NEWS RELEASE

Marinus Pharmaceuticals Provides Business Update and Reports Third Quarter 2021 Financial Results

11/9/2021

- European Medicines Agency has accepted the Marketing Authorization Application for ganaxolone in CDKL5 deficiency disorder for review under accelerated assessment
- European Commission granted orphan drug designation to ganaxolone in tuberous sclerosis complex; Phase 3 TrustTSC program on track to initiate patient enrollment Q1 2022
- Continued improvements in site activation for Phase 3 RAISE trial in refractory status epilepticus
- Nine abstracts accepted for poster session presentations and virtual analyst and investor event planned for December 6 at American Epilepsy Society 2021 Annual Meeting
- Received additional $30 million of funding under the existing Oaktree Capital Management, L.P. credit agreement

RADNOR, Pa.--(BUSINESS WIRE)-- **Marinus Pharmaceuticals**, Inc. (Nasdaq: MRNS), a pharmaceutical company dedicated to the development of innovative therapeutics to treat rare seizure disorders, today provided an update on its clinical and regulatory development activities and reported its financial results for the third quarter ended September 30, 2021.

“The third quarter provided steady progress across the wide breadth of our clinical programs and commercialization planning efforts. Our goal is to have ganaxolone available commercially, both in the U.S. and Europe, by the middle of next year following regulatory approvals,” said Scott Braunstein, M.D., Chief Executive Officer of Marinus Pharmaceuticals. “We also made significant advancements on the regulatory and patent exclusivity front, continued to build our commercial leadership team and expanded our patient-centric advocacy framework. In addition, we have prioritized second generation ganaxolone with a goal of beginning Phase 1 clinical trials early next year.”

Joseph Hulihan, M.D., Chief Medical Officer of Marinus, added, “Clinical trials in both our oral and IV franchises remain on track. We have advanced ganaxolone across multiple indications where there is high unmet medical need in the U.S. and in Europe. In particular, we are pleased that the European Medicines Agency has accepted our Marketing Authorization Application for review. We plan to have a significant presence at the American Epilepsy Society’s 2021 Annual Meeting where we will further detail the potential of ganaxolone with nine abstracts accepted for presentation.”

Pipeline Update

CDKL5 Deficiency Disorder (CDD)

- Commercialization planning efforts on track
  - Leadership in place and key discussions have been initiated with both government and commercial payers
  - Oral ganaxolone’s proposed U.S. brand name has been established and trademarked, subject to final FDA approval; trade name: ZTALMY® (Zuh-tal-mee)
  - As previously announced, the U.S. Food and Drug Administration (FDA) PDUFA target action date is set for March 20, 2022 and the FDA has indicated that it is not currently planning to hold an advisory committee meeting
  - Global commercial strategy advancing in collaboration with Orion Corporation
- European Medicines Agency (EMA) has accepted the Marketing Authorization Application (MAA) for review under accelerated assessment
  - EMA’s Committee for Medicinal Products for Human Use (CHMP) opinion on the MAA is expected in Q2 2022 and the European Commission (EC) decision is anticipated in early Q3 2022
- Recently **announced** collaboration with the Loulou Foundation and six other biotech and pharmaceutical organizations to undertake a comprehensive observational study in CDD to better understand the natural history and the utility of various clinical assessments
- Committed to identifying opportunities throughout the world to improve the lives of more patients, including growing the CDD Expanded Access Program to Europe

Tuberous Sclerosis Complex (TSC)

- European Commission granted orphan drug designation to ganaxolone in TSC
- Patient enrollment in the Phase 3 TrustTSC trial expected to begin Q1 2022 with topline data expected 1H 2024

Status Epilepticus*

- Continued improvements in site activation for Phase 3 RAISE trial in refractory status epilepticus (RSE)
    - Data continues to be expected in 2H 2022
- Phase 3 RAISE II trial in RSE (for European registration) expected to begin enrollment in 1H 2022
- Phase 2 RESET trial in established status epilepticus (ESE) is planned to begin U.S. enrollment in Q1 2022
- Marinus' U.S. patent application granted for ganaxolone dosing and method of treatment for status epilepticus with a 2040 expiry date
- Additional EEG and safety data from Phase 2 RSE trial was presented during Neurocritical Care Society Annual Meeting in October
    - P25:Intravenous Formulation of Ganaxolone for the Treatment of Refractory Status Epilepticus: Safety Analysis of the Renal Function in a Phase 2 Open-Label, Dose-Ranging Study
    - P274:The Use of Quantitative EEG Spectral Analysis to Characterize IV Ganaxolone PK/PD characteristics in Patients with Refractory Status Epilepticus

Second Generation Formulation and Lennox-Gastaut Syndrome (LGS)

- Five formulations have been selected, out of which two candidates are anticipated to be chosen for clinical and regulatory development
    - First candidate expected in clinic Q1 2022; second candidate expected in clinic by mid-2022
- Phase 2 LGS trial expected to begin 2H 2022 utilizing second generation formulation
- Sustained release formulation development expected to begin in 2022
- Prodrug program continues to advance with candidate selection targeted for mid-2022

*Ganaxolone development for RSE is being funded, in part, by the Biomedical Advanced Research and Development Authority (BARDA), part of the Office of the Assistant Secretary for Preparedness and Response at the U.S. Department of Health and Human Services, under contract number 75A50120C00159

Financial Update

- At September 30, 2021, the company had cash, cash equivalents, and investments of $145.1 million, compared to $140.0 million at December 31, 2020. During the third quarter, cash inflows included an approximately $30 million upfront payment associated with our European collaboration with Orion and an additional $30 million of funding from our credit agreement with Oaktree.
- Marinus recognized $1.1 million and $4.8 million in Biomedical Advanced Research and Development Authority (BARDA) federal contract revenue for the three and nine months ended September 30, 2021, respectively, as compared to $0.2 million for both the three and nine month periods ended September 30,

