REFINITIV

DELTA REPORT

10-Q

MRNS - MARINUS PHARMACEUTICALS,

10-Q - JUNE 30, 2024 COMPARED TO 10-Q - MARCH 31, 2024

The following comparison report has been automatically generated

TOTAL DELTAS	1143
CHANGES	210
DELETIONS	413
ADDITIONS	520

REFINITIV

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, JUNE 30, 2024

OR

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

For the transition period from to

COMMISSION FILE NUMBER 001-36576

Graphic

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, Suite 500

100 Matsonford Rd

Radnor, PA 19087

(Address of registrant's principal executive offices, including zip code)

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

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	Trading Symbol(s)	Name of Each Exchange on Which Registered
Common Stock, par value $0.001 per share	MRNS	Nasdaq Global Market

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 May 3, 2024 August 7, 2024, was: 54,933,774. 55,084,038.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY
INDEX TO FORM 10-Q
FOR THE QUARTER ENDED MARCH 31, JUNE 30, 2024

PART I – FINANCIAL INFORMATION

Unless the context otherwise requires, all references in this Quarterly Report on Form 10-Q to the "Company," "Marinus," "we," "us," and "our" include Marinus Pharmaceuticals, Inc. and its wholly owned subsidiary, Marinus Pharmaceuticals Emerald Limited, an Ireland company.

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PART I

FINANCIAL INFORMATION

Item 1. Consolidated Financial Statements

MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY

CONSOLIDATED BALANCE SHEETS
(in thousands, except share and per share amounts)
(unaudited)

	March 31, 2024	December 31, 2023
ASSETS		
Current assets:		
Cash and cash equivalents	$ 104,253	$ 120,572
Short-term investments	9,000	29,716
Accounts receivable, net	3,513	3,799
Inventory	5,783	2,413
Prepaid expenses and other current assets	8,727	8,746
Total current assets	131,276	165,246
Property and equipment, net	3,735	3,843
Other assets	2,339	1,819
Total assets	$ 137,350	$ 170,908
LIABILITIES AND STOCKHOLDERS' (DEFICIT) EQUITY		
Current liabilities:		
Accounts payable	$ 6,125	$ 4,003
Current portion of notes payable	15,401	11,551
Current portion of revenue interest financing payable	2,511	2,211
Accrued expenses	18,721	22,859
Total current liabilities	42,758	40,624
Notes payable, net of deferred financing costs	58,072	61,423
Revenue interest financing payable, net of deferred financing costs	34,642	33,766
Contract liabilities, net	17,730	17,545
Other long-term liabilities	582	785
Total liabilities	153,784	154,143
Stockholders' (deficit) equity:		
Common stock, $0.001 par value; 150,000,000 shares authorized, 54,938,349 issued and 54,931,042 outstanding at March 31, 2024 and 54,585,428 issued and 54,578,121 outstanding at December 31, 2023	55	55
Additional paid-in capital	594,106	588,656
Treasury stock at cost, 7,307 shares at March 31, 2024 and December 31, 2023	—	—
Accumulated other comprehensive loss	—	(20)
Accumulated deficit	(610,595)	(571,926)
Total stockholders' (deficit) equity	(16,434)	16,765
Total liabilities and stockholders' (deficit) equity	$ 137,350	$ 170,908

	June 30, 2024	December 31, 2023
ASSETS		
Current assets:		
Cash and cash equivalents	$ 64,676	$ 120,572
Short-term investments	—	29,716
Accounts receivable, net	3,435	3,799
Inventory	4,873	2,413
Prepaid expenses and other current assets	8,898	8,746
Total current assets	81,882	165,246
Property and equipment, net	3,720	3,843
Other assets	1,481	1,819
Total assets	$ 87,083	$ 170,908
LIABILITIES AND STOCKHOLDERS' (DEFICIT) EQUITY		
Current liabilities:		
Accounts payable	$ 6,237	$ 4,003
Current portion of notes payable	11,550	11,551
Current portion of revenue interest financing payable	2,849	2,211
Accrued expenses	15,231	22,859
Total current liabilities	35,867	40,624
Notes payable, net of deferred financing costs	45,075	61,423
Revenue interest financing payable, net of deferred financing costs	35,431	33,766
Contract liabilities, net	17,844	17,545
Other long-term liabilities	211	785
Total liabilities	134,428	154,143
Stockholders' (deficit) equity:		
Common stock, $0.001 par value; 150,000,000 shares authorized, 54,971,226 issued and 54,963,919 outstanding at June 30, 2024 and 54,585,428 issued and 54,578,121 outstanding at December 31, 2023	55	55
Additional paid-in capital	599,023	588,656
Treasury stock at cost, 7,307 shares at June 30, 2024 and December 31, 2023	—	—
Accumulated other comprehensive loss	—	(20)
Accumulated deficit	(646,423)	(571,926)
Total stockholders' (deficit) equity	(47,345)	16,765
Total liabilities and stockholders' (deficit) equity	$ 87,083	$ 170,908

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,	
	2024	2023
Revenue:		
Product revenue, net	$ 7,509	$ 3,332
Federal contract revenue	152	7,048
Collaboration revenue	18	—
Total revenue	7,679	10,380
Expenses:		
Research and development	24,118	27,933
Selling, general and administrative	18,626	15,204
Cost of product revenue	756	206
Total expenses	43,500	43,343
Loss from operations	(35,821)	(32,963)
Interest income	1,462	2,343
Interest expense	(4,346)	(4,147)
Other income, net	36	37
Net loss applicable to common shareholders	$ (38,669)	$ (34,730)
Per share information:		
Net loss per share of common stock—basic and diluted	$ (0.68)	$ (0.67)
Basic and diluted weighted average shares outstanding	56,851,811	51,769,685
Other comprehensive income:		
Unrealized gain on available-for-sale securities	20	74
Total comprehensive loss	$ (38,649)	$ (34,656)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Revenue:				
Product revenue, net	$ 7,951	$ 4,249	$ 15,460	$ 7,581
Federal contract revenue	87	1,814	239	8,862
Collaboration revenue	18	18	36	18
Total revenue	8,056	6,081	15,735	16,461
Expenses:				
Research and development	20,897	21,412	45,015	49,345

Selling, general and administrative	16,710	15,722	35,336	30,926
Restructuring Costs	1,950	—	1,950	—
Cost of product revenue	735	386	1,491	592
Total expenses	40,292	37,520	83,792	80,863
Loss from operations	(32,236)	(31,439)	(68,057)	(64,402)
Interest income	1,109	2,128	2,571	4,471
Interest expense	(4,617)	(4,208)	(8,963)	(8,355)
Other (expense) income, net	(84)	47	(48)	84
Loss before income taxes	(35,828)	(33,472)	(74,497)	(68,202)
Benefit for income taxes	—	1,538	—	1,538
Net loss applicable to common shareholders	$ (35,828)	$ (31,934)	$ (74,497)	$ (66,664)
Per share information:				
Net loss per share of common stock—basic and diluted	$ (0.63)	$ (0.61)	$ (1.31)	$ (1.28)
Basic and diluted weighted average shares outstanding	57,064,095	52,551,918	56,957,953	52,162,962
Other comprehensive income:				
Unrealized (loss) gain on available-for-sale securities	—	(188)	20	(114)
Total comprehensive loss	$ (35,828)	$ (32,122)	$ (74,477)	$ (66,778)

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,	
	2024	2023
Cash flows from operating activities		
Net loss	$ (38,669)	$ (34,730)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	131	157
Amortization of debt issuance costs	609	480
Accretion of revenue interest financing debt, net of cash paid	1,065	1,440

Amortization of discount on short-term investments	(149)	(228)
Stock-based compensation expense	5,193	3,741
Amortization of net contract asset/liability	(337)	(413)
Noncash lease expense	47	53
Noncash lease liability	224	96
Write off of fixed assets	—	62
Changes in operating assets and liabilities:		
Net contract asset/liability	522	544
Prepaid expenses and other current assets, non-current assets, inventory and accounts receivable	(3,833)	(8,963)
Accounts payable and accrued expenses	(2,264)	(3,717)
Net cash used in operating activities	(37,461)	(41,478)
Cash flows from investing activities		
Maturities of short-term investments	20,885	—
Purchases of short-term investments	—	(51,995)
Net cash provided by (used in) investing activities	20,885	(51,995)
Cash flows from financing activities		
Proceeds from exercise of stock options	257	—
Other cash flows from financing activities	—	(174)
Net cash provided by (used in) financing activities	257	(174)
Net decrease in cash and cash equivalents	(16,319)	(93,647)
Cash and cash equivalents—beginning of period	120,572	240,551
Cash and cash equivalents—end of period	$ 104,253	$ 146,904
Supplemental disclosure of cash flow information		
Unrealized gain on short-term investments	$ 20	$ 74
Cash paid for interest during the period	$ 2,671	$ 2,156

	Six Months Ended June 30,	
	2024	**2023**
Cash flows from operating activities		
Net loss	$ (74,497)	$ (66,664)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	271	284
Amortization of debt issuance costs	1,246	1,102
Accretion of revenue interest financing debt, net of cash paid	2,106	2,916
Amortization of discount on short-term investments	(149)	(669)
Stock-based compensation expense	10,110	7,632
Amortization of net contract asset/liability	(741)	(837)
Noncash lease expense	443	102
Noncash lease liability	87	196
Write off of fixed assets	—	62
Write off of right-of-use assets	757	—
Financing costs included in interest expense	300	—
Changes in operating assets and liabilities:		
Net contract asset/liability	1,040	1,569

Prepaid expenses and other current assets, non-current assets, inventory and accounts receivable	(3,160)	(7,441)
Accounts payable and accrued expenses	(6,115)	(4,088)
Net cash used in operating activities	(68,302)	(65,836)
Cash flows from investing activities		
Maturities of short-term investments	29,885	5,000
Purchases of short-term investments	—	(51,995)
Purchases of property and equipment	(38)	—
Net cash provided by (used in) investing activities	29,847	(46,991)
Cash flows from financing activities		
Proceeds from exercise of stock options	257	484
Prepayments of long-term debt, including financing costs	(17,698)	
Other cash flows from financing activities	—	(421)
Net cash (used in) provided by financing activities	(17,441)	63
Net decrease in cash and cash equivalents	(55,896)	(112,764)
Cash and cash equivalents—beginning of period	120,572	240,551
Cash and cash equivalents—end of period	$ 64,676	$ 127,787
Supplemental disclosure of cash flow information		
Unrealized gain (loss) on short-term investments	$ 20	$ (114)
Property and equipment in accounts payable	$ 60	$ —
Cash paid for interest during the period	$ 5,611	$ 4,336
Cash paid for income taxes during the period	$ —	$ 163

See accompanying notes to consolidated financial statements.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY
CONSOLIDATED STATEMENTS OF (DEFICIT) EQUITY
(in thousands)
(unaudited)

	Series A Convertible Preferred Stock		Common Stock		Additional Paid-in	Treasury Stock		Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Shares	Amount	Capital	Shares	Amount	Income (Loss)	Deficit	(Deficit) Equity
Balance, December 31, 2022	4,300	$ 4,043	49,642,767	$ 50	$542,428	7,307	$ —	$ —	$ (430,521)	$ 116,000
Stock-based compensation expense	—	—	—	—	3,741	—	—	—	—	3,741

Net issuance of common stock in connection with the vesting of restricted stock	—	—	22,350	—	—	—	—	—	—	—
Unrealized gain on short-term investments	—	—	—	—	—	—	—	74	—	74
Net Loss	—	—	—	—	—	—	—	—	(34,730)	(34,730)
Balance, March 31, 2023	4,300	$ 4,043	49,665,117	$ 50	$546,169	7,307	$ —	74	$ (465,251)	$ 85,085
Balance, December 31, 2023	—	$ —	54,578,121	$ 55	$588,656	7,307	$ —	$ (20)	$ (571,926)	$ 16,765
Stock-based compensation expense	—	—	—	—	5,193	—	—	—	—	5,193
Exercise of stock options	—	—	57,665	—	257	—	—	—	—	257
Net issuance of common stock in connection with the vesting of restricted stock	—	—	295,256	—	—	—	—	—	—	—
Unrealized gain on short-term investments	—	—	—	—	—	—	—	20	—	20
Net loss	—	—	—	—	—	—	—	—	(38,669)	(38,669)
Balance, March 31, 2024	—	$ —	54,931,042	$ 55	$594,106	7,307	$ —	$ —	$ (610,595)	$ (16,434)

	Series A Convertible Preferred Stock		Common Stock		Additional Paid-in	Treasury Stock		Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Shares	Amount	Capital	Shares	Amount	Income (Loss)	Deficit	(Deficit) Equity
Balance, December 31, 2022	4,300	$ 4,043	49,642,767	$ 50	$542,428	7,307	$ —	$ —	$ (430,521)	$ 116,000
Stock-based compensation expense	—	—	—	—	3,741	—	—	—	—	3,741
Net issuance of common stock in connection with the vesting of restricted stock	—	—	22,350	—	—	—	—	—	—	—
Unrealized gain on short-term investments	—	—	—	—	—	—	—	74	—	74
Net Loss	—	—	—	—	—	—	—	—	(34,730)	(34,730)
Balance, March 31, 2023	4,300	$ 4,043	49,665,117	$ 50	$546,169	7,307	$ —	74	$ (465,251)	$ 85,085
Stock-based compensation expense	—	—	—	—	3,891	—	—	—	—	3,891
Net issuance of common stock in connection with the vesting of restricted stock	—	—	11,625	—	—	—	—	—	—	—
Exercise of stock options	—	—	72,440	—	485	—	—	—	—	485
Conversion of convertible preferred stock into common	(4,300)	(4,043)	860,000	1	4,042	—	—	—	—	—
Unrealized loss on short-term investments	—	—	—	—	—	—	—	(188)	—	(188)
Net loss	—	—	—	—	—	—	—	—	(31,934)	(31,934)
Balance, June 30, 2023	—	$ —	50,609,182	$ 51	$554,587	7,307	$ —	(114)	$ (497,185)	$ 57,339
Balance, December 31, 2023	—	$ —	54,578,121	$ 55	$588,656	7,307	$ —	$ (20)	$ (571,926)	$ 16,765

Stock-based compensation expense	—	—	—	—	5,193	—	—	—	—	5,193
Exercise of stock options	—	—	57,665	—	257	—	—	—	—	257
Net issuance of common stock in connection with the vesting of restricted stock	—	—	295,256	—	—	—	—	—	—	—
Unrealized gain on short-term investments	—	—	—	—	—	—	—	20	—	20
Net loss	—	—	—	—	—	—	—	—	(38,669)	(38,669)
Balance, March 31, 2024	—	$ —	54,931,042	$ 55	$594,106	7,307	$ —	$ —	$ (610,595)	$ (16,434)
Stock-based compensation expense	—	—	—	—	4,917	—	—	—	—	4,917
Net issuance of common stock in connection with the vesting of restricted stock	—	—	32,877	—	—	—	—	—	—	—
Net loss	—	—	—	—	—	—	—	—	(35,828)	(35,828)
Balance, June 30, 2024	—	$ —	54,963,919	$ 55	$599,023	7,307	$ —	—	$ (646,423)	$ (47,345)

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 commercial-stage pharmaceutical company dedicated to the development of innovative therapeutics for the treatment of seizure disorders, including rare genetic epilepsies and status epilepticus (SE). On March 18, 2022, the U.S. Food and Drug Administration (FDA) approved our new drug application (NDA) for the use of ZTALMY® (ganaxolone) oral suspension CV for the treatment of seizures associated with Cyclin-dependent Kinase-like 5 (CDKL5) Deficiency Disorder (CDD) in patients two years of age and older. ZTALMY, our first FDA approved product, became available for commercial sale and shipment in the third quarter of 2022. On July 28, 2023, the European Commission (EC) granted marketing authorization for ZTALMY for the adjunctive treatment of epileptic seizures associated with CDD in patients two to 17 years of age. ZTALMY may be continued in patients 18 years of age and older. We have an exclusive collaboration agreement with Orion Corporation (Orion) for European commercialization of ganaxolone for ZTALMY. Orion is preparing continues to prepare for commercial launches of ZTALMY in select European countries in the second half of 2024. On July 18, 2024, we announced that the China

National Medical Products Administration has approved ganaxolone oral suspension for the treatment of epileptic seizures in patients two years of age and older with CDD. We have a collaboration agreement with Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia) for the commercialization of ganaxolone in Mainland China, Hong Kong, Macau and Taiwan.

We are also developing ganaxolone for the treatment of other rare genetic epilepsies, including Tuberous Sclerosis Complex (TSC), . Top-line results from our Phase 3 TSC (TrustTSC) clinical trial are expected in the first half of the fourth quarter of 2024. TSC is a rare, multisystem genetic disorder caused by inherited mutations in the TSC1 gene or TSC2 gene. TSC is often characterized by non-cancerous tumors, skin abnormalities, and severe neurological manifestations including refractory seizures and neurodevelopmental delays. TSC is a leading cause of genetic epilepsy.

We recently announced top-line results from our Phase 3 RAISE trial of intravenous (IV) ganaxolone for the treatment of Refractory Status Epilepticus (RSE). SE Status Epilepticus (SE) is a life-threatening condition characterized by continuous, prolonged seizures or rapidly recurring seizures without intervening recovery of consciousness. If SE is not treated urgently, permanent neuronal damage may occur, which contributes to high rates of morbidity and mortality. Patients with SE who do not respond to first-line benzodiazepine treatment are classified as having Established Status Epilepticus (ESE) and those who then progress to and subsequently fail at least one second-line antiepileptic drug (AED) are classified as having RSE. The top-line results from RAISE showed that the trial met its first co-primary endpoint, a statistically significant proportion of patients had status epilepticus cessation within 30 minutes of initiating IV ganaxolone compared to placebo, but failed to achieve statistical significance on its second co-primary endpoint, the proportion of patients not progressing to IV anesthesia for 36 hours following initiation of IV ganaxolone compared to placebo. We continue to analyze the full RAISE dataset and plan to request a meeting with the FDA to discuss a potential path forward for IV ganaxolone in RSE.

We are developing ganaxolone in formulations for two different routes of administration: intravenous (IV) IV and oral. The different formulations are intended to maximize potential therapeutic applications of ganaxolone for adult and pediatric patient populations, in both acute and chronic care. While the precise mechanism by which ganaxolone exerts its therapeutic effects in the treatment of seizures is unknown, its anticonvulsant effects are thought to result from positive allosteric modulation of the gamma-aminobutyric acid type A ($GABA_A$) receptor in the central nervous system (CNS). system. Ganaxolone is a synthetic analog of allopregnanolone, an endogenous neurosteroid, and targets both synaptic and extrasynaptic $GABA_A$. This unique receptor binding profile may contribute to the anticonvulsant, antidepressant and anxiolytic effects shown by neuroactive steroids in animal models, clinical trials or both.

Liquidity

Since inception, we have incurred negative cash flows from our operations, and other than for the three months ended September 30, 2022 due to a one-time net gain from the sale of our Priority Review Voucher (PRV), we have incurred net losses. We incurred a Net loss of $38.7 million $74.5 million for the three six months ended March 31, 2024 June 30, 2024. There is no

assurance that profitable operations will be achieved in the future, and if achieved, could be sustained on a continuing basis. In addition, development activities, clinical and preclinical testing, and commercialization of ganaxolone (in indications other than CDD in the U.S.) will require significant additional financing. Our accumulated deficit as of March 31, 2024 June 30, 2024 was $610.6 million $646.4 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, government funding, collaborations, licensing transactions and other commercial transactions or other sources, and revenues from product sales. 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 continued development and commercialization of ganaxolone.

Management's operating plan, which underlies the analysis of our ability to continue as a going concern, involves the estimation of the amount and timing of future cash inflows and outflows. Actual results could vary from the operating plan. We follow the provisions of Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) Topic 205-40, Presentation of Financial Statements—Going Concern, which requires management

to assess our ability to continue as a going concern within one year after the date the financial statements are issued. We had Cash and cash equivalents and Short-term investments of $113.3 million $64.7 million as of March 31, 2024 June 30, 2024. We believe that such amount is not sufficient to fund our operations for the one-year period after the date these financial statements are issued. As a result, there is substantial doubt about our ability to continue as a going concern through the one-year period from the date these financial statements are issued. Cost reduction activities are being were implemented with expected impact beginning in the second quarter of 2024. 2024 as further discussed in Note 13. Management's plans that are intended to further mitigate this risk include securing additional funding in the future from one or more equity or debt financings, government funding, collaborations, licensing transactions, other commercial or strategic transactions or other sources. However, there can be no assurance that we will be successful in raising additional capital or that such capital, if available, will be on terms acceptable to us. We have and will continue to evaluate alternatives to extend our operations beyond the one-year period after the date the financial statements are issued.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited interim consolidated financial statements include the accounts of Marinus Pharmaceuticals, Inc. (a Delaware corporation) as well as the accounts of Marinus Pharmaceuticals Emerald Limited (an Ireland company incorporated in February 2021), a wholly owned subsidiary requiring consolidation. Marinus Pharmaceuticals Emerald Limited serves as a corporate presence in the European Union for regulatory purposes. 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 U.S. (GAAP) for annual financial statements. In the opinion of management, these unaudited interim consolidated financial statements reflect all adjustments, consisting primarily of normal recurring accruals, necessary for a fair presentation of our financial position and results of operations and cash flows for the periods presented. The results of operations for interim periods are not necessarily indicative of the results for the full year. These unaudited interim consolidated financial statements should be read in conjunction with the audited financial statements for the year ended December 31, 2023 and accompanying notes thereto included in our Annual Report on Form 10-K filed with the SEC on March 5, 2024.

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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Product Revenue, net

We recognize ZTALMY revenue in accordance with ASC 606 – Revenue from contracts with customers. Our revenue recognition analysis consists of the following steps: (i) identification of the promised goods in the contract; (ii) determination of whether the promised goods are performance obligations, including whether they are capable of being distinct; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue as we satisfy each performance obligation.

Our first FDA approved product, ZTALMY, became available for commercial sale and shipment in the third quarter of 2022. We have three customers, one of which, Orsini Pharmaceutical Services, LLC (Orsini), a specialty pharmacy that dispenses ZTALMY directly to patients, represents approximately 99% of our ZTALMY revenue to date. Our contract with Orsini has a single performance obligation to deliver ZTALMY upon receipt of a purchase order, which is satisfied when Orsini receives ZTALMY. We recognize ZTALMY revenue at the point in time when control of ZTALMY is transferred to Orsini, which is upon delivery to Orsini. The transaction price that we recognize for

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ZTALMY revenue includes an estimate of variable consideration. Shipping and handling costs to Orsini are recorded as selling, general and administrative expenses. The components of variable consideration include:

Trade Discounts and Allowances. We provide contractual discounts, including incentive prompt payment discounts and chargebacks. Each of these potential discounts is recorded as a reduction of ZTALMY revenue and Accounts receivable in the period in which the related ZTALMY revenue is recognized. We estimate the amount of variable consideration for all discounts and allowances using the expected value method.

Product Returns and Recall. We provide for ZTALMY returns in accordance with our Return Good Policy. We estimate the amount of ZTALMY that may be returned using the expected value method, and we present this amount as a reduction of ZTALMY revenue in the period the related ZTALMY revenue is recognized. In the event of a recall, we will promptly notify Orsini and will reimburse Orsini for direct administrative expenses incurred in connection with the recall as well as the cost of replacement product.

Government Rebates. We are subject to discount obligations under state Medicaid programs, Medicare and the Tricare Retail Refund Program. We estimate reserves related to these discount programs and record these obligations in the same period the related revenue is recognized, resulting in a reduction of ZTALMY revenue.

Patient Assistance. We offer a voluntary co-pay patient assistance program intended to provide financial assistance to eligible patients with a prescription drug co-payment required by payors and coupon programs for cash payors. The calculation of the Current liability for this assistance is based on an estimate of claims and the cost per claim that we expect to receive associated with ZTALMY that has been recognized as Product revenue but remains in the distribution channel inventories at the end of each reporting period.

Federal Contract Revenue

We recognize Federal contract revenue from the BARDA Contract in the period in which the allowable research and development expenses are incurred, and receivables associated with this revenue are included within Accounts receivable, net on our interim consolidated balance sheets. This revenue is not within the scope of ASC 606 – Revenue from contracts with customers.

