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DELTA REPORT

10-Q

MRNS - MARINUS PHARMACEUTICALS,

10-Q - MARCH 31, 2024 COMPARED TO 10-Q - SEPTEMBER 30, 2023

The following comparison report has been automatically generated

TOTAL DELTAS	1674
CHANGES	219
DELETIONS	712
ADDITIONS	743

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 SEPTEMBER 30, 2023 MARCH 31, 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. ☒ YesYes ☐ 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 November 3, 2023 May 3, 2024, was: 54,573,581 54,933,774.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY
INDEX TO FORM 10-Q
FOR THE QUARTER ENDED SEPTEMBER 30, 2023 MARCH 31, 2024

PART I – FINANCIAL INFORMATION

PART II – OTHER 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)

	September 30, 2023	December 31, 2022	March 31, 2024	December 31, 2023
ASSETS				
Current assets:				
Cash and cash equivalents	$ 140,437	$ 240,551	$ 104,253	$ 120,572
Short-term investments	35,919	—	9,000	29,716
Accounts receivable, net	4,281	6,348	3,513	3,799
Inventory	2,736	77	5,783	2,413
Prepaid expenses and other current assets	11,667	5,402	8,727	8,746
Total current assets	195,040	252,378	131,276	165,246
Property and equipment, net	3,909	4,236	3,735	3,843
Other assets	1,857	2,904	2,339	1,819
Total assets	$ 200,806	$ 259,518	$ 137,350	$ 170,908
LIABILITIES AND STOCKHOLDERS' EQUITY				
LIABILITIES AND STOCKHOLDERS' (DEFICIT) EQUITY				
Current liabilities:				
Accounts payable	$ 2,626	$ 4,461	$ 6,125	$ 4,003
Current portion of notes payable	7,700	—	15,401	11,551
Current portion of revenue interest financing payable	1,901	1,020	2,511	2,211
Accrued expenses	18,328	19,536	18,721	22,859
Total current liabilities	30,555	25,017	42,758	40,624

Notes payable, net of deferred financing costs	64,783	71,018	58,072	61,423
Revenue interest financing payable, net of deferred financing costs	32,855	29,857	34,642	33,766
Contract liabilities, net	17,105	16,285	17,730	17,545
Other long-term liabilities	971	1,341	582	785
Total liabilities	146,269	143,518	153,784	154,143
Stockholders' equity:				
Series A convertible preferred stock, $0.001 par value; 25,000,000 shares authorized, no shares issued and outstanding at September 30, 2023; 4,300 shares issued and outstanding at December 31, 2022	—	4,043		
Common stock, $0.001 par value; 150,000,000 shares authorized, 54,580,797 issued and 54,573,490 outstanding at September 30, 2023 and 49,650,074 issued and 49,642,767 outstanding at December 31, 2022	55	50		
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	584,710	542,428	594,106	588,656
Treasury stock at cost, 7,307 shares at September 30, 2023 and December 31, 2022	—	—		
Treasury stock at cost, 7,307 shares at March 31, 2024 and December 31, 2023			—	—
Accumulated other comprehensive loss	(71)	—	—	(20)
Accumulated deficit	(530,157)	(430,521)	(610,595)	(571,926)
Total stockholders' equity	54,537	116,000		
Total liabilities and stockholders' equity	$ 200,806	$ 259,518		
Total stockholders' (deficit) equity			(16,434)	16,765
Total liabilities and stockholders' (deficit) equity			$ 137,350	$ 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) INCOME LOSS
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,		Three Months Ended March 31,	
	2023	2022	2023	2022	2024	2023
Revenue:						
Product revenue, net	$ 5,429	$ 555	$ 13,010	$ 555	$ 7,509	$ 3,332
Federal contract revenue	1,891	1,785	10,753	5,088	152	7,048
Collaboration revenue	18	—	36	12,673	18	—
Total revenue	7,338	2,340	23,799	18,316	7,679	10,380

Expenses:						
Research and development	23,661	19,002	73,006	58,488	24,118	27,933
Selling, general and administrative	14,868	13,389	45,794	42,187	18,626	15,204
Cost of product revenue	455	48	1,047	48	756	206
Cost of IP license fee	—	—	—	1,169		
Total expenses	38,984	32,439	119,847	101,892	43,500	43,343
Loss from operations	(31,646)	(30,099)	(96,048)	(83,576)	(35,821)	(32,963)
Interest income	1,895	514	6,366	610	1,462	2,343
Interest expense	(4,242)	(2,634)	(12,597)	(6,982)	(4,346)	(4,147)
Gain from sale of priority review voucher, net	—	107,375	—	107,375		
Other income (expense), net	1,021	(114)	1,105	(1,179)		
(Loss) income before income taxes	(32,972)	75,042	(101,174)	16,248		
(Provision) benefit for income taxes	—	(1,752)	1,538	(1,752)		
Net (loss) income	$ (32,972)	$ 73,290	$ (99,636)	$ 14,496		
Net income allocated to preferred shareholders	—	1,656	—	336		
Net (loss) income applicable to common shareholders	$ (32,972)	$ 71,634	$ (99,636)	$ 14,160		
Other income, net					36	37
Net loss applicable to common shareholders					$ (38,669)	$ (34,730)
Per share information:						
Net (loss) income per share of common stock—basic	$ (0.61)	$ 1.93	$ (1.89)	$ 0.38		
Net (loss) income per share of common stock—diluted	$ (0.61)	$ 1.89	$ (1.89)	$ 0.37		
Basic weighted average shares outstanding	53,920,109	37,202,269	52,755,114	37,084,060		
Diluted weighted average shares outstanding	53,920,109	37,910,511	52,755,114	38,393,754		
Other comprehensive income (loss):						
Unrealized gain (loss) on available-for-sale securities	43	—	(71)	—		
Total comprehensive (loss) income	$ (32,929)	$ 73,290	$ (99,707)	$ 14,496		
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)

See accompanying notes to consolidated financial statements.

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MARINUS PHARMACEUTICALS, INC. AND SUBSIDIARY
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)
(unaudited)

	Nine Months Ended September 30,		Three Months Ended March 31,	
	2023	2022	2024	2023
Cash flows from operating activities				
Net (loss) income	$ (99,636)	$ 14,496		
Adjustments to reconcile net (loss) income to net cash used in operating activities:				
Gain from sale of PRV, net of transaction costs	—	(107,375)		
Net loss			$ (38,669)	$ (34,730)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	426	336	131	157
Amortization of debt issuance costs	1,659	1,133	609	480
Accretion of revenue interest financing debt	4,421	—		
Accretion of revenue interest financing debt, net of cash paid			1,065	1,440
Amortization of discount on short-term investments	(995)	—	(149)	(228)
Stock-based compensation expense	11,638	11,091	5,193	3,741
Amortization of net contract asset/liability	(1,259)	(1,000)	(337)	(413)
Noncash lease expense	146	194	47	53
Noncash lease liability	300	252	224	96
Write off of fixed assets	61	169	—	62
Issuance of common stock for cost of license agreement	—	1,169		
Unrealized loss on foreign currency transactions	—	930		
Changes in operating assets and liabilities:				
Refund liability	—	(22,163)		
Net contract asset/liability	2,079	11,057	522	544
Prepaid expenses and other current assets, non-current assets, inventory and accounts receivable	(6,290)	(2,313)	(3,833)	(8,963)
Accounts payable and accrued expenses	(3,559)	1,052	(2,264)	(3,717)
Net cash used in operating activities	(91,009)	(90,972)	(37,461)	(41,478)
Cash flows from investing activities				
Proceeds from sale of PRV, net of transaction costs	—	107,375		
Proceeds from sale of property and equipment	9	—		
Maturities of short-term investments	17,000	—	20,885	—
Purchases of short-term investments	(51,995)	—	—	(51,995)
Purchases of property and equipment	(85)	(1,682)		
Net cash (used in) provided by investing activities	(35,071)	105,693		
Net cash provided by (used in) investing activities			20,885	(51,995)
Cash flows from financing activities				
Proceeds from exercise of stock options	783	1,763	257	—
Proceeds from notes payable, net of fees	—	28,838		
Payments of revenue interesting financing debt	(737)	—		
Proceeds from equity offerings, net of offering costs	25,920	—		
Net cash provided by financing activities	25,966	30,601		
Net (decrease) increase in cash and cash equivalents	(100,114)	45,322		
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	240,551	122,927	120,572	240,551
Cash and cash equivalents—end of period	$ 140,437	$ 168,249	$ 104,253	$ 146,904

Supplemental disclosure of cash flow information				
Unrealized loss on short-term investments	$ (71)	$ —		
Financing costs	$ 97	$ —		
Unrealized gain on short-term investments			$ 20	$ 74
Cash paid for interest during the period	$ 6,540	$ —	$ 2,671	$ 2,156
Cash paid for income taxes during the period	$ 903	$ —		
Property and equipment in deposits placed in service	$ —	$ 1,665		

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'	Series A Convertible Preferred Stock		Common Stock		Additional Paid-in	Treasury Stock	
	Shares	Amount	Shares	Amount	Capital	Shares	Amount	Income	Deficit	Equity	Shares	Amount	Shares	Amount	Capital	Shares	Amount
Balance, December 31, 2021	4,575	$ 4,302	36,790,254	$ 37	$459,852	7,307	$ —	$ —	$ (410,705)	$ 53,486							
Stock-based compensation expense	—	—	—	—	3,378	—	—	—	—	3,378							
Exercise of stock options	—	—	225,165	—	1,733	—	—	—	—	1,733							
Issuance of stock related to IP license agreement with Ovid	—	—	123,255	—	1,169	—	—	—	—	1,169							
Net Loss	—	—	—	—	—	—	—	—	(19,361)	(19,361)							
Balance, March 31, 2022	4,575	$ 4,302	37,138,674	$ 37	$466,132	7,307	$ —	$ —	$ (430,066)	$ 40,405							
Stock-based compensation expense	—	—	—	—	3,817	—	—	—	—	3,817							

Net issuance of common stock in connection with the vesting of restricted stock	—	—	2,508	—	—	—	—	—	—	—
Exercise of stock options	—	—	2,968	—	14	—	—	—	—	14
Conversion of convertible preferred stock into common	(275)	(259)	55,000	—	259	—	—	—	—	—
Net loss	—	—	—	—	—	—	—	—	(39,433)	(39,433)
Balance, June 30, 2022	4,300	$ 4,043	37,199,150	$ 37	$470,222	7,307	$ —	—	$ (469,499)	$ 4,803
Stock-based compensation expense	—	—	—	—	3,895	—	—	—	—	3,895
Exercise of stock options	—	—	3,304	—	16	—	—	—	—	16
Net settlement of restricted shares	—	—	(5,810)	—	—	—	—	—	—	—
Conversion of convertible preferred stock into common	—	—	—	—	—	—	—	—	—	—
Net income	—	—	—	—	—	—	—	—	73,290	73,290
Balance, September 30, 2022	4,300	$ 4,043	37,196,644	$ 37	$474,133	7,307	—	$ —	(396,209)	82,004

