to assist the Company with transitional matters for a mutually agreed period of time.

### Item 6. Exhibits

#### Exhibit

#### Number Exhibit Description

- |      |   |
|------|---|
| 31.1 | Certification of Chief Executive Officer pursuant to Rule 13a-14(a) or 15a-14(a) under the Exchange Act (filed herewith). |
| 31.2 | Certification of Chief Financial Officer pursuant to Rule 13a-14(a) or 15a-14(a) under the Exchange Act (filed herewith). |

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32.1	Certification Pursuant to 18 U.S.C. Section 1350 of principal executive officer and principal financial officer (filed herewith).
99.1	Press release, dated May 11, 2015, of Marinus Pharmaceuticals, Inc. (filed herewith)
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Signature	Title	Date
/s/ Christopher M. Cashman Christopher M. Cashman	President and Chief Executive Officer (principal executive officer) and Director	May 14, 2015
/s/ Edward F. Smith Edward F. Smith	Chief Financial Officer and Treasurer (principal financial and accounting officer)	May 14, 2015

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EXHIBITS INDEX

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32.1	Certification Pursuant to 18 U.S.C. Section 1350 of principal executive officer and principal financial officer (filed herewith).
99.1	Press release, dated May 11, 2015, of Marinus Pharmaceuticals, Inc. (filed herewith)
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document