Short-term Investments

We classify our Short-term investments as available-for-sale securities, which include U.S. government agency debt securities and U.S. treasury debt securities with original maturities of greater than three months. These securities are carried at fair market value, with unrealized gains and losses reported in Other comprehensive loss and Accumulated other comprehensive income (loss) within stockholders' equity.equity (deficit). We did not have any investments as of June 30, 2024. All of our investments were short-term in nature as of March 31, 2024 June 30, 2023.

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Accounts Receivable, net

Net trade receivables related to ZTALMY sales, which are recorded in Accounts receivable, net on the consolidated balance sheets, were approximately $2.8 $2.9 million and $2.6 million as of March 31, 2024 June 30, 2024 and December 31, 2023, respectively. As of both March 31, 2024 June 30, 2024 and December 31, 2023, we had no allowance for doubtful accounts. An allowance for doubtful accounts is determined based on our assessment of the credit worthiness and financial condition of our customers, aging of receivables, as well as the general economic environment. Any allowance would reduce the net receivables to the amount that is expected to be collected. We have three customers, one of which, Orsini Pharmaceutical Services, LLC (Orsini), a specialty pharmacy that dispenses ZTALMY directly to patients, represents approximately 99% of our ZTALMY revenue to date. Payment terms for Orsini are 30 days from the shipment date.

Excluding net trade receivables, Accounts receivable, net represents amounts due to us under the BARDA contract for valid expenditures expected to be reimbursed to us under the terms of the BARDA contract and current amounts due to us from Orion Corporation (Orion) under the collaboration agreement (Note 12).

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Inventory

Inventories are recorded using actual costs and may consist of raw materials (ganaxolone API), work in process and finished goods. We began capitalizing Inventory related to ZTALMY subsequent to the March 2022 FDA approval of ZTALMY, as the related costs were expected to be recoverable through the commercialization and subsequent sale of ZTALMY. Prior to FDA approval of ZTALMY, costs estimated at approximately $2 million $2.0 million for commercially saleable product and materials were incurred and included in Research and development expenses. As a result, Cost of product revenues related to ZTALMY initially reflected a lower average per unit cost of materials and will continue continued to reflect a lower average per unit cost of materials through do so into the second quarter of 2024, as previously expensed inventory is was utilized for commercial production and sold to customers. We expect Cost of product revenues related to ZTALMY to begin to reflect the current on-going average per unit cost of materials for the remainder of 2024 and thereafter.

Debt Issuance Costs

Debt issuance costs incurred in connection with Note payable (Note 10) and Revenue interest financing payable (Note 11) are amortized to Interest expense over the term of the respective financing arrangement using the effective-interest method. Debt issuance costs, net of related amortization, are deducted from the carrying value of the related debt.

Contract Liabilities, net

When consideration is received, or such consideration is unconditionally due, from a customer prior to completing our performance obligation to the customer under the terms of a contract, a Contract liability is recorded. Contract liabilities expected to be recognized as revenue or a reduction of expense within the 12 months following the balance sheet date are classified as Current liabilities. Contract liabilities not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as Long-term liabilities. In accordance with ASC 210-20, our Contract liabilities were partially offset by our Contract assets at March 31, 2024 June 30, 2024, as further discussed in Note 12.

Liability Related to Revenue Interest Financing and Non-Cash Interest Expense

In October 2022, we recognized a liability related to the Revenue Interest Financing Agreement with Sagard Healthcare Royalty Partners, LP (Sagard) under ASC 470-10 *Debt* and ASC 835-30 *Interest - Imputation of Interest*. The initial funds received by us from Sagard pursuant to the terms of the Revenue Interest Financing Agreement were recorded as a liability and will be accreted under the effective interest method upon the estimated amount of future royalty payments to be made pursuant to the Revenue Interest Financing Agreement. The issuance costs were recorded as a direct deduction to the carrying amount of the liability and will be amortized under the effective interest method over the estimated period the liability will be repaid. We estimated the total amount of future product revenue to be

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generated over the life of the Revenue Interest Financing Agreement, and a significant increase or decrease in these estimates could materially impact the liability balance and the related Interest expense. If the timing or amounts of any estimated future revenue and related payments change, we will prospectively adjust the effective interest and the related amortization of the liability and related issuance costs. The liability related to the Revenue Interest Financing Agreement with Sagard is further discussed in Note 11.

Collaboration and Licensing Revenue

We may enter into collaboration and licensing arrangements for research and development, manufacturing, and commercialization activities with counterparties for the development and commercialization of our product candidates. These arrangements may contain multiple components, such as (i) licenses, (ii) research and development activities, and (iii) the manufacturing of certain material. Payments pursuant to these arrangements may

include non-refundable and refundable payments, payments upon the achievement of significant regulatory, development and commercial milestones, sales of product at certain agreed-upon amounts, and royalties on product sales. The amount of variable consideration is constrained until it is probable that the revenue is not at a significant risk of reversal in a future period.

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In determining the appropriate amount of revenue to be recognized as we fulfill our obligations under a collaboration agreement, we perform the following steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are capable of being distinct; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue as we satisfy each performance obligation.

We must develop estimates and assumptions that require judgment to determine the underlying stand-alone selling price for each performance obligation, which determines how the transaction price is allocated among the performance obligations. The estimation of the stand-alone selling price may include such estimates as forecasted revenues and costs, development timelines, discount rates and probabilities of regulatory and commercial success. We also apply significant judgment when evaluating whether contractual obligations represent distinct performance obligations, allocating transaction price to performance obligations within a contract, determining when performance obligations have been met, assessing the recognition and future reversal of variable consideration and determining and applying appropriate methods of measuring progress for performance obligations satisfied over time.

3. Cash, Cash Equivalents and Short-Term Investments

As of March 31, 2024 June 30, 2024, our Cash and cash equivalents included $1.4 million $1.0 million of cash accounts in banking institutions and $102.9 $63.7 million in money market funds. As of December 31, 2023, our Cash and cash equivalents included $1.3 million of cash accounts in banking institutions and $119.3 million in money market funds. Our Cash and cash equivalents are maintained in federally insured financial institutions in excess of the federally insured limit. Included in Other assets at March 31, 2024 and December 31, 2023 was $0.1 million and $0.2 million, respectively, $0.2 million of accrued interest receivable related to our Short-term investments. At June 30, 2024, we had no accrued interest receivable or Short-term investments.

The following table provides details regarding our portfolio of Short-term investments (in thousands) as of March 31, 2024 and December 31, 2023:

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
March 31, 2024				

U.S. Treasury securities	$ 9,000	$ 155	$ (155)	$ 9,000
Total	$ 9,000	$ 155	$ (155)	$ 9,000

December 31, 2023	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Va
U.S. Treasury securities	$ 26,852	$ 138	$ (155)	$26,8
U.S. Government Agency securities	2,884	31	(34)	2,8
Total	$ 29,736	$ 169	$ (189)	$29,7

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4. 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. As of March 31, 2024 June 30, 2024 and December 31, 2023, all of our financial assets and liabilities were classified as Level 1 or Level 2 valuations.

We estimate the fair values of our financial instruments categorized as Level 2 in the fair value hierarchy, including U.S. Treasury securities and U.S. Government Agency 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.

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	Level 1	Level 2	Level 3	Total
March 31, 2024								
June 30, 2024								
Assets								
Cash	$ 1,420	$ —	$ —	$ 1,420	$ 984	$ —	$ —	$ 984
Money market funds (cash equivalents)	102,833	—	—	102,833	63,692	—	—	63,692
U.S. Treasury securities	—	9,000	—	9,000	—	—	—	—
Total assets	$ 104,253	$ 9,000	$ —	$ 113,253	$ 64,676	$ —	$ —	$ 64,676
December 31, 2023								
Assets								
Cash	$ 1,255	$ —	$ —	$ 1,255	$ 1,255	$ —	$ —	$ 1,255
Money market funds (cash equivalents)	119,317	—	—	119,317	119,317	—	—	119,317
U.S. Treasury securities	—	26,835	—	26,835	—	26,835	—	26,835
Agency securities	—	2,881	—	2,881	—	2,881	—	2,881
Total assets	$ 120,572	$ 29,716	$ —	$ 150,288	$120,572	$29,716	$ —	$150,288

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5. Inventory

Inventories are stated at actual costs and consisted of the following (in thousands):

	March 31, 2024	December 31, 2023
Raw materials	$ 3,600	$ 436
Work in process	1,591	1,075
Finished goods	592	902
Total Inventories	$ 5,783	$ 2,413

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	June 30, 2024	December 31, 2023
Raw materials	$ 3,200	$ 436
Work in process	945	1,075
Finished goods	728	902
Total Inventories	$ 4,873	$ 2,413

6. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	March 31, 2024	December 31, 2023	June 30, 2024	December 31, 2023
Payroll and related costs	$ 2,830	$ 7,746	$ 3,783	$ 7,746
Clinical trials and drug development	5,694	4,701	3,764	4,701
Accrued license agreement payment	2,000	4,000	—	4,000
Professional fees	1,669	1,236	1,269	1,236
Selling and commercial liabilities	4,502	3,901	4,475	3,901
Restructuring costs			952	—
Short-term lease liabilities	1,306	774	778	774
Other	720	501	210	501
Total accrued expenses	$ 18,721	$ 22,859	$ 15,231	$ 22,859

7. Loss Per Share of Common Stock

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

The pre-funded warrants to purchase common stock issued in connection with the November 2022 offering are included in the calculation of Basic and diluted net loss per share as the exercise price of $0.001 per share is non-substantive and is virtually assured. The pre-funded warrants are more fully described in Note 8.

The following potentially dilutive securities have been excluded from the computation of diluted weighted-average shares of common stock outstanding, as they would be anti-dilutive:

	Three Months Ended March 31,		June, 30	
	2024	2023	2024	2023
Convertible preferred stock	—	860,000		
Restricted stock awards and restricted stock units	2,293,878	1,542,115	2,449,334	1,489,119
Stock options	8,671,041	7,143,397	8,859,400	7,220,150
	10,964,919	9,545,512	11,308,734	8,709,269

8. Stockholders' Equity

In 2005, we adopted the 2005 Stock Option and Incentive Plan (2005 Plan) that authorizes us to grant stock options, restricted stock and other equity-based awards. As of March 31, 2024, no options to purchase shares of common stock were outstanding pursuant to grants in connection with the 2005 Plan. No additional shares are available for issuance under the 2005 Plan. The amount, terms of grants, and exercisability provisions are determined and set by our board of directors.

Effective August 2014, we adopted our 2014 Equity Incentive Plan, as amended (2014 Plan), that authorizes us to grant stock options, restricted stock, and other equity-based awards, subject to adjustment in accordance with the 2014 Plan. As of March 31, 2024 June 30, 2024, 6,389,758 6,160,958 options to purchase shares of common stock were outstanding pursuant to grants

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in connection with the 2014 Plan, and 260,569 no shares of common stock were available for future issuance. The amount, terms of grants, and exercisability provisions are were determined and set by our board of directors. In accordance with the 2014 Plan, on January 1, 2024, the shares of common stock available for future grants under the 2014 Plan was increased to 3,090,220. No additional share are available for issuance under the 2014 Plan.

Effective May 2024, we adopted our 2024 Equity Incentive Plan (2024 Plan), that authorizes us to grant stock options, restricted stock, and other equity-based awards, up to a maximum of 4,000,000 awards, subject to adjustment in accordance with the 2024 Plan, plus shares that become available for grant as a result of the termination or forfeiture of awards under the 2014 Plan. As of June 30, 2024, 726,717 options to purchase shares of common stock were outstanding pursuant to grants in connection with the 2024 Plan, and 3,532,098 shares of common stock were available for future issuance.

Stock Options

There were 8,671,041 8,859,400 stock options outstanding as of March 31, 2024 June 30, 2024 at a weighted average exercise price of $9.69 $9.08 per share, including 2,281,283 1,971,725 stock options outstanding outside of the 2014 Plan, and 2024 Plans, granted as inducements to new employees. During the three six months ended March 31, 2024 June 30, 2024, 1,617,094 2,391,211 options were granted to employees and directors at a weighted average exercise price of $9.76 $7.20 per share. Of the options granted, 1,540,519 options were granted pursuant to the 2014 Plan, 726,717 options were granted pursuant to the 2024 Plan and 76,575 123,975 were granted outside of the 2014 Plan and 2024 Plans as inducements for new employees.

Restricted Stock and Restricted Stock Units

All issued and outstanding restricted shares of common stock units are time-based, and generally become vested within two three years of the grant date, pursuant to the 2014 Plan. and 2024 Plans. Compensation expense is recorded ratably over the requisite service period. Compensation expense related to restricted stock units is measured based on the fair value using the closing market price of our common stock on the date of the grant. As of March 31, 2024, we did not have any restricted shares of common stock outstanding.

During the three six months ended March 31, 2024 June 30, 2024, we granted 1,350,244 1,920,135 restricted stock units, which generally vest within three years of the grant date, pursuant to the 2014 Plan. and 2024 Plans. As of March 31, 2024 June 30, 2024, we had 2,293,878 2,449,334 restricted stock units outstanding.

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

	Three Months Ended March 31,		Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023	2024	2023
Research and development	$ 1,832	$ 1,342	$1,444	$1,468	$ 3,276	$2,810
Selling, general and administrative	3,361	2,399	3,143	2,423	6,504	4,822
Restructuring costs			330	—	330	—
Total	$ 5,193	$ 3,741	$4,917	$3,891	$10,110	$7,632

Preferred Stock

As of March 31, 2024 June 30, 2024 all shares of our Series A Convertible Preferred Stock (Preferred Stock) had been converted and none remained outstanding. In the three and six months ended March 31, 2023 June 30, 2024, there were no conversions of shares of our Preferred Stock into shares of our common stock. In the six months ended June 30, 2023, 4,300 shares of our Preferred stock remained outstanding, convertible were converted into 860,000 shares of our common stock. In the three months ended March 31, 2023 June 30, 2023, there were no conversions of shares of our Preferred Stock into shares of our common stock.

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Underwritten Public Offering

In connection with an underwritten public offering in November 2022 and the closing of the related exercise of the underwriters' option in December 2022, we issued a total of 12,421,053 shares of common stock and 2,105,264 pre-funded warrants (the Pre-funded Warrants) resulting in aggregate net proceeds, after underwriting discounts and

commissions in the public offering and fees, of $64.5 million. The exercise price and the number of shares of common stock issuable upon exercise of each Pre-funded Warrant are subject to appropriate adjustment in the event of certain stock dividends and distributions, stock splits, stock combinations, reclassifications or similar events affecting the common stock as well as upon any distribution of assets, including cash, stock or other property, to our stockholders. The Pre-funded Warrants are exercisable at any time, will not expire and are exercisable in cash or by means of a cashless exercise. A holder of Pre-funded Warrants may not exercise such Pre-funded Warrants if the holder, together

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with its affiliates, would beneficially own more than 9.99% of the number of shares of common stock outstanding immediately after giving effect to such exercise. A holder of Pre-funded Warrants may increase or decrease this percentage not in excess of 19.99% by providing at least 61 days' prior notice to us.

Sales Pursuant to Equity Distribution Agreement

On July 9, 2020, we entered into an Equity Distribution Agreement (EDA) with JMP Securities LLC (JMP), as amended by the March 31, 2023 Amendment No. 1 to the EDA (Amended EDA), to create an at the market equity program under which we from time to time may offer and sell shares of our common stock without a maximum aggregate offering price. The Amended EDA was entered into in connection with our filing of a Registration Statement on Form S-3 (File No. 333-271041) with the SEC (the 2023 Registration Statement), which includes a prospectus supplement covering the offering, issuance and sale by us of up to $75,000,000 of shares of common stock that may be issued and sold under the Amended EDA. Subject to the terms and conditions of the Amended EDA, JMP will be entitled to a commission of up to 3.0% of the gross proceeds from each sale of shares of our common stock. We did not sell any shares of our common stock during the three and six months ended March 31, 2024 June 30, 2024 and March 31, 2023 June 30, 2023 under the EDA. As of June 30, 2024, we had up to $48.6 million of shares of common stock that may be issued and sold under the Amended EDA

9. Commitments and Contingencies

Leases

We have entered into one operating lease for real estate and several operating leases for clinical site equipment. Our real estate operating lease has with a term of 78 months, and includes including renewal terms which that can extend the lease term by 60 months, which we include in the lease term when it is reasonably certain that we will exercise the option. As of March 31, 2024 June 30, 2024, our operating lease for real estate had a remaining lease term of 18 months. Our operating leases for clinical site equipment each have a term of 18 months and include renewal terms that can extend the lease terms monthly at the end of each applicable term. As of March

31, 2024, our operating leases for clinical site equipment had an average remaining term of 14 15 months. The right-of-use (ROU) assets are asset is included in Other assets on our interim consolidated balance sheets as of March 31, 2024 June 30, 2024 and December 31, 2023, and represent represents our right to use the underlying assets asset for the applicable lease terms. Our obligations to make lease payments are included in both Accrued expenses and Other long-term liabilities on our interim consolidated balance sheets as of March 31, 2024 June 30, 2024 and December 31, 2023. Additionally, we entered into several operating leases for clinical site equipment which were subsequently cancelled due to the discontinuation of the RAISE II trial. Our operating leases for clinical site equipment each had a term of 18 months prior to the May 2024 terminations of each lease. The ROU assets were included in Other assets on our consolidated balance sheets as of December 31, 2023, and represented our right to use the underlying assets for the applicable lease terms. The ROU assets related to the clinical site equipment operating leases were written off and included in restructuring costs for the six months ended June 30, 2023. Our obligations to make lease payments were included in both Accrued expenses and Other long-term liabilities on our consolidated balance sheets as of December 31, 2023. All ROU assets were initially measured at cost, which comprise 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 asset(s) 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.

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As of March 31, 2024 June 30, 2024 and December 31, 2023, ROU assets were $1.5 million $0.7 million and $1.0 million, respectively, and operating lease liabilities were $1.9 million $1.0 million and $1.4 million, respectively. We have entered into various short-term operating leases, primarily for clinical trial equipment, with an initial term of twelve months or less. These leases are not recorded on our balance sheets. All operating lease expense is recognized on a straight-line basis over the lease term. During the three six months ended March 31, 2024 June 30, 2024 and 2023, we recognized $1.3 million and $0.3 million, respectively, in total lease costs, of which $0.8 million recognized during the six months ended June 30, 2024 were restructuring costs resulting from the RAISE II trial equipment lease terminations as noted above. During the three months ended June 30, 2024 and June 30, 2023, we recognized $1.0 million and $0.1 million, respectively, in total lease costs, of which $0.8 million recognized during the three months ended June 30, 2024 were restructuring costs resulting from the RAISE II trial equipment lease terminations as noted above. In all periods, we recognized less than $0.1 million related to short-term operating leases for each period. 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 ROU assets and lease liabilities 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. The borrowing

rate used to determine the initial value of the ROU asset and lease liability related to our operating lease for clinical site equipment was approximately 7.0%.

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, 2024 June 30, 2024, we recognized $0.8 million of impairment losses for our ROU asset related to the RAISE II trial equipment leases. These costs were including in restructuring costs in our consolidated statements of operations and comprehensive loss for the three and six months ended June 30, 2024. As of December 31, 2023, we have had not recognized any impairment losses for our ROU assets.

Maturities of operating lease liabilities as of June 30, 2024 were as follows (in thousands):

Remainder of 2024	$	422
2025		641
		1,063
Less: imputed interest		(74)
Total lease liabilities	$	989
Current operating lease liabilities	$	778
Non-current operating lease liabilities		211
Total lease liabilities	$	989

Contingencies

On June 5, 2024, a securities class action lawsuit captioned Bishins v. Marinus Pharmaceuticals, Inc., et. al., Case 2:24-cv-02430, was filed against us and certain of our officers in the U.S. District Court for the Eastern District of Pennsylvania. The complaint alleges violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934, as amended (Exchange Act) and Rule 10b-5 promulgated thereunder on the basis of purportedly materially false and misleading statements and omissions concerning our RAISE and RAISE II clinical trials. The complaint seeks, among other things, unspecified damages, attorneys' fees, expert fees, and other costs. Motions to appoint lead plaintiffs and lead counsel for the action were due on August 5, 2024. One purported stockholder filed a motion by the August 5 deadline. That motion is currently pending, and we intend to move to dismiss the complaint once a schedule has been set. We intend to vigorously defend against this action.

We currently are not able to estimate the possible cost to us from this action, as the pending lawsuit is currently at an early stage, and we cannot be certain how long it may take to resolve the pending lawsuit or the possible amount of any damages that we may be required to pay.

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Maturities of operating lease liabilities as of March 31, 2024 were as follows (in thousands):

Remainder of 2024	$	1,073
2025		955
		2,028
Less: imputed interest		(140)
Total lease liabilities	$	1,888
Current operating lease liabilities	$	1,306
Non-current operating lease liabilities		582
Total lease liabilities	$	1,888

10. Notes Payable

On May 11, 2021 (Closing Date) and as amended on May 17, 2021, May 23, 2022, October 28, 2022 and October 28, 2022 June 6, 2024 (Credit Agreement), we entered into the Credit Agreement with Oaktree Fund Administration, LLC as administrative agent (Oaktree) and the lenders party thereto (collectively, the Lenders) that provided for a five-year senior secured term loan facility in an aggregate original principal amount of up to $125.0 million that was available to us in five tranches (collectively, the Term Loans).

Upon entering into the Credit Agreement in May 2021, we borrowed $15.0 million in term loans from the Lenders (Tranche A-1 Term Loans); upon receipt of written acceptance by the FDA of our NDA filing relating to the use of ganaxolone for CDD in September 2021, we borrowed $30.0 million of tranche A-2 term loans from the Lenders (Tranche A-2 Term Loans); and in March 2022, we borrowed $30.0 million in term loans from the Lenders that became available as a result of the approval by the FDA of ZTALMY oral suspension for the treatment of seizures associated with CDD in patients two years of age and older (Tranche B Term Loans). In May 2022, we entered into an amendment (the Credit Agreement Amendment) to extend the commitment date for the tranche C Term Loans (Tranche C Term Loans) commitment from June 30, 2023 to December 31, 2023, and to eliminate the commitment fees associated with the Tranche C Term Loans. Also in May 2022, we delivered to Oaktree a separate notice of

commitment termination with respect to the tranche D term loans (Tranche D Term Loans) commitment. In October 2022, we entered into an amendment to, among other things, allow for the consummation of the Revenue Interest Financing Agreement with Sagard and the transactions thereunder. In addition, the Credit Agreement Amendment increased the exit fee due by us upon any repayment, whether as a prepayment or a scheduled repayment, of the principal of the loans under the Credit Agreement from 2.00% to 2.67%. In August 2023, we delivered to Oaktree a separate notice of commitment termination with respect to the $25.0 million of Tranche C Term Loans commitment. Following In June 2024, we entered into an amendment to remove the termination minimum liquidity covenant therein and reduce the remaining quarterly principal payments due in 2024 thereunder by 50%. On the June 6, 2024 amendment effective date, we also made a one-time prepayment of $15.0 million of the Tranche C Term Loan Commitment, outstanding tranche B loans, together with payment of the accrued and unpaid interest thereon and applicable exit and prepayment fees. Additionally, during the six months ended June 30, 2024, we began paying the required quarterly principal payments. As of June 30, 2024, the loans under the Credit Agreement consist consisted of $75.0 million $58.1 million of previously drawn Term Loans with no additional funds available thereunder.

The Until the June 6, 2024 amendment, the Credit Agreement contains contained a minimum liquidity covenant that requires required us to maintain cash and cash equivalents of at least $15.0 million from the funding date of the Tranche B Term Loans until the maturity of the Term Loans.

The Term Loans will be guaranteed by certain of our future subsidiaries (Guarantors). Our obligations under the Credit Agreement are secured by a pledge of substantially all of our assets and will be secured by a pledge of substantially all of the assets of the Guarantors.

