MARINUS PHARMACEUTICALS INC
Form 10-Q
May 01, 2019
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

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

FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2019

OR

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

COMMISSION FILE NUMBER 001-36576

MARINUS PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

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

5 Radnor Corporate Center

100 Matsonford Rd, Suite 100

Radnor, PA 19087

(Address of registrant's principal executive offices)

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

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted 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 such files). Yes No.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	Accelerated filer

Non-accelerated filer Smaller reporting company

Emerging growth company

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

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

The number of outstanding shares of the registrant's common stock, par value $0.001 per share, as of April 30, 2019 was: 52,554,625.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

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FOR THE QUARTER ENDED MARCH 31, 2019

PART I – FINANCIAL INFORMATION

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PART I

FINANCIAL INFORMATION

Item 1. Financial Statements

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED BALANCE SHEETS

(in thousands, except share and per share amounts)

(unaudited)

	March 31, 2019	December 31, 2018
ASSETS		
Current assets:		
Cash and cash equivalents	$ 60,848	$ 67,727
Short-term investments	—	4,998
Prepaid expenses and other current assets	2,024	1,215
Total current assets	62,872	73,940
Property and equipment, net	2,216	1,294
Other assets	2,531	—
Total assets	$ 67,619	$ 75,234
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,932	$ 2,472
Accrued expenses	4,745	4,437
Total current liabilities	6,677	6,909
Other long-term liabilities	3,197	—
Total liabilities	9,874	6,909
Stockholders' equity:		
Preferred stock, $0.001 par value; 25,000,000 shares authorized, no shares issued and outstanding	—	—

Common stock, $0.001 par value; 100,000,000 shares authorized, 52,583,856 issued and 52,554,625 outstanding at March 31, 2019 and 52,548,244 issued and 52,519,013 outstanding at December 31, 2018	53	53
Additional paid-in capital	251,628	249,727
Treasury stock at cost, 29,231 shares at March 31, 2019 and December 31, 2018	—	—
Accumulated other comprehensive loss	—	(2)
Accumulated deficit	(193,936)	(181,453)
Total stockholders' equity	57,745	68,325
Total liabilities and stockholders' equity	$ 67,619	$ 75,234

See accompanying notes to consolidated financial statements.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except share and per share amounts)

(unaudited)

	Three Months Ended March 31,	
	2019	2018
Expenses:		
Research and development	$ 8,872	$ 3,927
General and administrative	3,667	2,187
Loss from operations	(12,539)	(6,114)
Interest income	96	116
Other expense	(40)	(1)
Net loss	$ (12,483)	$ (5,999)
Per share information:		
Net loss per share of common stock—basic and diluted	$ (0.24)	$ (0.15)
Basic and diluted weighted average shares outstanding	52,465,207	40,373,083
Net loss	$ (12,483)	$ (5,999)
Other comprehensive loss:		
Unrealized gain (loss) on available-for-sale securities	2	(11)
Total comprehensive loss	$ (12,481)	$ (6,010)

See accompanying notes to consolidated financial statements.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

(unaudited)

	Three Months Ended March 31,	
	2019	2018
Cash flows from operating activities		
Net loss	$ (12,483)	$ (5,999)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	36	31
Stock-based compensation expense	1,836	1,127
Loss on disposal of fixed assets	42	—
Noncash lease expense	46	—
Noncash lease liability interest	(55)	—
Amortization of discount on investments	(1)	(25)
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(847)	(720)
Accounts payable and accrued expenses	(238)	(563)
Net cash used in operating activities	(11,664)	(6,149)
Cash flows from investing activities		
Maturities of short-term investments	5,000	—
Deposit on property and equipment	(80)	—
Purchases of property and equipment	(52)	(8)
Net cash provided by (used in) investing activities	4,868	(8)
Cash flows from financing activities		
Proceeds from exercise of stock options	65	—
Financing costs	(148)	—
Repayments of short-term bank borrowings	—	(193)
Net cash used in financing activities	(83)	(193)
Net decrease in cash and cash equivalents	(6,879)	(6,350)
Cash and cash equivalents—beginning of period	67,727	33,531
Cash and cash equivalents—end of period	$ 60,848	$ 27,181
Supplemental disclosure of cash flow information		
Property and equipment in accounts payable	$ 48	$ —
Operating lease liability	$ 3,357	$ —
Operating right-of-use asset	$ 2,458	$ —

See accompanying notes to consolidated financial statements.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

(in thousands)

(unaudited)

	Common Stock Shares	Amount	Additional Paid-in Capital	Treasury Stock Shares	Amount	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
Balance, December 31, 2018	52,548,244	$ 53	$ 249,727	29,231	$ —	$ (2)	$ (181,453)	$ 68,325
Stock-based compensation expense	—	—	1,836	—	—	—	—	1,836
Exercise of stock options	55,812	—	65	—	—	—	—	65
Forfeiture of restricted stock	(20,200)	—	—	—	—	—	—	—
Unrealized loss on investments	—	—	—	—	—	2	—	2
Net loss	—	—	—	—	—	—	(12,483)	(12,483)
Balance, March 31, 2019	52,583,856	$ 53	$ 251,628	29,231	$ —	$ —	$ (193,936)	$ 57,745
Balance, December 31, 2017	40,549,936	$ 41	$ 202,790	29,231	$ —	$ (96)	$ (144,727)	$ 58,008
Stock-based compensation expense	—	—	1,127	—	—	—	—	1,127
Unrealized loss on investments	—	—	—	—	—	(11)	—	(11)
Net loss	—	—	—	—	—	—	(5,999)	(5,999)
Balance, March 31, 2018	40,549,936	$ 41	$ 203,917	29,231	$ —	$ (107)	$ (150,726)	$ 53,125

See accompanying notes to consolidated financial statements.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Description of the Business and Liquidity

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

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, 2019 was $193.9 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 debt, potential collaborations and revenues from potential future product sales, if any. We have not generated positive cash flows from operations, and there are no assurances that we will be successful in obtaining an adequate level of financing for the development and commercialization of our planned product candidates.

In connection with the closing of a secondary public offering during the fourth quarter of 2018, we issued a total of 12,000,000 shares of common stock resulting in aggregate net proceeds, after underwriting discounts and commissions and other offering expenses, of $42.1 million.