Balance, December 31, 2022	4,300	$ 4,043	49,642,767	$ 50	$542,428	7,307	$ —	$ —	$ (430,521)	$ 116,000	4,300	$ 4,043	49,642,767	$ 50	$542,428	7,307	$ —
Stock-based compensation expense	—	—	—	—	3,741	—	—	—	—	3,741	—	—	—	—	3,741	—	—
Net issuance of common stock in connection with the vesting of restricted stock	—	—	22,350	—	—	—	—	—	—	—	—	—	22,350	—	—	—	—
Unrealized gain on short-term investments	—	—	—	—	—	—	—	74	—	74	—	—	—	—	—	—	—

Net loss	—	—	—	—	—	—	—	—	(34,730)	(34,730)														
Net Loss											—	—	—	—	—	—	—							
Balance, March 31, 2023	4,300	$ 4,043	49,665,117	$ 50	$546,169	7,307	$ —	$ 74	$ (465,251)	$ 85,085	4,300	$ 4,043	49,665,117	$ 50	$546,169	7,307	$ —							
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	$ —							
Stock-based compensation expense	—	—	—	—	4,006	—	—	—	—	4,006	—	—	—	—	5,193	—	—							
Exercise of stock options	—	—	50,338	—	298	—	—	—	—	298	—	—	57,665	—	257	—	—							
Net issuance of common stock in connection with the vesting of restricted stock	—	—	224,170	—	—	—	—	—	—	—	—	—	295,256	—	—	—	—							

Issuance of common stock in connection with at-the-market facility offering (average price of $7.17 per share), net of expenses of $529	—	—	3,689,800	4	25,819	—	—	—	—	25,823								
Unrealized gain on short-term investments	—	—	—	—	—	—	—	43	—	43	—	—	—	—	—	—	—	
Net loss	—	—	—	—	—	—	—	—	(32,972)	(32,972)	—	—	—	—	—	—	—	
Balance, September 30, 2023	—	$ —	54,573,490	$ 55	$584,710	7,307	—	$ (71)	(530,157)	54,537								
Balance, March 31, 2024											—	$ —	54,931,042	$ 55	$594,106	7,307	$ —	

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, 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®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. In June 2022, the U.S. Drug Enforcement Administration (DEA) published an interim final rule in the Federal Register placing ganaxolone and its salts in schedule V (CV) of the Controlled Substances Act (CSA), which rule became final on December 9, 2022. ZTALMY, our first FDA approved product, became available for commercial sale and shipment in the third quarter of 2022. On July 28, 2023 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. With the EC marketing authorization granted for ZTALMY, We have an exclusive collaboration agreement with Orion Corporation (Orion), our for European commercialization partner of ganaxolone for ZTALMY in Europe, has announced it has begun preparations ZTALMY. Orion is preparing for the launch of ZTALMY, 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 launches of ZTALMY in one or more select European countries. countries in 2024.

We are also developing ganaxolone for the treatment of other rare genetic epilepsies, including Tuberous Sclerosis Complex (TSC), and for the treatment of refractory status epilepticus Refractory Status Epilepticus (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 are classified as having RSE.

We are developing ganaxolone in formulations for two different routes of administration: intravenous (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, and for both acute hospital care and chronic at home-administration settings. 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). Ganaxolone is a synthetic analog of allopregnanolone, an endogenous neurosteroid. Ganaxolone acts at neurosteroid, and targets both synaptic and extrasynaptic $GABA_A$ receptors, a target known for its anti-seizure, . This unique receptor binding profile may contribute to the anticonvulsant, antidepressant and anxiolytic potential. effects shown by neuroactive steroids in animal models, clinical trials or both.

COVID-19 affected our clinical operations and timelines. For example, our Randomized Therapy In Status Epilepticus (RAISE) trial for RSE is conducted in hospitals, primarily intensive care units in academic medical centers, which experienced high rates of COVID-19 admissions. Several of these sites participating in the RAISE trial experienced COVID-related difficulties, including staff turnover and the need to devote significant resources to patients with COVID-19, which resulted in site initiation and enrollment delays for the RAISE trial. Given these COVID-19-related challenges and the interruption in drug supply in mid-2022, we previously adjusted our expectation for our top-line data readout for the RAISE trial. In May 2022, we resumed screening and recruitment for the RAISE trial. We now expect our interim analysis with top-line data readout for the RAISE trial to be available in the second quarter of 2024, if the pre-defined stopping criteria from the planned interim analysis are met.

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 and negative cash flows from our operations. losses. We incurred a net Net loss of $99.6 million $38.7 million for the nine three months ended September 30, 2023 March 31, 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 September 30, 2023 March 31, 2024 was $530.2 million $610.6 million, and we expect to incur substantial losses in future periods.

We plan to finance our future operations with a combination of proceeds from the issuance of equity securities, the issuance of 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

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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

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to assess our ability to continue as a going concern within one year after the date the financial statements are issued. We believe that our existing cash, had Cash and cash equivalents and short-term Short-term investments of $113.3 million as of September 30, 2023, will be March 31, 2024. We believe that such amount is not sufficient to fund our operating expenses and capital expenditure requirements, as well as maintain the minimum cash balance required under our debt facility, operations for the one-year period after the date the these financial statements are issued. However, we will need As a result, there is substantial doubt about our ability to secure continue as a going concern through the one-year period from the date these financial statements are issued. Cost reduction activities are being implemented, with expected impact beginning in the second quarter of 2024. 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 sources. However, there can be no assurance that we will be successful in order raising additional capital or that such capital, if available, will be on terms acceptable to carry out all of us. We have and will continue to evaluate alternatives to extend our commercialization and planned research and development activities with respect to ganaxolone. 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, 2022 December 31, 2023 and accompanying notes thereto included in our Annual Report on Form 10-K filed with the SEC on March 9, 2023 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.

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 over approximately 99% of our ZTALMY revenue to date. Our

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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 an contractual discounts, including incentive prompt payment discount to Orsini as explicitly stated in the contract with Orsini. This discount discounts and chargebacks. Each of these potential discounts is recorded as a reduction of ZTALMY revenue and accounts 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 product 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 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 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 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 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 Accumulated other comprehensive income (loss) within stockholders' equity. All of our investments were short-term in nature as of September 30, 2023 March 31, 2024.

Accounts Receivable, net

Net trade receivables related to ZTALMY sales, which are recorded in Accounts receivable, net accounts receivable on the consolidated balance sheets, were approximately $2.3 $2.8 million and $1.3 million $2.6 million as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023, respectively. As of both September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023, we had no allowance for doubtful accounts. An allowance for doubtful accounts is determined based on our assessment of the creditworthiness 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 approximately 30 days from the shipment date.

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Excluding net trade receivables, accounts 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 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 for commercially saleable product and materials were incurred and included in research Research and development expenses. As a result, cost Cost of product revenues related to ZTALMY initially reflected a lower average per unit cost of materials, and will initially continue to reflect a lower average per unit cost of materials into approximately through the first half second quarter of 2024, as previously expensed inventory is utilized for commercial production and sold to customers.

Debt Issuance Costs

Debt issuance costs incurred in connection with Note payable (Note 10) and revenue Revenue interest financing payable (Note 11) are amortized to interest 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 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 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 Long-term liabilities. In accordance with ASC 210-20, our contract Contract liabilities are were partially offset by our contract Contract assets at March 31, 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*Debt* and ASC 835-30 *Interest - Imputation of Interest.* *Interest*. The initial funds we 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 as further discussed in Note 11. repaid. We estimated the total amount of future product revenue to be 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 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 September 30, 2023 March 31, 2024, our cash Cash and cash equivalents included $1.2 million $1.4 million of cash accounts in banking institutions and $139.3 $102.9 million in money market funds. As of December 31, 2022 December 31, 2023, our cash Cash and cash equivalents included $10.6 1.3 million of cash accounts in banking institutions and $230.0 million $119.3 million in money market funds. Our cash Cash and cash equivalents are maintained in federally insured financial institutions in excess of the federally insured limit. Included in

other Other assets at September 30, 2023 March 31, 2024 and December 31, 2023 was $0.1 million and $0.2 million, respectively, of accrued interest receivable related to our short-term Short-term investments.

The following table provides details regarding our portfolio of short-term Short-term investments (in thousands) as of September 30, 2023 March 31, 2024 and December 31, 2023:

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value	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								
U.S. Treasury securities	$ 28,624	$ 97	$ (155)	$ 28,566	$ 26,852	$ 138	$ (155)	$ 26,835
U.S. Government Agency securities	7,366	21	(34)	7,353	2,884	31	(34)	2,881
Total	$ 35,990	$ 118	$ (189)	$ 35,919	$ 29,736	$ 169	$ (189)	$ 29,716

We did not have any short-term investments as of December 31, 2022.

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.

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- 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 September 30, 2023 March 31, 2024 and December 31, 2023, all of our financial assets and liabilities were classified as Level 1 or Level 2 valuations. As of December 31, 2022, all of our financial assets and liabilities were classified as Level 1 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
September 30, 2023								
March 31, 2024								
Assets								
Cash	$ 1,161	$ —	$ —	$ 1,161	$ 1,420	$ —	$ —	$ 1,420
Money market funds (cash equivalents)	139,276	—	—	139,276	102,833	—	—	102,833
U.S. Treasury securities	—	28,566	—	28,566	—	9,000	—	9,000
U.S. Government Agency securities	—	7,353	—	7,353				
Total assets	$ 140,437	$ 35,919	$ —	$ 176,356	$104,253	$ 9,000	$ —	$113,253
December 31, 2022								
December 31, 2023								
Assets								
Cash	$ 10,569	$ —	$ —	$ 10,569	$ 1,255	$ —	$ —	$ 1,255
Money market funds (cash equivalents)	229,982	—	—	229,982	119,317	—	—	119,317
U.S. Treasury securities					—	26,835	—	26,835
Agency securities					—	2,881	—	2,881
Total assets	$ 240,551	$ —	$ —	$ 240,551	$120,572	$29,716	$ —	$150,288

5. Inventory

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

	September 30, 2023	December 31, 2022	March 31, 2024	December 31, 2023
Raw materials	$ 1,410	$ —	$ 3,600	$ 436
Work in process	571	—	1,591	1,075
Finished goods	755	77	592	902
Total Inventories	$ 2,736	$ 77	$ 5,783	$ 2,413

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6. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2023	December 31, 2022	March 31, 2024	December 31, 2023
Payroll and related costs	$ 7,159	$ 7,061	$ 2,830	$ 7,746
Clinical trials and drug development	5,303	5,725	5,694	4,701
Accrued license agreement payment			2,000	4,000
Professional fees	594	1,417	1,669	1,236
Accrued tax provision	—	2,445		
Third-party commercial expenses	3,708	1,880		
Selling and commercial liabilities			4,502	3,901
Short-term lease liabilities	704	637	1,306	774
Other	860	371	720	501
Total accrued expenses	$ 18,328	$ 19,536	$18,721	$22,859

7. Net (Loss) Income Loss Per Share of Common Stock

Basic net (loss) income loss per share of common stock is computed by dividing net (loss) income Net loss attributable to common stockholders by the weighted Weighted average number of shares of common stock outstanding during each period, without consideration for potential dilutive shares of common stock. period. Diluted net (loss) income 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. Diluted net (loss) income per share of common stock is computed by dividing In computing the net (loss) income attributable to common stockholders by the weighted-average number of common share equivalents outstanding for the period determined using the treasury-stock method and if-converted method, as applicable. Basic and diluted net (loss) income loss per share attributable applicable to common stockholders, is presented in conformity with the two-class method required Weighted average number of shares remains the same for participating securities, which include convertible preferred stock.