The Term Loans mature on May 11, 2026 (Maturity Date). The Term Loans bear interest at a fixed per annum rate (subject to increase during an event of default) of 11.50%, and we are required to make quarterly interest payments until the Maturity Date. We are also required to make quarterly principal payments beginning on June 30, 2024 in an amount equal to 2.5% of the aggregate amount of the previously drawn Term Loans, and continuing until 2025, at which time we are required to make quarterly principal payments in an amount equal to 5.0% of the aggregate amount of the previously drawn Term Loans outstanding on June 30, 2024, and continuing until the Maturity Date. On the Maturity Date, we are required to pay in full all outstanding Term Loans and other amounts owed under the Credit Agreement.

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At the time of borrowing any tranche of the Term Loans, we were required to pay an upfront fee of 2.0% of the aggregate principal amount borrowed at that time. In addition, a commitment fee of 75 basis points per annum began to accrue on each of the tranche B, C, and D commitments for the period beginning 120 days after the funding date of the Tranche A-2 Term Loans and continued until the applicable tranche was either funded or terminated, at which time the related commitment fees were due. The Tranche A-2 Term Loans were funded on September 27, 2021, and as such, we began accruing the commitment fees for tranche B, C, and D Term Loans 120 days later, on January 25,

2022. We drew down the additional $30.0 million of Tranche B Term Loans in March 2022, and paid less than $0.1 $0.1 million in

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commitment fees related to Tranche B Term Loans. The May 2022 amendment eliminated the commitment fees related to the Tranche C Term Loans, and separately, we terminated the Tranche D Term Loans in May 2022 and the Tranche C Term Loans in August 2023.

We may prepay all or any portion of the Term Loans, and are required to make mandatory prepayments of the Term Loans from the proceeds of asset sales, casualty and condemnation events, and prohibited debt issuances, subject to certain exceptions. All mandatory and voluntary prepayments of the Term Loans are subject to prepayment premiums equal to (i) 4% of the principal prepaid if prepayment occurs after May 11, 2023 but on or before May 11, 2024, or (ii) 2% of the principal prepaid if prepayment occurs after May 11, 2024 but on or before May 11, 2025. If prepayment occurs after May 11, 2025, no prepayment premium is due. The June 2024 prepayment of $15.0 million resulted in an additional $0.3 million of prepayment premiums that are included in interest expense for the six months ended June 30, 2024.

In addition, we are required to pay an exit fee in an amount equal to 2.67% of all principal repaid, whether as a mandatory prepayment, voluntary prepayment, or a scheduled repayment. Prior to the October 28, 2022 amendment to the Credit Agreement, the exit fee was 2.0%. The increase in the exit fee resulted in an additional $0.5 million of debt issuance costs that are classified as a contra-liability on the consolidated balance sheets and is being recognized as Interest expense over the term of the loan using the effective interest method.

In addition to the minimum liquidity covenant, we We are subject to a number of affirmative and restrictive covenants under the Credit Agreement, including limitations on our ability and our subsidiaries' abilities, among other things, to incur additional debt, grant or permit additional liens, make investments and acquisitions, merge or consolidate with others, dispose of assets, pay dividends and distributions, and enter into affiliate transactions, subject to certain exceptions. As of March 31, 2024 June 30, 2024, we were in compliance with all covenants.

Upon the occurrence of certain events, including but not limited to our failure to satisfy our payment obligations under the Credit Agreement, the breach of certain of our other covenants under the Credit Agreement, the occurrence of cross defaults to other indebtedness, or defaults related to enforcement action by the FDA or other Regulatory Authority or recall of ganaxolone, Oaktree and the Lenders will have the right, among other remedies, to accelerate all amounts outstanding under the Term Loans and declare all principal, interest, and outstanding fees immediately due and payable.

In March 2022, we borrowed $30.0 million upon the approval by the FDA of ZTALMY for CDD and incurred debt issuance costs of $1.8 million, including the exit fee of $0.6 million, that are classified as contra-liabilities on our consolidated balance sheets and are being recognized as Interest expense over the term of the loan using the effective interest method.

In September 2021, we borrowed $30.0 million upon receipt of written acceptance by the FDA of our NDA filing relating to the use of ganaxolone in the treatment of CDD and incurred debt issuance costs of $1.2 million, including the exit fee of $0.6 million, that are classified as contra-liabilities on our consolidated balance sheets and are being recognized as Interest expenses over the term of the loan using the effective-interest method.

In May 2021, we borrowed $15.0 million upon entering into the Credit Agreement and incurred debt issuance costs of $4.4 million, including the exit fee of $0.3 million, that are classified as a contra-liabilities on the consolidated balance sheet and are being recognized as Interest expenses over the term of the loan using the effective-interest method.

For the three six months ended March 31, 2024 June 30, 2024, we recognized interest expense of $2.7 million $5.3 million, of which $2.2 million $4.3 million was interest on the Term Loans and $0.5 million $1.0 million was non-cash interest expense related to the amortization of debt issuance costs.

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The following table summarizes the composition of Notes payable as reflected on the consolidated balance sheet as of March 31, 2024 June 30, 2024 (in thousands):

Gross proceeds	$ 75,000	$ 75,000
Contractual exit fee	2,003	2,003
Principal payment including associated exit fee		(17,326)
Unamortized debt discount and issuance costs	(3,530)	(3,052)
Total note payable	$ 73,473	$ 56,625
Current portion of note payable	15,401	11,550
Non-current portion of note payable	58,072	45,075
Total note payable	$ 73,473	$ 56,625

The aggregate maturities of Notes payable as of March 31, 2024 June 30, 2024 are as follows (in thousands):

Remainder of 2024	$ 11,250	$ 3,750
2025	15,000	15,000
2026	48,750	39,375
Total	$ 75,000	$58,125

11. Revenue Interest Financing Agreement

On October 28, 2022 (Closing Date), we entered into a revenue interest financing agreement (Revenue Interest Financing Agreement) with Sagard Healthcare Royalty Partners, LP (Sagard) pursuant to which we received $32.5 million (Investment Amount) to provide funding for our development and commercialization of ganaxolone and related pharmaceutical products, including the commercial launch of ZTALMY, and for working capital and general administrative purposes. On June 6, 2024, we entered into the first amendment to the Revenue Interest Financing Agreement (the Amendment) with Sagard, to, among other things, remove the minimum liquidity covenant therein so long as the obligations under the Oaktree Credit Agreement are outstanding.

In exchange for the Investment Amount, we have agreed to make quarterly payments to Sagard (Payments) as follows: (i) for each calendar quarter from and after the Closing Date through and including the quarter ended June 30, 2026, an amount equal to 7.5% of (a) our U.S. net sales of ZTALMY and all other pharmaceutical products that contain ganaxolone (Net Sales), in each case with any dosage form, dosing regimen, or strength, or any improvements related thereto (collectively, Included Products) and (b) certain other payments received by us in connection with the manufacture, development and sale of the Included Products in the U.S. (Other Included Payments, and, together with Net Sales, Product Revenue); and (ii) for each calendar quarter following the calendar quarter ended June 30, 2026, an amount equal to (x) 15.0% of the first $100 million in annual Product Revenue of the Included Products and (y) 7.5% of annual Product Revenue of the Included Products in excess of $100 million.

The Payments are subject to a hard cap equal to 190% of the Investment Amount (Hard Cap) or $61.8 million. Sagard's right to receive payments will terminate when Sagard has received payments in respect of the Included Products, including any additional payments described below, equal to the Hard Cap. Further, we have the right to make voluntary prepayments to Sagard, and such payments will be credited against the Hard Cap.

If Sagard has not received aggregate payments equaling at least 100% of the Investment Amount by December 31, 2027 or at least 190% of the Investment Amount by December 31, 2032 (each, a Minimum Amount), then we will be obligated to make a cash payment to Sagard in an amount sufficient to gross up Sagard up to the applicable Minimum Amount within a specified period of time after each reference date.

The obligations under the Revenue Interest Financing Agreement, including the Payments, will be guaranteed by certain of our future subsidiaries that are required to become a party thereto as guarantors (Guarantors). Our obligations under the Revenue Interest Financing Agreement and the guarantee of such obligations are secured, subject to customary

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permitted liens and other agreed upon exceptions and subject to an intercreditor agreement with Oaktree as administrative agent for the lenders under our credit agreement (as described below, the Credit Agreement), by a pledge of substantially all of our and the Guarantors' assets that relate to, or are used or held for use for, the development, manufacture, use and/or

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commercialization of ZTALMY and all other pharmaceutical products that contain ganaxolone in the U.S., including the Product Revenue, pursuant to the terms of the Security Agreement dated as of the Closing Date by and among us, the Guarantors from time to time party thereto, and Sagard (Security Agreement).

At any time, we have the right, but not the obligation (Call Option), to repurchase all, but not less than all, of Sagard's interest in the Payments at a repurchase price (Put/Call Price) equal to: (a) on or before the third anniversary of the Closing Date, 160% of the Investment Amount; (b) after the third anniversary but on or prior to the fourth anniversary of the Closing Date, 180% of the Investment Amount; and (c) after the fourth anniversary of the Closing Date, 190% of the Investment Amount, in each case, less the aggregate of all of our payments in respect of the Payments made to Sagard prior to such date.

The Revenue Interest Financing Agreement contains certain restrictions on our and our subsidiaries' abilities, among other things, to incur additional debt, grant or permit additional liens, make investments and acquisitions, dispose of assets, pay dividends and distributions and enter into affiliate transactions, in each case, subject to certain exceptions. In addition, the Revenue Interest Financing Agreement contains a financial covenant that requires us, after the repayment of the loans under the Credit Agreement with Oaktree, to maintain at all times cash and cash equivalents in certain deposit accounts in an amount at least equal to (i) from the Closing Date until the repayment of the loans under the Credit Agreement, $15.0 million and (ii) thereafter, $10.0 million.

In connection with the Revenue Interest Financing Agreement, on the Closing Date, we entered into the Credit Agreement Amendment with Oaktree which is fully described in Note 10.

Issuance costs pursuant to the Revenue Interest Financing Agreement consisted primarily of advisory and legal fees and totaled $2.6 million. $2.6 million. In June 2024, an additional $0.1 million of issuance costs was incurred related to the Amendment. These issuance costs were recorded as a direct deduction to the carrying amount of the liability and will be amortized under the effective interest method over the estimated period the liability will be repaid. For the three six months ended March 31, 2024 June 30, 2024, we we estimated an effective annual interest rate of approximately 18% 17%. Over the course of the Revenue Interest Financing Agreement, the actual interest rate will be affected by the amount and timing of net ZTALMY revenue recognized and changes in the timing of forecasted net ZTALMY revenue. On a quarterly basis, we reassess the expected timing of the net ZTALMY revenue, recalculate the amortization and effective interest rate and adjust the accounting prospectively as needed.

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The following table summarizes the activity of the Revenue Interest Financing Agreement for the three six months ended March 31, 2023 June 30, 2023 and March 31, 2024 June 30, 2024 (in thousands):

For the three months ended March 31, 2023		
Revenue Interest Financing Balance at December 31, 2022	$	30,877
Non-cash interest expense in the three months ended March 31, 2023		1,440
Amortization of debt discount in the three months ended March 31, 2023		71
Payments made in the three months ended March 31, 2023		(174)
Revenue Interest Financing Balance at March 31, 2023	$	32,214
Current portion of revenue interest financing liability		1,321
Long-term portion of revenue interest financing liability		30,893
Revenue Interest Financing Balance at March 31, 2023	$	32,214
For the three months ended March 31, 2024		
Revenue Interest Financing Balance at December 31, 2023	$	35,977
Non-cash interest expense in the three months ended March 31, 2024		1,556
Amortization of debt discount in the three months ended March 31, 2024		111
Payments made in the three months ended March 31, 2024		(491)
Revenue Interest Financing Balance at March 31, 2024	$	37,153
Current portion of revenue interest financing liability	$	2,511
Long-term portion of revenue interest financing liability		34,642
Revenue Interest Financing Balance at March 31, 2024	$	37,153

For the six months ended June 30, 2023		
Revenue Interest Financing Balance at December 31, 2022	$	30,877
Non-cash interest expense in the six months ended June 30, 2023		2,916
Amortization of debt discount in the six months ended June 30, 2023		134
Payments made in the six months ended June 30, 2023		(421)
Revenue Interest Financing Balance at June 30, 2023	$	33,506
Current portion of revenue interest financing liability		1,556
Long-term portion of revenue interest financing liability		31,950
Revenue Interest Financing Balance at June 30, 2023	$	33,506

For the six months ended June 30, 2024		
Revenue Interest Financing Balance at December 31, 2023	$	35,977
Non-cash interest expense in the six months ended June 30, 2024		3,156
Amortization of debt discount in the six months ended June 30, 2024		197
Payments made in the six months ended June 30, 2024		(1,050)
Revenue Interest Financing Balance at June 30, 2024	$	38,280
Current portion of revenue interest financing liability	$	2,849
Long-term portion of revenue interest financing liability		35,431
Revenue Interest Financing Balance at June 30, 2024	$	38,280

12. Collaboration Revenue

Orion Collaboration Agreement

In July 2021, we entered into a collaboration agreement (Orion Collaboration Agreement) with Orion. The Orion Collaboration Agreement falls under the scope of ASC Topic 808, Collaborative Arrangements (ASC 808) as both parties are active participants in the arrangement that are exposed to significant risks and rewards. While this arrangement is in the scope of ASC 808, we analogize to ASC 606 for some aspects of this arrangement, including for the delivery of a good or service (i.e., a unit of account). Revenue recognized by analogizing to ASC 606 is recorded as collaboration revenue on the consolidated statements of operations.

Under the terms of the Orion Collaboration Agreement, we granted Orion an exclusive, royalty-bearing, sublicensable license to certain of our intellectual property rights with respect to commercializing biopharmaceutical products incorporating our product candidate ganaxolone (Licensed Products) in the European Economic Area, the United Kingdom and Switzerland (collectively, the Territory) for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions (Field), initially in the indications of CDD, TSC and RSE. We will be responsible for the continued development of Licensed Products and regulatory interactions related thereto, including conducting and sponsoring all clinical trials, provided that Orion may conduct certain post-approval studies in the Territory. Orion will be responsible, at Orion's sole cost and expense, for the commercialization of any Licensed Product in the Field in the Territory.

Under the terms of the Orion Collaboration Agreement, we received a €25.0 million ($29.6 million) upfront payment from Orion in July 2021. In connection with the upfront fee, we agreed to provide Orion with the results of a planned genotoxicity study on the M2 metabolite of ganaxolone, a "Combined Micronucleus & Comet study in vivo." In May 2022, the final study report was received, which confirmed that no genotoxicity was found, as measured by formation of micronuclei in the bone marrow or comet morphology in the liver. In the event that the results of such study were positive, based on the criteria set forth in the study's protocol, Orion would have had the right to terminate

we would have been required to refund Orion seventy-five percent (75%) of the upfront fee. We are eligible to receive up to an additional €97 million in R&D reimbursement and cash milestone payments based on specific clinical and commercial achievements, as well as tiered royalty payments based on net sales ranging from the low double-digits to high teens for the oral programs and the low double-digits to low 20s for the IV program. Also, as part of the overall arrangement, we have agreed to supply the Licensed Products to Orion at an agreed upon price.

The Orion Collaboration Agreement shall remain effective until the date of expiration of the last to expire Royalty Term, which is defined as the period beginning on the date of the first commercial sale Licensed Product in such country and ending on the latest to occur of (a) the tenth (10th) anniversary of the first commercial sale of Licensed Product in such country, (b) the expiration of the last-to-expire licensed patent covering the manufacture, use or sale of such Licensed Product in such country, and (c) the expiration of regulatory exclusivity period, if any, for such Licensed Product in such country. The Orion Collaboration Agreement has a term of at least ten (10) years since a commercial sale has yet to occur. The Orion Collaboration Agreement allows for termination in certain specific events, such as material breach, in the event Orion challenges the validity, enforceability or scope of the licensed patent rights, termination for forecast failure, insolvency and force majeure, none of which are probable at contract inception.

In accordance with the guidance, we identified the following commitments under the arrangement: (i) exclusive rights to develop, use, sell, have sold, offer for sale and import any product comprised of Licensed Product (License) (ii) development and regulatory activities (Development and Regulatory Activities), and (iii) requirement to supply Orion with the Licensed Product at an agreed upon price (Supply of Licensed Product). We determined that these three commitments represent distinct performance obligations for purposes of recognizing revenue or reducing expense, which we will recognize such revenue or expense, as applicable, as we fulfill these performance obligations.

At contract inception, we determined that the non-refundable portion of the upfront payment plus the research and development reimbursement constitutes the transaction price as of the outset of the Orion Collaboration Agreement. The refundable portion of the upfront payment and the future potential regulatory and development milestone payments were fully constrained at contract inception as the risk of significant revenue reversal related to these amounts had not yet been resolved. During 2022, the refundable portion of the upfront payment was determined to be included in the transaction price as the final genotoxicity study on the M2 metabolite of ganaxolone was received as described above and the remaining $12.7 million of the upfront payment was recorded as collaboration revenue in the year ended December 31, 2022. The achievement of the future potential milestones is not within our control and is subject to certain research and development success and therefore carry significant uncertainty. As a result of the July 2023 EC approval of ZTALMY oral suspension for the adjunctive treatment of epileptic seizures associated with CDD in patients two to 17 years of age, we are now eligible under the Orion Collaboration Agreement to receive a commercial milestone payment of €10 million, if commercial sales of ZTALMY commence in the Territory, due upon the

earlier of (1) the first commercial sale of ZTALMY within two of a select set of countries consisting of Germany, France, Italy, Spain, and the United Kingdom and (2) the 18-month anniversary of the first commercial sale of ZTALMY in the Territory. We will reevaluate the likelihood of achieving these milestones at the end of each reporting period and adjust the transaction price in the period the risk is resolved. In addition, we will recognize any consideration related to sales-based milestones and royalties when the subsequent sales occur since those payments relate primarily to the License, which was delivered by us to Orion upon entering into the Orion Collaboration Agreement.

The transaction price was allocated to the three performance obligations based on the estimated stand-alone selling prices at contract inception. The stand-alone selling price of the License was based on a discounted cash flow approach and considered several factors including, but not limited to, discount rate, development timeline, regulatory risks, estimated market demand and future revenue potential using an adjusted market approach. The stand-alone selling price of the Development and Regulatory Activities and the Supply of Licensed Product was estimated using the expected cost-plus margin approach.

As of December 31, 2023, there was a total contract liability of $13.7 million and a total contract asset of $2.9 million. In accordance with ASC 210-20, the contract liability was offset by the contract asset related to the reimbursement of research and development costs, which resulted in a net contract liability of $10.8 million as of December 31, 2023.

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Transaction Price and Net Contract Liability as of December 31, 2023:

	Transaction Price	Cumulative Collaboration Revenue Recognized as of December 31, 2023	Contract Liability
License	$ 21,660	$ 21,660	$ -
Development and Regulatory Services	6,717	2,511	4,206
Supply of Licensed Product	9,503	-	9,503
	$ 37,880	$ 24,171	$ 13,709
Less Total Contract Asset			2,912
Net Contract Liability			$ 10,797

During the three six months ended March 31, 2024 June 30, 2024, we amortized $0.3 million $0.7 million of the transaction price associated with the Development and Regulatory Services as a reduction of research and development costs. These reductions to the transaction price resulted in a total contract liability of $13.4 million $13.0 million as of March 31, 2024 June 30, 2024. In accordance with ASC 210-20, the contract liability of $13.4 million $13.0

million is offset by the contract asset of $2.4 million $1.8 million related to the reimbursement of research and development costs, resulting in a net contract liability of $11.0 $11.1 million as of March 31, 2024 June 30, 2024.

Transaction Price and Net Contract Liability as of March 31, 2024 June 30, 2024:

	Transaction Price	Cumulative Collaboration Revenue Recognized as of March, 2024	Contract Liability	Transaction Price	Cumulative Collaboration Revenue Recognized as of June 30, 2024	Contract Liability
License	$ 21,660	$ 21,660	$ -	$ 21,660	$ 21,660	$ -
Development and Regulatory Services	6,717	2,848	3,869	6,717	3,252	3,465
Supply of Licensed Product	9,503	-	9,503	9,503	-	9,503
	$ 37,880	$ 24,508	$ 13,372	$ 37,880	$ 24,912	$12,968
Less Total Contract Asset			2,372			1,836
Net Contract Liability			$ 11,000			$11,132

We incurred $2.0 million of incremental costs in connection with obtaining the Orion Collaboration Agreement. These contract acquisition costs were allocated consistent with the transaction price, resulting in $1.1 million of expense recorded to selling, general and administrative expense commensurate with the recognition of the License performance obligation and $0.9 million recorded as capitalized contract costs, included in other current assets and other assets, which are being amortized as Development and Regulatory Services and Supply of Licensed Product obligations are met.

Tenacia Collaboration Agreement

On November 16, 2022 (Effective Date), we entered into a Collaboration and Supply Agreement (Tenacia Collaboration Agreement) with Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia). The Tenacia Collaboration Agreement falls under the scope of ASC Topic 808, Collaborative Arrangements (ASC 808) as both parties are active participants in the arrangement that are exposed to significant risks and rewards. While this arrangement is in the scope of ASC 808, we analogize to ASC 606 for some aspects of this arrangement, including for the delivery of a good or service (i.e., a unit of account). Revenue recognized by analogizing to ASC 606 is recorded as collaboration revenue on the consolidated statements of operations.

Under the terms of the Tenacia Collaboration Agreement, we granted Tenacia an exclusive, royalty-bearing, sublicensable license to certain of our intellectual property rights to develop, commercialize and otherwise exploit certain products incorporating certain oral and intravenous formulations of our product candidate ganaxolone (Licensed Products) in Mainland China, Hong Kong, Macau and Taiwan (collectively, Territory) for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions (Field), initially for the treatment of cyclin-dependent

kinase-like 5 deficiency disorder, tuberous sclerosis complex and SE (including refractory and established SE) (collectively, the Initial Indications). The collaboration can be expanded to include additional indications and formulations of ganaxolone pursuant to a right of first negotiation.

Under the terms of the Tenacia Collaboration Agreement, Tenacia agreed to pay us an upfront cash payment of $10 million (the Upfront Fee) within forty-five (45) days after the Effective Date, which was paid in December 2022. In addition to the Upfront Fee, Tenacia has agreed to make cash payments to us upon the achievement of certain development, regulatory and sales-based milestones related to (i) the Initial Indications and (ii) the first new formulation or pro-drug of ganaxolone or any back-up compound of ganaxolone in a new indication (Selected Product) for which the parties amend the Tenacia Collaboration Agreement in connection with Tenacia's exercise of its right of first negotiation and for which there is no other Licensed Product approved in China (for clarity, the milestone payments under this clause (ii) will only apply to one Selected Product), up to an aggregate amount of $256 million. Of the milestones, $15 million relates to regulatory approvals with separate milestones related to each of oral and intravenous formulations and the Selected Product, and an aggregate of $241 million of sales-based milestones are connected to annual revenue thresholds specific to each of the oral, intravenous and Selected Product formulations of ganaxolone. Tenacia has further agreed to pay us tiered royalty payments based on annual net sales of Licensed Products ranging from the low double digits to the mid-teens for each of the oral formulation, intravenous formulation and Selected Product formulation of Licensed Products. Tenacia's obligations to pay royalties to us with respect to sales of a Licensed Product in each particular jurisdiction of the Territory will commence on the date of first commercial sale in such jurisdiction and expire upon the latest of (i) ten years following the first commercial sale of such Licensed Product in such jurisdiction, (ii) the expiration of the last-to-expire valid claim of any licensed patent rights that covers such Licensed Product in such jurisdiction and (iii) the expiration of all regulatory exclusivities for such Licensed Product in such jurisdiction. Royalty payments are subject to reduction in specified circumstances as set forth in the Tenacia Collaboration Agreement, including if net sales decrease by a certain percentage after the introduction of a generic product.

Tenacia will be primarily responsible for the development of Licensed Products in the Territory and regulatory interactions related thereto, including conducting and sponsoring clinical studies in the Field in the Territory to support regulatory filings in the Territory. All regulatory approvals filed by Tenacia in the Territory will be in the name of and owned by us unless otherwise required by applicable law, in which case such regulatory approvals would be in the name of and owned by Tenacia for the benefit of us. We and Tenacia have agreed to enter into clinical and commercial supply agreements pursuant to which we will supply Tenacia with its requirements of Licensed Products necessary for Tenacia to develop and commercialize Licensed Products in the Field in the Territory. The parties entered into the clinical and commercial supply agreement in May 2023. The agreement contains pricing, delivery, acceptance, payment, termination, forecasting, and other terms consistent with the Tenacia Collaboration Agreement, as well as certain quality assurance, indemnification, liability and other standard industry terms. Tenacia will be responsible for, at Tenacia's sole cost and expense, obtaining regulatory approval and commercializing the Licensed Product in the Field in Mainland China. Tenacia is enrolling enrolled patients in our Phase 3 randomized, double blind, placebo-controlled trial (TrustTSC trial) of adjunctive ganaxolone.