2. Summary of Significant Accounting Policies

Basis of Presentation

The unaudited interim consolidated financial statements included herein have been prepared pursuant to the rules and regulations of the Securities and Exchange Commission (SEC). Accordingly, they do not include all information and disclosures necessary for a presentation of our financial position, results of operations and cash flows in conformity with generally accepted accounting principles in the United States of America (GAAP) for annual financial statements. In the opinion of management, these unaudited interim consolidated financial statements reflect the elimination of all intercompany accounts and transactions and all adjustments, consisting primarily of normal recurring accruals, necessary for a fair presentation of our financial position and results of operations and cash flows for the periods presented. The results of operations for interim periods are not necessarily indicative of the results for the full year. These unaudited interim consolidated financial statements should be read in conjunction with the audited consolidated financial statements for the year ended December 31, 2018 and accompanying notes thereto included in our annual report on Form 10-K filed with the SEC on March 12, 2019.

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. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

Recent Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board (FASB) established Topic 842, Leases, by issuing Accounting Standards Update (ASU) No. 2016-02, which requires lessees to recognize leases on-balance sheet and disclose key information about leasing arrangements. Topic 842 was subsequently amended by ASU No. 2018-01, Land Easement Practical Expedient for Transition to Topic 842; ASU No. 2018-10, Codification Improvements to Topic 842, Leases; and ASU No. 2018-11, Targeted Improvements. The new standard establishes a right-of-use model that requires a lessee to recognize a right-of-use (ROU) asset and lease liability on the balance sheet for all leases with a term longer than 12 months, and leases are classified as finance or operating, with classification affecting the pattern and classification of expense recognition in the income statement.

We adopted ASU 2016-02 in the first quarter of 2019 utilizing the modified retrospective transition method on the effective date. Consequently, financial information has not been updated and the disclosures required under the new standard have not been provided for dates and periods before January 1, 2019. Upon adoption, we elected the ‘package of practical expedients,’ which permitted us not to reassess under the new standard our prior conclusions about lease identification, lease classification and initial direct costs. We did not elect the use-of-hindsight practical expedient nor the practical expedient pertaining to land easements, the latter not being applicable to us. The adoption of ASU 2016-02 on January 1, 2019 resulted in the recognition of right-of-use assets of $2.5 million and lease liabilities for operating leases of $3.4 million on our interim consolidated balance sheets, with no material impact to our interim consolidated statements of operations, cash flows or stockholders’ equity. The operating lease liabilities were determined based on the present value of the remaining minimum retal payments and the operating lease asset was determined based on the value of the lease liability, adjusted for the lease incentive of $0.9 million. See Note 8 for further information regarding the impact of the adoption of ASU 2016-02 on our interim consolidated 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.

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

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

Valuation Techniques - Level 2 Inputs

We estimate the fair values of our financial instruments categorized as level 2 in the fair value hierarchy, including U.S. Treasury securities, by taking into consideration valuations obtained from third-party pricing services. The pricing services use industry standard valuation models, including both income- and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, benchmark yields, issuer credit spreads, benchmark securities, and other observable inputs. We obtain a single price for each financial instrument and do not adjust the prices obtained from the pricing service. We validate the prices provided by our third-party pricing services by reviewing their pricing methods, obtaining market values from other pricing sources and comparing them to the share prices presented by the third-party pricing services. After completing our validation procedures, we did not adjust or override any fair value measurements provided by our third-party pricing services as of December 31, 2018. As of March 31, 2019 there were no Level 2 inputs.

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, 2019				
Assets				
Money market funds (cash equivalents)	$ 24,465	$ —	$ —	$ 24,465
U.S. Treasury securities	—	—	—	—
Total assets	$ 24,465	$ —	$ —	$ 24,465
December 31, 2018				
Assets				
Money market funds (cash equivalents)	$ 14,049	$ —	$ —	$ 14,049
U.S. Treasury securities	—	4,998	—	4,998
Total assets	$ 14,049	$ 4,998	$ —	$ 19,047

4. Accrued Expenses

At March 31, 2019 and December 31, 2018 accrued expenses consisted of the following (in thousands):

December 31,

	March 31, 2019	2018
Payroll and related costs	$ 1,243	$ 1,364
Clinical trials and drug development	2,771	2,781
Professional fees	356	204
Other	375	88
Total accrued expenses	$ 4,745	$ 4,437

5. Property and Equipment

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

	March 31, 2019	December 31, 2018
Laboratory equipment	$ 1,756	$ 1,756
Leasehold improvements	899	—
Office furniture and equipment	134	148

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

	2,789	1,904
Less: accumulated depreciation	(573)	(610)
	$ 2,216	$ 1,294

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 7, and summarized in the table below:

	March 31, 2019	2018
Restricted stock	32,400	125,867
Stock options	6,492,571	4,624,320
	6,524,971	4,750,187

7. Stockholders’ Equity

In 2005, we adopted the 2005 Stock Option and Incentive Plan (2005 Plan) that authorizes us to grant options, restricted stock and other equity-based awards. As of March 31, 2019, 330,450 options to purchase shares of common stock were outstanding pursuant to grants in connection with the 2005 Plan. No additional shares are available for issuance under the 2005 Plan.

In August 2014, we adopted our 2014 Equity Incentive Plan, amended in May 2017 (2014 Plan), that authorizes us to grant options, restricted stock, and other equity-based awards, subject to adjustment in accordance with the 2014 Plan. The amount, terms of grants, and exercisability provisions are determined and set by our board of directors. As of March 31, 2019, 5,696,121 options to purchase shares of common stock and 32,400 shares of restricted stock were outstanding pursuant to grants in connection with the 2014 Plan, and 1,880,929 shares of common stock were available for future issuance. The amount, terms of grants, and exercisability provisions are determined and set by our board of directors.

Stock Options

There were 6,492,571 stock options outstanding as of March 31, 2019 at a weighted-average exercise price of $5.13 per share. During the three months ended March 31, 2019, 1,813,000 options were granted to employees and directors at a weighted-average exercise price of $3.91 per share.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

	Three Months Ended March 31,	
	2019	2018
Research and development	$ 649	$ 313
General and administrative	1,177	778
Total	$ 1,826	$ 1,091

Restricted Stock

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

We did not issue any restricted shares of common stock during the three months ended March 31, 2019 or 2018. As of March 31, 2019 there were 32,400 restricted shares of common stock outstanding, and 52,600 shares vested during the three months ended March 31, 2019.