Under the two-class method, undistributed earnings are allocated to common stock and convertible preferred stock both calculations due to the extent fact that each preferred security may share when a Net loss exists, dilutive shares are not included in earnings as if all of the earnings for the period had been distributed. The total earnings allocated to common stock is then divided by the number of outstanding shares to which the earnings calculation. These potentially dilutive securities are allocated to determine the earnings per share. The two-class method is not applicable during periods with a net loss, as the holders of the convertible preferred stock have no obligation to fund losses. 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 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.

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The computations for basic and diluted net (loss) income per share were as follows (in thousands, except per-share data):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Numerator				
Net (loss) income	$ (32,972)	$ 73,290	$ (99,636)	$ 14,496
Less: Net income attributable to preferred shareholders	—	(1,656)	—	(336)
Net (loss) income attributable to common shareholders	(32,972)	71,634	(99,636)	14,160
Denominator				
Basic weighted average shares outstanding	53,920,109	37,202,269	52,755,114	37,084,060
Effect of dilutive securities	—	708,242	—	1,309,694
Diluted weighted average shares outstanding	53,920,109	37,910,511	52,755,114	38,393,754
Basic net (loss) income per share of common stock	$ (0.61)	$ 1.93	$ (1.89)	$ 0.38
Diluted net (loss) income per share of common stock	$ (0.61)	$ 1.89	$ (1.89)	$ 0.37

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

	Three Months Ended September 30,		Nine Months Ended September 30,		Three Months Ended March 31,	
	2023	2022	2023	2022	2024	2023
Convertible preferred stock					—	860,000
Restricted stock awards and restricted stock units	1,265,316	—	1,265,316	—	2,293,878	1,542,115
Stock options	7,188,661	4,735,841	7,188,661	4,566,687	8,671,041	7,143,397
	8,453,977	4,735,841	8,453,977	4,566,687	10,964,919	9,545,512

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 September 30, 2023 March 31, 2024, 577 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 September 30, 2023 March 31, 2024, 5,063,925 6,389,758 options to purchase shares of common stock were outstanding pursuant to grants

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in connection with the 2014 Plan, and 775,794 260,569 shares of common stock were available for future issuance. The amount, terms of grants, and exercisability provisions are determined and set by our board of directors. In accordance with the 2014 Plan, on January 1, 2023 January 1, 2024, the shares of common stock available for future grants under the 2014 Plan was increased by 2,020,111. to 3,090,220.

Stock Options

There were 7,188,661 8,671,041 stock options outstanding as of September 30, 2023 March 31, 2024 at a weighted average exercise price of $9.75 $9.69 per share, including 2,124,159 2,281,283 stock options outstanding outside of the 2014 Plan, granted as inducements to new employees. During the nine three months ended September 30, 2023 March 31, 2024, 1,971,291 1,617,094 options were granted to employees and directors at a weighted average exercise price of $6.34 $9.76 per share. Of the options granted, 1,550,766 1,540,519 options were granted pursuant to the 2014 Plan and 420,525 76,575 were granted outside of the 2014 Plan as inducements for new employees.

Restricted Stock and Restricted Stock Units

All issued and outstanding restricted shares of common stock are time-based, and become vested within two years of the grant date, pursuant to the 2014 Plan. Compensation expense is recorded ratably over the requisite service period. Compensation expense related to restricted stock is measured based on the fair value using the closing market price of our common stock on the date of the grant. As of September 30, 2023 March 31, 2024, we did not have any restricted shares of common stock outstanding.

During the nine three months ended September 30, 2023 March 31, 2024, we granted 974,940 1,350,244 restricted stock units, which generally vest within three years of the grant date, pursuant to the 2014 Plan. As of September 30, 2023 March 31, 2024, we had 1,265,316 2,293,878 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 September 30,		Nine Months Ended September 30,		Three Months Ended March 31,	
	2023	2022	2023	2022	2024	2023
Research and development	$ 1,519	$ 1,394	$ 4,329	$ 4,069	$1,832	$1,342
Selling, general and administrative	2,487	2,501	7,309	7,022	3,361	2,399
Total	$ 4,006	$ 3,895	$ 11,638	$ 11,091	$5,193	$3,741

Preferred Stock

As of September 30, 2023 March 31, 2024 all shares of our Series A Convertible Preferred Stock (Preferred Stock) had been converted and none remained outstanding. In the nine three months ended September 30, 2023 March 31, 2023, 4,300 shares of our Preferred stock were converted remained outstanding, convertible into 860,000 shares of our common stock.

In the three months ended March 31, 2023, there were no conversions of shares of our Preferred Stock Issued in Connection with Ovid License Agreement

On March 29, 2022, pursuant to an exclusive patent license agreement with Ovid Therapeutics Inc. (Ovid), we issued 123,255 into shares of our common stock to Ovid. The shares were issued in reliance upon the exemption from the registration requirements of the Securities Act of 1933, as amended (the Securities Act) provided by Section 4(a)(2) of the Securities Act and Regulation D thereunder as sales by an issuer not involving any public offering (see Part II, Item 2. *Unregistered Sales of Equity Securities and Use of Proceeds*). The fair value of these shares is reflected in operating expenses for the nine months ended September 30, 2022. stock.

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

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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 Agreement) 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 Agreement 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 Agreement. EDA. Subject to the terms and conditions of the Amended Agreement, 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. In the nine months ended September 30, 2023, we sold approximately 3.7 million shares of our common stock pursuant to the Amended Agreement, which consisted of net proceeds of approximately $25.9 million. We did not sell any shares of our common stock during the nine three months ended September 30, 2022 March 31, 2024 and March 31, 2023 under the EDA.

9. Leases

We have entered into one operating lease for real estate. This estate and several operating leases for clinical site equipment. Our real estate operating lease has a term of 78 months, and includes renewal terms which 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 September 30, 2023 March 31, 2024, our operating lease had a remaining lease term of 24 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 months. The right-of-use (ROU) asset is assets are included in "Other assets" Other assets on our interim consolidated balance sheets as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023, and represents represent our right to use the underlying asset assets for the applicable lease term. terms. Our obligations to make lease payments are included in both "Accrued expenses" Accrued expenses and "Other Other long-term liabilities" liabilities on our interim consolidated balance sheets as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023. The ROU asset was assets were initially measured at cost, which comprises 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 asset is assets

are subsequently measured throughout the lease term at the carrying amount of the lease liability, plus initial direct costs, plus (minus) any prepaid (accrued) lease payments, less the unamortized balance of lease incentives received.

As of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023, ROU assets were $1.0 million $1.5 million and $1.3 million $1.0 million, respectively, and operating lease liabilities were $1.5 million $1.9 million and $2.0 million $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 each of the three months ended September 30, 2023 March 31, 2024 and 2022, 2023, we recognized $0.3 million and $0.1 million, respectively, in total lease costs. During each costs, of the nine months ended September 30, 2023 and 2022, we recognized $0.4 million in total least costs. In all periods, we recognized which less than $0.1 million in short-term lease costs $0.1 million related to short-term operating leases. leases for each period.

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 and December 31, 2023, we have not recognized any impairment losses for our ROU assets.

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asset is impaired, and if so, the amount of the impairment loss to recognize. As of September 30, 2023 and December 31, 2022, we have not recognized any impairment losses for our ROU assets.

Maturities of operating lease liabilities as of September 30, 2023 March 31, 2024 were as follows (in thousands):

Remaining three months of 2023	$ 206	
2024	840	
Remainder of 2024		$1,073
2025	642	955
	1,688	2,028
Less: imputed interest	(180)	(140)
Total lease liabilities	$ 1,508	$1,888
Current operating lease liabilities	$ 704	$1,306
Non-current operating lease liabilities	804	582
Total lease liabilities	$ 1,508	$1,888

10. Notes Payable

On May 11, 2021 (Closing Date) and as amended on May 17, 2021, May 23, 2022 and October 28, 2022 (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 in 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 a revenue interest financing agreement (Revenue the Revenue Interest Financing Agreement) Agreement with Sagard Health Royalty Partners (Sagard) and the transactions thereunder. In addition, the October 2022 amendment 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 the termination of the Tranche C Term Loan Commitment, the loans under the Credit Agreement consist of $75.0 million of previously drawn Term Loans with no additional funds available thereunder.

The Credit Agreement contains a minimum liquidity covenant that requires 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

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until the Maturity Date. We are also required to make quarterly principal payments beginning on June 30, 2024 in an amount equal to 5.0% of the aggregate amount of the 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 million in 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 plusa "make-whole" amount equal to the interest that would have accrued through May 11, 2023 if prepayment occurred on or before May 11, 2023, (ii) 4% of the principal prepaid if prepayment occurs after May 11, 2023 but on or before May 11, 2024, or (iii) (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.

We are also required to make mandatory prepayments of the Term Loans upon an event of default under the Credit Agreement resulting from the occurrence of a change of control.

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 2022 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 Interest expense over the term of the loan using the effective interest method.

In addition to the minimum liquidity covenant, 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 September 30, 2023 March 31, 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 expenses 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,

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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 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 Interest expenses over the term of the loan using the effective-interest method.

For the nine three months ended September 30, 2023 March 31, 2024, we recognized interest expense of $8.0 million $2.7 million, of which $6.5 million $2.2 million was interest on the Term Loans and $1.5 million $0.5 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 September 30, 2023 March 31, 2024 (in thousands):

Gross proceeds	$	75,000	$75,000
Contractual exit fee		2,003	2,003
Unamortized debt discount and issuance costs		(4,520)	(3,530)
Total note payable	$	72,483	$73,473
Current portion of note payable		7,700	15,401
Non-current portion of note payable		64,783	58,072
Total note payable	$	72,483	$73,473

The aggregate maturities of Notes payable as of September 30, 2023 March 31, 2024 are as follows (in thousands):

Remainder of 2023	$	—	
2024		11,250	
Remainder of 2024			$11,250
2025		15,000	15,000
2026		48,750	48,750
Total	$	75,000	$75,000

11. Revenue Interest Financing Agreement

On October 28, 2022 (Closing Date), we entered into the Revenue a revenue interest financing agreement (Revenue Interest Financing Agreement 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.

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.