The term of the Tenacia Collaboration Agreement extends for so long as royalties are payable anywhere in the Territory. Subject to the terms of the Tenacia Collaboration Agreement, (i) for a specified period of time after the Effective Date, Tenacia may terminate the Tenacia Collaboration Agreement in its entirety for any or no reason upon written notice to us, and (ii) either party may terminate the Tenacia Collaboration Agreement for the other party's material breach following a cure period or insolvency.

In accordance with the guidance, we identified the following commitments under the arrangement: (i) grant to Tenacia the exclusive rights to develop, commercialize and otherwise exploit Licensed Product in the Field in the Territory (License) and (ii) requirement to supply Tenacia with the Licensed Product at an agreed upon price (Supply of Licensed Product). We determined that these two commitments represent distinct performance obligations for purposes of recognizing revenue or reducing expense, which it will recognize such revenue or expense, as applicable, as it fulfills these performance obligations.

The transaction price was allocated to the two performance obligations based on the estimated stand-alone selling prices at contract inception. The stand-alone selling price of the License was based on a discounted cash flow

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approach and considered several factors including, but not limited to, discount rate, development timeline, regulatory risks, estimated market demand and future revenue potential using an adjusted market approach. The stand-alone selling price of the Supply of Licensed Product was estimated using the expected cost-plus margin approach.

There was no activity during each of the three and six months ended March 31, 2024 June 30, 2024 and 2023. The cumulative collaboration revenue recognized as of March 31, 2024 June 30, 2024 and December 31, 2023 is $3.0 million, which was the $3.0 million transaction price associated with the License as revenue at contract inception. No license revenue was recorded during each of the three and six months ended March 31, 2024 June 30, 2024 and 2023. There was a total contract liability of $7.0 million as of both March 31, 2024 June 30, 2024 and December 31, 2023. In accordance with ASC 210-20, the contract liability of $7.0 million is offset by a contract asset of $0.7 million, resulting in a net contract liability of $6.3 million as of both March 31, 2024 June 30, 2024 and December 31, 2023.

Transaction Price and Net Contract Liability as of March 31, 2024 June 30, 2024 and December 31, 2023:

	Cumulative Collaboration			Cumulative Collaboration		
	Transaction Price	Revenue Recognized as of March 31, 2024 and December 31, 2023	Contract Liability	Transaction Price	Revenue Recognized as of June 30, 2024 and December 31, 2023	Contract Liability
License	$ 2,998	$ 2,998	$ -	$ 2,998	$ 2,998	$ -
Supply of Licensed Product	7,002	-	7,002	7,002	-	7,002

	$ 10,000	$ 2,998	$ 7,002	$ 10,000	$ 2,998	$ 7,002
Less Total Contract Asset			700			700
Net Contract Liability			$ 6,302			$ 6,302

We incurred $1.0 million of incremental costs in obtaining the Tenacia Collaboration Agreement. These contract acquisition costs were allocated consistent with the transaction price, resulting in $0.1 million of expense recorded to Selling, general and administrative expense and $0.2 million recorded to Cost of collaboration revenue, commensurate with the recognition of the License performance obligation, and $0.7 million recorded as capitalized contract costs, which will be amortized as Supply of License Product obligations are met.

Biologix Distribution and Supply Agreement

In May 2023, we entered into an exclusive distribution and supply agreement (Biologix Agreement) with Biologix FZCo (Biologix), whereby Biologix has the right to distribute and sell ganaxolone in Algeria, Bahrain, Egypt, Iraq, Jordan, Kingdom of Saudi Arabia, Kuwait, Lebanon, Libya, Morocco, Oman, Qatar, Tunisia and United Arab Emirates. In exchange for distribution rights, we will be the exclusive supplier of our products to Biologix on terms set forth in the respective agreements in exchange for a negotiated purchase price for the products. Upon execution of the Biologix Agreement, we received an upfront payment of $0.5 million which is to be recognized over the term of the Biologix Agreement. We may be entitled to additional fees upon regulatory milestones. In the three and six months ended March 31, 2024 June 30, 2024 and June 30, 2023, we recorded less than $0.1 million of Collaboration revenue related to the Biologix Agreement. There was a total Contract liability of $0.4 million and $0.5 million at March 31, 2024. As the Biologix Agreement was entered into in May 2023, there was no contract liability at March 31, 2023. June 30, 2024 and June 30, 2023, respectively.

13. Subsequent Events Restructuring Costs.

On April 15, 2024, we announced that the independent Data Monitoring Committee (DMC) completed its review of the interim analysis of the RAISE trial. The trial did not meet the pre-defined interim analysis stoppingcriteria on the co-primary endpoints, and the DMC recommendation was that the RAISE trial may continue without

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modification. We have decided to complete enrollment in the RAISE trial at 100 patients with top-line results expected in the summer of 2024.

Cost reduction activities are being implemented with expected impact beginning in the second quarter of 2024. coprimary endpoints. On April 30, 2024, we implemented a reduction-in-force (RIF) which impacted approximately 20% of our workforce. workforce, and implemented additional cost reduction activities with impact beginning in the second quarter of 2024. We incurred approximately $2.0 million of restructuring costs in the six months ending June 30, 2024, which primarily consisted of severance payments, employee benefits and related costs, as well as noncash stock compensation expense and contract termination costs related to clinical site equipment no longer being utilized. At June

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30, 2024, we had unpaid restructuring costs totaling $1.0 million which are expected to be fully paid in the third quarter of 2024 and are included in accrued expenses at June 30, 2024 (see Note 6).

The following table summarizes the restructuring balances at June 30, 2024 (in thousands):

Restructuring costs payable at December 31, 2023	$	—
Restructuring costs incurred the six months ended June 30, 2024:		
Personnel costs, including severance and related costs		1,193
Clinical equipment contract termination costs		759
Restructuring costs paid in the six months ended June 30, 2024		(670)
Less noncash stock-based compensation expense related to RIF		(330)
Total Restructuring costs payable at June 30, 2024	$	952

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," "may," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," "will," or "would," and or the negative of these terms, or other comparable terminology intended to identify statements about the future. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement

contained in this 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 plans to continue to successfully commercialize ganaxolone in Cyclin-dependent Kinase-like 5 (CDKL5) Deficiency Disorder (CDD) in the U.S.;
- our expectations about the results from development of ganaxolone for Refractory Status Epilepticus (RSE) after the Phase 3 RAISE trial and the top-line data from such trial; assessments;
- our expectations that our cost reduction activities, being which were implemented with expected impact beginning in the second quarter of 2024, will be sufficient to fund our operating expenses and capital expenditure requirements as well as maintain the minimum cash balance required under our debt facility, into the first second quarter of 2025;
- our plans to meet our post-approval commitments to the U.S. Food and Drug Administration (FDA) and the European Commission (EC) for ganaxolone;
- our expectations regarding the commercialization of ganaxolone in the European Union (EU), including the timing thereof;
- the potential benefits of ganaxolone in indications other than CDD, and our ability to develop ganaxolone for additional indications, including Refractory Status Epilepticus (RSE), RSE, Tuberous Sclerosis Complex (TSC) and Lennox Gastaut Syndrome (LGS);

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- the status, timing and results of preclinical studies and clinical trials;
- the design of and enrollment in clinical trials, availability of data from ongoing clinical trials, expectations for regulatory approvals and the attainment of clinical trial results that will be supportive of regulatory approvals;
- the timing of seeking marketing approval of ganaxolone in specific additional indications;

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- our ability to maintain marketing approval for ganaxolone for CDD and obtain regulatory approval for ganaxolone in other indications;
- the possibility that we expand the targeted indication footprint and explore new potential formulations of ganaxolone;
- our estimates of expenses and future revenue and profitability;
- our estimates regarding our capital requirements and our needs for additional financing;
- our estimates of the size of the potential markets for ganaxolone;
- our expectations regarding our collaborations with Orion Corporation (Orion), Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia) and Biologix FZCo (Biologix), including the expected amounts and timings of milestone, royalty and other payments, including research and development reimbursement, if applicable, pursuant thereto;
- our ability to attract collaborators with acceptable development, regulatory and commercial expertise;
- the benefits and contractual requirements 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 expected future sales of ganaxolone for CDD, revenue contributions from our contract (BARDA Contract) with the Biomedical Advanced Research and Development Authority (BARDA), corporate collaborations, license agreements, and other collaborative efforts for the development and commercialization of ganaxolone for CDD and in other indications being developed for ganaxolone;
- our ability to create and maintain an effective sales and marketing infrastructure where we elect to market and sell ganaxolone directly;
- the pricing and the timing and amount of reimbursement for ganaxolone;
- the success of other competing therapies that may become available;
- the manufacturing capacity and supply for ganaxolone;
- the possibility that third parties, such as Ovid Therapeutics, Inc. (Ovid), 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;
- the outcome of our post-grant review of one of Ovid's patents and Ovid's Inter Partes Review challenge of one of our patents;

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- the possibility that we expand and diversify our product pipeline through acquisitions of additional drug candidates that fit our business strategy;

- our ability to maintain and protect our intellectual property rights;
- our results of operations, financial condition, liquidity, prospects, and growth strategies;
- our ability to, among other actions, secure additional financing or strategic transactions and continue as a going concern;

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- the enforceability of the exclusive forum provisions in our fourth amended and restated certificate of incorporation; and
- the industry in which we operate and trends which may affect the industry or us.

You should refer to Part II Item 1A. *Risk Factors* of this Quarterly Report on Form 10-Q and Part I Item 1A. *Risk Factors* of our Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 5, 2024 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, 2023, which are included in our Annual Report on Form 10-K filed with the SEC on March 5, 2024.

Overview

We are a commercial-stage pharmaceutical company dedicated to the development of innovative therapeutics for the treatment of seizure disorders, including rare genetic epilepsies and status epilepticus (SE). On March 18, 2022, the U.S. Food and Drug Administration (FDA) FDA approved our new drug application (NDA) for the use of ZTALMY® (ganaxolone) oral suspension CV for the treatment of seizures associated with Cyclin-dependent Kinase-like 5 (CDKL5) Deficiency Disorder (CDD) CDD in patients two years of age and older. ZTALMY, our first FDA

approved product, became available for commercial sale and shipment in the third quarter of 2022. On July 28**July 28, 2023,** 2023, the European Commission (EC) EC granted marketing authorization for ZTALMY for the adjunctive treatment of epileptic seizures associated with CDD in patients two to 17 years of age. ZTALMY may be continued in patients 18 years of age and older. We have an exclusive collaboration agreement with Orion Corporation (Orion) for European commercialization of ganaxolone for ZTALMY. Orion is preparing continues to prepare for commercial launches of ZTALMY in select European countries in the second half of 2024. On July 18, 2024, we announced that the China National Medical Products Administration (CNMPA) has approved ganaxolone oral suspension for the treatment of epileptic seizures in patients two years of age and older with CDD. We have a collaboration agreement with Tenacia for the commercialization of ganaxolone in Mainland China, Hong Kong, Macau and Taiwan.

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We are also developing ganaxolone for the treatment of other rare genetic epilepsies, including Tuberous Sclerosis Complex (TSC), TSC. Top-line results from our Phase 3 TSC (TrustTSC) clinical trial are expected in the first half of the fourth quarter of 2024. TSC is a rare, multisystem genetic disorder caused by inherited mutations in the TSC1 gene or TSC2 gene. TSC is often characterized by non-cancerous tumors, skin abnormalities, and severe neurological manifestations including refractory seizures and neurodevelopmental delays. TSC is a leading cause of genetic epilepsy.

We recently announced top-line results from our RAISE trial of intravenous (IV) ganaxolone for the treatment of Refractory Status Epilepticus (RSE). RSE. SE is a life-threatening condition characterized by continuous, prolonged seizures or rapidly recurring seizures without intervening recovery of consciousness. If SE is not treated urgently, permanent neuronal damage may occur, which contributes to high rates of morbidity and mortality. Patients with SE who do not respond to first-line benzodiazepine treatment are classified as having Established Status Epilepticus (ESE) and those who then progress to and subsequently fail at least one second-line antiepileptic drug (AED) are classified as having RSE. The top-line results from RAISE showed that the trial met its first co-primary endpoint, a statistically significant proportion of patients had status epilepticus cessation within 30 minutes of initiating IV ganaxolone compared to placebo: 80% vs. 13%, respectively ($p < 0.0001$), but failed to achieve statistical significance on its second co-primary endpoint, the proportion of patients not progressing to IV anesthesia for 36 hours following initiation of IV ganaxolone compared to placebo: 63% vs. 51%, respectively (p=0.162). We continue to analyze the full RAISE trial dataset and plan to request a meeting with the FDA to discuss a potential path forward for IV ganaxolone in RSE.

We are developing ganaxolone in formulations for two different routes of administration: intravenous (IV) IV and oral. The different formulations are intended to maximize potential therapeutic applications of ganaxolone for adult and pediatric patient populations, in both acute and chronic care. While the precise mechanism by which ganaxolone exerts its therapeutic effects in the treatment of seizures is unknown, its anticonvulsant effects are thought to result from positive allosteric modulation of the gamma-aminobutyric acid type A ($GABA_A$) receptor in the central

nervous system (CNS). system. Ganaxolone is a synthetic analog of allopregnanolone, an endogenous neurosteroid, and targets both synaptic and

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extrasynaptic $GABA_A$. This unique receptor binding profile may contribute to the anticonvulsant, antidepressant and anxiolytic effects shown by neuroactive steroids in animal models, clinical trials or both.

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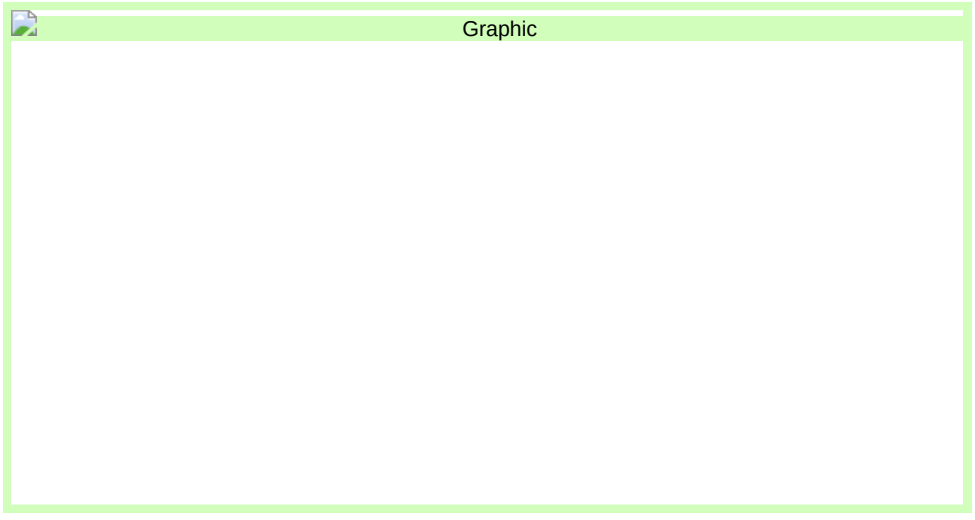
Our Products Pipeline

We are pursuing the development of ganaxolone for selected indications based on the mechanism of action and clinical profile of ganaxolone, including the following programs:



Our Oral Product and Product Candidates

ZTALMY® (ganaxolone) oral suspension CV

ZTALMY is an oral suspension given three times per day that we have developed for the treatment of CDD-associated seizures. ZTALMY was approved by the FDA in March 2022 for the treatment of seizures associated with CDD in patients two years of age and older. ZTALMY, our first FDA approved product, became available for commercial sale and shipment in the third quarter of 2022. We recorded ZTALMY net product revenue of $7.5 million $8.0 million and $3.3 million $15.5 million for the three and six months ended March 31, 2024 June 30, 2024, respectively. We recorded ZTALMY net product revenue of $4.2 million and 2023, $7.6 million for the three and six months ended June 30, 2023, respectively. On July 28, 2023, the EC granted marketing authorization for ZTALMY for the adjunctive treatment of epileptic seizures associated with CDD in patients two to 17 years of age. ZTALMY may be continued in patients 18 years of age and older. With the EC marketing authorization granted for ZTALMY, Orion, our commercialization partner for ZTALMY in Europe, announced it has begun preparations for the expected launch of ZTALMY in Europe in the second half of 2024, including engaging in the required processes for obtaining pricing and reimbursement approval in the various European countries. The pricing and reimbursement process can be time-consuming and may delay Orion's commercial launch of ZTALMY in one or more European countries.

CDD is a serious and rare genetic disorder that is caused by a mutation of the *CDKL5* gene, located on the X chromosome. CDD is a severely debilitating and potentially fatal genetic condition, which occurs with an estimated frequency of 1:40,000 live births in the U.S. It predominantly affects females and is characterized by early onset, difficult to control seizures and severe neurodevelopmental impairment. The *CDKL5* gene encodes proteins essential for normal brain structure and function. Most children affected by CDD have neurodevelopmental deficits such as difficulty walking, talking and taking care of themselves. Many also suffer from scoliosis, gastrointestinal dysfunction or sleep disorders. Genetic testing is available to determine if a patient has a mutation in the *CDKL5* gene.

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In June 2017, we were granted FDA orphan drug designation for ganaxolone for the treatment of CDD. The designation provides the drug developer with a seven-year period of U.S. marketing exclusivity, tax credits for clinical research costs, the ability to apply for annual grant funding, clinical research trial design assistance and waiver of Prescription Drug User Fee Act filing fees. In July 2020, the FDA granted Rare Pediatric Disease Designation (RPD Designation) for ganaxolone for the treatment of CDD. The FDA grants RPD Designation for diseases that affect fewer than 200,000 people in the U.S. and in which the serious or life-threatening manifestations occur primarily in individuals 18 years of age and younger. Upon FDA approval of ZTALMY for CDD in March 2022, the FDA awarded us a Rare Pediatric Disease Priority Review Voucher (PRV), which we monetized in August 2022 for $110.0 million in cash. In August 2022, we received a letter from Purdue Neuroscience Company (Purdue) in which Purdue claimed that it was owed $5.5 million by us from the sale of the PRV pursuant to the Purdue License Agreement. We responded to Purdue that we did not agree with their claim. In February 2024, following discussions with Purdue, we agreed to pay Purdue $4 million $4.0 million in respect of its claim. The first $2 million $2.0 million installment was paid

to Purdue in March 2024, and the second $2 million $2.0 million installment will be was paid on or before June 15, 2024. in June 2024.

In November 2019, the European Medicines Agency's (EMA) Committee for Orphan Medicinal Products (COMP) granted orphan drug designation for ganaxolone for the treatment of CDD. Prior to the grant of the marketing authorization, the COMP was required to determine whether the orphan drug designation criteria were still met. On May 26, 2023, the COMP provided a positive opinion to maintain the orphan drug designation for ganaxolone for CDD in the EU.

The U.S. and EC approvals of ZTALMY for CDD are based on data from a Phase 3 double-blind placebo-controlled trial (Marigold Trial), in which 101 patients were randomized and treated with ZTALMY. Clinical trial patients receiving ZTALMY showed a median 30.7% reduction in 28-day major motor seizure frequency, compared to a median 6.9% reduction for those receiving placebo, achieving the trial's primary endpoint (p=0.0036). At two years in the open label extension phase of the Marigold Trial, patients (n=50) treated with ZTALMY experienced a median 48.2% reduction in major motor seizure frequency. These data suggest that patients who remain on treatment long-term may demonstrate continued reductions in seizure frequency. The most common adverse events (AEs) in the double-blind

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portion of the Marigold Trial were somnolence (36.0% in the ganaxolone group compared to 15.7% in the placebo group), pyrexia (18.0% and 7.8%, respectively) and salivary hypersecretion (6.0% and 2.0%, respectively).

We own families of patents and pending patent applications that claim certain formulations of ganaxolone and cover certain therapeutic uses of ganaxolone, including for treating CDD. The 20-year terms for patents, and applications that issue as patents, in these families run from 2026 through 2042, absent any available patent term adjustments or extensions. We have also licensed from Ovid certain patents that claim certain therapeutic uses of ganaxolone for the treatment of CDD. The licensed patents include a granted U.S. patent, and pending applications in the U.S. and Europe. The 20-year term for these licensed patents and applications that issue as patents will run through 2037, absent any available patent term adjustments.

U.S. Commercial Strategy. Since ZTALMY was approved by the FDA, we have been focused on the implementation and execution of an integrated launch plan to make ZTALMY available to CDD patients in the U.S. through a specialty pharmacy. Key commercial strategies have included and continue to include: (1) executing our supply chain network and quality management system to assure product is available to patients; (2) driving clinical awareness of ZTALMY as the first and only FDA approved product indicated specifically for seizures associated with CDD; (3) deploying our field sales force to target physicians who treat this rare pediatric patient population; (4) engaging commercial and government payers with the objective of obtaining insurance coverage; and (5) enhancing our internal capabilities (such as Finance, Human Resources, Information Technology, Data Analytics and Compliance) to support our first launch as a commercial company.

U.S. Marketing Strategy. Our marketing strategy in the U.S. is to reinforce that seizures are central to the constellation of CDD symptoms, establish ZTALMY as central to the comprehensive management of seizures associated with CDD, and ensure that patients have seamless access to ZTALMY from prescription through fulfillment. Our marketing campaign for ZTALMY is active, and our integrated commercial launch activities initiated in the third quarter of 2022.

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U.S. Sales Strategy. Our U.S. commercial strategy divides our sales force includes territories into 16 U.S. territories where our experienced regional account managers experienced in rare disease. Our field force is targeting identified target key accounts and centers of excellence for CDD. Based on our market research, we estimate the addressable patient population for ZTALMY for CDD in the U.S. is approximately 2,000 pediatric patients. As this is the first product approved by the FDA specifically for seizures associated with CDD and the International Classification of Diseases, Tenth Revision (ICD10) code for CDD was established in 2021, there is limited data available for this specific market. We have strengthened both our market access and field force teams, and both payer and customer engagement are ongoing.

U.S. Market Access. We have established a payer and reimbursement account team with the objective of obtaining and maintaining reimbursement (coverage) of ZTALMY in the U.S. We are focusing our efforts on reimbursement from commercial payers where pharmacy benefit managers (PBMs) control the majority of commercial pharmacy-benefit lives and government payers, primarily Medicaid for the target population for CDD. We expect approximately 50% of the CDD patient population will access primary coverage through Fee-for-Service or Managed Medicaid, with the remaining approximately 50% accessing primary coverage through commercial payers, with the top PBMs having significant influence. The prescribing and fulfillment process for ZTALMY in the U.S. is managed through ZTALMY One™, a comprehensive patient support program. Enrollment in the program offers various support and information to help caregivers and patients prescribed ZTALMY access their ZTALMY prescription and assist in determining eligibility for and access to co-pay support or free drug programs.

U.S. Specialty Pharmacy. We are utilizing Orsini Pharmaceutical Services, LLC (Orsini), a specialty pharmacy, to provide services for patients in the U.S., including patient enrollment, benefit verification and investigation, prior authorization support, patient education and drug counseling, dispensing of product and shipment coordination.

U.S. Specialty Distributor. We are utilizing ASD Specialty Healthcare, LLC (ASD), a specialty distributor, to provide distribution services in the U.S. in connection with ZTALMY to institutional inpatient pharmacies, U.S. governmental customers, including any Department of Veterans Affairs or Department of Defense sites, and Kaiser Permanente facilities.

Infrastructure. We continue to enhance our internal capabilities and processes to support a commercial stage commercial-stage company. We have implemented a healthcare compliance program to guide our compliance with rules and regulations regarding pharmaceutical sales.