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

	Three Months Ended March 31,	
	2019	2018
Research and development	$ 5	$ 7
General and administrative	5	28
Total	$ 10	$ 35

8. Leases

We have entered into operating leases for real estate. These leases have terms which range from 36 to 78 months, and include renewal terms which can extend the lease terms by 24 to 60 months, which are included in the lease term when it is reasonably certain that we will exercise the option. As of March 31, 2019, our operating leases had a weighted average remaining lease term of 77 months. These operating leases are included in "Other assets" on our interim consolidated balance sheet as of March 31, 2019, and represent our right to use the underlying asset for the lease term. Our obligations to make lease payments are included in "Accrued expenses" and "Other long-term liabilities" on our interim consolidated balance sheet as of March 31, 2019. The ROU assets are initially measured at cost, which comprises the initial amount of the lease liability adjusted for lease payments made at or before the lease commencement date, plus any initial direct costs incurred, less any lease incentives received. The ROU assets are subsequently measured throughout the lease term at the carrying amount of the lease liability, plus initial direct costs, plus (minus) any prepaid (accrued) lease payments, less the unamortized balance of lease incentives received. Our ROU assets have been adjusted for $0.9 million in lease incentives.

Based on the present value of the lease payments for the remaining lease term of our existing leases, we initially recognized ROU assets of $2.5 million and lease liabilities for operating leases of $3.4 million during the first quarter of 2019. Operating lease right-of-use assets and liabilities commencing after January 1, 2019 are

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

recognized at commencement date based on the present value of lease payments over the lease term. As of March 31, 2019, total right-of-use assets and operating lease liabilities were $2.4 million and $3.4 million, respectively. We have entered into various short-term operating leases, primarily for clinical study equipment, with an initial term of twelve months or less. These leases are not recorded on our interim consolidated balance sheets. All operating lease expense is recognized on a straight-line basis over the lease term. During the three months ended March 31, 2019, we recognized $0.2 million in total lease costs, which was comprised of $0.2 million in operating lease costs for right-of-use assets and less than $0.1 million in short-term lease costs related to short-term operating leases.

Because the rate implicit in each lease is not readily determinable, we use our incremental borrowing rate to determine the present value of the lease payments. The weighted average incremental borrowing rate used to determine the initial value of right-of-use assets and lease liabilities during the three months ended March 31, 2019 was 11.0%, derived from a corporate yield curve based on a synthetic credit rating model using a market signal analysis. We have certain contracts for real estate which may contain lease and non-lease components which we have elected to treat as a single lease component.

ROU assets for operating leases are periodically reduced by impairment losses. We use the long-lived assets impairment guidance in ASC Subtopic 360-10, Property, Plant, and Equipment – Overall, to determine whether an ROU asset is impaired, and if so, the amount of the impairment loss to recognize. As of March 31, 2019, we have not recognized any impairment losses for our ROU assets.

We monitor for events or changes in circumstances that require a reassessment of one of its leases. When a reassessment results in the remeasurement of a lease liability, a corresponding adjustment is made to the carrying amount of the corresponding ROU asset unless doing so would reduce the carrying amount of the ROU asset to an amount less than zero. In that case, the amount of the adjustment that would result in a negative ROU asset balance is recorded in profit or loss.

Maturities of operating lease liabilities as of March 31, 2019 were as follows (in thousands):

Remaining nine months of 2019	$ 213
2020	807
2021	818
2022	807
2023	823
Thereafter	1,482

	$ 4,950
Less: imputed interest	(1,537)
Total lease liabilities	$ 3,413
Current operating lease liabilities	216
Non-current operating lease liabilities	3,197
Total lease liabilities	$ 3,413

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

9. Commitments

Severance Arrangement

In March 2019, we entered into a Severance Agreement and General Release (Severance Agreement) with Christopher M. Cashman (Cashman), our former Chief Executive Officer. In connection with this Severance Agreement, we agreed to pay certain severance benefits for one year to Cashman, including salary and benefits continuation and a prorated bonus totaling $0.6 million. As of March 31, 2019, $0.5 million in severance benefits remained unpaid. In addition, certain of Cashman's outstanding stock option agreements were modified to accelerate vesting and extend the exercise period, resulting in additional compensation cost of $0.4 million.

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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 t or other comparable terminology intended to identify statements about the future. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Quarterly Report on Form 10-Q, we caution you that these statements are based on a combination of facts and factors currently known by us and our expectations of the future, about which we cannot be certain.

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

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

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

You should refer to Part II Item 1A. “Risk Factors” of this Quarterly Report on this Form 10-Q for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. As a result of these factors, we cannot assure you that the forward-looking statements in this Quarterly Report on Form 10-Q will prove to be accurate. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame or at all. We undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

You should read this Quarterly Report on Form 10-Q and the documents that we reference in this Quarterly Report on Form 10-Q and have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

The following Management’s Discussion and Analysis of Financial Condition and Results of Operations should be read in conjunction with: (i) the interim consolidated financial statements and related notes thereto which are included in this Quarterly Report on Form 10-Q; and (ii) our annual consolidated financial statements for the year ended December 31, 2018 which are included in our Annual Report on Form 10-K filed with the SEC on March 12, 2019.

Overview

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

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Our Pipeline

We are developing ganaxolone in two different routes of administration (IV and oral formulations) intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings where there is a mechanistic rationale for ganaxolone to provide a benefit, including the following indications:

CDKL5 deficiency disorder (CDD)

We are currently enrolling patients into a pivotal Phase 3 clinical study (Marigold Study) evaluating the use of oral ganaxolone in children and young adults with CDD. The Marigold Study is a global, double blind, placebo controlled, study that will enroll between 70 and 100 patients between the ages of 2 and 21 with a confirmed disease related CDKL5 gene variant and allopregnanolone sulfate levels below a pre-specified limit. Patients will undergo a six-week prospective baseline period to collect seizure data, followed by a 17-week double-blind treatment phase. Patients randomized to ganaxolone will titrate over four weeks to a dose of up to 600 mg of oral liquid suspension three times a day and maintain that dose for the following 13-weeks, in addition to their existing anti seizure treatment. Following the double blind treatment period, all patients that meet certain eligibility requirements will have the opportunity to receive ganaxolone in the open label phase of the study. The study's primary efficacy endpoint is percent reduction in seizures. Secondary outcome measures will include non seizure related endpoints to capture certain changes in behavior and sleep. We plan to complete enrollment in this study by the end of 2019 with top-line data expected by mid-2020.

