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Marinus Pharmaceuticals Provides Business Update and Reports Fourth Quarter and Full Year 2021 Financial Results

03/21/2022


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- Received FDA approval of ZTALMY® (ganaxolone) for the treatment of seizures associated with CDKL5 deficiency disorder; first approved treatment for this rare form of genetic epilepsy
- Awarded a Rare Pediatric Disease Priority Review Voucher by the FDA
- Eligible for an additional \$30 million of funding under the existing Oaktree Capital Management, L.P. credit agreement
- Patient screening commenced for Phase 3 TrustTSC trial in tuberous sclerosis complex
- Resupply of clinical trial materials has been moved up to May 2022 for Phase 3 RAISE trial in refractory status epilepticus
- Marinus to host conference call today at 8:00 a.m. ET / 5 a.m. PT

RADNOR, Pa.--(BUSINESS WIRE)-- [Marinus Pharmaceuticals, Inc.](#) (Nasdaq: MRNS), a pharmaceutical company dedicated to the development of innovative therapeutics to treat seizure disorders, today reported business highlights and financial results for the fourth quarter and year ended December 31, 2021.

"This year has already proven to be pivotal for Marinus following the FDA's approval of ZTALMY®, the first and only treatment for seizures associated with CDKL5 deficiency disorder," said Scott Braunstein, M.D., Chief Executive Officer of Marinus Pharmaceuticals. "This achievement further validates our clinical development plans for ganaxolone as an innovative treatment option across a range of seizure disorders and sets the stage as we move into our next chapter of growth as a commercial stage company. We look forward to building on this momentum as the approval enables the potential for significant non-dilutive funding in the near-term to execute on our business strategy and invest in our future."

Commercial Preparedness - CDKL5 Deficiency Disorder

ZTALMY® (ganaxolone) oral suspension received U.S. Food and Drug Administration (FDA) approval on March 18, 2022 for the treatment of seizures associated with cyclin-dependent kinase-like 5 deficiency disorder (CDD) in patients two years of age and older.

- ZTALMY is expected to be commercially available in the U.S. through a specialty pharmacy beginning in July following scheduling by the U.S. Drug Enforcement Administration, which is expected within 90 days of FDA approval
- To support the CDD community, Marinus plans to launch The ZTALMY One™ Program, a comprehensive patient services program to provide assistance with product access, ongoing support to patients, caregivers and their medical teams, and financial support to eligible patients
- Commercial launch leadership is in place; 16 field representatives are expected to be onboarded in April and key discussions have been initiated with both government and commercial payers


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- Topline data targeted first half of 2024

Status Epilepticus

- Expect to resupply clinical trial material and resume recruitment for Phase 3 RAISE trial in refractory status epilepticus (RSE) by end of May
- Continued U.S. site activations in RAISE trial with 52 sites now open
 - Planning to expand trial to include sites in Canada
 - Topline Phase 3 data readout is anticipated in the second half of 2023
- Phase 2 RESET trial in established status epilepticus is planned to begin U.S. enrollment in the second half of 2022
 - Topline data anticipated by year end 2023
- Phase 3 RAISE II trial in RSE (for European registration) now expected to begin enrollment in the first half of 2023

Next Generation Ganaxolone and Lennox-Gastaut Syndrome (LGS)

- Second generation ganaxolone formulation Phase 1 topline data on track for mid-2022 readout
 - If Phase 1 data supports further clinical development, Phase 2 LGS trial expected to begin in the second half of 2022 utilizing second generation formulation
 - Expect to initiate modified release candidate development in 2022
- The company has identified two prodrug candidates that are targeted to advance into initial Investigational New Drug (IND) enabling studies in the second half of 2022

Second generation ganaxolone formulation is being developed to improve pharmacokinetic (PK) characteristics and expand the therapeutic utility, including efficacy, dosing frequency and tolerability.

General Business and Financial Update

- A Rare Pediatric Disease Priority Review Voucher (PRV) was awarded to Marinus by the U.S. Food and Drug Administration upon approval of ZTALMY®. The company intends to monetize the PRV to fund on-going operations, including continued clinical development and commercialization efforts for ganaxolone.
- FDA approval of ZTALMY® makes available through December 31, 2022 an additional \$30 million of funding under the existing Oaktree Capital Management, L.P. credit agreement, subject to the satisfaction of certain customary conditions described in the credit agreement.
- In March 2022, Marinus entered an exclusive license agreement with Ovid Therapeutics to license patents and patent applications in the U.S., the EU and certain other countries in Europe related to the use of ganaxolone for the treatment of CDKL5 deficiency disorder.

Financial Results

- At December 31, 2021, the company had cash, cash equivalents, and short-term investments of \$122.9 million, compared to \$140.0 million at December 31, 2020.
- Recognized \$1.5 million and \$6.4 million in Biomedical Advanced Research and Development Authority (BARDA) federal contract revenue for the three and twelve months ended December 31, 2021, respectively, as compared to \$1.5 million and \$1.7 million for the three and twelve months ended December 31, 2020.
- Research and development (R&D) expenses were \$18 million and \$73.5 million for the three and twelve months ended December 31, 2021, respectively, as compared to \$13 million and \$51.1 million, respectively, for the same periods in the prior year; the increase was due primarily to increased R&D headcount, increased clinical trial activity, including the RSE and TSC trials, and drug development activities for both the oral and IV formulations.
- General and administrative (G&A) expenses were \$10.6 million and \$37.3 million for the three and twelve months ended December 31, 2021, respectively, as compared to \$6 million and \$18.5 million, respectively, for the same periods in the prior year; the primary drivers of the change were increased headcount to support scale up of the company's operations and [separations for commercial launch](#).
- Separately, and as a result of the European collaboration agreement with Orion, we booked full year 2021 collaboration revenue of \$9 million and a one-time cost of collaboration expense of \$1.5 million, both of which were fully recorded in the third quarter of 2021.