2020. The BARDA federal contract was entered into in September 2020.

- Research and development (R&D) expenses were $18.4 million and $55.5 million for the three and nine months ended September 30, 2021, respectively, as compared to $11.3 million and $38.1 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 on-going activities associated with the CDD indication.
- General and administrative (G&A) expenses were $9.5 million and $26.7 million for the three and six months ended September 30, 2021, respectively, as compared to $4.6 million and $12.5 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, commercial preparation activities, and contract acquisition costs associated with the Orion collaboration.
- Separately, and as a result of the European collaboration agreement with Orion, collaboration revenue of $9.0 million and a one-time cost of collaboration expense of $1.5 million were both recorded in the third quarter 2021.
- The company reported net losses of $19.5 million and $70.5 million for the three and nine months ended September 30, 2021, respectively; cash used in operating activities decreased to $33.7 million for the nine months ended September 30, 2021, compared to $44.5 million for the same period a year ago.
- Readers are referred to, and encouraged to read in its entirety, the company's Quarterly Report on Form 10-Q for the three months ended September 30, 2021, to be filed with the Securities and Exchange Commission, which includes further detail on the European partnership with Orion and the company's business plans, operations, financial condition, and results of operations.

Corporate Guidance

- For the fiscal year 2021, the company expects BARDA revenues in the range of $6 to $8 million and total GAAP operating expenses, inclusive of G&A and R&D, to be in the range of $111 to $116 million, of which the company expects stock-based compensation to be approximately $14 million. Previous guidance provided for 2021 included BARDA contract revenues in the range of $7 to $10 million and total GAAP operating expenses in the range of $113 to $118 million, including approximately $16 million of stock-based compensation.

	September 30, 2021	December 31, 2020
ASSETS		
Cash and cash equivalents	145,101	138,509
Investments	—	1,474
Other assets	13,772	10,479
Total assets	158,873	150,462
LIABILITIES AND STOCKHOLDERS' EQUITY		

Current liabilities	37,676	10,729
Long Term Debt, Net	40,579	-
Other long-term liabilities	2,124	2,534
Total liabilities	80,379	13,263
Total stockholders' equity	78,494	137,199
Total liabilities and stockholders' equity	158,873	150,462

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Revenue:				
Federal contract revenue	1,127	171	4,838	171
Collaboration revenue	8,987	—	8,987	—
Total revenue	10,114	171	13,825	171
Expenses:				
Research and development	18,353	11,306	55,506	38,062
General and administrative	9,452	4,564	26,656	12,543
Cost of collaboration revenue	1,478	—	1,478	—
Loss from operations	-19,169	-15,699	-69,815	-50,434
Interest income	17	79	57	459
Interest expense	-678	—	-1,029	—
Currency translation and other expense, net	323	-39	316	-31
Net loss and comprehensive loss	-19,507	-15,659	-70,471	-50,006
Deemed dividends on convertible preferred stock	—	—	—	-8,880
Net loss applicable to common shareholders	-19,507	-15,659	-70,471	-58,886
Per share information:				
Net loss per share of common stock—basic and diluted	-0.53	-0.51	-1.92	-2.29
Basic and diluted weighted average shares outstanding	36,744,591	30,552,947	36,667,472	25,737,981

About Ganaxolone

Ganaxolone, a positive allosteric modulator of GABAA receptors, is an investigational product being developed in intravenous and oral formulations intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Ganaxolone exhibits anti-seizure and anti-anxiety activity via its effects on synaptic and extrasynaptic GABAA receptors. Ganaxolone has been studied in more than 1,900 pediatric and adult subjects across various indications at therapeutically relevant dose levels and treatment regimens for up to more than two years.

About Marinus Pharmaceuticals

Marinus Pharmaceuticals, Inc. is a pharmaceutical company dedicated to the development of innovative therapeutics to treat seizure disorders. Ganaxolone is a positive allosteric modulator of GABAA receptors that acts on a well-characterized target in the brain known to have anti-seizure, antidepressant and anti-anxiety effects. Ganaxolone is being developed in IV and oral dose formulations intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Marinus completed the first ever Phase 3 pivotal trial in children with CDKL5 deficiency disorder last year, is planning to conduct a Phase 3 trial in tuberous sclerosis complex, and a Phase 3 trial in refractory status epilepticus is ongoing. For more information visit **www.marinuspharma.com**.

Forward-Looking Statements

To the extent that statements contained in this press release are not descriptions of historical facts regarding Marinus, they are forward-looking statements reflecting the current beliefs and expectations of management made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Words such as "may", "will", "expect", "anticipate", "estimate", "intend", "believe", and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements. Examples of forward-looking statements contained in this press release include, among others, statements regarding our expected clinical development plans, enrollment in our clinical trials, regulatory communications and submissions and product launches for ganaxolone, and the timing thereof; our expectations and beliefs regarding the FDA and EMA with respect to our product candidates; our expectations regarding the Orion Corporation collaboration; 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.

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, the risk that the FDA will require additional clinical trials or data; any delays in review of the NDA submission by the FDA for any reason, including the COVID-19 pandemic; the timing of regulatory filings for our product candidates; the potential that regulatory authorities, including the FDA and EMA, may not grant or may delay approval for our product candidate; 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 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 programs; the company's ability to develop sales and marketing capabilities, whether alone or with potential future collaborators; the rate and degree of market acceptance of the company's product candidates; 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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Sasha Damouni Ellis
Vice President, Corporate Affairs & Investor Relations
Marinus Pharmaceuticals, Inc.
484-253-6792
sdamouni@marinuspharma.com

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