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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 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 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 an amendment to 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. 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 nine three months ended September 30, 2023 March 31,

2024, we estimated an effective annual interest rate of approximately 18%. 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 will 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 nine three months ended September 30, 2023 March 31, 2023 and March 31, 2024 (in thousands):

For the three months ended March 31, 2023		
Revenue Interest Financing Balance at December 31, 2022	$ 30,877	$30,877
Non-cash interest expense in the nine months ended September 30, 2023	4,421	
Amortization of debt discount in the nine months ended September 30, 2023	194	
Payments made in the nine months ended September 30, 2023	(736)	
Revenue Interest Financing Balance at September 30, 2023	$ 34,756	
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	$ 1,901	1,321
Long-term portion of revenue interest financing liability	32,855	30,893
Revenue Interest Financing Balance at September 30, 2023	$ 34,756	
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

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 the such study were positive, based on the criteria set forth in the study's protocol, Orion would have had the right to terminate the Orion Collaboration Agreement within ninety (90) days after its receipt of the final report of such study, report, and in which case

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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 research and development 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

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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 carries 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 Euro, €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 or 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, 2022 December 31, 2023, we allocated the transaction price to the performance obligations as described below and recorded the remaining $12.7 million of the upfront payment as collaboration revenue during the year ended December 31, 2022. During 2022, we amortized $1.1 million of the transaction price associated with the Development and Regulatory Services as a reduction of research and development costs. These reductions to transaction price resulted in there was a total contract liability of $15.1 million as $13.7 million and a total contract asset of December 31, 2022 $2.9 million. In accordance with ASC 210-20, the contract liability of $15.1 million was offset by a the contract asset of $5.1 million related to the reimbursement of research and development costs, resulting which resulted in a net contract liability of $10.0 million $10.8 million as of December 31, 2022 December 31, 2023.

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Transaction Price and Net Contract Liability as of December 31, 2022 December 31, 2023:

	Transaction Price	Cumulative Collaboration Revenue Recognized as of December 31, 2022	Contract Liability	Transaction Price	Cumulative Collaboration Revenue Recognized as of December 31, 2023	Contract Liability
License	$ 21,660	$ 21,660	$ -	$ 21,660	$ 21,660	$ -
Development and Regulatory Services	6,717	1,158	5,559	6,717	2,511	4,206
Supply of Licensed Product	9,503	-	9,503	9,503	-	9,503
	$ 37,880	$ 22,818	$ 15,062	$ 37,880	$ 24,171	$13,709

Less Total Contract Asset	5,079	2,912
Net Contract Liability	$ 9,983	$10,797

During the nine three months ended September 30, 2023 March 31, 2024, we amortized $1.2 million $0.3 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 $13.8 million $13.4 million as of September 30, 2023 March 31, 2024. In accordance with ASC 210-20, the contract liability of $13.8 million $13.4 million is offset by a the contract asset of $3.5 million $2.4 million related to the reimbursement of research and development costs, resulting in a net contract liability of $10.3 $11.0 million as of September 30, 2023 March 31, 2024.

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

	Transaction Price	Cumulative Collaboration Revenue Recognized as of September 30, 2023	Contract Liability	Transaction Price	Cumulative Collaboration Revenue Recognized as of March, 2024	Contract Liability
License	$ 21,660	$ 21,660	$ -	$ 21,660	$ 21,660	$ -
Development and Regulatory Services	6,717	2,417	4,300	6,717	2,848	3,869
Supply of Licensed Product	9,503	-	9,503	9,503	-	9,503
	$ 37,880	$ 24,077	$ 13,803	$ 37,880	$ 24,508	$13,372
Less Total Contract Asset			3,464			2,372
Net Contract Liability			$ 10,339			$11,000

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.

We reevaluate the transaction price and the total estimated costs expected to be incurred to satisfy the performance obligations and adjust the deferred revenue at the end of each reporting period. Such changes will result in a change to the amount of collaboration revenue recognized and deferred revenue.

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.

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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

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kinase-like 5 deficiency disorder, tuberous sclerosis complex and SE (including RSE) 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 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

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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.

As of December 31, 2022, we allocated the transaction price to the performance obligations as described below. There was no activity in during each of the three and nine months ended September 30, 2023. March 31, 2024 and 2023. The cumulative collaboration revenue recognized as of September 30, 2023 March 31, 2024 and December 31, 2023 is $3.0 million, which was the $3.0 million transaction price associated with the License as revenue for the year ended December 31, 2022. at contract inception. No license revenue was recorded in during each of the three and nine months ended September 30, 2023. March 31, 2024 and 2023. There was a total contract liability of $7.0 million as of both September 30, 2023 March 31, 2024 and December 31, 2022 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 September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023.

Transaction Price and Net Contract Liability as of September 30, 2023 March 31, 2024 and December 31, 2022 December 31, 2023:

	Cumulative Collaboration			Cumulative Collaboration		
	Transaction Price	Revenue Recognized as of September 30, 2023 and December 31, 2022	Contract Liability	Transaction Price	Revenue Recognized as of March 31, 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

In December 2022, we 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, Selling, general and administrative expense and $0.2 million recorded to cost Cost of collaboration revenue, in the period ended December 31, 2022,

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.

We reevaluate the transaction price and the total estimated costs expected to be incurred to satisfy the performance obligations and adjust the deferred revenue at the end of each reporting period. Such changes will result in a change to the amount of collaboration revenue recognized and deferred revenue.

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 agreement. Biologix Agreement. We may be entitled to additional fees upon regulatory milestones. In the three and nine months ended September 30, 2023 March 31, 2024, we recorded less than $0.1 million $0.1 million of collaboration Collaboration revenue related to the Biologix Agreement. There was a total contract Contract liability of $0.5 million $0.4 million at September 30, 2023 March 31, 2024. As the Biologix Agreement was entered into in May 2023, there was no contract liability at December 31, 2022 March 31, 2023.

13. Subsequent Events

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 stopping criteria 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. On April 30, 2024, we implemented a reduction-in-force (RIF) which impacted approximately 20% of our workforce.

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 Deficiency Disorder (CDD) in the U.S.;
- our expectations about the results from the RAISE trial and the top-line data from such trial;

- our expectations that our cost reduction activities being 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 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 EU, 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), Tuberous Sclerosis Complex (TSC) and Lennox Gastaut Syndrome (LGS);
- 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 potential benefits of ganaxolone, including in indications other than CDD;
- the timing of seeking marketing approval of ganaxolone in specific additional indications;

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- our ability to maintain marketing approval for ganaxolone in 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;

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- 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 in 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 possibility that we expand and diversify our product pipeline through acquisitions of additional drug candidates that fit our business strategy;
- our belief that our existing cash and cash equivalents 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, for at least twelve months from the date these financial statements are issued;
- 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;
- the extent to which our business may be adversely impacted by the effects of the COVID-19 coronavirus pandemic or by other pandemics, epidemics or outbreaks;

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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.

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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 9, 2023 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, 2022 December 31, 2023, which are included in our Annual Report on Form 10-K filed with the SEC on March 9, 2023 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. 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®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 for commercial launches of ZTALMY in select European countries in 2024.

We are also developing ganaxolone for the treatment of other rare genetic epilepsies, including Tuberous Sclerosis Complex (TSC), and for the treatment of Refractory Status Epilepticus (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 are classified as having RSE.

We are developing ganaxolone in formulations for two different routes of administration: intravenous (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, and for both acute hospital care and chronic at home-administration settings. 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). Ganaxolone is a synthetic analog of allopregnanolone, an endogenous neurosteroid. Ganaxolone acts at neurosteroid, and targets both synaptic and extrasynaptic $GABA_A$ receptors, a target known for its anti-seizure, antidepressant and anxiolytic potential.

COVID-19

COVID-19 affected our clinical operations and timelines. For example, our Randomized Therapy In Status Epilepticus (RAISE) trial for RSE is conducted in hospitals, primarily intensive care units in academic medical centers, which experienced high rates of COVID-19 admissions. Several of these sites participating in the RAISE trial experienced COVID-related difficulties, including staff turnover and the need to devote significant resources to patients with COVID-19, which resulted in site initiation and enrollment delays for the RAISE trial. Given these COVID-19-related challenges and the interruption in drug supply in mid-2022, we previously adjusted our expectation for our top-line data readout for the RAISE trial. In May 2022, we resumed screening and recruitment for the RAISE trial. We now expect our interim analysis with top-line data readout for the RAISE trial to be available in the second quarter of 2024, if the pre-defined stopping criteria from the planned interim analysis are met.

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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.

Our Products 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 2 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 $5.4 million $7.5 million and $13.0 million in $3.3 million for the three and nine months ended September 30, 2023, March 31, 2024 and 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 launch of ZTALMY, 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.

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, as well as 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 in 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 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 in respect of its claim. The first $2 million installment was paid to Purdue in March 2024, and the second $2 million installment will be paid on or before June 15, 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 is was required to determine whether the orphan drug designation criteria are were still met. On May 26, 2023, the COMP provided a positive opinion to maintain the orphan drug designation for ganaxolone in for CDD in the EU.

The US U.S. and EC approvals of ZTALMY in for CDD are based on data from a Phase 3 double-blind placebo-controlled trial (Marigold Study) 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 Study, 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 Study 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.

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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 U.S. CDD patients in the U.S. through a specialty pharmacy. Key launch commercial strategies have included and continue to include: (1) establishing 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) developing 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.

U.S. Sales Strategy. Our U.S. commercial sales force includes 16 regional account managers experienced in rare disease. Our field force is targeting identified key accounts and centers of excellence for CDD. Based on our market research, we estimate the addressable patient population for ZTALMY in for CDD in the U.S. is approximately 2,000 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 cross-functional 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. patients, , including patient enrollment, benefit verification and investigation, prior authorization support, patient education and drug counseling, dispensing of product and shipment coordination. We recorded our first sales of ZTALMY to Orsini in the third quarter of 2022.

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.

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Infrastructure. We continue to enhance our internal capabilities and processes to support a 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

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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 Administration 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 completed and 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, and 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; a CNS distribution study of the M17 metabolite in rats; and in vitro studies to assess the drug interaction potential of the M17 metabolite. rats. We expect to be able to complete these remaining required FDA studies within and are working with the requested FDA timeframe. with respect to the timing of their completion and submission.

Managed Access Program (MAPS). We have recently 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 and on 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. In September 2023, the The clinical study report (CSR) for Study 1042-HME-1001 and the was submitted in September 2023. The ganaxolone Steady-State Metabolite Study report, were submitted. The remaining post-marketing authorization measures include: completing the final Study 1042-CDD-3001 CSR with the open-label trial completion; 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

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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 M17 in vitro drug-drug interaction (DDI) Studies; conducting a M17 in vivo pharmacokinetic (PK) Study with Brain Penetrance; 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

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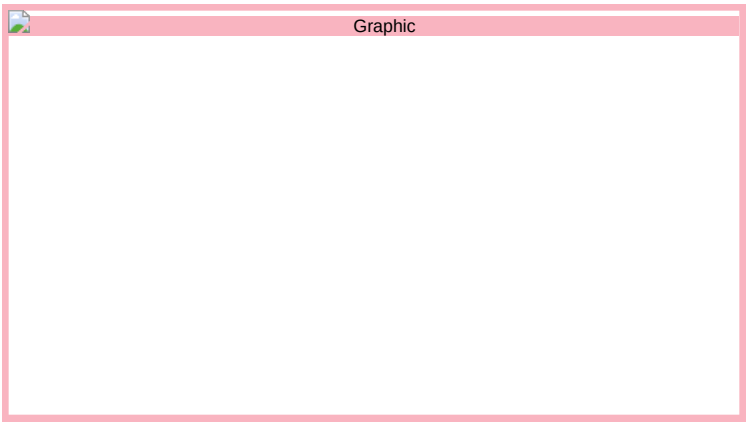
carcinogenicity study in rats with ganaxolone, a 2-year carcinogenicity study in rats with M2, and a juvenile toxicity study with M2. We expect to be able complete the remaining required studies within the requested EMA timeframe.