Manufacture of Commercial Supply. We have executed commercial supply agreements for ganaxolone active pharmaceutical ingredient (API) with our current manufacturer and also with our current supplier for finished bulk drug product. Additionally, we have executed a master supply agreement with a second API supplier to undertake certain process development activities and, if successful, provide commercial supplies of API and/or API intermediates.

Regulated as a Controlled Substance in the U.S. On June 1, 2022, the Drug Enforcement Agency (DEA) published an interim final rule in the Federal Register placing ganaxolone and its salts in schedule V of the Controlled Substances Act (CSA), which rule became final December 9, 2022. Under the CSA, drugs are classified into five (5) distinct categories or schedules depending upon the drug's acceptable medical use and the drug's abuse or dependency potential. Schedule V is defined by the DEA as drugs with lower potential for abuse than schedule IV and consist of preparations containing limited quantities of certain narcotics. ZTALMY became available for commercial sale and shipment in the third quarter of 2022. As a controlled substance, ganaxolone is subject to the applicable CSA requirements such as registration, security, recordkeeping and reporting, storage manufacturing, distribution, importation and other requirements.

FDA Post-Marketing Requirements. In connection with FDA approval of ZTALMY for CDD, we have several post-marketing commitments. The Phase 1 renal impairment study commitment was submitted to the FDA in May 2022, the Phase 1 hepatic impairment study and the thorough QTc study were submitted to the FDA in December 2022, the extractable/leachable study results on the container closure system were submitted to the FDA in July 2023, the M17 in vitro drug-drug interaction (DDI) study was submitted in August 2023, and the M17 in vivo PK study with Brain Penetrance was submitted in December 2023. The remaining post-marketing requirements include: 2-year carcinogenicity studies of ganaxolone and the major human unconjugated plasma metabolite, M2, in rats; a 26-week

carcinogenicity of ganaxolone in transgenic mice; and a juvenile animal toxicity study of M2 in rats. We expect to be able to complete these remaining required FDA studies and are working with the FDA with respect to the timing of

their completion and submission.

Managed Access Program (MAPS). We have initiated a global managed access program with Uniphar Durbin Ireland LTD to support physician access to ZTALMY for appropriate patients with seizures associated with CDD in geographies where there is no available patient access, local regulatory criteria and program eligibility are satisfied, and we do not already have a commercial distribution relationship in place.

Marketing Authorization Application

In August 2021, the Committee for Medicinal Products for Human Use (CHMP) of the EMA granted our request for accelerated assessment of ganaxolone for the treatment of seizures associated with CDD. The marketing authorization application (MAA) for ganaxolone was submitted to the EMA on October 11, 2021, and on October 28, 2021, we received formal notification from the EMA that the CDD MAA was validated. With this validation, the EMA began its formal review of the MAA under the centralized procedure. On May 26, 2023, the CHMP adopted a positive opinion recommending approval of ZTALMY. On July 28, 2023, the EC approved ZTALMY oral suspension for the adjunctive treatment of epileptic seizures associated with CDD in patients two to 17 years of age. ZTALMY may be continued in patients 18 years of age and older. The EC decision is applicable to all 27 EU member states plus Iceland, Norway and Liechtenstein. ZTALMY is the first treatment in the EU indicated for the treatment of seizures associated with CDD.

EC Post-Authorization Measures. In connection with the EC approval of ZTALMY for CDD, we have several post-marketing authorization measures. The clinical study report (CSR) for Study 1042-HME-1001 was submitted in September 2023. The ganaxolone Steady-State Metabolite Study report, the final Study 1042-CDD-3001 CSR with the open-label trial completion, the M17 in vitro DDI study, and the M17 in vivo PK study with Brain Penetrance were submitted in December 2023. The updated Environmental Risk Assessment, including a Sediment Dwelling Organism toxicity study, was submitted in February 2024. The remaining post-marketing authorization measures include: participating in Study

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LLF001 (CANDID observational study) and providing annual updates; participating in the CDD-IPR-CDD-0 CDKL5 Deficiency Disorder International Patient Registry and providing six monthly updates; conducting a toxicity study with a sediment dwelling organism and an updated Environmental Risk Assessment; developing a sodium benzoate-free suspension and assessing the compatibility of the oral suspension with food, drinks, enteral tubes, shake time and stand time; conducting a 26-Week Oral Gavage Toxicity Study of M2; conducting a M2 Embryo-fetal Development study; and conducting a 26-week Oral Gavage Carcinogenicity Study of ganaxolone and M2. The EMA also requested weight of evidence (WoE) assessments to evaluate the need for a 2-year carcinogenicity study in rats with ganaxolone, a 2-year carcinogenicity study in rats with M2, and a juvenile toxicity study with M2. We expect to be able complete the remaining required studies within and are working with the requested EMA timeframe. with respect to the timing of their completion and submission.

Our Pipeline

We are pursuing development Ganaxolone for CDD approved by the China National Medical Products Administration

On July 18, 2024, we announced that the CNMPA approved ganaxolone oral suspension for the treatment of epileptic seizures in patients two years of age and older with CDD. In November 2022, we entered into a collaboration agreement with Tenacia which granted Tenacia the right to develop and commercialize ganaxolone in Mainland China, Hong Kong, Macau and Taiwan in exchange for selected indications based on the mechanism of action royalties and clinical profile of ganaxolone, including the following programs:

other payments associated with net sales and commercial and regulatory milestones.

Tuberous Sclerosis Complex (TSC)

TSC is a rare genetic disorder that causes non-malignant tumors in the brain, skin, kidney, heart, eyes, and lungs. Rarely, patients may develop malignant tumors of the kidney, breast or thyroid gland. The condition is caused by inherited mutations in either the *TSC1* or *TSC2* gene. It occurs with a frequency of approximately 1:6,000 live births, with a genetic mutation being found in 85% of patients. While the disease phenotype can be extremely variable, epilepsy occurs with a frequency of up to 85%. TSC is a leading cause of genetic epilepsy, often manifesting in the first year of life as either focal seizures or infantile spasms. There are currently few disease-specific treatments approved for seizures

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in TSC. Orphan drug designation for ganaxolone for the treatment in TSC was granted by the FDA in August 2021 and by the EMA in October 2021.

In August 2021, we announced top-line data from our open-label Phase 2 trial evaluating the safety and efficacy of adjunctive oral ganaxolone in 23 patients with TSC-associated seizures. The trial enrolled 23 patients ages 2 to 32, who entered a four-week baseline period followed by a 12-week treatment period, during which they received up to 600 mg of ganaxolone (oral liquid suspension) three times a day. Patients who completed the initial 12-week treatment period were able to continue ganaxolone during an extension phase of the trial. The primary endpoint was the percent

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change in 28-day TSC-associated seizure frequency during the 12-week treatment period relative to the four-week baseline period. Secondary outcome measures included the percentage of patients experiencing a greater than or equal to 50% reduction in 28-day TSC-associated seizure frequency through the end of the 12-week treatment period compared to the 4-week baseline period.

The primary endpoint showed a median 16.6% reduction in 28-day frequency of TSC-associated seizures relative to the four-week baseline period. A secondary endpoint showed that the proportion of patients that achieved at least a 50% seizure reduction was 30.4%. During the trial, patients with focal seizures (n=19) showed a median 25.2% reduction in focal seizure frequency. Ganaxolone was generally well-tolerated with somnolence reported as the most common AE. In addition, one serious adverse event (SAE) of worsening seizures occurred, which was assessed by the investigator as treatment related. Four patients discontinued the trial due to AEs. Based on the reports of somnolence from the Phase 2 TSC trial, a revised titration schedule has been implemented in the Phase 3 TSC TrustTSC trial designed to improve tolerability while titrating to effective therapeutic ganaxolone levels. To date, there is a less than 7% discontinuation rate in the overall blinded study population in the Phase 3 TSC TrustTSC trial.

In response to our request for an end-of Phase 2 meeting with the FDA regarding a proposed Phase 3 TSC trial, the FDA provided written responses to our questions in lieu of a meeting. We believe the written responses show overall alignment on the clinical development plan in TSC, and with the FDA approval of CDD, that a single trial could serve as necessary support for regulatory approval of TSC in the U.S. In response to our request for Protocol Assistance, which is a special form of scientific advice available for developers of designated orphan medicines for rare diseases, the EMA provided written feedback in December 2021 in lieu of a meeting. We believe the written responses from the EMA, like those from the FDA, show overall alignment on the clinical development plan in TSC. After commencing site initiations in the U.S. and Europe in the first quarter of 2022, we are actively began enrolling patients in this TrustTSC global Phase 3 randomized, double blind, placebo-controlled trial (TrustTSC trial) of adjunctive ganaxolone in approximately 128 patients with TSC-related seizures. Based on the sample size of 128 trial participants, the trial provides 90% power to detect a 25% difference in seizure reductions between ganaxolone and placebo. The trial has 93 fully enrolled 129 patients in May of 2024, with trial sites activated, including several TSC centers of excellence, predominantly in the U.S., Western Europe, Canada, Australia, China and Israel. The primary endpoint is percent change in 28-day frequency of TSC-associated seizures, and we plan to announce top-line data in the first half of the fourth quarter of 2024.

In July 2023, the USPTOU.S. Patent and Trademark Office (USPTO) granted us a patent (U.S. Patent No. 11,701,367) on a method of certain methods for treating TSC-related epilepsy by administering oral ganaxolone. This issued patent expires in 2040. This In May 2024, the USPTO granted us a second patent is a member of a patent family we own that includes pending (U.S. Patent No. 11,980,625) on methods for treating TSC-related epilepsy by administering oral ganaxolone. We continue to prosecute patent applications that claim in this family relating to certain therapeutic regimens for the treatment of TSC.

Status Epilepticus (SE)

SE is a life-threatening condition characterized by continuous, prolonged seizures or rapidly recurring seizures without intervening recovery of consciousness. If SE is not treated urgently, permanent neuronal damage may occur, which contributes to high rates of morbidity and mortality. Patients with SE who do not respond to first-line benzodiazepine treatment are classified as having Established Status Epilepticus (ESE) and those who then progress to and subsequently fail at least one second-line antiepileptic drug (AED) are classified as having RSE. In RSE, synaptic $GABA_A$ receptors are internalized into the neuron, resulting in decreased responsiveness to drugs such as benzodiazepines. RSE unresponsiveness to one or more second-line AEDs may require treatment with IV anesthesia to terminate seizures and prevent neuronal injury and other complications. The IV anesthetic is increased to a level that induces deep coma and is maintained at that rate for 24 hours or more. SE that recurs following an attempted wean of IV anesthesia is classified as super refractory status epilepticus (SRSE). In April 2016, we were granted FDA orphan drug designation for the IV formulation of ganaxolone for the treatment of SE, which includes RSE.

In January 2021, we enrolled the first patient in the Phase 3 RAISE trial, a randomized, double-blind, placebo-controlled trial in patients with RSE, who have failed two or more antiseizure medications. The RAISE trial has approximately 70 trial sites, primarily in the U.S. and Canada. It is designed to enroll approximately 124 patients who will be randomized to receive ganaxolone or placebo added to standard of care. We reached alignment with the FDA on a protocol amendment, including a proposal for an interim analysis when two-thirds of the patients (approximately 82) have completed assessment of the primary and key secondary trial endpoints. The enrollment target for the interim

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analysis was completed in the first quarter of 2024. On April 15, 2024, we announced that the independent Data Monitoring Committee (DMC) completed its review of the interim analysis. The trial did not meet the pre-defined interim analysis stopping criteria on the co-primary endpoints, and the DMC recommendation was that the RAISE trial may continue without modification. We have decided to complete enrollment in the RAISE trial at 100 patients with top-line results expected in the summer of 2024. Those results will be used to determine whether to continue development of IV ganaxolone in RSE. We remain blinded to the RAISE trial data. The co-primary endpoints for the RAISE trial are (1) proportion of patients who experience SE cessation within 30 minutes without use of other IV antiseizure medications, and (2) proportion of patients without progression to IV anesthesia for 36 hours. In June 2022, we announced that we amended the protocol for the RAISE trial to expand eligibility criteria, including allowing

patients previously treated with up to 18 hours of high-dose IV anesthesia to enroll. Previously, we had excluded patients treated with high-dose IV anesthetics for any duration.

In February 2022, we temporarily paused the RAISE trial after routine monitoring of stability batches of clinical supply material indicated that it became necessary to reduce the shelf life to less than the anticipated 24 months to meet product stability testing specifications. We notified the FDA of this issue and our plans to proactively pause the trial, and we subsequently provided additional information to the FDA to support resuming trial activities. In May 2022, we announced that the trial had resumed utilizing new batches of the original buffer IV formulation of ganaxolone, and we implemented a reduced shelf life of 12 months. In agreement with the FDA, ganaxolone clinical supplies with the original buffer IV formulation would be stored under refrigerated conditions for the entire duration of clinical use. The shelf life of the original buffer IV formulation was updated to 18 months under refrigerated conditions, based on stability data which was submitted in the subsequent IND amendment in February 2023. Subsequently, we manufactured the IV ganaxolone formulation with a new buffer and are targeting a shelf life of 24 months at room temperature, pending the results of ongoing stability monitoring. The FDA agreed that in principle a buffer change in the ganaxolone IV formulation is acceptable but requested that additional information be submitted prior to use of the new buffer formulation in clinical trials. We submitted an IND amendment to the FDA in May 2023. All sites have been resupplied with the new buffer formulation, which we believe will not require refrigeration and is expected to have a shelf life of 24 months.

We commenced a separate Phase 3 RSE trial to support an MAA in Europe (RAISE II trial) in 2023. We have decided to discontinue the RAISE II trial. We plan to assess top-line results from the RAISE trial which we expect in the summer of 2024. Future development of IV ganaxolone in RSE will be assessed following review of the RAISE trial top-line data.

We gained alignment on the RAISE II trial design at a meeting with the EMA in the first quarter of 2021. The RAISE II trial was designed as a double blind, placebo-controlled registration trial targeting enrollment of 70 patients who have failed first-line benzodiazepine treatment and at least one second-line IV AED. Under the protocol, patients would receive either ganaxolone or placebo, administered in combination with a standard-of-care second-line IV AED. The simultaneous administration of a standard-of-care AED with the trial drug was designed to provide data complementary to that from the RAISE trial. There are two additional key differences between the RAISE and RAISE II trials. First, unlike the RAISE trial, which specified progression to IV anesthesia as constituting treatment failure, any escalation of care – whether an additional second-line IV AED or an IV anesthetic – will fulfill criteria for treatment failure in RAISE II. This aligns more closely with the European standard of practice for RSE in which IV anesthesia is used less commonly than in the U.S. Second, the primary endpoint for the RAISE II trial is based on a responder analysis, with response defined as SE cessation within 30 minutes and no escalation of care within 36 hours, rather than the co-primary endpoints in the RAISE trial, which require statistical significance to be achieved independently on both the 30-minute and 36-hour outcomes. Analysis of the RAISE data is expected to inform future development of IV ganaxolone in refractory status epilepticus, including whether the RAISE II trial or a similar trial design would move forward.

In 2023, we discontinued the RESET trial, a Phase 2 trial evaluating ganaxolone for the treatment of ESE. We have focused our resources for IV ganaxolone on our RSE trials (i.e., completing the RAISE trial and accelerating enrollment in the RAISE II trial), as well as developing a proof-of-concept trial in SRSE. SRSE is a life-threatening neurological emergency with high morbidity and mortality, and we have provided ganaxolone to physicians who have requested it for SRSE treatment under emergency investigational new drug (eIND) applications. To date, 29 patients

have been treated for SRSE with ganaxolone. Based on our observations of treatment outcomes in these patients and pending the results of the top-line RAISE trial data which are expected in the summer of 2024, we plan to submit a protocol to the FDA for an open-label, proof-of-concept trial of ganaxolone in approximately 20 patients with SRSE.

On September 7, 2021, the U.S. Patent and Trademark Office (USPTO) granted us a patent (US 11,110,100) on a method of treating SE which includes claims related to our clinical therapeutic regimen for the treatment of SE using IV ganaxolone. This issued patent expires in 2040. That patent is a member of a patent family we own that includes pending patent applications that claim certain therapeutic regimens for the treatment of SE, including RSE, using intravenous ganaxolone. The USPTO granted us a second patent for SE on June 20, 2023 (US 11,679,117) with new claims that cover therapeutic regiments in which high doses of ganaxolone are administered, which we believe is relevant for some patients, and strengthens our intellectual property portfolio for the treatment of SE, including SRSE, using ganaxolone.

In July 2022, the USPTO issued U.S. Patent No. 11,395,817 (Ovid ‘817 Patent) to Ovid Therapeutics, Inc. (Ovid) with claims that may encompass our product candidate for the treatment of SE. On March 15, 2023, we filed a petition seeking post-grant review (PGR) of the Ovid ‘817 Patent with the USPTO Patent Trial and Appeal Board (PTAB). Our petition for PGR argues that the claims of the Ovid ‘817 Patent are unpatentable on multiple grounds. Ovid filed a preliminary response to our petition on June 20, 2023. In Ovid’s reply to our request for PGR, Ovid disclaimed claims 1-21, 23 and 24 of the Ovid ‘817 Patent, which has the effect of erasing these claims from the patent, irrespective of the outcome of the PGR. On August 17, 2023, the PTAB issued a decision granting institution of our petition seeking PGR of the Ovid ‘817 Patent. In instituting the PGR, the PTAB stated that it was more likely than not that we would be able to invalidate the remaining claims (22 and 25-31) of the Ovid ‘817 Patent during the proceeding. The decision to institute is not a final decision on the patentability of the claims. The final decision will be based on the full record developed during the proceeding. The PGR process is ongoing, oral arguments are scheduled for May 22, 2024, and a final decision is expected by August of 2024. If we prevail in the PGR, the Ovid ‘817 Patent will not be enforceable against us. If we do not prevail in the PGR proceeding, the decision can be appealed to the Court of Appeals for the Federal Circuit. If an appeal is not successful, our ability to challenge the Ovid ‘817 Patent in court will be limited in certain respects.

On February 20, 2024, the USPTO issued U.S. Patent No. 11,903,930 (Ovid ‘930 Patent) to Ovid with claims that may encompass our product candidate for the treatment of SE. On March 5, 2024, the USPTO issued U.S. Patent No. 11,918,563 (Ovid ‘563 Patent) to Ovid with claims that may encompass our product candidate for the treatment of SE. We are evaluating the Ovid ‘930 Patent and the Ovid ‘563 Patent.

Ovid may file lawsuits against us alleging infringement of its patents. Any such proceedings, in the PTAB or courts, regardless of their outcome, would likely result in the expenditure of significant financial resources and the diversion of management’s time and resources. In addition, any such proceeding may cause negative publicity, adversely impact patients, and, while unlikely, we may be prohibited from marketing or selling ganaxolone for SE, including RSE, during such proceedings or if we are not successful in such proceedings. If Ovid does decide to bring an infringement lawsuit, we do not expect that it will be filed before a U.S. commercial launch of ganaxolone for RSE based upon the “safe harbor” provisions of the Drug Price Competition and Patent Term Restoration Act of 1984

(Hatch-Waxman Act). We may need to acquire or obtain a license to certain Ovid patents to market or sell ganaxolone for RSE in the U.S., which may not be available on commercially acceptable terms or at all. If we are not able to acquire the certain Ovid patents or negotiate a license on acceptable terms, and if our product is determined to infringe Ovid's patents and such patents are determined to be valid, then we may be forced to pay Ovid royalties, damages and costs, or, although unlikely, we may be prevented from commercializing ganaxolone for RSE in the U.S. altogether, which would have a material adverse impact on our business.

The Ovid '817 Patent, the Ovid '930 Patent and the Ovid '563 Patent claims are limited to the use of ganaxolone in the treatment of SE and do not cover or impact our marketing and sales of ZTALMY for the treatment of seizures associated with CDD.

On March 24, 2024, Ovid filed an Inter Partes Review (IPR) challenge of our U.S. Patent 11,110,100 one of our patents for the use of ganaxolone in treating SE and RSE. We intend to file our preliminary response to the IPR by July

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11, 2024. Following our response, the PTAB will have 3 months to determine whether or not to institute the Ovid IPR. If the PTAB institutes the IPR, it will not be a final decision on the patentability of the patent's claims. If instituted, a final decision on the patentability of the claims would be issued by the PTAB in October 2025 after consideration of the full record developed during the proceeding. If we do not prevail in the IPR proceeding, the decision can be appealed to the Court of Appeals for the Federal Circuit. If an appeal is not successful, our ability to obtain patent protection for use of IV ganaxolone in the treatment of SE or RSE may be limited. The inability to obtain meaningful patent protection for the use of IV ganaxolone in the treatment of SE or RSE could have a material adverse impact on our business.

Clinical Development in Lennox-Gastaut Syndrome (LGS), other epileptic encephalopathies, and Prodrug Development and Second-generation Formulation

We plan to expand our investment in ZTALMY to explore its potential in the treatment of other rare epilepsies. Preliminary planning is underway for a clinical trial that would assess oral ganaxolone for the treatment of a broad range of developmental and epileptic encephalopathies, including LGS, targeted to begin in the first half of 2025, pending the top-line data results from our Trust TSC TrustTSC trial. LGS is a severe form of epilepsy that typically begins between one and eight years of age. Affected children have neurodevelopmental impairments and intractable seizures, including focal, atonic, tonic, generalized tonic-clonic and atypical absence seizures. In March 2023, the FDA granted orphan drug designation to ganaxolone for the treatment of LGS. This designation applies to the active moiety of ganaxolone and is not dependent on the formulation. Given the overlap in seizure types and etiologies with CDD and other disorders where

ganaxolone has therapeutic potential, such as CDD and TSC, we believe that LGS represents a promising opportunity for ZTALMY prodrug, or our second-generation the ganaxolone formulation. prodrug.

The development of ganaxolone prodrug compounds continues to advance with a lead oral and IV candidates candidate having been selected. We anticipate completion of IND-enabling investigational new drug (IND)-enabling studies for the oral prodrug in the first second half of 2025, followed by an IND filing by the end of 2025 and the initiation of a Phase 1 trials trial(s) in 2025, early 2026, pending the top-line data results from our Trust TSC TrustTSC trial.

Additionally, top-line data from a single ascending dose (SAD) Phase 1 trial in healthy volunteers utilizing the first candidate for a second generation formulation of ganaxolone demonstrated linear PK properties at doses of up to 1200 mg. Data from a subsequent phase 1 multiple ascending dose (MAD) trial also demonstrated linear kinetics through the range of doses assessed. Based on these results, we intend to apply extended-release technologies to the formulation, which could provide consistent exposure that maintains trough concentrations within the therapeutic range, minimizes peak dose-related side effects and allows once- or twice-daily dosing. The linear kinetics observed in the MAD trial, along with predictable dose-exposure relationships, may allow physicians to individualize dosing to patient needs.

On September 27, 2023 November 7, 2023, the USPTO issued a Notice of Allowance in an U.S. Patent No. 11,806,336 to Ovid patent application with claims that encompass our product candidate for the treatment of LGS. This patent issued, U.S. Patent No. 11,806,336, on November 7, 2023. The claims in the Ovid LGS patent cover the use of ganaxolone in the treatment of LGS and do not cover or impact the use of ganaxolone in any other indication. Ovid may file a lawsuit against us alleging infringement of its LGS patent claims. Any such proceeding, regardless of the outcome, would likely result in the expenditure of significant financial resources and the diversion of management's time and resources. In addition, any such proceeding may cause negative publicity, adversely impact patients, and we may be prohibited from marketing or selling ganaxolone for LGS during such proceeding or if we are not successful in such proceedings. If Ovid does decide to bring an infringement lawsuit, we do not expect that it will be filed before a commercial launch of ganaxolone for LGS based upon the "safe harbor" provisions of the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman Act). We may need to acquire or obtain a license to the Ovid LGS patent to market or sell ganaxolone for LGS, which may not be available on commercially acceptable terms or at all. If we are not able to acquire the Ovid LGS patent or negotiate a license on acceptable terms, and if our product is determined to infringe Ovid's patent and such patent is determined to be valid, then we may be forced to pay Ovid royalties, damages and costs, or we may be prevented from commercializing ganaxolone for LGS altogether, which would have a material adverse impact on our business.