PCDH19-related epilepsy (PCDH19-RE)

In March 2019, Marinus announced the initiation of the Violet Study, a global, double-blind, randomized, placebo-controlled pivotal Phase 3 study evaluating ganaxolone in children with PCDH19-RE. The study will enroll up to 70 patients between the ages of 1 and 17 with a confirmed PCDH19 mutation. Patients enrolled in the study will be stratified into one of two biomarker groups based on baseline allopregnanolone sulfate levels and randomized (ganaxolone or placebo) within each stratum. The study will consist of an 8-week prospective baseline period to collect seizure data, followed by a 17-week double-blind treatment phase. Patients randomized to ganaxolone will titrate over four weeks to a dose of up to 600 mg of oral liquid suspension three times a day and maintain that dose for the following

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13-weeks. After the double-blind period, all patients who meet certain eligibility criteria will have the opportunity to receive ganaxolone in an open label phase of the study. We expect to begin screening patients for enrollment into the study in the second quarter of 2019 with top-line data from the study anticipated in 2021.

Refractory Status Epilepticus (RSE)

Status epilepticus (SE) is a life threatening occurrence of continuous or intermittent seizures lasting more than five minutes in duration without recovery of consciousness during the five or more minutes duration. If SE is not treated immediately, permanent neuronal damage may occur, which contributes to high rates of morbidity and mortality. In RSE, patients who fail to respond to at least two antiepileptic drugs (AEDs) certain synaptic $GABA_A$ receptors are internalized, and thereby unavailable to drugs that target these receptors, such as benzodiazepines. RSE patients who do not respond to AEDs are generally placed under IV anesthesia as a last resort to attempt to stop the seizures and prevent further damage to the brain and death. These patients are referred to as having super refractory status epilepticus (SRSE).

We are believe there is strong rationale for ganaxolone as a potential treatment option for RSE. In RSE, ganaxolone leverages extrasynaptic $GABA_A$ receptors, where, in contrast to the third line SRSE setting in which the patients have been placed into a medically induced coma, target receptors are more likely to be both viable and available. We are enrolling patients with RSE in a Phase 2 multiple ascending dose study with ganaxolone IV. Initial data from this proof of concept study in approximately 15-20 patients are expected in the third quarter of 2019.

Postpartum Depression (PPD)

In December 2018, we announced positive top line results from Part 1 of our Magnolia Study. The primary endpoints of the study were safety and pharmacokinetics. In the study, 58 patients with PPD were randomized on a 1:1 basis to receive one of three ascending fixed IV 60 hour (48-hour fixed infusion followed by 12-hour taper) dose regimens of ganaxolone or placebo. Data from the placebo groups were combined. A bolus injection of ganaxolone prior to the infusion was also explored in the third cohort to test the safety and tolerability of a very short, high dose infusion. Patients with a Hamilton Rating Scale for Depression (HAM D17) score of ≥26 were considered for enrollment in the study. HAM D17 measurements were conducted by a centralized rater and taken at various timepoints spanning from baseline to day 34.

Ganaxolone was safe and well tolerated in all dose groups. Consistent with previous ganaxolone studies, the most common reported adverse events were sedation and dizziness. There were no serious adverse events reported, no discontinuations due to a treatment related adverse event and, consistent with prior studies, there were no reports of loss of consciousness. There was a dose response relationship seen for three groups of patients receiving ganaxolone IV at median doses of 60, 90 and 140 μg/kg/h. No dose group was powered to generate statistical significance in HAM-D17 scores as comparing ganaxolone to placebo; however, we did see strong trends in the efficacy of ganaxolone to reduce HAM-D17 scores in PPD patients. The 140 μg/kg/h dose group (n=10) demonstrated the largest HAM D17 reduction, with a HAM D17 reduction of 15.1 (5.6 > placebo), 16.9 (4.2 > placebo) and 15.7 (4.1 > placebo) points from baseline at 48 hours, 60 hours and day 34, respectively. Patients in the 140 μg/kg/h dose group had a mean response rate, as defined as having a ≥50% reduction from baseline, of 67% and 75% at 60 hours and day 34, respectively. The mean remission rate, as determined by a HAM D17 ≤7, was 33% and 50% at 60 hours and day 34, respectively. The Clinical Global Impression of Improvement (CGI I) as well as the Edinburgh Postnatal Depression Survey (EPDS) and Spielberger State Trait Anxiety 6 (STAI 6) showed consistent trends as the HAM D17.

Based on these results, we advanced to Part 2 of the Magnolia Study that has enrolled 33 patients and will evaluate a six hour 20 mg/hr IV (> 140 μg/kg/h exposure) infusion followed by a once daily dinnertime 900 mg dose of oral ganaxolone for 28 days of treatment. We have fully enrolled Part 2, and data are expected in the first half of the third quarter of 2019.

Marinus is also conducting a PPD study with oral ganaxolone alone, the Amaryllis Study. Patients with a HAM D17 score of ≥ 20 but < 26 are being considered for enrollment in the study. Cohorts of patients enrolled in the initial open label phase of the study receive ascending dose regimens with oral ganaxolone. The primary endpoints of the study are safety and pharmacokinetics. In December 2018, we provided an interim update from our Amaryllis study

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where a low dose and a moderate dose of ganaxolone was studied. Patients who received a once-daily 675 mg evening (moderate) dose who took oral ganaxolone for four weeks experienced a mean reduction in HAM D17 scores of 13.2 points at day 28 from a baseline of 24.7 and a mean reduction of 15.7 points at day 35. Oral ganaxolone was generally safe and well tolerated with no serious adverse events reported and no discontinuations due to treatment related adverse events.