Our Pipeline

We are developing ganaxolone in indications where there is a mechanistic rationale for ganaxolone to provide a benefit, including the following indications:



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 requires 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 pivotal RAISE trial. The RAISE trial is a randomized, double-blind, placebo-controlled clinical trial in patients with RSE. We expect approximately 70 trial sites in hospitals, primarily across the U.S. and Canada, to participate. The RAISE trial is designed to enroll approximately 124 patients, to be randomized to receive ganaxolone or placebo added to standard of care. With this number of patients, the trial is designed to provide over 90% power to detect a 30% efficacy difference between ganaxolone and placebo. 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 the trial. We anticipate reaching the enrollment target for the interim analysis in the first quarter of 2024 with topline data now expected in the second quarter of 2024, if the pre-defined stopping criteria from the planned interim analysis are met. We believe positive interim RAISE trial results could be adequate for regulatory filing purposes in RSE.

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The co-primary endpoints for the RAISE trial are (1) proportion of patients with RSE who experience seizure cessation within 30 minutes of treatment initiation without other medications for SE treatment, and (2) proportion of patients with no progression to IV anesthesia for 36 hours following initiation of the trial drug. In June 2022, we announced that we amended the protocol for the RAISE trial to expand eligibility criteria to support recruitment. We broadened the inclusion criteria to permit patients previously treated with up to 18 hours of high-dose IV anesthesia to qualify for the trial, rather than excluding patients treated with anesthetics at high doses for any duration. We believe this

will facilitate the enrollment of patients transferred to the ICU from other hospitals or the emergency room, who may already have received high doses of anesthetic medication for less than 18 hours.

Several academic medical centers and intensive care units participating in the RAISE trial experienced COVID-related difficulties, including staff turnover and the need to devote significant resources to patients with COVID-19, which resulted in site initiation and enrollment delays. Additionally, 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 current IV formulation of ganaxolone, and we implemented a reduced shelf life of 12 months. In agreement with the FDA, ganaxolone clinical supplies with the current IV formulation will be stored under refrigerated conditions for the entire duration of clinical use. Based on ongoing stability data, the shelf life of the current IV formulation has been updated to 18 months under refrigerated conditions, which was submitted in the subsequent IND amendment in February 2023. 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 additional information be submitted prior to use of the new formulation in the clinical trials. An IND amendment was submitted to the FDA in May 2023. All sites have been resupplied with the new formulation, which we believe will not require refrigeration and is expected to have a two-year shelf life.

Planning continues for a separate Phase 3 RSE trial to support an MAA in Europe (RAISE II trial). We gained alignment on the trial design at a meeting with the EMA in the first quarter of 2021. Due to the delay in clinical trial supply mentioned for the RAISE trial, the RAISE II trial initiation is planned for the fourth quarter of 2023. The RAISE II trial will be a double blind, placebo-controlled pivotal registration trial expected to enroll 70 patients who have failed first-line benzodiazepine treatment and at least one second-line AED. Patients will receive either ganaxolone or placebo, administered in combination with a standard-of-care second-line AED. The simultaneous administration of a standard-of-care AED with the trial drug is expected to provide data complementary to that from the RAISE trial. There are two additional key differences between the RAISE and RAISE II trials. First, rather than specifying progression to IV anesthesia as a treatment failure, under the RAISE II protocol any escalation of care will constitute a treatment failure. This could be IV anesthesia or another second-line IV AED. Second, the primary analysis for the RAISE II trial will be 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.

We have recently decided to discontinue the RESET trial, a Phase 2 trial evaluating ganaxolone in the treatment of ESE, and focus our resources for IV ganaxolone on completing the RAISE trial and accelerating the RAISE II trial and further investigate potential development opportunities in SRSE. The RESET trial proved complex and costly to enroll.

SRSE is a life-threatening neurological emergency with high morbidity and mortality. We do not currently have a clinical trial ongoing in SRSE, however, physicians have received ganaxolone for 21 patients in SRSE under emergency investigational new drug (EIND) applications that have been approved by the FDA. We plan to engage with the FDA to request input on potential pursuing development of ganaxolone for SRSE in light of the encouraging data generated from patients treated under the EINDs to date.

In September 2021, the U.S. Patent and Trademark Office (USPTO) granted us a patent on a method of treating SE, including dosing regimens. This issued patent expires in 2040. That patent is a member of a patent family we own

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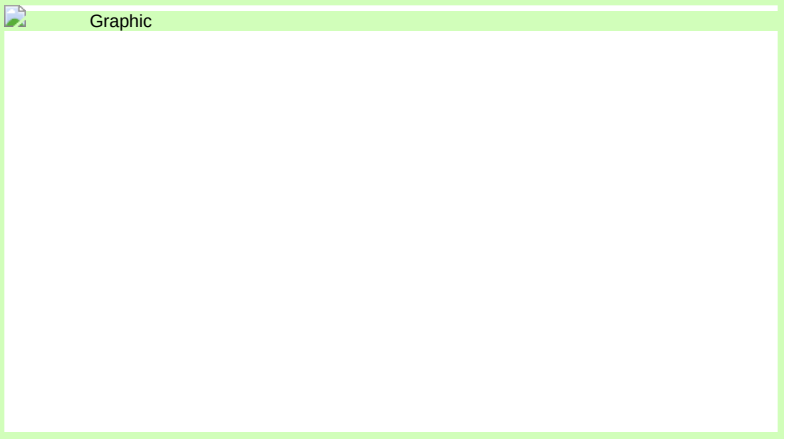
that includes pending patent applications that claim certain therapeutic regimens for the treatment of SE, including RSE, using intravenous ganaxolone. The USPTO granted us a second patent for SE on June 20, 2023, which includes claims related to our clinical therapeutic regimen for the treatment of SE using IV ganaxolone. These new claims 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 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 U.S. Patent and Trademark Office 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 next steps in the PGR are replies by us and Ovid as well as depositions of the experts. The oral arguments are currently scheduled for May 22, 2024. The decision to institute is not a final decision on the patentability of the claims. The final decision will be selected indications based on the full record developed during the proceeding. If we do not prevail in the PGR proceeding, the decision can be appealed to the Court mechanism 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.

The Ovid '817 Patent claims cover the use action and clinical profile of ganaxolone, in including the treatment of SE and do not cover or impact our marketing and sales of ZTALMY for the treatment of seizures associated with CDD. If we prevail in the PGR, the Ovid '817 Patent will not be enforceable against us. following programs:

Ovid may file a lawsuit 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 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 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, 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 we may be prevented from commercializing ganaxolone for RSE altogether, which would have a material adverse impact on our business.



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 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 (CALM trial) evaluating the safety and efficacy of adjunctive oral ganaxolone in 23 patients with seizures associated with TSC. TSC-associated seizures. The CALM 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 met eligibility criteria completed the initial 12-week treatment period were able to continue ganaxolone treatment during a 24-week extension. an extension phase of the trial. The primary endpoint was the percent

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percent 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. Additionally, Based on the data reports of somnolence 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. The interpretation of these findings is limited by the small sample size and open-label design of the trial. A formal Phase 1 drug-drug interaction trial was completed in the fourth quarter of 2022, demonstrating a lack of significant interaction between ganaxolone and Epidiolex. Additionally, the revised titration schedule for all subjects has been implemented in the Phase 3 TSC trial has been adjusted designed to maximize tolerability. 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 trial.

In response to our request for an End of 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. We believe that, based on the FDA's written responses, TSC, and with the FDA approval of CDD, that a single trial could serve as necessary support for regulatory approval for 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 first quarter of 2022 U.S. and dosing the first patient Europe in the second first quarter of 2022, we are actively enrolling patients in the U.S. and Western Europe for this global Phase 3 randomized, double blind, placebo-controlled trial (TrustTSC trial) of adjunctive ganaxolone in approximately 128 patients. With this number patients with TSC-related seizures. Based on the sample size of patients, 128 trial participants, the trial is designed to provide provides 90% power to detect a 25% efficacy difference in seizure reductions between ganaxolone and placebo. We expect to expand the The trial to include up to 90 has 93 sites activated, including several TSC centers of excellence, predominantly in the U.S., Western Europe, Canada, Australia, China and Israel. The primary endpoint for the TrustTSC trial is percent change in 28-day frequency of TSC-associated seizures. We seizures, and we plan to announce top-line data from in the TrustTSC trial by mid-2024. fourth quarter of 2024.

In July 2023, the USPTO granted us a patent on a method of treating TSC-related epilepsy by administering oral ganaxolone. This issued patent expires in 2040. This patent is a member of a patent family we own that includes pending patent applications that claim certain therapeutic regimens for the treatment of TSC.

Second-Generation Formulation, Clinical Development 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 Lennox-Gastaut Syndrome (LGS) 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 Prodrug Development

Top-line data from prevent neuronal injury and other complications. The IV anesthetic is increased to a single ascending dose (SAD) Phase 1 trial in healthy volunteers utilizing 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 first candidate for a second-generation IV formulation of ganaxolone demonstrated linear pharmacokinetic (PK) properties at doses for the treatment of up to 1200 mg and PK characteristics that may allow for twice-daily dosing. Additionally, as shown in the figure below, while area under the curve (AUC), SE, which quantifies the total exposure to drug over a period of time, demonstrated linear kinetics through the full range dosed, C_{max}, which is the maximum serum concentration that a drug achieves after administration, showed less substantial increases at the higher end of the dose range. These findings suggest that efficacy could be improved using higher doses of ganaxolone without corresponding increases in dose-related side effects, such as somnolence. This would permit greater individualization of dosing to optimize efficacy and tolerability. 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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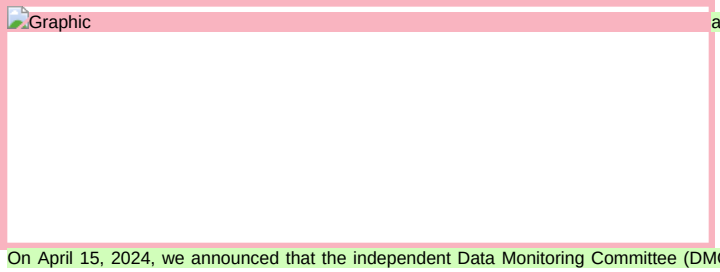

analysis was completed in the first quarter of 2024. On April 15, 2024, we announced that the independent Data Monitoring Committee (DMC) completed its review of the interim analysis. The trial did not meet the pre-defined interim analysis stopping criteria on the co-primary endpoints, and the DMC recommendation 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

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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 believe that are evaluating the data support further clinical development of this formulation of ganaxolone. We received preliminary feedback from Ovid '930 Patent and the FDA on the design of the multiple ascending dose (MAD) trial. The FDA requested additional data, including the SAD clinical trial report, prior to initiating the MAD trial. The information was provided to the FDA and, upon review, the FDA indicated that we may proceed with the proposed trial. We initiated the MAD trial in the third quarter of 2023 with preliminary data expected by the end of 2023. Ovid '563 Patent.