Our IV Product Candidates

Status Epilepticus (SE)

SE is a life-threatening condition characterized by continuous, prolonged seizures or rapidly recurring seizures without intervening recovery of consciousness. If SE is not treated urgently, permanent neuronal damage may occur, which contributes to high rates of morbidity and mortality. Patients with SE who do not respond to first-line benzodiazepine treatment are classified as having ESE and those who then progress to and subsequently fail at least one second-line AED are classified as having RSE. In RSE, synaptic $GABA_A$ receptors are internalized into the neuron, resulting in decreased responsiveness to drugs such as benzodiazepines. RSE unresponsiveness to one or more second-line AEDs may require treatment with IV anesthesia to terminate seizures and prevent neuronal injury and other complications. The IV anesthetic is increased to a level that induces deep coma and is maintained at that rate for 24 hours or more. SE that recurs following an attempted wean of IV anesthesia is classified as super refractory status epilepticus (SRSE). In April 2016, we were granted FDA orphan drug designation for the IV formulation of ganaxolone for the treatment of SE, which includes RSE.

In January 2021, we enrolled the first patient in the RAISE trial, a randomized, double-blind, placebo-controlled trial in patients with RSE, who have failed two or more antiseizure medications. The RAISE trial has approximately 70 trial sites, primarily in the U.S. and Canada. It was designed to enroll approximately 124 patients who were randomized to receive ganaxolone or placebo added to standard of care. We reached alignment with the FDA on a protocol amendment, including a proposal for an interim analysis when two-thirds of the patients (approximately 82) had completed assessment of the primary and key secondary trial endpoints. The enrollment target for the interim analysis was completed in the first quarter of 2024. On April 15, 2024, we announced that the trial did not meet the pre-defined interim analysis stopping criteria on the co-primary endpoints. We decided to complete enrollment in the RAISE trial at 100 patients. On June 17, 2024, we announced top-line results of the RAISE trial, which showed that the trial met its first co-primary endpoint, with a statistically significant proportion of patients achieving SE cessation within 30 minutes of initiating IV ganaxolone compared to placebo: 80% vs. 13%, respectively ($p< 0.0001$). However, the trial failed to achieve statistical significance on its second co-primary endpoint, the proportion of patients not progressing to IV anesthesia for 36 hours following initiation of IV ganaxolone compared to placebo: 63% vs. 51%, respectively ($p=0.162$). The incidence of SAEs was similar between the treatment and placebo arms (n=19 for IV ganaxolone, n=18

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for placebo), with hypotension being more commonly seen in the IV ganaxolone arm. Other secondary endpoints, including escalation of treatment with either IV ASMs or IV anesthesia within 24 hours and reduction in electroencephalogram (EEG) seizure burden, at 36 hours favored ganaxolone. We continue to analyze the full RAISE trial dataset and plan to request a meeting with the FDA to discuss a potential path forward for IV ganaxolone in RSE.

To date, we have provided IV ganaxolone for treatment of SRSE in 31 patients under emergency IND applications. Pending the outcome of discussions with the FDA regarding IV ganaxolone in RSE, it is possible that we may re-initiate clinical programs for IV ganaxolone including a proof-of-concept study in SRSE.

We commenced a separate Phase 3 RSE trial to support an MAA in Europe (RAISE II) in 2023. In the second quarter of 2024, we decided to discontinue the RAISE II trial.

On September 7, 2021, the USPTO granted us a patent (U.S. Patent No. 11,110,100) on a method of treating SE which includes claims related to our clinical therapeutic regimen for the treatment of SE using IV ganaxolone. This issued patent expires in 2040. That patent is a member of a patent family we own that includes pending patent applications that claim certain therapeutic regimens for the treatment of SE, including RSE, using intravenous ganaxolone. The USPTO granted us a second patent for SE on June 20, 2023 (U.S. Patent No.11,679,117) with claims that cover therapeutic regiments in which high doses of ganaxolone are administered, which we believe is relevant for some patients, and strengthens our intellectual property portfolio for the treatment of SE, including SRSE, using ganaxolone.

In July 2022, the USPTO issued U.S. Patent No. 11,395,817 (Ovid '817 Patent) to Ovid Therapeutics, Inc. (Ovid) with claims that may encompass our product candidate for the treatment of SE. On March 15, 2023, we filed a petition seeking post-grant review (PGR) of the Ovid '817 Patent with the USPTO Patent Trial and Appeal Board (PTAB). Our petition for PGR argues that the claims of the Ovid '817 Patent are unpatentable on multiple grounds. Ovid filed a preliminary response to our petition on June 20, 2023. In Ovid's reply to our request for PGR, Ovid disclaimed claims 1-21, 23 and 24 of the Ovid '817 Patent, which has the effect of erasing these claims from the patent, irrespective of the outcome of the PGR. On August 17, 2023, the PTAB issued a decision granting institution of our petition seeking PGR of the Ovid '817 Patent. In instituting the PGR, the PTAB stated that it was more likely than not that we would be able to invalidate the remaining claims (22 and 25-31) of the Ovid '817 Patent during the proceeding. On August 1, 2024, the PTAB issued its final written decision on the PGR (*Marinus Pharms., Inc. v. Ovid Therapeutics, Inc.*, PGR2023-00020, Paper 41 (Aug. 1, 2024)), concluding that all remaining claims—claims 22 and 25-31—were unpatentable as obvious. The decision is publicly available on the Patent Office's website (PTAB Open Data (uspto.gov)). If Ovid wishes to request rehearing or review of the decision, Ovid could file for rehearing or seek Director review at the PTAB within 30 days from the August 1, 2024 PTAB decision date. If Ovid wishes to appeal the decision to the U.S. Court of Appeals for the Federal Circuit, Ovid could file a notice of appeal to the Federal Circuit 63 days from the August 1, 2024 PTAB decision date. If Ovid chooses to file for rehearing or seek Director review with the PTAB before appealing to the Federal Circuit, the time to file a notice of appeal would run from the date the PTAB acts on the rehearing or Director review request. The entire process of a Federal Circuit appeal can take approximately 15 to 24 months to complete.

On February 20, 2024, the USPTO issued U.S. Patent No. 11,903,930 (Ovid '930 Patent) to Ovid with claims that may encompass our product candidate for the treatment of SE. On March 5, 2024, the USPTO issued U.S. Patent No. 11,918,563 (Ovid '563 Patent) to Ovid with claims that may encompass our product candidate for the treatment of SE. We are evaluating the Ovid '930 Patent and the Ovid '563 Patent.

Ovid may file lawsuits against us alleging infringement of its patents. Any such proceedings, in the PTAB or courts, regardless of their outcome, would likely result in the expenditure of significant financial resources and the diversion of management's time and resources. In addition, any such proceeding may cause negative publicity, adversely impact patients, and, while unlikely, we may be prohibited from marketing or selling ganaxolone for SE, including RSE, during such proceedings or if we are not successful in such proceedings. If Ovid does decide to bring an infringement lawsuit, we do not expect that it will be filed before a U.S. commercial launch of ganaxolone for RSE based upon the "safe harbor" provisions of the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman Act). We may need to acquire or obtain a license to certain Ovid patents to market or sell ganaxolone for RSE in the U.S.,

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which may not be available on commercially acceptable terms or at all. If we are not able to acquire the certain Ovid patents or negotiate a license on acceptable terms, and if our product is determined to infringe Ovid's patents and such patents are determined to be valid, then we may be forced to pay Ovid royalties, damages and costs, or, although unlikely, we may be prevented from commercializing ganaxolone for RSE in the U.S. altogether, which would have a material adverse impact on our business.

The Ovid '817 Patent, the Ovid '930 Patent and the Ovid '563 Patent claims are limited to the use of ganaxolone in the treatment of SE and do not cover or impact our marketing and sales of ZTALMY for the treatment of seizures associated with CDD.

On March 24, 2024, Ovid filed an Inter Partes Review (IPR) challenge of one of our patents for the use of ganaxolone in treating SE and RSE (U.S. Patent No. 11,110,100). We filed our preliminary response to the IPR on June 26, 2024. Following our response, the PTAB will have 3 months to determine whether or not to institute the Ovid IPR. If the PTAB institutes the IPR, it will not be a final decision on the patentability of the patent's claims. If instituted, a final decision on the patentability of the claims is expected to be issued by the PTAB in October 2025 after consideration of the full record developed during the proceeding. If we do not prevail in the IPR proceeding, the decision can be appealed to the Court of Appeals for the Federal Circuit. If an appeal is not successful, our ability to obtain patent protection for use of IV ganaxolone in the treatment of SE or RSE may be limited. The inability to obtain meaningful patent protection for the use of IV ganaxolone in the treatment of SE or RSE could have a material adverse impact on our business.

Operations

Our operations to date have consisted primarily of organizing and staffing our company, developing ganaxolone, including conducting preclinical studies and clinical trials, raising capital, partnering ZTALMY in certain geographies and the early commercialization of ZTALMY. We have funded our operations primarily through sales of equity and debt securities. We recorded $7.5 million $8.0 million and $3.3 million $15.5 million of ZTALMY net sales in the three and six months ended March 31, 2024 June 30, 2024, respectively. We recorded $4.2 million and 2023, $7.6 million of ZTAMLY net sales in the three and six months ended June 30, 2023, respectively. Since inception, we have incurred negative cash flows from our operations, and other than for the three months ended September 30, 2022 due to a one-time net gain from the sale of our Priority Review Voucher (PRV), PRV, we have incurred net losses. We have generated limited product revenues, and there is no assurance that profitable operations will be achieved in the future, and if achieved, could be sustained on a continuing basis. We incurred Net losses of $38.7 million $35.8 million and $34.7 million $74.5 million for the three and six months ended March 31, 2024 June 30, 2024, respectively. We incurred Net losses of $31.9 million and 2023, $66.7 million for the three and six months ended June 30, 2023, respectively. Our accumulated deficit as of March 31, 2024 June 30, 2024 was $610.6 $646.4 million.

We anticipate that we will continue to incur substantial losses in future periods as we:

- conduct multiple later stage clinical trials in targeted indications;
- continue the research, development and scale-up manufacturing capabilities to optimize ganaxolone and dose forms for which we may obtain regulatory approval;
- establish and implement sales, marketing and distribution capabilities to continue to commercialize ganaxolone;
- conduct other preclinical studies and clinical trials to support the filing of NDAs with the FDA, MAAs with the EMA and other marketing authorization filings with 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;

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- hire additional clinical, manufacturing, scientific and commercial personnel; and
- add or enhance operational, financial and management information systems and personnel, including personnel to support our drug development efforts.

We had cash, Cash and cash equivalents and short-term investments of $113.3 million $64.7 million as of March 31, 2024 June 30, 2024. We believe that our existing cash, cash equivalents and short-term investments as of March 31, 2024 June 30, 2024 will be sufficient to fund our operating expenses and capital expenditure requirements as well as maintain the minimum cash balance required under our debt facility, into the first second quarter of 2025. This expectation includes cost reduction activities that are being were implemented with expected impact beginning in the second quarter of 2024. We will need to secure additional funding in the future, from one or more equity or debt financings, government funding, collaborations, licensing transactions, other commercial transactions or other sources in order to carry out all of our commercialization and planned research and development activities with respect to ganaxolone.

Financial Overview

Product Revenue, net

Our first FDA approved product, ZTALMY, became available for commercial sale and shipment in the third quarter of 2022. We have three customers, one of which, Orsini, Pharmaceutical Services, LLC (Orsini), a specialty pharmacy that dispenses ZTALMY directly to patients, represents approximately 99% of our ZTALMY revenue to date. Our contract with Orsini has a single performance obligation to deliver ZTALMY upon receipt of a purchase order, which is satisfied when Orsini receives ZTALMY. We recognize ZTALMY revenue at the point in time when control of

ZTALMY is transferred to Orsini, which is upon delivery to Orsini. The transaction price that we recognize for ZTALMY revenue includes an estimate of variable consideration. Shipping and handling costs to Orsini are recorded as selling, general and administrative expenses. The components of variable consideration include:

Trade Discounts and Allowances. We provide contractual discounts, including incentive prompt payment discounts and chargebacks. Each of these potential discounts are recorded as a reduction of ZTALMY revenue and accounts receivable in the period in which the related ZTALMY revenue is recognized. We estimate the amount of variable consideration for discounts and allowances using the expected value method.

Product Returns and Recall. We provide for ZTALMY returns in accordance with our Return Good Policy. We estimate the amount of ZTALMY that may be returned using the expected value method, and we present this amount as a reduction of ZTALMY revenue in the period the related ZTALMY revenue is recognized. In the event of a recall, we will promptly notify Orsini and will reimburse Orsini for direct administrative expenses incurred in connection with the recall as well as the cost of replacement product.

Government Rebates. We are subject to discount obligations under state Medicaid programs, Medicare, and the Tricare Retail Refund Program. We estimate reserves related to these discount programs and record these obligations in the same period the related Product revenue is recognized, resulting in a reduction of Product revenue.

Patient Assistance. We offer a voluntary co-pay patient assistance program intended to provide financial assistance to eligible patients with a prescription drug co-payment required by payors and coupon programs for cash payors. The calculation of the current liability for this assistance is based on an estimate of claims and the cost per claim that we expect to receive associated with ZTALMY that has been recognized as Product revenue but remains in the distribution channel inventories at the end of each reporting period.

Federal Contract Revenue

In September 2020, we entered into a contract (BARDA Contract) with the Biomedical Advanced Research and Development Authority (BARDA), a division of the U.S. Department of Health and Human Services' Office of the Assistant Secretary for Preparedness and Response. Under the BARDA Contract, we received an award of up to an estimated $51 million for development of IV-administered ganaxolone for the treatment of RSE. The BARDA Contract provides for funding to support, on a cost-sharing basis, the completion of a Phase 3 clinical trial of IV-administered

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ganaxolone in patients with RSE, which covers the RAISE trial, funding of pre-clinical preclinical studies to evaluate IV-administered ganaxolone as an effective treatment for RSE due to chemical nerve gas agent exposure, and funding of certain ganaxolone manufacturing scale-up and regulatory activities. In March 2022, we entered into an amendment with BARDA to extend the end date of our base performance period for funding under the BARDA Contract from September 1, 2022 to December 31, 2023. In September 2022, we entered into an amendment with BARDA that, among other things, (i) provides for the exercise of BARDA's option under the BARDA Contract to support U.S. onshoring of the manufacturing capabilities for ganaxolone API (Option 2), (ii) changes the end date of our performance period under Option 2 from December 31, 2026 to July 31, 2025, (iii) increases the government cost share amount under Option 2 from approximately $11.5 million to approximately $12.3 million, and (iv) increases our cost share amount under Option 2 from approximately $4.9 million to approximately $5.3 million. In September 2023, we entered into an amendment with BARDA to extend the end date of our base performance period for funding under the BARDA Contract from December 31, 2023 to September 30, 2024.

The BARDA Contract consists of an approximately four-year base period, including the extension periods, during which BARDA agreed to provide up to approximately $21 million of funding for the RAISE trial on a cost share basis and funding of additional preclinical studies of ganaxolone in nerve agent exposure models. As of December 31, 2023, the entire base period funding of approximately $21 million had been recorded. Following successful completion of the RAISE trial and preclinical studies in the contract period, the BARDA Contract provides for approximately $31 million of additional BARDA funding for three options in support of ganaxolone manufacturing, supply chain, clinical, regulatory and toxicology activities, including the $12.3 million exercise of Option 2 as described above. Under the BARDA Contract, we will be responsible for cost sharing in the amount of approximately $33 million and BARDA will

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be responsible for approximately $52 million if all development options are completed. The contract period-of-performance (base period plus option exercises) is up to approximately five years.

We recognize Federal contract revenue from the BARDA Contract in the period in which the allowable research and development expenses are incurred. We expect Federal contract revenue to decrease as the entire base period funding of approximately $21 million had been recorded as of December 31, 2023. As such, funding is currently limited to Option 2, which supports onshoring of the manufacturing capabilities for ganaxolone API.

Collaboration Revenue

In July 2021, we entered into a collaboration agreement (Orion Collaboration Agreement) with Orion Corporation (Orion). Orion. Under the terms of the Orion Collaboration Agreement, we granted Orion an exclusive, royalty-bearing, sublicensable license to certain of our intellectual property rights with respect to commercializing biopharmaceutical products incorporating ganaxolone (Licensed Products) in the European Economic Area, the United Kingdom and Switzerland (collectively, the Territory) for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions (Field), initially in the indications of CDD, TSC and RSE.

Under the terms of the Orion Collaboration Agreement, we received a €25.0 million ($29.6 million at then-existing exchange rate) upfront payment from Orion in July 2021. We are eligible to receive up to an additional €97 million in R&D reimbursement and cash milestone payments based on specific clinical and commercial achievements. Also, as part of the overall arrangement, we have agreed to supply the Licensed Products to Orion at an agreed upon price.

We identified the following commitments under the arrangement: (i) exclusive rights to develop, use, sell, have sold, offer for sale and import any product comprised of Licensed Product (License); (ii) development and regulatory activities (Development and Regulatory Activities); and (iii) requirement to supply Orion with the Licensed Product at an agreed upon price (Supply of Licensed Product). We determined that these three commitments represent distinct performance obligations for purposes of recognizing revenue and will recognize license and collaboration revenue or a reduction of expense as we fulfill each performance obligation.

On November 16, 2022, we entered into a Collaboration and Supply Agreement (Tenacia Collaboration Agreement) with Tenacia Biotechnology (Shanghai) Co., Ltd. (Tenacia). Under the terms of the Tenacia Collaboration Agreement, we granted Tenacia an exclusive, royalty-bearing, sublicensable license to certain of our intellectual

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property rights to develop, commercialize and otherwise exploit certain products incorporating certain oral and intravenous formulations of the our product candidate ganaxolone (Licensed Products) in Mainland China, Hong Kong, Macau and Taiwan (collectively, Territory) for the diagnosis, prevention and treatment of certain human diseases, disorders or conditions (Field), initially for the treatment of CDD, TSC and SE (including RSE) (collectively, Initial Indications). The collaboration can be expanded to include additional indications and formulations of ganaxolone pursuant to a right of first negotiation.

Under the terms of the Tenacia Collaboration Agreement, Tenacia agreed to pay us an upfront cash payment of $10 million (Upfront Fee) within forty-five (45) days after the Effective Date, which was received in December 2022. In addition to the Upfront Fee, Tenacia has agreed to make cash payments to us upon the achievement of certain development, regulatory and sales-based milestones related to (i) the Initial Indications and (ii) the first new formulation or pro-drug of ganaxolone or any back-up compound of ganaxolone in a new indication (Selected Product) for which the parties amend the Tenacia Collaboration Agreement in connection with Tenacia's exercise of its right of first negotiation and for which there is no other Licensed Product approved in China (for clarity, the milestone payments under this clause (ii) will only apply to one Selected Product), up to an aggregate amount of $256 million. Of the milestones, $15 million relates to regulatory approvals with separate milestones related to each of oral and intravenous formulations and the Selected Product, and an aggregate of $241 million of sales-based milestones are connected to annual revenue thresholds specific to each of the oral, intravenous and Selected Product formulations of ganaxolone. Tenacia has further agreed to pay us tiered royalty payments based on annual net sales of Licensed Products ranging from the low double digits to the mid-teens for each of the oral formulation, intravenous formulation

and Selected Product formulation of Licensed Products. Tenacia's obligations to pay royalties to us with respect to sales of a Licensed Product in each

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particular jurisdiction of the Territory will commence on the date of first commercial sale in such jurisdiction and expire upon the latest of (i) ten years following the first commercial sale of such Licensed Product in such jurisdiction, (ii) the expiration of the last-to-expire valid claim of any licensed patent rights that covers such Licensed Product in such jurisdiction and (iii) the expiration of all regulatory exclusivities for such Licensed Product in such jurisdiction. Royalty payments are subject to reduction in specified circumstances as set forth in the Tenacia Collaboration Agreement, including if net sales decrease by a certain percentage after the introduction of a generic product.

Tenacia will be primarily responsible for the development of Licensed Products in the Territory and regulatory interactions related thereto, including conducting and sponsoring clinical studies in the Field in the Territory to support regulatory filings in the Territory. All regulatory approvals filed by Tenacia in the Territory will be in the name of and owned by us unless otherwise required by applicable law, in which case such regulatory approvals would be in the name of and owned by Tenacia for the benefit of us. We and Tenacia agreed to enter into clinical and commercial supply agreements pursuant to which we will supply Tenacia with its requirements of Licensed Products necessary for Tenacia to develop and commercialize Licensed Products in the Field in the Territory. The parties entered into one such clinical and commercial supply agreement in May 2023. The agreement contains pricing, delivery, acceptance, payment, termination, forecasting, and other terms consistent with the Tenacia Collaboration Agreement, as well as certain quality assurance, indemnification, liability and other standard industry terms. Tenacia will be responsible for, at Tenacia's sole cost and expense, obtaining regulatory approval and commercializing the Licensed Product in the Field in Mainland China. Tenacia is enrolling enrolled patients in our Phase 3 randomized, double blind, placebo-controlled trial (TrustTSC trial) of adjunctive ganaxolone.

The term of the Tenacia Collaboration Agreement extends for so long as royalties are payable anywhere in the Territory. Subject to the terms of the Tenacia Collaboration Agreement, (i) for a specified period of time after the Effective Date, Tenacia may terminate the Tenacia Collaboration Agreement in its entirety for any or no reason upon written notice to us, and (ii) either party may terminate the Tenacia Collaboration Agreement for the other party's material breach following a cure period or insolvency.

In accordance with the guidance, we identified the following commitments under the arrangement: (i) grant to Tenacia the exclusive rights to develop, commercialize and otherwise exploit Licensed Product in the Field in the Territory (License) and (ii) requirement to supply Tenacia with the Licensed Product at an agreed upon price (Supply of Licensed Product). We determined that these two commitments represent distinct performance obligations for purposes

of recognizing revenue or reducing expense, which we will recognize such revenue or expense, as applicable, as we fulfill these performance obligations.

We have also entered into agreements for commercialization of ganaxolone in other territories with (i) NovaMedica LLC (NovaMedica), whereby NovaMedica has the right to market and sell ganaxolone in Armenia, Azerbaijan, Belarus, Georgia, Kazakhstan, Kyrgyzstan, Moldova, Russia, Tajikistan, Turkmenistan, Ukraine and Uzbekistan, and (ii) Biologix FZCo (Biologix), whereby Biologix has the right to distribute and sell ganaxolone in Algeria, Bahrain, Egypt, Iraq, Jordan, Kingdom of Saudi Arabia, Kuwait, Lebanon, Libya, Morocco, Oman, Qatar, Tunisia and United Arab Emirates. In exchange for these rights, we will be the exclusive supplier of our products to NovaMedica and Biologix on terms set forth in the respective agreements in exchange for a negotiated purchase price for the products. As of the first quarter of 2023, we had initiated limited sales of ZTALMY to NovaMedica to support on-going early access programs associated with patients from the Marigold Trial. We are in the process of initiating a global managed access program with Uniphar Durbin Ireland LTD to support physician access to ZTALMY for appropriate patients with seizures associated with CDD in geographies where there is no available patient access, local regulatory criteria and program eligibility are satisfied, and we do not already have a commercial distribution relationship in place. We continue to assess opportunities in other markets to further expand the distribution and commercialization of ganaxolone globally.

Research and Development Expenses

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

- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;
- expenses incurred under agreements with clinical research organizations (CROs) and investigative sites that conduct our clinical trials and preclinical studies;
- the cost of acquiring, developing and manufacturing clinical trial materials;
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, enterprise technology, and other supplies;

- costs associated with preclinical activities and regulatory operations; and
- costs associated with developing new formulations and prodrugs of ganaxolone.

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

We have and will incur substantial costs beyond our present and planned clinical trials in order to file an NDA and Supplemental NDAs, or MAAs outside the U.S., for ganaxolone for various clinical indications, and in each case, the nature, design, size and cost of further clinical trials and other studies will depend in large part on the outcome of preceding studies and trials and discussions with regulators. It is difficult to determine with certainty the costs and duration of our current or future clinical trials and preclinical studies, or if, when or to what extent we will generate revenue from the commercialization and sale of ganaxolone if we obtain regulatory approval in other indications. We may never succeed in achieving regulatory approval for ganaxolone in other indications and, if approved, we may not be successful in commercialization of ganaxolone in other indications. The duration, costs and timing of clinical trials and development of ganaxolone will depend on a variety of factors, including the uncertainties of future clinical trials and preclinical studies, uncertainties in clinical trial enrollment rate and significant and changing government regulation.