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	December 31, 2021	December 31, 2020		
	(unaudited)			
ASSETS				
Cash and cash equivalents	122,927	138,509		
Investments	—	1,474		
Other assets	13,913	10,479		
Total assets	136,840	150,462		
LIABILITIES AND STOCKHOLDERS' EQUITY				
Current liabilities	40,566	10,729		
Long Term Debt, Net	40,809	—		
Other long-term liabilities	1,979	2,534		
Total liabilities	83,354	13,263		
Total stockholders' equity	53,486	137,199		
Total liabilities and stockholders' equity	136,840	150,462		
	Three Months Ended		Twelve Months Ended	
	December 31,		December 31,	
	2021	2020	2021	2020
	(unaudited)		(unaudited)	
Revenue:				
Federal contract revenue	1,520	1,546	6,358	1,718
Collaboration revenue	-	—	8,987	—
Total revenue	1,520	1,546	15,345	1,718
Expenses:				
Research and development	18,014	13,044	73,520	51,106
General and administrative	10,622	6,005	37,278	18,549
Cost of collaboration revenue	—	—	1,478	—
Loss from operations	-27,116	-17,503	-96,931	-67,937
Interest income	23	40	80	499
Interest expense	-1,553	—	-2,582	—
Other income (expense), net	341	-6	657	-37
Net loss and comprehensive loss	-28,305	-17,469	-98,776	-67,475
Deemed dividends on convertible preferred stock	—	—	—	-8,880
Net loss applicable to common shareholders	-28,305	-17,469	-98,776	-76,355
Per share information:				
Net loss per share of common stock—basic and diluted	-0.77	-0.55	-2.69	-2.80
Basic and diluted weighted average shares outstanding	36,746,112	31,832,970	36,663,340	27,270,055

Conference Call Information

Monday, March 21, at 8:00 a.m. ET

Domestic: +1 (888) 550-5280

International: (646) 960-0813

Webcast Link: <https://events.q4inc.com/attendee/991025183>

Conference ID: 2696394

About Marinus Pharmaceuticals

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Marinus is a pharmaceutical company dedicated to the development of innovative therapeutics to treat seizure disorders. Ganaxolone is a neuroactive steroid GABA_A receptor modulator that acts on a well-characterized target in the brain known to have anti-seizure effects. It is being developed in IV and oral dose

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ongoing financial support to eligible patients and caregivers; our expectations regarding BARDA funding; our expectations and beliefs regarding the FDA and EMA with respect to our product candidates; our expectations regarding the development of new formulations and prodrug candidates; our expectations regarding the Orion Corporation collaboration; our expectations regarding our license agreement with Ovid Therapeutics; our expectations to monetize the PRV; our expectations regarding drawing down on the Oaktree credit facility; our expectations regarding the resupply of clinical trial materials for the RAISE trial and the expected timing thereof; our expectation regarding the impact of the COVID-19 pandemic on our business and clinical development plans; our financial projections; and the potential safety and efficacy of ganaxolone, as well as its therapeutic potential in a number of indications; and other statements regarding the company's future operations, financial performance, financial position, prospects, objectives and other future event.

Forward-looking statements in this press release involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, our ability to establish commercial infrastructure and capabilities to launch ZTALMY; physician and patient acceptance of ZTALMY; our ability to obtain adequate market access for ZTALMY; the varying interpretation of clinical data; the scheduling of ZTALMY by the U.S. Drug Enforcement Administration; our ability to comply with the FDA's requirement for additional post-marketing studies in the required time frames; the timing of regulatory filings for our other product candidates; the potential that regulatory authorities, including the FDA and EMA, may not grant or may delay approval for our product candidates; uncertainties and delays relating to the design, enrollment, completion, and results of clinical trials; unanticipated costs and expenses; early clinical trials may not be indicative of the results in later clinical trials; clinical trial results may not support regulatory approval or further development in a specified indication or at all; actions or advice of the FDA or EMA may affect the design, initiation, timing, continuation and/or progress of clinical trials or result in the need for additional clinical trials; our ability to obtain and maintain regulatory approval for our product candidate; our ability to develop new formulations of ganaxolone or prodrugs; our ability to obtain, maintain, protect and defend intellectual property for our product candidates; the potential negative impact of third party patents on our or our collaborators' ability to commercialize ganaxolone; delays, interruptions or failures in the manufacture and supply of our product candidate; the size and growth potential of the markets for the company's product candidates, and the company's ability to service those markets; the company's cash and cash equivalents may not be sufficient to support its operating plan for as long as anticipated; the company's expectations, projections and estimates regarding expenses, future revenue, capital requirements, and the availability of and the need for additional financing; the company's ability to obtain additional funding to support its clinical development and commercial programs; the potential for Orion to breach the collaboration or terminate the agreement in accordance with its terms; the potential for Orion to recoup a percentage of the upfront fee depending on the additional pre-clinical testing; the effect of the COVID-19 pandemic on our business, the medical community, regulators and the global economy; and the availability or potential availability of alternative products or treatments for conditions targeted by us that could affect the availability or commercial potential of our product candidate. This list is not exhaustive and these and other risks are described in our periodic reports, including the annual report on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, filed with or furnished to the Securities and Exchange Commission and available at www.sec.gov. Any forward-looking statements that we make in this press release speak only as of the date of this press release. We assume no obligation to update forward-looking statements whether as a result of new information, future events or otherwise, after the date of this press release.

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