The development 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 prodrug compounds continues 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 advance, 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 lead oral 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 candidates selected, 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, Prodrug Development and Phase 1 data targeted for 2024. Second-generation Formulation

We plan to pursue expand our investment in ZTALMY to explore its potential in the development treatment of the second-generation formulation 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 epileptic encephalopathies, including LGS, targeted to begin in the first half of 2025, pending the top-line data results from our Trust TSC 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 other disorders where ganaxolone has therapeutic potential, to reduce seizures, such as CDD and TSC, we believe that LGS represents a promising opportunity for ZTALMY, prodrug, or our second-generation ganaxolone development. formulation.

The development of ganaxolone prodrug compounds continues to advance with lead oral and IV candidates having been selected. We expect to finalize anticipate completion of IND-enabling studies for the clinical program design for LGS oral prodrug in the first quarter half of

2024, 2025, followed by IND filing and initiation of Phase 1 trials in 2025, pending the top-line data results from our Trust TSC 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, that we initiated in the third quarter of 2023. along with predictable dose-exposure relationships, may allow physicians to individualize dosing to patient needs.

On September 27, 2023, the USPTO issued a Notice of Allowance in an Ovid patent application with claims that encompass our product candidate for the treatment of LGS. This patent is scheduled to issue issued, U.S. Patent No. 11,806,336, on November 7, 2023. The claims in the Ovid LGS patent application 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.

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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 raising capital. the early commercialization of ZTALMY. We have funded our operations primarily through sales of equity and debt securities. ZTALMY, our first FDA approved product, became available for commercial sale and shipment in the third quarter of 2022. We recorded $5.4 million $7.5 million and $13.0 million $3.3 million of ZTALMY net ZTALMY sales in the three and nine months ended September 30, 2023, March 31, 2024 and 2023, respectively. Since inception, we have incurred negative cash flows from our operations, and other than for

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the three months ended September 30, 2022 due to a one-time net gain from the sale of our PRV, Priority Review Voucher (PRV), we have incurred net losses and negative cash flows from our operations. 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 Net losses of $33.0 million $38.7 million and $99.6 million $34.7 million for the three and nine months ended September 30, 2023, respectively. Due to the one-time receipt of proceeds from the sale of the PRV in the third quarter of 2022, we generated net income of $73.3

million March 31, 2024 and $14.5 million for the three and nine months ended September 30, 2022, 2023, respectively. Our accumulated deficit as of September 30, 2023 March 31, 2024 was $530.2 million, and $610.6 million.

We anticipate that we expect to will continue to incur substantial losses in future periods. We anticipate that our operating expenses will increase substantially as we carry out all of our planned commercialization and continued research and development activities with respect to ganaxolone.

We anticipate that our expenses will increase substantially 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;
- continue to 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;
- 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 equivalents and short-term investments of $176.4 million $113.3 million as of September 30, 2023 March 31, 2024. We believe that our existing cash, cash equivalents and short-term investments as of September 30, 2023 March 31, 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, for at least twelve months from into the date these financial statements first quarter of 2025. This expectation includes cost reduction activities that are issued. However, we being 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 over 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

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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 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 Product revenue is recognized, resulting in a reduction of product 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 ganaxolone in patients with RSE, which covers the RAISE trial, funding of pre-clinical 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 will 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 contract 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

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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.

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We recognize federal Federal contract revenue from the BARDA Contract in the period in which the allowable research and development expenses are incurred. We expect federal Federal contract revenue to increase decrease as the costs associated with our RAISE trial increase.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. Orion Corporation (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) 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. 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 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

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 the 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 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 we will recognize such revenue or expense, as applicable, as it fulfills 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 distribution 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 have had initiated limited sales of ZTALMY to NovaMedica to support on-going early access programs associated with patients from the Marigold Study. 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.

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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 and services; supplies;

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- 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. approval in other indications. We may never succeed in achieving regulatory approval for ganaxolone. 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.

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"*Risk Factors* section of our Annual Report on Form 10-K filed on March 9, 2023 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, 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.

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 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 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 for commercially saleable product and materials were incurred and included in research Research and development expenses. As a result, cost Cost of product revenues related to ZTALMY initially reflected a lower average per unit cost of materials, and will initially continue to reflect a lower average per unit cost of materials into approximately through the first half second quarter of 2024, as previously expensed inventory is utilized for commercial production and sold to customers.

Cost of IP License Fee

In March 2022, we entered into an exclusive patent license agreement (License Agreement) with Ovid. Under the License Agreement, we have an exclusive, non-transferable (except as provided in the License Agreement), royalty-bearing, sublicensable license under certain of Ovid's patent(s) and patent applications to develop, make, have made, commercialize, promote, distribute, sell, offer for sale and import, ganaxolone, including any analogues or derivatives, including its salts, and pharmaceutical formulations of the foregoing (Licensed Products), in the U.S., the member states of the EU, Iceland, Lichtenstein, Norway, the United Kingdom, and Switzerland (Territory) for the treatment of CDD in humans (Field). Under the License Agreement, we have the sole right and responsibility for, and control over, all development, manufacturing, and commercialization activities, including all regulatory activities, with respect to the

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Licensed Products in the Field in the Territory. In addition, all regulatory approvals and related filings with respect to the Licensed Products in the Field in the Territory will be in the name of, and be owned solely by, us. We were required, at Ovid's option exercisable in accordance with the License Agreement, to (i) pay to Ovid the sum of $1.5 million in cash; or (ii) issue to Ovid 123,255 shares of our common stock, which option to obtain shares of our common stock was exercisable within the five-business day period following the filing of our Annual Report on Form 10-K for the year ended December 31, 2021 on March 24, 2022. On March 29, 2022, we issued 123,255 shares of our common stock to Ovid, per Ovid's option in accordance with the License Agreement. As such, we recorded $1.2 million of IP license fee expenses related to the Ovid License Agreement in the nine months ended September 30, 2022.

The License Agreement also provides for payment of royalties by us to Ovid in the low single digits on net sales by us, our affiliates and sublicensees, of Licensed Products in the Field in the Territory. Such royalties are subject to reduction in the event of generic competition in accordance with the License Agreement. We may terminate the License Agreement at any time without cause on thirty days' prior written

notice. Either party may terminate the License Agreement for the other party's material breach or insolvency subject to certain cure periods. Also, Ovid has the right to terminate the License Agreement if there has not been a first commercial sale of any Licensed Products in the Field in the Territory on or before June 30, 2025. In the event of termination, all licenses granted under the License Agreement will terminate.

Interest Income

Interest income consists principally of interest income earned on cash Cash and cash equivalents and investment 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, (Expense), net

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

Benefit (Provision) for Income Taxes

We recorded a $1.5 million benefit for income taxes due to the identification of a discrete item of tax determined upon preparation of our 2022 tax return in the nine months ended September 30, 2023. We did not record a benefit or provision for income taxes in the three months ended September 30, 2023. We recorded a $1.8 million provision for income taxes in the three and nine months ended September 30, 2022 due to net income related to the one-time receipt of proceeds from the sale of the PRV. adjustments.

Results of Operations

Product Revenue, net

We recognized $5.4 million $7.5 million and $13.0 million $3.3 million of ZTALMY net product revenue for the three and nine months ended September 30, 2023 March 31, 2024 and March 31, 2023, respectively. We recognized $0.6 million of ZTALMY net product revenue for both The increase in the three and nine months ended September 30, 2022. March 31, 2024 compared to the 2023 period was primarily due to an increase in patients in the three months ended March 31, 2024 as ZTALMY had only recently become available for commercial sale and shipment in the comparable 2023 period.

Federal Contract Revenue

We recognized $1.9 million $0.2 million and $10.8 million $7.0 million of federal contract revenue for the three and nine months ended September 30, 2023 as a result of the BARDA Contract. We recognized $1.8 million March 31, 2024 and $5.1 million of federal contract revenue for the three and nine months ended September 30, 2022, 2023, respectively, as a result of the BARDA Contract. The

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increase decrease in the nine three months ended September 30, 2023 March 31, 2024 compared to the 2022 2023 period primarily relates related to expenses incurred in connection with on-going validation of a new third-party supplier of ganaxolone API. API in the 2023 period.

Collaboration Revenue

Collaboration revenue, which resulted from our agreement with Biologix, was less than $0.1 million for the three and nine months ended September 30, 2023 March 31, 2024. No collaboration revenue was recorded in the three months ended September 30, 2022 March 31, 2023. Collaboration revenue was $12.7 million for the nine months ended September 30, 2022 a result

Table of revenue recognition related to the previously refundable upfront payment pursuant to the Orion Collaboration Agreement. In connection with the upfront fee related to the Orion Collaboration Agreement, we agreed to provide Orion with the results of an ongoing genotoxicity study. In May 2022, the final study report showed that no genotoxicity was found, as measured by formation of micronuclei in the bone marrow or comet morphology in the liver. As a result of the study's findings, we were not required to refund Orion any of the upfront fee and Orion does not have the right to terminate the Orion Collaboration Agreement based on the study outcome. During the nine months ended September 30, 2022, we allocated the previously refundable portion of the upfront payment to the transaction price and recognized the related revenue. Contents

Research and Development Expenses

We record direct research 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, in thousands. program. The primary drivers of our research Research and development expenditures are currently in our product development programs in 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 September 30,		Nine Months Ended September 30,		Three Months Ended March 31,	
	2023	2022	2023	2022	2024	2023
CDKL5 deficiency disorder (1)	$ 406	$ 909	$ 2,461	$ 2,973	$ 218	$ 1,240
PCDH19-related epilepsy (2)	120	—	245	1,063	47	94
Tuberous Sclerosis (3)	3,491	2,590	10,865	6,897	2,827	3,788
Drug Development – Suspension (4)	1,267	1,115	2,425	2,770	1,538	270
Oral Indications Subtotal	5,284	4,614	15,996	13,703	4,630	5,392
Status epilepticus (5)	4,117	2,661	10,697	6,484	4,216	3,002
Drug Development – IV (6)	1,305	708	10,733	4,688	1,423	8,489
IV Indications Subtotal	5,422	3,369	21,430	11,172	5,639	11,491
Other research and development (7)	2,193	2,356	4,021	7,751	1,864	1,196
Indirect research and development (8)	10,762	8,663	31,559	25,862	11,985	9,854
Total	$ 23,661	$ 19,002	$ 73,006	$ 58,488	$24,118	$27,933

(1) The decrease in the three months ended September 30, 2023 March 31, 2024 compared to the 2022 2023 period was due primarily to decreased clinical trial activity. The decrease in the nine months ended September 30, 2023 compared to the 2022 period was also due primarily to decreased clinical trial activity but was partially offset by increased costs due to increased clinical site close-out activities related in the three months ended March 31, 2024 as compared to the Marigold Study 2023 period and increased activity associated with the MAA application and review in the first quarter of 2023. three months ended March 31, 2023 with no comparable costs in the 2024 period.

(2) The increase decrease in the three months ended September 30, 2023 March 31, 2024 compared to the 2022 2023 period was due primarily to increased reduced clinical trial close-out costs. activity.

(3) The decrease in the nine three months ended September 30, 2023 March 31, 2024 compared to the 2022 period was due to reduced clinical activity, specifically completion of the open label extension portion of the PCDH-19 trial.