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In addition, the probability of success for our clinical programs will depend on numerous factors, including competition, manufacturing capability and commercial viability. See the *Risk Factors* section of our Annual Report on Form 10-K filed on March 5, 2024 for more information with respect to such factors. Our continued 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.

Selling, General and Administrative Expenses

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

Restructuring Costs

Restructuring costs primarily consist of severance payments, employee benefits and related costs, as well as noncash stock compensation expense and contract termination costs. Restructuring costs are expensed when incurred.

Cost of Product Revenue

Cost of product revenue includes the cost of inventory sold, which includes direct manufacturing and supply chain costs. Also included in Cost of product revenue are royalty payments owed to Purdue Neuroscience Company (Purdue) and Ovid in accordance with the respective license agreements. We began capitalizing Inventory related to

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ZTALMY subsequent to the March 2022 FDA approval of ZTALMY, as the related costs were expected to be recoverable through the commercialization and subsequent sale of ZTALMY. Prior to FDA approval of ZTALMY, costs estimated at approximately $2 million $2.0 million for commercially saleable product and materials were incurred and included in Research and development expenses. As a result, Cost of product revenues related to ZTALMY initially reflected a lower average per unit cost of materials, and will continue continued to reflect a lower average per unit cost of materials through do so into the second quarter of 2024, as previously expensed inventory is was utilized for commercial production and sold to customers. We expect Cost of product revenues related to ZTALMY to begin to reflect the current on-going average per unit cost of materials for the remainder of 2024 and thereafter.

Interest Income

Interest income consists principally of interest earned on Cash and cash equivalents and Short-term investments balances.

Interest Expense

Interest expense consists of interest expense and amortization of debt discount related to our Notes Payable and our Revenue Interest Financing Payable.

Other Income, net

Other income and expense consist principally of non-operational transactions, gains or losses on disposal of fixed assets held for sale, foreign currency transactions, and fair value adjustments.

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Results of Operations

Product Revenue, net

We recognized $7.5 million $8.0 million and $3.3 million $4.2 million of ZTALMY net product revenue for the three months ended March 31, 2024 June 30, 2024 and March 31, 2023 June 30, 2023, respectively. We recognized $15.5 million and $7.6 million of ZTALMY net product revenue for the six months ended June 30, 2024 and June 30, 2023, respectively. The increase increases in the both the three and six months ended March 31, 2024 June 30, 2024 compared to the 2023 period was periods were primarily due to an increase in patients in the three and six months ended March 31, 2024 June 30, 2024 as ZTALMY had only recently become available for commercial sale and shipment in the comparable 2023 period. periods.

Federal Contract Revenue

We recognized $0.2 million $0.1 million and $7.0 million $1.8 million of federal contract revenue for the three months ended March 31, 2024 June 30, 2024 and 2023, June 30, 2023, respectively, as a result of the BARDA Contract. We recognized $0.2 million and $8.9 million of federal contract revenue for the six months ended June 30, 2024 and June 30, 2023, respectively, as a result of the BARDA Contract. The decrease decreases in both the three and six months ended March 31, 2024 June 30, 2024 compared to the 2023 period periods primarily related to expenses incurred in connection with on-going validation of a new third-party supplier of ganaxolone API in the 2023 period. periods, and no revenue related to the base period funding in 2024. As of December 31, 2023, the entire base period funding had been recorded, resulting in no revenue for the base period funding in the three and six months ended June 30, 2024.

Collaboration Revenue

Collaboration revenue, which resulted from our agreement with Biologix, was less than $0.1 million for the three and six months ended March 31, 2024. No collaboration revenue was recorded in the three months ended March 31, 2023 June 30, 2024 and June 30, 2023.

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Research and Development Expenses

We record direct Research and development expenses, consisting principally of external costs, such as fees paid to investigators, consultants, central laboratories and CROs in connection with our clinical trials, and costs related to manufacturing, to specific product development programs. We do not allocate costs related to purchasing clinical trial materials, employee and contractor-related costs, costs associated with our facility expenses, including depreciation or other indirect costs, to specific product programs because these costs are deployed across multiple product programs under research and development and, as such, are not separately classified. The table below shows our research and development expenses incurred with respect to each active program. The primary drivers of our

Research and development expenditures are currently in our product development programs for CDD, RSE, and TSC. We did not allocate research and development expenses to any other specific product development programs during the periods presented (in thousands):

	Three Months Ended March 31,		Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023	2024	2023
CDKL5 deficiency disorder (1)	$ 218	$ 1,240	$ 209	$ 816	$ 427	$ 2,055
PCDH19-related epilepsy (2)	47	94	—	31	47	125
Tuberous Sclerosis (3)	2,827	3,788	3,816	3,585	6,643	7,374
Drug Development – Suspension (4)	1,538	270	1,229	888	2,767	1,158
Oral Indications Subtotal	4,630	5,392	5,254	5,320	9,884	10,712
Status epilepticus (5)	4,216	3,002	4,263	3,578	8,479	6,580
Drug Development – IV (6)	1,423	8,489	870	939	2,293	9,428
IV Indications Subtotal	5,639	11,491	5,133	4,517	10,772	16,008
Other research and development (7)	1,864	1,196	1,168	632	3,032	1,828
Indirect research and development (8)	11,985	9,854	9,342	10,943	21,327	20,797
Total	$ 24,118	$ 27,933	$20,897	$21,412	$45,015	$49,345

(1) The decrease in the three months ended March 31, 2024 June 30, 2024 compared to the 2023 period was due to decreased clinical trial activity in the three months ended March 31, 2024 June 30, 2024. The decrease in the six months ended June 30, 2024 compared to the 2023 period was due to decreased clinical trial activity in the six months ended June 30, 2024 as compared to the 2023 period and increased activity associated with the MAA application and review in the three six months ended March 31, 2023 June 30, 2023 with no comparable costs in the 2024 period.

(2) Costs remained relatively consistent in the three and six months ended June 30, 2024 compared to the 2023 periods.

(3) The decrease increase in the three months ended March 31, 2024 June 30, 2024 compared to the 2023 period was primarily due to increased Phase 3 TSC trial activity in the 2024 period. The decrease in the six months ended June 30, 2024 compared to the 2023 period was due primarily to reduced clinical activity.

(3) The decrease increased global site activity in the three months ended March 31, 2024 compared to the 2023 period which was due primarily to decreased partially offset by increased Phase 3 TSC trial activity in the three months ended March 31, 2024 and increased global site activity in the 2023 2024 period.

(4) The increase increases in the three and six months ended March 31, 2024 June 30, 2024 compared to the 2023 period was periods were due primarily to higher manufacturing development activity related to clinical trial batches than in the prior 2023 period. periods.

(5) The increase increases in the three and six months ended March 31, 2024 June 30, 2024 compared to the 2023 period was periods were due primarily to increased RAISE and RAISE II Phase 3 trial activity. and close out activities in the 2024 period.

(6) The decrease decreases in the three and six months ended March 31, 2024 June 30, 2024 compared to the 2023 period was periods were due primarily to the start-up costs associated with validation of a new third-party U.S. supplier of ganaxolone API in the 2023 period with no comparable costs in the three months ended March 31, 2024. periods.

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(7) The increase in the three and six months ended March 31, 2024 June 30, 2024 compared to the 2023 period was periods were due primarily to an increase in toxicology and other safety studies, as well as an increase in post-marketing studies. Other research and development expenses include external expenses associated with preclinical development of ganaxolone, including safety studies, stability studies, preclinical studies, including animal toxicology and pharmacology studies and studies related to post-marketing commitments, and other professional fees.

(8) The increase decrease in the three months ended March 31, 2024 June 30, 2024 compared to the 2023 period was primarily related to decreases in personnel costs and manufacturing activities. The increase in the six months ended June 30, 2024 compared to the 2023 period was primarily related to increased costs related to software and personnel, costs which were partially offset by a decrease in support of our increased activity in preclinical studies, including safety and post-marketing studies, and manufacturing activities. Indirect research and development expenses include personnel costs and non-study specific research and development costs.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $18.6 million $16.7 million and $15.2 million $35.3 million for the three and six months ended March 31, 2024 June 30, 2024, respectively. Selling, general and 2023, administrative expenses were $15.7 million and $30.9 million for the three and six months ended June 30, 2023, respectively. The primary drivers of the increase for the three months ended March 31, 2024 June 30, 2024 compared to the 2023 period were $1.5 million $0.8 million in increased commercial costs, $1.0 million professional fees, $0.7 million in increased stock-based compensation costs, $0.6 million in increased personnel costs, and $0.6 million in increased professional fees, commercial costs, which were partially offset by a decrease of $0.5 million in decreased personnel costs, $0.3 million in decreased software related expenses. expenses and $0.3 million in decreased consulting fees. The primary drivers of the increase for the six months ended June 30, 2024 compared to the 2023 period were $2.1 million in increased commercial costs, $1.7 million in increased professional fees and $1.7 million in increased stock-based compensation costs, which were partially offset by $0.6 million in decreased software related expenses and $0.5 million in decreased consulting costs.

Restructuring Costs

Restructuring costs were approximately $2.0 million for the three and six months ended June 30, 2024, with no comparable costs in 2023, and primarily consisted of severance payments, employee benefits and related costs, as well as noncash stock compensation expense and contract termination costs.

Interest Income

Interest income was $1.5 million $1.1 million and $2.6 million for the three and six months ended March 31, 2024 June 30, 2024 compared to $2.3 million $2.1 million and $4.5 million for the three and six months ended March 31, 2023 June 30, 2023 and consisted of interest earned on Cash and cash equivalents and Short-term investments. The decrease was decreases were due to a decrease in Cash and cash equivalent and Short-term investments in the three and six months ended March 31, 2024 June 30, 2024 as compared to the three and six months ended March 31, 2023 June 30, 2023.

Interest Expense

Interest expense was $4.3 million $4.6 million and $9.0 million for the three and six months ended March 31, 2024 June 30, 2024, respectively, compared to $4.1 million $4.2 million and $8.4 million for the three and six months ended March 31, 2023 June 30, 2023. Interest expense for the three six months ended March 31, 2024 June 30, 2024 included $2.2 million $4.6 million of interest paid and $0.5 million $1.0 million of debt amortization in connection with our Note Payable (Note 10 in accompanying notes to consolidated financial statements), and $1.6 million $3.2 million of non-cash interest expense and $0.1 million $0.2 million of debt amortization related to our Revenue interest financing payable (Note 11 in accompanying notes to consolidated financial statements). Interest expense for the six months ended June 30, 2023 included $4.3 million of interest paid and $1.0 million of debt amortization in connection with our Note Payable, and $2.9 million of non-cash interest expense and $0.1 million of debt amortization related to our revenue interest financing payable,

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Other Income (expense), net

Other income (expense), net was less than $0.1 million for each of the three and six months ended March 31, 2024 June 30, 2024 and 2023. Other income (expense), net consists principally of non-operational transactions, gains or losses on disposal of fixed assets held for sale, foreign currency transactions, and fair value adjustments adjustments.

Liquidity and Capital Resources

Since inception, we have incurred negative cash flows from our operations, and other than for the three months ended September 30, 2022 due to a one-time net gain from the sale of our PRV, we have incurred net losses. We incurred a Net loss of $38.7 million $74.5 million for the three six months ended March 31, 2024 June 30, 2024. Our Cash used in operating activities was $37.5 million $68.3 million for the three six months ended March 31,

2024 June 30, 2024 compared to $41.5 million $65.8 million for the three six months ended March 31, 2023 June 30, 2023. Historically, we have financed our operations principally through the sale of common stock, notes payable, preferred stock and convertible debt.

In August 2022, we received a letter from Purdue in which Purdue claimed that it was owed $5.5 million by us from the sale of the PRV pursuant to the Purdue License Agreement. We responded to Purdue that we did not agree with their claim. In February 2024, following discussions with Purdue, we agreed to pay Purdue $4 million $4.0 million in respect of its

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claim. We paid the first installment of $2 million $2.0 million in March 2024. At March 31, 2024 we had $2.0 million accrued related to 2024 and the second $2 million installment which will be paid on or before June 15, 2024. of $2.0 million in June 2024.

In September 2023, in connection with the amended Equity Distribution Agreement (EDA) with JMP Securities LLC (JMP), we received net proceeds totaling approximately $25.9 million from the sale of 3.7 million shares of our common stock at an average price of $7.17 per share.

As of March 31, 2024 June 30, 2024, we had Cash and cash equivalents and $64.7 million. We did not have any Short-term investments as of $113.3 million June 30, 2024. We believe that our existing Cash and cash equivalents and Short-term investments as of March 31, 2024 June 30, 2024 will be sufficient to fund our operating expenses and capital expenditure requirements as well as maintain the minimum cash balance required under our debt facility, into the first second quarter of 2025. This expectation includes cost reduction activities that are being were implemented with expected impact beginning in the second quarter of 2024. We will need to secure additional funding in the future, from one or more equity or debt financings, government funding, collaborations, licensing transactions, other commercial transactions or other sources in order to carry out all of our commercialization and planned research and development activities with respect to ganaxolone.

Oaktree Credit Agreement

On May 11, 2021 (Closing Date) and as amended on May 17, 2021, May 23, 2022, October 28, 2022 and October 28, 2022 June 6, 2024 (Credit Agreement), we entered into the Credit Agreement with Oaktree Fund Administration, LLC as administrative agent (Oaktree) and the lenders party thereto (collectively, Lenders) that provided for a five-year senior secured term loan facility in an aggregate original principal amount of up to $125.0 million that was available to us in five tranches (collectively, Term Loans). As of March 31, 2024 June 30, 2024, we had drawn on three tranches with no additional funds available thereunder.

We received $15.0 million of Tranche A-1 Term Loans on the Closing Date, $30.0 million of Tranche A-2 Term Loans in September 2021 after formal acceptance by the FDA of an NDA filing relating to the use of ganaxolone in the treatment of CDD, and $30.0 million of Tranche B Term Loans in March 2022 after FDA approval of ZTALMY for CDD.

In connection with the second amendment to the Credit Agreement, we prepaid $15.0 million of the Tranche B Term Loans in June 2024 as well as the associated prepayment penalties, exit fee and accrued interest. Additionally, during the six months ended June 30, 2024, we began paying the required quarterly principal payments. As of June 30, 2024, the loans under the Credit Agreement consisted of $58.1 million of previously drawn Term Loans with no additional funds available thereunder.

The Term Loans mature on May 11, 2026 (Maturity Date). The Term Loans bear interest at a fixed per annum rate (subject to increase during an event of default) of 11.50%, and we are required to make quarterly interest payments until the Maturity Date. We are also required to make quarterly principal payments beginning on June 30, 2024 in an

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amount equal to 2.5% of the aggregate amount of the previously drawn Term Loans, and continuing until 2025, at which time we are required to make quarterly principal payments in an amount equal to 5.0% of the aggregate amount of the previously drawn Term Loans outstanding on June 30, 2024, and continuing until the Maturity Date. On the Maturity Date, we are required to pay in full all outstanding Term Loans and other amounts owed under the Credit Agreement.

At the time of borrowing any tranche of the Term Loans, we were required to pay an upfront fee of 2.0% of the aggregate principal amount borrowed at that time.

In connection with the Revenue Interest Financing Agreement with Sagard as described below, on October 28, 2022, we entered into an amendment to the Credit Agreement to, among other things, allow for the consummation of the Revenue Interest Financing Agreement and the transactions thereunder, and paid $0.3 million in administrative fees in connection with the execution of the amendment. In addition, the amendment increased the exit fee due by us upon any repayment, whether as a prepayment or a scheduled repayment, of the principal of the loans under the Credit Agreement from 2.00% to 2.67%.

Sagard Financing Agreement

In October 2022, we entered into a revenue interest financing agreement (the Revenue Interest Financing Agreement) with Sagard Healthcare Royalty Partners, LP (Sagard) pursuant to which we received $32.5 million.

In exchange for the Investment Amount, we agreed to make quarterly payments to Sagard (Payments) as follows: (i) for each calendar quarter from and after the closing date of such financing through and including the quarter ended June 30, 2026, an amount equal to 7.5% of (a) our U.S. net sales of ZTALMY and all other pharmaceutical products that contain ganaxolone (Net Sales), in each case with any dosage form, dosing regimen, or strength, or any improvements related thereto (collectively, Included Products) and (b) certain other payments received by us in connection with the manufacture, development and sale of the Included Products in the U.S. (Other Included Payments, and, together with Net Sales, Product Revenue); and (ii) for each calendar quarter following the calendar quarter ended June 30, 2026, an amount equal to (x) 15.0% of the first $100 million in annual Product Revenue of the Included Products and (y) 7.5% of annual Product Revenue of the Included Products in excess of $100 million.

The Payments are subject to a hard cap equal to 190% ($61.8 million) of the Investment Amount (Hard Cap). Sagard's right to receive payments will terminate when Sagard has received payments in respect of the Included Products, including any additional payments described below, equal to the Hard Cap. Further, we have the right to make voluntary prepayments to Sagard, and such payments will be credited against the Hard Cap.

If Sagard has not received aggregate payments equaling at least 100% of the Investment Amount by December 31, 2027 or at least 190% of the Investment Amount by December 31, 2032 (each, Minimum Amount), then we will be obligated to make a cash payment to Sagard in an amount sufficient to gross up Sagard up to the applicable Minimum Amount within a specified period of time after each reference date.

BARDA Contract

In September 2020, we entered into a contract (BARDA Contract) with the Biomedical Advanced Research and Development Authority (BARDA), a division of the U.S. Department of Health and Human Services' Office of the Assistant Secretary for Preparedness and Response. Under the BARDA Contract, we received an award of up to an estimated $51 million for development of IV-administered ganaxolone for the treatment of RSE. The BARDA Contract provides for funding to support, on a cost-sharing basis, the completion of a Phase 3 clinical trial of IV-administered ganaxolone in patients with RSE, which covers the RAISE trial, funding of pre-clinical preclinical studies to evaluate IV-administered ganaxolone as an effective treatment for RSE due to chemical nerve gas agent exposure, and funding of certain ganaxolone manufacturing scale-up and regulatory activities. In March 2022, we entered into an amendment with BARDA to extend the end date of our base performance period for funding under the BARDA Contract from September 1, 2022 to December 31, 2023. In September 2022, we entered into an amendment with BARDA that, among other things, (i) provides for the exercise of BARDA's option under the BARDA Contract to support U.S. onshoring of the

manufacturing capabilities for ganaxolone API (Option 2), (ii) changes the end date of our performance period under Option 2 from December 31, 2026 to July 31, 2025, (iii) increases the government cost share amount under Option 2 from approximately $11.5 million to approximately $12.3 million, and (iv) increases our cost share amount under Option 2 from approximately $4.9 million to approximately $5.3 million. In September 2023, we entered into an amendment with BARDA to extend the end date of our base performance period for funding under the BARDA Contract from December 31, 2023 to September 30, 2024.

The BARDA Contract consists of an approximately four-year base period, including the extension periods, during which BARDA agreed to provide up to approximately $21 million of funding for the RAISE trial on a cost share basis and funding of additional preclinical studies of ganaxolone in nerve agent exposure models. As of December 31, 2023, the entire base period funding of approximately $21 million had been recorded. Following successful completion of the RAISE trial and preclinical studies in the base period and extension periods, the BARDA Contract provides for approximately $31 million of additional BARDA funding for three options in support of ganaxolone manufacturing, supply chain, clinical, regulatory and toxicology activities, including the $12.3 million exercise of Option 2 as described above. Under the BARDA Contract, we will be responsible for cost sharing in the amount of approximately $33 million and BARDA will be responsible for approximately $52 million if all development options are completed. The contract period-of-performance (base period plus option exercises) is up to approximately five years.

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Equity Financings

In connection with the closing of an equity financing in November 2022 and the December 2022 exercise of the related underwriters' option, we issued a total of 12,421,053 shares of common stock and 2,105,264 pre-funded warrants to purchase common stock in an underwritten public offering resulting in aggregate net proceeds of $64.5 million, after deducting the underwriting discounts and commissions and offering expenses paid or payable by us.

Equity Distribution Agreement

On July 9, 2020, we entered into an Equity Distribution Agreement (EDA) with JMP Securities LLC (JMP), as amended by the March 31, 2023 Amendment No. 1 to the EDA (Amended EDA), to create an at the market equity program under which we from time to time may offer and sell shares of our common stock without a specified maximum aggregate offering price. The Amended EDA was entered into in connection with our filing of a Registration Statement on Form S-3 (File No. 333-271041) with the SEC (the 2023 Registration Statement), which includes a prospectus supplement covering the offering, issuance and sale by us of up to $75,000,000 of shares of common stock that may be issued and sold under the Amended EDA. Subject to the terms and conditions of the Amended EDA, JMP will be entitled to a commission of up to 3.0% of the gross proceeds from each sale of shares of our common stock. We did not sell any shares of our common stock during each of the three and six months ended March

31, 2024 June 30, 2024 and March 31, 2023 June 30, 2023 under the EDA. As of June 30, 2024, we had up to $48.6 million of shares of common stock that may be issued and sold under the Amended EDA.

Cash Flows

Operating Activities. Cash used in operating activities was $37.5 million $68.3 million and $41.5 million $65.8 million for the three six months ended March 31, 2024 June 30, 2024 and 2023, respectively. Excluding the noncash impacts primarily related to depreciation and amortization, debt issuance costs, interest accretion net of cash paid, stock-based compensation, and changes in the net contract assets/liabilities related to the Orion, Tenacia and Biologix Collaboration Agreements, the change in cash used in operating activities for the three six months ended March 31, 2024 June 30, 2024 compared to the same period in 2023, was primarily the result of decreases in the changes in accounts payable, accrued expenses, including the $2.0 million $4.0 million installment to be paid to Purdue, other long term-liabilities, prepaid expenses, other current assets, inventory and accounts receivable as and an increase in operating expenses remained relatively consistent. for the six months ended June 30, 2024 as compared to the 2023 period, a portion of which was due to restructuring costs including $0.7 million of personnel related restructuring costs paid in the six months ended June 30, 2024.

Investing Activities. Cash provided by investing activities for the three six months ended March 31, 2024 June 30, 2024 represents $20.9 million $29.9 million in maturities of Short-term investments. investments, which was partially offset by less than $0.1 million in purchases

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of lab equipment. Cash used in investing activities for the three six months ended March 31, 2023 June 30, 2023 represents $52.0 million in purchases of short-term Short-term investments, which was partially offset by $5.0 million in maturities of Short-term investments.

Financing Activities. Cash provided by used in financing activities for the three six months ended March 31, 2024 June 30, 2024 represents $17.7 million in principal prepayments, including associated financing costs and exit fees, on our Long-term debt and $0.3 million in proceeds from the exercise of stock options. Cash used in provided by financing activities for the three six months ended March 31, 2023 June 30, 2023 was less than $0.2 million $0.1 million and represented $0.5 million in proceeds from the exercise of stock options, which was partially offset by $0.4 million of other financing activities.

Funding Requirements

Since inception, we have incurred negative cash flows from our operations, and other than for the three months ended September 30, 2022 due to a one-time net gain from the sale of our PRV, we have incurred net losses. We incurred a Net loss losses of $38.7 million $35.8 million and $74.5 million for the three and six months ended March 31, 2024. June 30, 2024, respectively. We have generated limited product revenues, and there is no assurance that profitable operations will be achieved in the future, and if achieved, could be sustained on a continuing basis.

We had Cash and cash equivalents and Short-term investments of $113.3 million $64.7 million as of March 31, 2024 June 30, 2024. We believe that our existing Cash and cash equivalents and Short-term investments as of March 31, 2024 June 30, 2024 will be sufficient to fund our operating expenses and capital expenditure requirements as well as maintain the minimum cash balance required under our debt facility, into the first second quarter of 2025. This expectation includes cost reduction activities that are being were implemented with expected impact beginning in the second quarter of 2024. We will need to secure additional funding in the future, from one or more equity or debt financings, government funding, collaborations, licensing

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transactions, other commercial transactions or other sources in order to carry out all of our commercialization and planned research and development activities with respect to ganaxolone. 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, or engage in federal contracts or other partnerships. If we raise additional funds through the issuance of convertible debt securities, these securities could have rights senior to those of our common stock and could contain covenants that restrict our operations. There can be no assurance that we will be able to obtain additional equity or debt financing on terms acceptable to us, if at all. Our failure to obtain sufficient funds on acceptable terms when needed could have a negative impact on our business, results of operations, and financial condition.