Based on data to date from this study and Part 1 of the Magnolia Study, we have enrolled an additional 43 patients into a higher dose cohort with the goal of further optimizing the oral ganaxolone dose regimen. Patients enrolled in this dose cohort receive 675 mg of oral ganaxolone at dinner and before bedtime for the first two days of treatment and then receive a once-daily 1125 mg dinnertime dose of oral ganaxolone for the remainder of the 28-day regimen. We have fully enrolled the Amaryllis Study and data are expected in the first half of the third quarter of 2019.

Our operations to date have consisted primarily of organizing and staffing our company and developing ganaxolone, including conducting preclinical testing and clinical studies. We have funded our operations primarily through sales of equity and debt securities. At March 31, 2019, we had cash and cash equivalents of $60.8 million. We have no products currently available for sale, have incurred operating losses since inception, have not generated any product sales revenue and have not achieved profitable operations. We incurred a net loss of $12.5 million for the three months ended March 31, 2019. Our accumulated deficit as of March 31, 2019 was $193.9 million, and we expect to continue to incur substantial losses in future periods. We anticipate that our operating expenses will increase substantially as we continue to advance our clinical-stage product candidate, ganaxolone.

We anticipate that our expenses will increase substantially as we:

- conduct later stage clinical studies in targeted indications, which could include CDD, PPD, RSE, PCDH19-RE, Lennox-Gastaut Syndrome, Fragile X Syndrome (FXS) and other indications;

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

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

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

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

- hire additional clinical, manufacturing and scientific personnel; and

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

We believe that our cash and cash equivalents as of March 31, 2019, will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2020. However, we will need to secure additional funding in the future, from one or more equity or debt financings, collaborations, or other sources, in order to carry out all of our planned research and development activities with respect to ganaxolone.

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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 studies and preclinical studies;

the cost of acquiring, developing and manufacturing clinical study 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 studies, 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 studies in order to file an NDA and Supplemental New Drug Applications (sNDAs), or equivalent Marketing Authorization Applications (MAA) outside the US, for ganaxolone for various clinical indications, and in each case, the nature, design, size and cost of further studies will depend in large part on the outcome of preceding studies and discussions with regulators. It is difficult to determine with certainty the costs and duration of our current or future clinical studies 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 studies and development of ganaxolone will depend on a variety of factors, including the uncertainties of future clinical studies and preclinical studies, uncertainties in clinical study enrollment rate and significant and changing government regulation.

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

General and Administrative Expenses

General and administrative expenses consist principally of salaries and related costs for executive and other administrative personnel and consultants, including stock-based compensation and travel expenses. Other general and administrative expenses include professional fees for legal, patent review, consulting and accounting services. General and administrative expenses are expensed when incurred. We expect that our general and administrative expenses will increase in the future as a result of employee hiring and scaling of our operations commensurate with supporting more advanced clinical studies and in preparation for commercial infrastructure. These increases will likely include increased costs for insurance, hiring of additional personnel, outside consultants, legal counsel and accountants, among other expenses.

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Interest Income

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

Results of Operations

Research and Development Expenses

Research and development expenses increased to $8.9 million for the three months ended March 31, 2019, as compared to $3.9 million for the same period in the prior year. The primary drivers of our research and development expenditures are currently in our programs in CDD, PPD, RSE and PCDH19-RE. We have initiated Phase 3 studies in CDD and PCDH19-RE, and Phase 2 studies in PPD and RSE.

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

	Three Months Ended March 31,	
	2019	2018
CDKL5 disorder (1)	$ 0.9	$ 0.2
Postpartum depression (2)	2.4	1.0
Refractory status epilepticus	0.5	0.4
PCDH19-RE (3)	0.7	0.1
General supportive studies (4)	1.2	0.4
Indirect research and development (5)	3.2	1.8
Total	$ 8.9	$ 3.9

(1) The increase was due primarily to the startup activities and eventual initiation of our Phase 3 study in 2018.
(2) The increase was due primarily to increased patient enrollment and the expansion of our Phase 2 study to include our oral formulation of ganaxolone.
(3) The increase was due primarily to the initiation of our Phase 3 study in 2019.
(4) General supportive studies include preclinical and manufacturing studies in support of all formulations and indications for ganaxolone. The increase was due primarily to further the advancement of ganaxolone in clinical studies to support a future NDA.
(5) Indirect research and development expenses in support of all our programs have increased due to the overall increase in preclinical, clinical, and manufacturing activities.

General and Administrative Expenses

General and administrative expenses were $3.7 million for the three months ended March 31, 2019 as compared to $2.2 million for the same period in the prior year, driven primarily by $1.0 million in severance expenses related to the departure of Christopher M. Cashman, our former Chief Executive Officer ($0.4 million of which was non-cash equity compensation expense), and approximately $0.5 million in professional fees and other costs associated with an increased scale of operations.

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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 $12.5 million for the three months ended March 31, 2019. Our cash used in operating activities was $11.7 million for the three months ended March 31, 2019 compared to $6.1 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, and the use of term loans. At March 31, 2019, we had cash and cash equivalents of $60.8 million. During 2018, we received net proceeds of $42.1 million through the sale of our common stock in connection with a secondary public offering.

Cash Flows

Operating Activities. Cash used in operating activities increased to $11.7 million for the months ended March 31, 2019 compared to $6.1 million for the same period a year ago. The increase was driven primarily by a $6.5 million increase in net loss, offset by a $0.7 million increase in stock-based compensation expense.

Investing Activities. Cash provided by investing activities for the three months ended March 31, 2019 represents the maturity of short-term investments of $5.0 million offset by payment of $0.1 million for property and equipment.

Financing Activities. Cash used in financing activities was $0.1 million for the three months ended March 31, 2019 due to payments of accrued financing costs offset by proceeds from the exercise of outstanding stock options. Cash used in financing activities for the three months ended March 31, 2018 was $0.2 million due to repayments of short-term bank borrowings.

Funding Requirements

We have never been profitable 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 studies for ganaxolone.

We believe that our cash, cash equivalents and investments as of March 31, 2019 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2020. 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 studies;
- 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 studies;
.

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;

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- the cost of manufacturing and formulating ganaxolone, or any other future product candidates, to internal and regulatory standards for use in preclinical studies, clinical studies 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;
- capital needed to attract and retain skilled personnel;
- the costs involved in preparing, filing, prosecuting, maintaining, defending and enforcing patent claims, including litigation costs and the outcome of such litigation; and
- the timing, receipt and amount of sales of, or royalties on, future approved products, if any.