(3) The increase in the three and nine months ended September 30, 2023 compared to the 2022 2023 period was due primarily to increased decreased Phase 3 TSC trial activity including in the three months ended March 31, 2024 and increased global site activity as compared to more limited Phase 3 activity in the 2022 2023 period.

(4) The increase in the three months ended September 30, 2023 March 31, 2024 compared to the 2022 2023 period was due primarily to higher manufacturing development activity related to clinical trial batches than in the prior 2022 2023 period. The decrease in the nine months ended September 30, 2023 compared to the 2022 period was due primarily to higher manufacturing development activity related to pre-validation and registration batches in the 2022 period, partially offset by the higher manufacturing development activity related to clinical trial batches.

(5) The increase in the three and nine months ended September 30, 2023 March 31, 2024 compared to the 2022 2023 period was due primarily to increased RAISE and RAISE II Phase 3 trial activity.

(6) The increase decrease in the three months ended September 30, 2023 March 31, 2024 compared to the 2022 2023 period was due primarily to the completion of registration batches with no comparable costs in the prior 2022 period. The increase in the nine months ended September 30, 2023 compared to the 2022 period was due primarily to 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 prior 2022 period. three months ended March 31, 2024.

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(7) The increase in the three months ended March 31, 2024 compared to the 2023 period was 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 decrease increase in the three and nine months ended September 30, 2023 March 31, 2024 compared to the 2022 2023 period was due primarily related to decreased activities associated with the Phase 1 clinical trials.

(8) increased personnel costs 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. The increases in the three and nine months ended September 30, 2023 compared to the 2022 periods were primarily related to increased personnel costs in support of our increased activity in preclinical, clinical, and manufacturing activities.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $14.9 million $18.6 million and $45.8 million $15.2 million for the three and nine months ended September 30, 2023, respectively, compared to $13.4 million March 31, 2024 and $42.2 million for the three and nine months ended September 30, 2022, 2023, respectively. The primary drivers of the increase for the three months ended September 30, 2023 March 31, 2024 compared to 2022 the 2023 period were $0.5 million $1.5 million in increased commercial costs, $0.5 million $1.0 million in increased consulting stock-based compensation costs, $0.4 million $0.6 million in increased personnel costs, and net other cost increases of $0.1 million. The primary drivers of the increase for the nine months ended September 30, 2023 compared to 2022 were $3.4 million in increased personnel costs, $1.5 million $0.6 million in increased professional fees, consulting costs and other general costs, $0.6 million in increased software related costs and $0.3 million in increased stock-based compensation costs. These increases which were partially offset by $2.2 million a decrease of $0.3 million in decreased commercial costs, primarily due to significant commercial-launch preparedness activities in 2022. software related expenses.

Interest Income

Interest income was $1.9 million and $6.4 million $1.5 million for the three and nine months ended September 30, 2023, respectively, March 31, 2024 compared to $0.5 million and $0.6 million $2.3 million for the three and nine months ended September 30, 2022, respectively. March 31, 2023 and consisted of interest earned on Cash and cash equivalents and Short-term investments. The increase is decrease was due to the increase a decrease in cash, Cash and cash equivalent and short-term Short-term investments and increased yield. in the three months ended March 31, 2024 as compared to the three months ended March 31, 2023.

Interest Expense

Interest expense was $4.2 million and $12.6 million $4.3 million for the three and nine months ended September 30, 2023, respectively, March 31, 2024 compared to $2.6 million and $7.0 million $4.1 million for the three and nine months ended September 30, 2022, respectively. March 31, 2023. Interest expense for the nine three months ended September 30, 2023 March 31, 2024 included $6.5 million $2.2 million of interest paid and $1.5 million $0.5 million of debt amortization in connection with our Note Payable (Note 10 in accompanying notes to consolidated financial statements), and $4.4 million $1.6 million of non-cash interest expense and $0.2 million $0.1 million of debt amortization related to our revenue Revenue interest financing payable (Note 11 in accompanying notes to consolidated financial statements). Interest expense for the nine months ended September 30, 2022 included $5.7 million of interest paid, $1.2 million of debt amortization, and $0.1 million related to commitment fees paid in connection with our Note Payable.

Other Income, (Expense), net

Other income, net was $1.0 million and $1.1 million less than $0.1 million for each of the three and nine months ended September 30, 2023, respectively, due primarily to the IRS Employee Retention Tax Credit program. March 31, 2024 and 2023. Other expense was $0.1 million and $1.2 million for the three and nine months ended September 30, 2022, respectively, which consisted income, net consists principally of foreign currency transaction non-operational transactions, gains or losses 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 and negative cash flows from our operations. losses. We incurred a net Net loss of $99.6 million $38.7 million for the nine three months ended September 30, 2023. Due to the one-time receipt of proceeds from the sale of the PRV of $110.0 million in the third quarter of 2022, we generated net income of $14.5 million for the nine months ended September 30, 2022 March 31, 2024. Our cash Cash used in operating activities was $91.0 million $37.5 million for the nine three months ended September 30, 2023 March 31, 2024 compared to $91.0 million $41.5 million for the nine three months ended September 30, 2022 March 31, 2023. Historically, we have financed our operations principally through the sale of common stock, notes payable, preferred stock and convertible debt.

In July 2022, we entered into the PRV Asset Purchase Agreement to sell our PRV, pursuant to which Novo Nordisk, Inc. paid us $110.0 million upon the closing of the transaction. 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. Our position communicated We responded to Purdue is that we do did not owe agree with their claim. In February 2024, following discussions with Purdue, any we agreed to pay Purdue $4 million in respect of its

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claim. We paid the proceeds from the sale first installment of the PRV. No associated payment by us has been made, and Purdue has not filed a specific claim to date.

In November 2022, $2 million in connection with an underwritten public offering of 10,526,316 shares of our common stock, pre-funded warrants to purchase 2,105,264 shares of common stock and the exercise of an option of 1,894,737 shares of common stock, March 2024. At March 31, 2024 we received approximately $64.5 million in total net proceeds after taking into account the exercise of the underwriters' option, in each case deducting the underwriting discounts and commissions and after deducting offering expenses paid or payable by us. Additionally, in November 2022, we received an upfront payment of $32.5 million pursuant had $2.0 million accrued related to the Revenue Interest Financing Agreement with Sagard, and in December 2022, we received an upfront payment of $10.0 million in connection with the Tenacia Collaboration Agreement.second $2 million installment which will be paid on or before June 15, 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 September 30, 2023 March 31, 2024, we had cash, Cash and cash equivalents and short-term Short-term investments of $176.4 million $113.3 million. We believe that our existing cash, Cash and cash equivalents and short-term Short-term investments as of September 30, 2023 March 31, 2024 will be sufficient to

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fund our operating expenses and capital expenditure requirements, as well as maintain the minimum cash balance required under our debt facility, for at least twelve months from into the date these financial statements first quarter of 2025. This expectation includes cost reduction activities that are issued. However, we being 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.

Orion Commercialization Agreement

On July 30, 2021, we entered into the Orion Collaboration Agreement, whereby Orion received exclusive rights to commercialize the oral and IV dose formulations of ganaxolone in the European Economic Area, United Kingdom and Switzerland (collectively, Territory) in multiple seizure disorders, including CDD, TSC and RSE. Under the agreement, we received a €25 million ($29.6 million) upfront fee. We are eligible to receive up to an additional €97 million in research and development 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. 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 Euro, if commercial sales of ZTALMY commence in the Territory, 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 or (2) the 18-month anniversary of the first commercial sale of ZTALMY in the Territory. Also, as part of the overall arrangement, we have agreed to supply the Licensed Products to Orion at an agreed upon price.

Tenacia Commercialization Agreement

On November 16, 2022, we entered into a collaboration and supply agreement with Tenacia, pursuant to which 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 RSE) (collectively, Initial Indications). The collaboration can be expanded to include additional indications and formulations of ganaxolone pursuant to a right of first negotiation.

In connection with the execution 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 and payment 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 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

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Collaboration Agreement, including if net sales decrease by a certain percentage after the introduction of a generic product.

Biologix Distribution and Supply Agreement

In May 2023, we entered into an exclusive distribution and supply agreement (Biologix Agreement) with Biologix, whereby Biologix has the right to distribute and sell ganaxolone inAlgeria, 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 Biologix Agreement 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 agreement. We may be entitled to additional fees upon regulatory milestones. In the nine months ended September 30, 2023, we recorded less than $0.1 million of collaboration revenue related to the Biologix Agreement.

NovaMedica Agreement

In June 2013, we entered into a clinical development and collaboration agreement with NovaMedica LLC (NovaMedica) for commercialization of ganaxolone, and in December 2022, as amended in May 2023, we entered into a manufacturing and supply agreement with 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. In exchange for distribution rights, we will be the exclusive supplier of our products to NovaMedica on terms set forth in the NovaMedica Agreement in exchange for a negotiated purchase price for the products. As of the first quarter of 2023, we have initiated limited sales of ZTALMY to NovaMedica to support on-going early access programs associated with patients from the Marigold Study.

Oaktree Credit Agreement

On May 11, 2021 (Closing Date) and as amended on May 17, 2021, May 23, 2022 and October 28, 2022 (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 September 30, 2023 March 31, 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.

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 5.0% of the aggregate amount of the 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

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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.

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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 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 and BARDA 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, under which 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 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 of 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 will 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

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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 Agreement) 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 Agreement 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 Agreement. EDA. Subject to the terms and conditions of the Amended Agreement, 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. In the nine months ended September 30, 2023, we sold 3.7 million shares of our common stock pursuant to the Amended Agreement, which consisted of net proceeds of approximately $25.9 million at an average price of $7.17 per share. We did not sell any shares of our common stock during each the nine three months ended September 30, 2022 March 31, 2024 and March 31, 2023 under the EDA.

IP License Agreement

In March 2022, we entered into the License Agreement with Ovid. Under the License Agreement, we have an exclusive, non-transferable (except as provided in the License Agreement), royalty-bearing, sublicensable license under certain of Ovid's patent(s) and patent applications to develop, make, have made, commercialize, promote, distribute, sell, offer for sale and import, ganaxolone, including any analogues or derivatives, including its salts, and pharmaceutical formulations of the foregoing (Licensed Products), in the U.S., the member states of the EU, Iceland, Lichtenstein, Norway, the United Kingdom, and Switzerland (Territory) for the treatment of CDD in humans (Field). Under the License Agreement, we have the sole right and responsibility for, and control over, all development, manufacturing, and commercialization activities, including all regulatory activities, with respect to the Licensed Products in the Field in the Territory. In addition, all regulatory approvals and related filings with respect to the Licensed Products in the Field in the Territory will be in the name of, and be owned solely by, us. We were required, at Ovid's option exercisable in accordance with the License Agreement, to (i) pay to Ovid the sum of $1.5 million in cash; or (ii) issue to Ovid 123,255 shares of our common stock, which option to obtain shares of our common stock was exercisable within the five-business day period following the filing of our Annual Report on Form 10-K for the year ended December 31, 2021 on March 24, 2022. On March 29, 2022, we issued 123,255 shares of our common stock to Ovid, per Ovid's option in accordance with the License Agreement. As such, we recorded $1.2 million of IP license fee expenses related to the Ovid License Agreement in the nine months ended September 30, 2022.