Our future capital requirements will depend on many factors, including:

- the results of our preclinical studies and clinical trials;
- the development, formulation and commercialization activities related to ganaxolone, including ZTALMY;
- the scope, progress, results and costs of researching and developing ganaxolone, including ZTALMY, or any other future product candidates, and conducting preclinical studies and clinical trials;
- the timing of, and the costs involved in, obtaining regulatory approvals for ganaxolone, including ZTALMY in indications other than CDD in the U.S., EU, or other significant markets, and any other future product candidates in these markets;
- the cost of commercialization activities for ZTALMY in CDD in the U.S., including marketing, sales and distribution costs;
- the cost of commercialization activities for ZTALMY, ganaxolone in any other indications, or any other future product candidates, are approved for sale, including marketing, sales and distribution costs;
- the cost of manufacturing and formulating ganaxolone, or any other future product candidates, to internal and regulatory standards for use in preclinical studies, clinical trials and, if approved, commercial sale;

- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of such agreements;
- our ability to receive funding under the BARDA Contract;
- our expectations regarding the amount and timing of milestone and royalty payments owed to us pursuant to our collaboration and supply agreements with Orion for the commercialization of ganaxolone in Europe, our collaboration and supply agreements with Tenacia for the commercialization of ganaxolone in Mainland China, Hong Kong, Macau and Taiwan and our exclusive distribution and supply agreement with Biologix for the distribution and supply of ganaxolone in the Middle East and North Africa region;
- our expectations regarding the amount and timing of milestone and royalty payments owed by us pursuant to our Revenue Interest Financing Agreement with Sagard;
- any product liability, infringement or other lawsuits related to ZTALMY or other indications being developed for ganaxolone and, if approved, 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, ZTALMY in CDD and on future approved products, if any.

Please see the *Risk Factors* section included in our Annual Report on Form 10-K for the year ended December 31, 2023 filed with the SEC on March 5, 2024 for additional risks associated with our substantial capital requirements.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements that have or are reasonably likely to have a current or future effect on our financial condition, changes in financial condition, revenues or expenses, results of operations, liquidity, capital expenditures or capital resources that are material to investors.

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, 2024 June 30, 2024, there were no significant changes to our critical accounting policies from those described in our annual consolidated financial statements for the year ended December 31, 2023, which we included in our Annual Report on Form 10-K and was filed with the SEC on March 5, 2024.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

We are a smaller reporting company as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended (Exchange Act), and are not required to provide the information under this item.

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Item 4. Controls and Procedures

(a) **Evaluation of Disclosure Controls and Procedures**.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, has evaluated the effectiveness of our disclosure controls and procedures (as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of the end of the period covered by this Quarterly Report on Form 10-Q to ensure that the information required to be disclosed by us in the 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 we file or submit under the Exchange Act is accumulated and communicated to our management, including our Chief Executive Officer and Chief Financial Officer, to allow timely decisions regarding required disclosures. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost benefit relationship of possible controls and procedures. Based on such evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of March 31, 2024 June 30, 2024.

(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(d) and 15d-15(d) of the Exchange Act that occurred during the quarter ended March 31, 2024 June 30, 2024 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 Other than as described below, 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.

On June 5, 2024, a securities class action lawsuit captioned Bishins v. Marinus Pharmaceuticals, Inc., et. al., Case 2:24-cv-02430, was filed against us and certain of our officers in the U.S. District Court for the Eastern District of Pennsylvania. The complaint alleges violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934, as amended (Exchange Act), and Rule 10b-5 promulgated thereunder on the basis of purportedly materially false and misleading statements and omissions concerning our RAISE and RAISE II clinical trials. The complaint seeks, among other things, unspecified damages, attorneys' fees, expert fees, and other costs. Motions to appoint lead plaintiffs and lead counsel for the action were due on August 5, 2024. One purported stockholder filed a motion by the August 5 deadline. That motion is currently pending, and we intend to move to dismiss the complaint once a schedule has been set. We intend to vigorously defend against this action.

Item 1A. Risk Factors

There have been no material changes to the risk factors previously disclosed in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023, except as follows:

We and certain of our officers have been named as defendants in a pending securities class action lawsuit. This lawsuit, and potential similar or related lawsuits, could result in substantial damages, divert management's time and attention from our business, and have a material adverse effect on our results of operations. This lawsuit, and any other lawsuits to which we are subject, will be costly to defend and is uncertain in its outcome.

On June 5, 2024, a securities class action lawsuit captioned Bishins v. Marinus Pharmaceuticals, Inc., et. al., Case 2:24-cv-02430 was filed against us and certain of our officers in the U.S. District Court for the Eastern District of Pennsylvania. The complaint alleges violations of Sections 10(b) and 20(a) of the Securities and Exchange Act of 1934, as amended (Exchange Act) and Rule 10b-5 promulgated thereunder on the basis of purportedly materially false and misleading statements and omissions concerning our RAISE and RAISE II clinical trials. The complaint seeks, among other things, unspecified damages, attorneys' fees, expert fees, and other costs. Motions to appoint

lead plaintiffs and lead counsel for the action were due on August 5, 2024. One purported stockholder filed a motion by the August 5 deadline. That motion is currently pending, and we intend to move to dismiss the complaint once a schedule has been set.

We intend to vigorously defend against this action. However, whether or not the claim is successful, litigation is often expensive and can divert management's attention and resources from other business concerns, which could adversely affect our business.

We currently are not able to estimate the possible cost to us from this action, as the pending lawsuit is currently at an early stage, and we cannot be certain how long it may take to resolve the pending lawsuit or the possible amount of any damages that we may be required to pay. If we are ultimately required to pay significant defense costs, damages or settlement amounts, such payments could adversely affect our operations.

We may be the target of similar litigation in the future. The market price of our common stock has experienced and may continue to experience volatility, and in the past, companies that have experienced volatility in the market price of their stock have been subject to securities litigation. Any future litigation could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business. We maintain liability insurance; however, if any costs or expenses associated with the pending lawsuit or any other litigation exceed our insurance coverage, we may be forced to bear some or all costs and expenses directly, which could adversely affect our business, financial condition, results of operations or stock price.

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An unfavorable outcome with the pending lawsuit or any other litigation could have a material adverse effect on our business, reputation, financial condition, results of operations and cash flows, which, in turn, may result in or may contribute to an inability by us to meet the financial covenant contained in the RIFA.

Our future success is dependent on the successful clinical development, regulatory approval and continued commercialization of ganaxolone, which is being studied in several indications and will require significant capital resources and years of additional clinical development effort.

In March 2022, we received FDA approval of ZTALMY for CDD in the U.S., and in July 2023, we received EC approval of ZTALMY for CDD in the EU, and we plan to develop ganaxolone in several other geographic regions and additional indications in oral and IV formulations. As a result, our business is dependent on our ability to successfully complete clinical development, scale-up manufacturing, obtain regulatory approval, and, if it is approved, commercialize ganaxolone in a timely manner. We cannot commercialize additional indications or formulations of ganaxolone in the U.S. in any other indication without first obtaining regulatory approval from the FDA; similarly, we cannot commercialize additional indications or formulations of ganaxolone outside of the U.S. without obtaining regulatory approval from comparable foreign regulatory authorities. Before obtaining regulatory approvals for the commercial sale of ganaxolone for a target indication, we must demonstrate with substantial evidence gathered in preclinical studies and clinical trials and, with respect to approval in the U.S., 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.

Ganaxolone is metabolized extensively in animals and humans. During the development of CDD, one major metabolite (M2) was present in plasma of humans that was not found in plasma of rats or dogs. The chemical structure of M2 has been identified. An activity assay, dose range finding study in rats and an in vivo micronucleus with comet analysis for the detection of genotoxicity have been conducted and the results submitted to the FDA. The M17 in vitro drug-drug interaction (DDI) study was submitted in August 2023, and the M17 in vivo PK study with Brain Penetrance was submitted in December 2023. Results from additional preclinical studies are required by the FDA as post-marketing requirement(s). These include: 2-year carcinogenicity studies of ganaxolone and the major human unconjugated plasma metabolite, M2, in rats; a 26-week carcinogenicity of ganaxolone in transgenic mice; and a juvenile animal toxicity study of the major human unconjugated plasma metabolite, M2, in rats. Additional post-marketing requirements included: phase 1 renal and hepatic impairment studies and a thorough QTc study; and extractable/leachable study results on the container closure system. The Phase 1 renal impairment study commitment was submitted to the FDA in May 2022. The Phase 1 hepatic impairment study and the thorough QTc study were completed and submitted to the FDA in December 2022. The extractable/leachable study results on the container closure system were submitted to the FDA in July 2023. We plan to complete the required FDA studies within the required FDA timeframe. However, there is a risk that the studies could take longer than expected to complete or the studies may have adverse findings which may require additional investments and have the potential to materially impact the label or our ability to market ZTALMY.

In connection with the EC approval of ZTALMY for CDD, we have several post-marketing authorization measures. The clinical study report (CSR) for Study 1042-HME-1001 was submitted in September 2023. The ganaxolone Steady-State Metabolite Study report, the final Study 1042-CDD-3001 CSR with the open-label trial completion, the M17 in vitro DDI study, and the M17 in vivo PK study with Brain Penetrance were submitted in December 2023. The remaining post-marketing authorization measures include: participating in Study LLF001 (CANDID observational study) and providing annual updates; participating in the CDD-IPR-CDD-0 CDKL5 Deficiency

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Disorder International Patient Registry and providing six monthly updates; conducting a toxicity study with a sediment dwelling organism and an updated Environmental Risk Assessment; developing a sodium benzoate-free suspension and assessing the compatibility of the oral suspension with food, drinks, enteral tubes, shake time and stand time; conducting a 26-Week Oral Gavage Toxicity Study of M2; conducting a M2 Embryo-fetal Development study; and conducting a 26-week Oral Gavage Carcinogenicity Study of ganaxolone and M2. The EMA also requested weight of evidence (WoE) WoE assessments to evaluate the need for a 2-year carcinogenicity study in rats with ganaxolone, a 2-year carcinogenicity study in rats with M2, and a juvenile toxicity study with M2. While we expect to be able complete the remaining required studies within the requested EMA timeframe, there is a risk that the studies could take longer or the studies may have adverse findings which may require additional investments and have the potential to materially impact the label or our ability to marker ZTALMY.

We are conducting In January 2021, we enrolled the first patient in the RAISE trial, a randomized, double-blind, placebo-controlled trial in patients with RSE, which is a life-threatening medical condition involving prolonged seizure activity in seriously ill patients. who have failed two or more antiseizure medications. The RAISE trial requires expertise has

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approximately 70 trial sites, primarily in electroencephalogram (EEG) interpretation, which may be subject the U.S. and Canada. It was designed to variability, and enroll approximately 124 patients who were randomized to receive ganaxolone or placebo added to standard of care. We reached alignment with the FDA or foreign regulatory authorities could find the data generated in this trial inadequate or difficult to interpret, which could delay, limit or prevent regulatory approval on a protocol amendment, including a proposal for this indication. In April 2024, the independent Data Monitoring Committee (DMC) completed its review an interim analysis when two-thirds of the RAISE patients (approximately 82) had completed assessment of the primary and key secondary trial endpoints. The enrollment target for the interim analysis. The analysis was completed in the first quarter of 2024. On April 15, 2024, we announced that the trial did not meet the pre-defined interim analysis stopping criteria on the co-primary endpoints, and the DMC recommendation was that the trial may continue without modification. endpoints. We have decided to complete enrollment in the RAISE trial at 100 patients with patients. On June 17, 2024, we announced top-line results expected in the summer of 2024. Based on this interim analysis, there is a substantial risk that the Phase 3 clinical trial of ganaxolone in RAISE will not generate data that is sufficient to support regulatory approvals for this indication. Additionally, the clinical trial endpoints of the RAISE trial, are based which showed that the trial met its first co-primary endpoint, with a statistically significant proportion of patients achieving SE cessation within 30 minutes of initiating IV ganaxolone compared to placebo: 80% vs. 13%, respectively ($p<0.0001$). However, the trial failed to achieve statistical significance on treatment outcomes, including its second co-primary endpoint, the proportion of patients not progressing to IV anesthesia for 36 hours following initiation of anesthesia IV ganaxolone compared to placebo: 63% vs. 51%, respectively ($p=0.162$). The incidence of SAEs was similar between the treatment and placebo arms (n=19 for treatment of RSE. Practice variability IV ganaxolone, n=18 for placebo), with hypotension being more commonly seen in the use IV ganaxolone arm. Other secondary endpoints, including escalation of treatment with either IV ASMs or IV anesthesia for SE treatment could adversely impact within 24 hours and reduction in electroencephalogram (EEG) seizure burden, at 36 hours favored ganaxolone. We continue to analyze the ability to show a treatment effect with ganaxolone. Even if the full RAISE trial shows that ganaxolone is effective, there is dataset and plan to request a risk that meeting with the FDA will require more safety data generated with to discuss a potential path forward for IV ganaxolone at the doses given to patients in this trial before approving an NDA or require post approval commitments to generate additional safety data as a condition of approval ganaxolone for use in RSE.

In August 2021, we reported data from an open-label, single-arm Phase 2 trial evaluating the safety and effectiveness of adjunctive oral ganaxolone treatment in 23 patients with TSC. The primary endpoint showed a median 16.6% reduction in 28-day frequency of TSC-associated seizures relative to the four-week baseline period. In addition, data from the Phase 2 TSC trial suggested that in patients on concomitant Epidiolex, early elevation of ganaxolone blood levels occurred and appeared to be linked to greater somnolence. A formal Phase 1 drug-drug interaction trial

was completed, demonstrating a lack of significant interaction between ganaxolone and Epidiolex. Additionally, the titration schedule for all subjects in the Phase 3 TSC TrustTSC trial has been adjusted to maximize tolerability. Undesirable side effects could delay clinical trials and result in the FDA or other regulatory authorities requiring us to conduct additional studies or trials for our product candidate either prior or post-approval, such as additional drug-drug interaction studies or safety or efficacy studies, or it may object to elements of our clinical development program. There is also a risk that the Phase 3 clinical TrustTSC trial of ganaxolone in TSC will generate data that is not sufficient to support regulatory approvals for this indication. indication.

Even if ganaxolone were to obtain approval from the FDA and comparable foreign regulatory authorities for TSC, RSE, or any other indication under development, 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 trial or risk management requirements. If we are unable to obtain regulatory approval for ganaxolone in these additional indications 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 indications for ganaxolone or any other product candidate that we may in-license, develop or acquire in the future. Furthermore, even with regulatory approval for ganaxolone, we will still need to develop a commercial organization, establish commercially viable pricing and obtain 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.

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We are conducting clinical development activities for ganaxolone across multiple indications, and such clinical development activities may not produce favorable results, which could adversely impact our ability to achieve regulatory approval for ganaxolone in such indications.

We are conducting clinical development activities for ganaxolone across multiple indications. Success in preclinical studies and early clinical trials in one indication does not ensure that later clinical trials in such indication or other indications will generate adequate data to demonstrate the efficacy and safety of ganaxolone in one or more indications. Furthermore, unfavorable clinical trial results in one ganaxolone indication may adversely impact our ability to continue to develop such indication or other ganaxolone indications. A number of companies in the pharmaceutical and biotechnology industries, including those with greater resources and experience, have suffered significant setbacks in clinical trials, even after seeing promising results in earlier studies and clinical trials. For example, while ganaxolone showed statistical separation from placebo in a Phase 2 clinical trial in adjunctive treatment of adults with focal onset seizures, it failed to show a similar statistically significant separation in a Phase 3 clinical trial 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 RSE and pediatric orphan genetic epilepsy indications. Further, in April 2024, the

independent DMC completed its review of the RAISE trial interim analysis and found that the trial did not meet the pre-defined interim analysis stopping criteria on the co-primary endpoints, and the DMC recommendation was that the trial may continue without modification. We have decided to complete enrollment in On June 17, 2024, we announced top-line results of the RAISE trial, at 100 patients with top-line results expected in the summer of 2024. Based on this interim analysis, there is a substantial risk which showed that the Phase 3 clinical trial met its first co-primary endpoint, with a statistically significant proportion of patients achieving SE cessation within 30 minutes of initiating IV ganaxolone compared to placebo: 80% vs. 13%, respectively (p<0.0001). However, the trial failed to achieve statistical significance on its second co-primary endpoint, the proportion of patients not progressing to IV anesthesia for 36 hours following initiation of IV ganaxolone compared to placebo: 63% vs. 51%, respectively (p=0.162). We continue to analyze the full RAISE trial dataset and plan to request a meeting with the FDA to discuss a potential path forward for IV ganaxolone in RAISE will not generate data that is sufficient to support regulatory approvals for this indication. RSE.

We do not know whether the clinical trials we may conduct will demonstrate adequate efficacy and safety to result in regulatory approval to market ganaxolone in any particular jurisdiction or indication. If clinical trials underway or conducted in the future do not produce favorable results, our ability to achieve regulatory approval for ganaxolone in those indications may be adversely impacted. Further, even if we believe the data collected from our clinical trials of ganaxolone are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Pre-clinical Preclinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us, which could delay, limit or prevent regulatory approval.

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

None.

Item 3. Defaults upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

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Item 5. Other Information

Trading Plans

During the three months ended March 31, 2024 June 30, 2024, none of our directors or officers (as defined in Rule 16a-1(f) of the Exchange Act) adopted a "Rule 10b5-1 trading arrangement" (as those terms are defined under Item 408 of Regulation S-K), except as follows:

Name	Title	Reporting Action	Plan Start Date	Plan End Date	Shares of Common Stock to be Sold	Intended to Satisfy Rule 10b5-1(c)?
Elan Ezickson	Director	Plan Adoption	January 18, 2025	January 31, 2025	1,600	Yes
Marvin H. Johnson, Jr.	Director	Plan Adoption	June 10, 2024	June 10, 2025	6,999	Yes
Sarah Noonberg, M.D., Ph.D.	Director	Plan Adoption	January 18, 2025	January 31, 2025	To be determined as sale of common stock to cover tax obligation related to expected January 2025 RSU vesting	Yes
Christina Shafer	Chief Commercial Officer	Plan Adoption	June 10, 2024	June 10, 2025	236,932	Yes

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Item 6. Exhibits

Exhibit Number	Exhibit Description
3.1	Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on August 7, 2014.)
3.2	Certificate of Amendment of the Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on April 2, 2020.)
3.3	Certificate of Amendment of the Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on May 27, 2020.)
3.4	Certificate of Amendment of the Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on September 22, 2020.)
3.5	Certificate of Amendment of the Fourth Amended and Restated Certificate of Incorporation. (Incorporated by reference to Exhibit 3.2 to Form 8-K current report filed on September 22, 2020.)
3.6	Amended and Restated By-laws. (Incorporated by reference to Exhibit 3.2 to Form 8-K current report filed on August 7, 2014.)
3.7	Certificate of Designations, Preferences and Rights of Series A Participating Convertible Preferred Stock. (Incorporated by reference to Exhibit 3.1 to Form 8-K current report filed on December 13, 2019.)
3.8	Delaware Certificate of Change of Registered Agent. (Incorporated by reference to Exhibit 3.8 to Form 10-Q quarterly report filed on May 12, 2022.)
4.1	Specimen Certificate evidencing shares of Marinus Pharmaceuticals, Inc.'s common stock. (Incorporated by reference to Exhibit 4.1 to Form S-1/A registration statement filed on July 18, 2014.)
4.2	Form of Pre-funded Warrant to Purchase Common Stock. (Incorporated by reference to Exhibit 4.1 to Form 8-K current report filed on November 10, 2022.)
10.1	Marinus Pharmaceuticals, Inc. 2024 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on May 24, 2024.)
10.2	Form of Nonqualified Stock Option Agreement for Non-Employee Directors under the Marinus Pharmaceuticals, Inc. 2024 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.3 to Form S-8 registration statement filed on June 6, 2024.)
10.3	Form of Restricted Stock Unit Agreement for Non-Employee Directors under the Marinus Pharmaceuticals, Inc. 2024 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.4 to Form S-8 registration statement filed on June 6, 2024.)
10.4	Form of Nonqualified Stock Option Agreement for Employees under the Marinus Pharmaceuticals, Inc. 2024 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.5 to Form S-8 registration statement filed on June 6, 2024.)
10.5	Form of Restricted Stock Unit Agreement for Employees under the Marinus Pharmaceuticals, Inc. 2024 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.6 to Form S-8 registration statement filed on June 6, 2024.)
10.6	Form of Nonqualified Stock Option Agreement for Employees granted as an Inducement Award under the Marinus Pharmaceuticals, Inc. 2024 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.7 to Form S-8 registration statement filed on June 6, 2024.)
10.7	Form of Incentive Stock Option Agreement for Employees under the Marinus Pharmaceuticals, Inc. 2024 Equity Incentive Plan. (Incorporated by reference to Exhibit 10.8 to Form S-8 registration statement filed on June 6, 2024.)

10.8	Second Amendment to Credit Agreement, dated June 6, 2024, by and among Marinus Pharmaceuticals, Inc., as Borrower, Oaktree Fund Administration, LLC, as Administrative Agent, and the other lenders party thereto. (Incorporated by reference to Exhibit 10.1 to Form 8-K current report filed on June 6, 2024.)
10.9	First Amendment to Revenue Interest Financing Agreement, dated June 6, 2024, by and between Marinus Pharmaceuticals, Inc. and Sagard Healthcare Royalty Partners, LP. (Incorporated by reference to Exhibit 10.2 to Form 8-K current report filed on June 6, 2024.)
31.1	Certification of Chief Executive Officer pursuant to Rule 13a-14(a) or 15a-14(a)15d-14(a) under the Exchange Act (filed herewith.)

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31.2	Certification of Chief Financial Officer pursuant to Rule 13a-14(a) or 15a-14(a)15d-14(a) under the Exchange Act (filed herewith.)
32.1	Certification Pursuant to 18 U.S.C. Section 1350 of principal executive officer and principal financial officer (furnished herewith.)
101.INS	XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation Linkbase
101.DEF	XBRL Taxonomy Extension Definition Linkbase
101.LAB	XBRL Taxonomy Extension Labels Linkbase
101.PRE	XBRL Taxonomy Extension Presentation Linkbase
104	Cover Page Interactive Data File formatted as Inline XBRL and contained in Exhibit 101

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SIGNATURES

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

Signature	Title	Date
/s/ SCOTT BRAUNSTEIN, M.D. Scott Braunstein, M.D.	President, Chief Executive Officer (Principal Executive Officer), Chairman of the Board and Director	May 8, August 13, 2024
/s/ STEVEN PFANSTIEL Steven Pfanstiel	Chief Operating Officer, Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)	May 8, August 13, 2024

Exhibit 31.1

Certification of Chief Executive Officer Pursuant to Exchange Act Rules 13a-14(a) or 15d-14(a)

I, Scott Braunstein, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Marinus Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 8, 2024 August 13, 2024

/s/ Scott Braunstein, M.D.

Scott Braunstein, M.D.
Chief Executive Officer and Director
(Principal Executive Officer)

Exhibit 31.2

Certification of Chief Financial Officer Pursuant to Exchange Act Rules 13a-14(a) or 15d-14(a)

I, Steven Pfanstiel, certify that:

1. I have reviewed this quarterly report on Form 10-Q of Marinus Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 8, 2024 August 13, 2024

/s/ Steven Pfanstiel

Steven Pfanstiel,
Chief Operating Officer, Chief Financial Officer and Treasurer
(Principal Financial and Accounting Officer)

Exhibit 32.1

Certification Pursuant to 18 U.S.C. Section 1350

In connection with the quarterly report of Marinus Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the quarter ended March 31, 2024 June 30, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned, in the capacities and on the date indicated below, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to his knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 8, 2024 August 13, 2024

/s/ Scott Braunstein
Chief Executive Officer and Director
(Principal executive officer)

Date: May 8, 2024 August 13, 2024

/s/ Steven Pfanstiel
Chief Operating Officer, Chief Financial Officer and Treasurer
(Principal financial and accounting officer)

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