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

Off-Balance Sheet Arrangements

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

Discussion of Critical Accounting Policies and Significant Judgments and Estimates

The preparation of financial statements in conformity with GAAP requires us to use judgment in making certain estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities and the reported amounts of revenues and expenses in our financial statements and accompanying notes. Critical accounting policies are those that are most important to the portrayal of our financial condition and results of operations and require difficult, subjective and complex judgments by management in order to make estimates about the effect of matters that are inherently uncertain. During the three months ended March 31, 2019, there were no significant changes to our critical accounting policies from those described in our annual financial statements for the year ended December 31, 2018, which we included in our Annual Report on Form 10-K and was filed with the SEC on March 12, 2019.

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 $60.8 million at March 31, 2019, consisting primarily of funds in cash and money market accounts. From time to time we will purchase various investments, including U.S. Treasury securities 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.

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Item 4. Controls and Procedures

(a) Evaluation of Disclosure Controls and Procedures.

Our management, with the participation of our Executive Chairman 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, 2019. 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 Executive Chairman and Chief Financial Officer have concluded that, as of March 31, 2019, 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 Executive Chairman and Chief Financial Officer, to allow timely decisions regarding required disclosure.

(b) Changes in Internal Control Over Financial Reporting

There was no change in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(f) and 15d-15(f) of the Exchange Act that occurred during the quarter ended March 31, 2019 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 incurred significant losses since our inception and anticipate that we will continue to incur losses in the future.

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

We have incurred significant operating losses since our inception, including a net loss of $12.5 million for the three months ended March 31, 2019. As of March 31, 2019, we had an accumulated deficit of $193.9 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. 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 pharmaceutical products, we are unable to predict the extent of any future losses or whether or when we will become profitable, if ever.

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

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

- successfully complete development activities, including enrollment of study participants, completion of the necessary clinical studies and attainment of clinical study results that will support regulatory approvals;

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

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

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

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

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

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

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

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

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

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

We believe that our cash and cash equivalents as of March 31, 2019, will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2020. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we currently expect. Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to the:

initiation, progress, timing, costs and results of preclinical studies and clinical studies, including patient enrollment in such studies, 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;

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effects of competing technological and market developments;

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

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

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

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

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

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

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

Risks Related to Our Business and Development of Our Product

Our future success is dependent on the successful clinical development, regulatory approval and commercialization of ganaxolone, which is currently undergoing four clinical studies 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 scale-up manufacturing and 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

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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 studies, generally including two adequate and well-controlled clinical studies, and, with respect to approval in the United States, to the satisfaction of the FDA, that ganaxolone is safe and effective for use for that target indication and that the manufacturing facilities, processes and controls are adequate. Even if ganaxolone were to obtain approval from the FDA and comparable foreign regulatory authorities, any approval might contain significant limitations, such as restrictions as to specified age groups, warnings, precautions or contraindications, or may be subject to burdensome post-approval study or risk management requirements. If we are unable to obtain regulatory approval for ganaxolone in one or more jurisdictions, or any approval contains significant limitations, we may not be able to obtain sufficient funding or generate sufficient revenue to continue the development of any other product candidate that we may in-license, develop or acquire in the future. Furthermore, even if we obtain regulatory approval for ganaxolone, we will still need to develop a commercial organization, establish commercially viable pricing and obtain approval for adequate reimbursement from third-party and government payers. If we are unable to successfully commercialize ganaxolone, we may not be able to earn sufficient revenue to continue our business.

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

Success in preclinical studies and early clinical studies does not ensure that later studies 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 studies, even after seeing promising results in earlier studies and studies. For example, while ganaxolone showed statistical separation from placebo in a Phase 2 study in adjunctive treatment of adults with focal onset seizures, ganaxolone failed to show a similar statistically significant separation in a Phase 3 study for the same indication. As a result, we discontinued our program in adult focal onset seizures and began to focus our efforts on advancing ganaxolone in postpartum depression, refractory status epilepticus, and pediatric orphan epilepsy indications. We do not know whether the clinical studies we may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market ganaxolone in any particular jurisdiction or indication. If clinical studies underway or conducted in the future do not produce favorable results, our ability to achieve regulatory approval for ganaxolone may be adversely impacted.

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

Ganaxolone is a novel compound and its potential therapeutic benefit is unproven. Our ability to generate revenue from ganaxolone, which we do not expect will occur for at least the next several years, if ever, will depend heavily on our successful development and commercialization after regulatory approval, which is subject to many potential risks and may not occur. Ganaxolone may interact with human biological systems in unforeseen, ineffective or harmful ways. If ganaxolone is associated with undesirable side effects or has characteristics that are unexpected, we may need

to abandon its development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in early stage testing for treating the target indications for ganaxolone have later been found to cause side effects that prevented further development of the compound. As a result of these and other risks described herein that are inherent in the development of novel therapeutic agents, we may never successfully develop, enter into or maintain third-party licensing or collaboration transactions with respect to, or successfully commercialize, ganaxolone, in which case we will not achieve profitability and the value of our stock may decline.

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

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

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We may experience delays in our ongoing or future clinical studies and we do not know whether planned clinical studies 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 studies of ganaxolone on clinical hold now or in the future. Clinical studies 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 study design that we are able to execute;
- delay or failure in obtaining authorization to commence a study or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical study;
- delay or failure in reaching agreement on acceptable terms with prospective CROs and clinical study sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and study 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 study at each site;
- withdrawal of clinical study sites from our clinical studies as a result of changing standards of care or the ineligibility of a site to participate in our clinical studies;
- delay or failure in recruiting and enrolling suitable study subjects to participate in a study;
- delay or failure in study subjects completing a study or returning for post-treatment follow-up;
- clinical sites and investigators deviating from a study protocol, failing to conduct the study in accordance with regulatory requirements, or dropping out of a study;
- inability to identify and maintain a sufficient number of study sites, many of which may already be engaged in other clinical study programs, including some that may be for competing product candidates with the same indication;
- failure of our third-party clinical study managers to satisfy their contractual duties or meet expected deadlines;
- delay or failure in adding new clinical study sites;
- ambiguous or negative interim results or results that are inconsistent with earlier results;
- feedback from the FDA, IRBs, data safety monitoring boards, or a comparable foreign regulatory authority, or results from earlier stage or concurrent preclinical studies and clinical studies, that might require modification to the protocol for the study;
- decision by the FDA, an IRB, a comparable foreign regulatory authority, or us, or recommendation by a data safety monitoring board or comparable foreign regulatory authority, to suspend or terminate clinical studies 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;