The License Agreement also provides for payment of royalties by us to Ovid in the low single digits on net sales by us, our affiliates and sublicensees, of Licensed Products in the Field in the Territory. Such royalties are subject to reduction in the event of generic competition in accordance with the License Agreement. We may terminate the License Agreement at any time without cause on thirty days' prior written notice. Either party may terminate the License Agreement for the other party's material breach or insolvency subject to certain cure periods. Also, Ovid has the right to terminate the License Agreement if there has not been a first commercial sale of any Licensed Products in the Field in the Territory on or before June 30, 2025. In the event of termination, all licenses granted under the License Agreement will terminate.

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Cash Flows

Operating Activities. Cash used in operating activities was $91.0 million for the nine months ended September 30, 2023 $37.5 million and remained consistent compared to $91.0 $41.5 million for the same period in 2022. three months ended March 31, 2024 and 2023, respectively. Excluding the noncash impacts primarily related to depreciation and amortization, debt issuance costs, interest accretion net of revenue interest financing and amortization of discounts on short-term investments, cash paid, stock-based compensation, cost of license agreement and changes in the net contract assets/liabilities related to the Orion, Tenacia and Biologix collaboration agreements, Collaboration Agreements, the change in cash used in operating activities for the nine three months ended September 30, 2023 March 31, 2024 compared to the same period in 2022, 2023, was primarily the result of decreases in the changes in accounts payable, accrued expenses, including the $2.0

million installment to be paid to Purdue, other long term-liabilities, prepaid expenses, other current assets, inventory and accounts receivable and an increase in as operating expenses. expenses remained relatively consistent.

Investing Activities. Cash provided by investing activities for the three months ended March 31, 2024 represents $20.9 million in maturities of Short-term investments. Cash used in investing activities for the nine three months ended September 30, 2023 primarily March 31, 2023 represents $52.0 million in purchases of short-term investments and partially offset by $17.0 million in maturities of short-term investments. Cash provided by investing activities for the nine months ended September 30, 2022 represents net proceeds of $107.4 million from the sale of the PRV, partially offset by $1.7 million in purchases of property and equipment.

Financing Activities. Cash provided by financing activities for the nine three months ended September 30, 2023 March 31, 2024 represents $25.9 million in net proceeds related to the sale of common stock in connection with the EDA as amended, $0.8 million $0.3 million in proceeds from the exercise of stock options, partially offset by $0.7 million of payments options. Cash used in connection with the Revenue Interest Financing Agreement with Sagard. Cash provided by financing activities for the nine three months ended September 30, 2022 includes $28.8 million in proceeds from notes payable, net of issuance costs, March 31, 2023 was less than $0.2 million and $1.8 million in proceeds from the exercise of stock options. represented 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 and negative cash flows from our operations. losses. We incurred a net Net loss of $99.6 million $38.7 million for the nine three months ended September 30, 2023 March 31, 2024. 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, Cash and cash equivalents and short-term Short-term investments of $176.4 million $113.3 million as of September 30, 2023 March 31, 2024. We believe that our existing cash, Cash and cash equivalents and short-term Short-term investments as of September 30, 2023 March 31, 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, for at least twelve months from into the date these financial statements first quarter of 2025. This expectation includes cost reduction activities that are issued. However, we being 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. Further, the continued spread of COVID-19 has also led to severe disruption and volatility in the global capital markets, which could increase our cost of capital and adversely affect our ability to access the capital markets in the future. 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 effects of the COVID-19 pandemic on our business, the medical community and the global economy;
- 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; 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 exclusive license collaboration and supply agreements with Orion for the commercialization of ganaxolone in Europe, our collaboration and Tenacia. 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, 2022 December 31, 2023 filed with the SEC on March 9, 2023 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 September 30, 2023 March 31, 2024, there were no

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significant changes to our critical accounting policies from those described in our annual consolidated financial statements for the year ended December 31, 2022 December 31, 2023, which we included in our Annual Report on Form 10-K and was filed with the SEC on March 9, 2023 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.

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 September 30, 2023 March 31, 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 September 30, 2023 March 31, 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 are not currently a party to any material legal proceedings, and we are not aware of any pending or threatened legal proceedings against us that we believe could have a material adverse effect on our business, operating results or financial condition.

Item 1A. Risk Factors

Except as set forth below, there 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, 2022. December 31, 2023, except as follows:

Third parties, such as Ovid Therapeutics, Inc., may initiate legal proceedings alleging that we are infringing their intellectual property rights, Our future success is dependent on the outcome successful clinical development, regulatory approval and continued commercialization of ganaxolone, which would be uncertain is being studied in several indications and could harm our business. will require significant capital resources and years of additional clinical development effort.

Our commercial success depends upon 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 develop, manufacture, market successfully complete clinical development, scale-up manufacturing, obtain regulatory approval, and, sell our products and product candidates, all if it is approved, commercialize ganaxolone in a timely manner. We cannot commercialize additional indications or formulations of which contain ganaxolone and to use our related technologies. We may become party to, or threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to one or more of our products, including interference or derivation proceedings before in the U.S. Patent and Trademark Office (USPTO). Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue commercializing one or more of our products. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Under certain circumstances, we could be forced, including by court order, to cease commercializing one or more of our products. In addition, in any such proceeding other indication without first obtaining regulatory approval from the FDA; similarly, we cannot commercialize additional indications or litigation, 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 could be found liable for monetary damages. A finding of infringement could prevent us from commercializing one or more of our products or force us to cease some of our business operations, which could materially harm our business. Any claims by third parties that we have misappropriated their confidential information or trade secrets could have a similar negative impact on our business.

While our product candidates are must demonstrate with substantial evidence gathered in preclinical studies and clinical trials we believe that the use of our product candidates in these preclinical studies and, clinical trials falls within the scope of the exemptions provided by 35 U.S.C. Section 271(e) with respect to approval in the U.S., which exempts from patent infringement liability activities reasonably related 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 submission an in vivo micronucleus with comet analysis for the detection of information 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 (Federal Development Patent Infringement Exemption). As our product candidates progress toward commercialization, the possibility of a patent infringement claim against us increases. While ganaxolone itself is off patent, we attempt to ensure that our product candidates in May 2022. The Phase 1 hepatic impairment study and the methods we employ thorough QTc study were completed and submitted to manufacture ganaxolone do not infringe other parties' patents 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 other proprietary rights. There can be no assurance they do

not, however, and competitors have the potential to materially impact the label or other parties may assert that we infringe their proprietary rights in any event. our ability to market ZTALMY.

On July 26, 2022, the USPTO issued U.S. Patent No. 11,395,817 (Ovid '817 Patent) to Ovid Therapeutics, Inc. (Ovid) with claims that 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 In connection with the U.S. Patent 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 Trademark Office Patent Trial 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 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 next steps providing annual updates; participating in the PGR are replies by us and Ovid as well as depositions of the experts. The oral arguments are currently scheduled for May 22, 2024. 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. If we do not prevail in 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 PGR proceeding, compatibility of the decision can 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. While we expect to be appealed 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 Court of Appeals for the Federal Circuit. If an appeal is not successful, label or our ability to challenge marker ZTALMY.

We are conducting the Ovid '817 Patent RAISE trial in court RSE, which is a life-threatening medical condition involving prolonged seizure activity in seriously ill patients. The RAISE trial requires expertise in electroencephalogram (EEG) interpretation, which may be subject to variability, and 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 for this indication. In April 2024, the independent Data Monitoring Committee (DMC) completed its review of the RAISE trial 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 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. Based on this interim analysis, there is a substantial risk that the Phase 3 clinical trial of ganaxolone in RAISE will be limited not generate data that is sufficient to support regulatory approvals for this indication. Additionally, the clinical trial endpoints of the RAISE trial are based on treatment outcomes, including initiation of anesthesia for treatment of RSE. Practice variability in certain respects. The Ovid '817 Patent claims cover the use of anesthesia for SE treatment could adversely impact the ability to show a treatment effect with ganaxolone. Even if the RAISE trial shows that ganaxolone is effective, there is a risk that the FDA will require more safety data generated with 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 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 trial of ganaxolone in TSC will generate data that is not sufficient to support regulatory approvals for this indication.

Even if ganaxolone were to obtain approval from the treatment 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 SE 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 do obtain adequate reimbursement from third-party and government payers. If we are unable to successfully commercialize ganaxolone, we may not cover or impact be able to earn sufficient revenue to continue our marketing and sales of ZTALMY for the treatment of seizures associated with CDD. 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 USPTO issued a Notice of Allowance in an Ovid patent application with claims that encompass our product candidate for the treatment of LGS. The patent is scheduled to issue on November 7, 2023. The claims in this Ovid LGS patent application cover the use of ganaxolone for the treatment of LGS efficacy and do not cover or impact the use safety of ganaxolone in any 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 indication.

Ovid may file a lawsuit against us alleging infringement ganaxolone indications. A number of its patents. Any such proceeding, companies in the PTAB or courts, regardless 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 their outcome, would likely 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 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 that the Phase 3 clinical trial of ganaxolone in RAISE will not generate data that is sufficient to support regulatory approvals for this indication. 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 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 future do not produce favorable results, our ability to achieve regulatory approval for ganaxolone in those indications may be prohibited from marketing or selling ganaxolone for SE, RSE and LGS during such proceedings or adversely impacted. Further, even 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 believe

the data collected from our clinical trials of ganaxolone for SE, RSE or LGS based upon the "safe harbor" provisions of the Hatch-Waxman Act. We may need to acquire or obtain a license to certain Ovid patents to market or sell ganaxolone for SE, RSE or LGS, which are promising, these data may not be available on commercially acceptable terms sufficient to support approval by the FDA or at all. If we are not able to acquire certain Ovid patents foreign regulatory authorities. Pre-clinical and clinical data can be interpreted in different ways. Accordingly, the FDA 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, foreign regulatory authorities could interpret these data in different ways from us, which could delay, limit or we may be prevented from commercializing ganaxolone for SE, RSE and LGS altogether, which would have a material adverse impact on our business. 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

None.**Trading Plans**

During the three months ended March 31, 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.)
31.1	Certification of Chief Executive Officer pursuant to Rule 13a-14(a) or 15a-14(a) under the Exchange Act (filed herewith.)
31.2	Certification of Chief Financial Officer pursuant to Rule 13a-14(a) or 15a-14(a) under the Exchange Act (filed herewith.)
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	November 7, 2023 May 8, 2024
/s/ STEVEN PFANSTIEL Steven Pfanstiel	Chief Operating Officer, Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)	November 7, 2023 May 8, 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: November 7, 2023 May 8, 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: November 7, 2023 May 8, 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 September 30, 2023 March 31, 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: November 7, 2023 May 8, 2024

/s/ Scott Braunstein

Chief Executive Officer and Director

(Principal executive officer)

Date: November 7, 2023 May 8, 2024

/s/ Steven Pfanstiel

Chief Operating Officer, Chief Financial Officer and Treasurer

(Principal financial and accounting officer)

DISCLAIMER

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