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- difficulties in manufacturing or obtaining from third parties sufficient quantities of a product candidate for use in clinical studies that meet internal and regulatory standards;

- lack of adequate funding to continue the clinical study, including the incurrence of unforeseen costs due to enrollment delays, requirements to conduct additional clinical studies 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 studies, is affected by many factors including the size and nature of the subject population, the proximity of subjects to clinical sites, the eligibility criteria for the study, the design of the clinical study, ability to obtain and maintain subject consents, risk that enrolled subjects will drop out before completion, competing clinical studies 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 studies for the indications we are investigating. Some of our clinical studies are directed at small patient populations. Patient enrollment in these studies could be particularly challenging. In the past, we have experienced delays in enrolling patients in studies directed at small patient populations. We rely on CROs and clinical study sites to ensure the proper and timely conduct of our clinical studies, 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 study 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 studies 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 studies 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 studies 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 studies, in some cases there were side effects, and some of the side effects were severe. The most frequent side effects were dizziness, fatigue and somnolence (or drowsiness). More side effects of the Central Nervous System (CNS) were categorized as severe as compared to side effects of other body systems.

If these side effects are reported in future clinical studies, or if other safety or toxicity issues are reported in our future clinical studies, 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 studies could reveal an unacceptably high severity and prevalence of side effects. In such an event, our clinical studies 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 studies and may result in potential product liability claims.

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

- we may be forced to suspend marketing of ganaxolone;
- regulatory authorities may withdraw their approvals of ganaxolone;
- regulatory authorities may require additional warnings on the label that could diminish the usage or otherwise limit the commercial success of ganaxolone;
- we may be required to conduct post-marketing studies;
- we could be sued and held liable for harm caused to subjects or patients; and
- our reputation may suffer.

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

Even if ganaxolone receives regulatory approval, we may still face 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, patient registry, 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;

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suspend any ongoing clinical studies;

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

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

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

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

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

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

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

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

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

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authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. If we are unable to obtain approval of ganaxolone by regulatory authorities in the European Union or another country or jurisdiction, the commercial prospects of ganaxolone may be significantly diminished and our business prospects could decline.

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

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

We have received orphan drug designation for treating CDD, PCDH19-RE, FXS and SE with ganaxolone and expect that we may in the future pursue orphan drug designations for ganaxolone for one or more additional indications. However, obtaining an orphan drug designation can be difficult and we may not be successful in doing so for additional ganaxolone indications or any future product candidates. Even if we were to obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product from the competition of different drugs for the same condition, which could be approved during the exclusivity period. In addition, 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 studies. If a disruption event were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. For example, the loss of data relating to completed, ongoing or planned clinical studies could result in delays in our regulatory approval efforts and cause us to incur significant additional costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of ganaxolone could be delayed.

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

Our operations could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made

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

Risks Related to the Commercialization of Our Product

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

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

- efficacy and safety of ganaxolone, or ganaxolone administered with other drugs, each as demonstrated in clinical studies and post-marketing experience;
- clinical indications for which ganaxolone is approved;
- acceptance by physicians and patients of ganaxolone as a safe and effective treatment;
- potential and perceived advantages of ganaxolone over alternative treatments;
- safety of ganaxolone seen in a broader patient group, including its use outside the approved indications should physicians choose to prescribe for such uses;
- prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA or other regulatory authorities;
- timing of market introduction of ganaxolone as well as competitive products;
- cost of treatment in relation to alternative treatments;
- availability of coverage and adequate reimbursement and pricing by government and private payers;
- relative convenience and ease of administration; and
- effectiveness of our sales and marketing efforts.

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

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

We do not currently have an organization for the sale, marketing and distribution of pharmaceutical products and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. In order

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

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

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

- differing regulatory requirements in foreign countries;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market (with low or lower prices) rather than buying them locally;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- 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.

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

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

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for coverage and reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may only be temporary. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payers and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payers often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to obtain coverage and profitable reimbursement rates from both government-funded and private payers for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

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

The development and commercialization of new drug products is highly competitive. We face competition with respect to ganaxolone, and will face competition with respect to any other product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies

that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing ganaxolone. Some of these competitive products and therapies are based on scientific approaches that are the same as, or similar to, our approach, and others are based on entirely different approaches. For example, there are several companies developing product candidates that target the same $GABA_A$, neuroreceptor that we are targeting or that are testing product candidates in the same indications that we are testing. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Ganaxolone is presently being developed as an antiepileptic and neuropsychiatric therapeutic. There are a variety of marketed therapies available for these patients.

Specifically, there are more than 25 approved AEDs available in the United States and worldwide, including the generic products levetiracetam, lamotrigine, carbamazepine, oxcarbazepine, valproic acid and topiramate. Recent market

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entrants include branded products developed by Lundbeck, UCB, Eisai, and Sunovion Pharmaceuticals. In addition, there are several drugs in development for the treatment of pediatric orphan indications, including compounds being developed by GW Pharmaceuticals, Zogenix, Zynerba and Ovid Therapeutics. Sage Therapeutics is developing molecules with a similar mechanism of action as ganaxolone for the treatment of PPD.

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

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

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

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific, management and commercial personnel, establishing clinical study sites and subject registration for clinical studies, 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 studies and will face an even greater risk if ganaxolone receives regulatory approval and we subsequently commercialize it. Product liability claims may be brought against us by study subjects enrolled in our clinical studies, 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 study sites or entire study programs;

injury to our reputation and significant negative media attention;

withdrawal of clinical study subjects;

significant costs to defend the related litigation;

substantial monetary awards to clinical study subjects or patients;

loss of revenue;

diversion of management and scientific resources from our business operations;

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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 product liability insurance coverage, 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 studies. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize ganaxolone.

We rely on third-party CROs to monitor and manage data for our ongoing preclinical and clinical programs. We rely on these parties for execution of our preclinical studies and clinical studies, and we control only some aspects of their activities. Nevertheless, we are responsible for ensuring that each of our preclinical studies and clinical studies 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 study sponsors, principal investigators and study sites. If we or any of our CROs fail to comply with applicable GCP, the clinical data generated in our clinical studies may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical studies 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 studies comply with GCP requirements. In addition, our clinical studies must be conducted with product produced under cGMP requirements. Failure to comply with these regulations may require us to repeat preclinical studies and clinical studies, 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 studies 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

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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 studies 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 studies 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 studies. We have no experience manufacturing ganaxolone on a commercial scale and have no manufacturing facility. We are dependent on third-party manufacturers for the manufacture of ganaxolone as well as on third parties for our supply chain, and if we experience problems with any such third parties, the manufacturing of ganaxolone could be delayed.

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

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured ganaxolone ourselves, including reliance on the third party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control, including a failure to

synthesize and manufacture ganaxolone or any products we may eventually commercialize in accordance with our specifications, and the possibility of termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or damaging to us. In addition, the FDA and other regulatory authorities would require that ganaxolone and any products that we may eventually commercialize be manufactured according to cGMP and similar foreign standards. Any failure by our third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of ganaxolone in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of ganaxolone. In addition, such failure could be the basis for the FDA to issue a warning letter, withdraw approvals for ganaxolone previously granted to us, or take other regulatory or legal action, including recall or seizure of outside supplies of ganaxolone, total or partial suspension of production, suspension of ongoing clinical studies, 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 study could considerably delay completion of our preclinical study or clinical study, product testing and potential regulatory approval of ganaxolone. If

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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 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 studies 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 studies. In addition, our preclinical studies and clinical studies to evaluate ganaxolone in patients suffering from posttraumatic stress disorder (PTSD) have been primarily conducted by the United States Department of Veterans Affairs, which also receives funding from the DoD. Programs funded by the United States government and its agencies, including the DoD, include provisions that confer on the government substantial rights and remedies, many of which are not typically found in commercial contracts, including powers of the government to:

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

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

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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.

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. Any reduction or delay in DoD funding to our collaborators may force us to seek alternative funding in order to progress these programs, which may not be available on non-dilutive terms, terms favorable to us or at all.

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

Our research and development activities involve the controlled use of potentially hazardous substances, including chemical and biological materials by our third-party manufacturers. Our manufacturers are subject to federal, state and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical, radioactive and hazardous materials. We cannot completely eliminate the risk of contamination or injury resulting from medical, radioactive or hazardous materials. As a result of any such contamination or injury we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability

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

In March 2010, 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 was signed into law. 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. In August 2011, the Budget Control Act of 2011 was signed into law, 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, the American Taxpayer Relief Act of 2012 was signed into law, 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

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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 payers are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. Furthermore, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the European Union will put additional pressure on product pricing, reimbursement and usage, which may adversely affect our future product sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

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

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

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

The Foreign Corrupt Practices Act (FCPA) prohibits any United States individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the

United States to comply with certain accounting provisions requiring such companies to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the DOJ. The SEC is involved with enforcement of the books and records provisions of the FCPA.

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

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

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

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

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

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

statements in connection with the delivery of or payment for healthcare benefits, items or services;

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

the Affordable Care Act requires manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or Children's Health Insurance Program, to report annually to HHS information related to payments and other transfers of value to physicians and teaching hospitals, and

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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 payers, including private insurers; some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state and foreign laws govern the privacy and security of health information in specified circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

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

Risks Related to Our Intellectual Property

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

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

The patent positions of biotechnology and pharmaceutical companies generally are highly uncertain, involve complex legal and factual questions and have in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patents, including those patent rights licensed to us by third parties, are highly uncertain. The steps we or our licensors have taken to protect our proprietary rights may not be adequate to preclude misappropriation of our proprietary information or infringement of our intellectual property rights, both inside and outside the United States. Further, the examination process may require us or our licensors to

narrow the claims for our pending patent applications, which may limit the scope of patent protection that may be obtained if these applications issue. The rights already granted under any of our currently issued patents or those licensed to us and those that may be granted under future issued patents may not provide us with the protection or competitive advantages we are seeking. If we or our licensors are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficient, our competitors could develop and commercialize technology and products similar or superior to ours, and our ability to successfully commercialize our technology and products may be adversely affected. It is also possible that we or our licensors will fail to identify patentable aspects of inventions made in the course of our development and commercialization activities before it is too late to obtain patent protection on them.

With respect to patent rights, we do not know whether any of our granted or issued patents will effectively prevent others from commercializing competitive technologies and products. Publications of discoveries in the scientific

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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.

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

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

Our commercial success depends upon our ability to develop, manufacture, market and sell ganaxolone, and to use our related technologies. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to ganaxolone, including interference or derivation proceedings before the United States Patent and Trademark Office (USPTO). Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue commercializing ganaxolone. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Under certain circumstances, we could be forced, including by court order, to cease commercializing ganaxolone. In addition, in any such proceeding or litigation, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing ganaxolone or force us to cease some of our business operations, which could materially harm our business. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar negative impact on our business.

While ganaxolone is in preclinical studies and clinical studies, we believe that the use of ganaxolone in these preclinical studies and clinical studies falls within the scope of the exemptions provided by 35 U.S.C. Section 271(e) in

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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.

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

Filing, prosecuting and defending patents on ganaxolone and any future product candidates throughout the world would be prohibitively expensive, and our or our licensors' intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws and practices of some foreign countries, particularly those relating to pharmaceuticals, do not protect intellectual property rights to the same extent as federal and state laws in the United States. For example, novel formulations and manufacturing processes may not be patentable in certain jurisdictions, and the requirements for patentability may differ in certain countries, particularly developing countries. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our patents, requiring us to engage in complex, lengthy and costly litigation or other proceedings. Generic drug manufacturers may develop, seek approval for, and launch generic versions of our products. Many countries, including European Union countries, India, Japan and China, have compulsory licensing laws under which a patent owner may be compelled under certain circumstances to grant licenses to third parties. In those countries, we may have limited remedies if patents are infringed or if we are compelled to grant a license to a third party, which could materially diminish the value of our patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from our intellectual property.

We may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions into or within the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products, and may export otherwise infringing products to territories where we have patent pr