NEWS RELEASE

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

8/10/2021

- Anticipate initiating a Phase 3 trial of adjunctive ganaxolone in tuberous sclerosis complex (TSC) in Q3 2021, targeting first patient enrolled during Q4 2021; continue to expect topline Phase 2, open label data in Q3 2021
- European Medicines Agency (EMA) is expected to accept accelerated assessment of ganaxolone for the treatment of seizures associated with CDKL5 deficiency disorder (CDD); Marinus plans to submit a marketing authorization application to the EMA by the end of Q3 2021
- Received notice of allowance from the U.S. Patent and Trademark Office for refractory status epilepticus (RSE) dosing and method of treatment patent application
- U.S. Food and Drug Administration (FDA) indicated alignment on overall trial design for the established status epilepticus (ESE) trial; company expects to begin U.S. enrollment in 1H 2022
- Submitted New Drug Application to FDA for ganaxolone for the treatment of seizures associated with CDD
- Entered exclusive collaboration with Orion Corporation for European commercialization of ganaxolone
- Site initiations for Phase 3 RSE trial were affected by COVID-19-related delays in Q2 2021; expect vast majority of sites to be open by end of Q3 2021, with topline data anticipated for release 2H 2022

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

“We have seen tremendous progress over the past few months, including the recent announcements of our European collaboration with Orion Corporation and the submission of our NDA for ganaxolone in CDKL5 deficiency disorder,” said Scott Braunstein, M.D., Chief Executive Officer of Marinus Pharmaceuticals. “Orion’s leadership in the

European rare orphan disease market and its ability to commercialize both oral and IV ganaxolone makes them a strong partner."

Dr. Braunstein added, "We are also pleased to report that the FDA indicated they are in overall alignment with the design of our TSC Phase 3 clinical trial, which we expect to initiate in the fourth quarter of the year. We anticipate the remainder of 2021 to be equally eventful."

Pipeline Update

CDKL5 Deficiency Disorder (CDD)

- Submitted New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for ganaxolone for the treatment of seizures associated with CDD in July 2021; expect to receive a filing notification letter from the FDA prior to the end of Q3 2021
    - The NDA includes a request for Priority Review, which, if granted, accelerates completion of the FDA review goal to six months following the 60-day filing review, compared to a standard review of 10 months
    - The NDA also includes a request for a rare pediatric disease (RPD) priority review voucher. The FDA granted RPD designation for ganaxolone for the treatment of CDD in July 2020
    - NDA acceptance for filing enables the opportunity to draw $30 million under the existing Oaktree Capital Management, L.P. facility, subject to the satisfaction of certain customary conditions described in the credit agreement
- European Medicines Agency (EMA) is expected to accept accelerated assessment of ganaxolone for the treatment of seizures associated with CDD; if granted, accelerated assessment would reduce target review time to 150 from 210 days
- Marinus expects to file a marketing authorization application with the EMA for ganaxolone for the treatment of seizures associated with CDD by the end of Q3 2021
- Marinus, with the support of its strategic collaborator, Orion Corporation, expects to announce a European expanded access program

Tuberous Sclerosis Complex (TSC)

- Top-line, open label, Phase 2 trial (CALM Study) data expected to be reported in Q3 2021
    - Trial evaluates the effectiveness, safety and tolerability of adjunctive ganaxolone treatment in patients with TSC
- Should ganaxolone be approved in CDD, the FDA has indicated a single pivotal TSC Phase 3 trial could be

sufficient for the approval
- A scientific advice meeting with the EMA is targeted for Q1 of 2022
- A global Phase 3 randomized, double blind, placebo-controlled trial in approximately 160 patients is expected to begin enrollment during Q4 2021

Status Epilepticus

- FDA indicated alignment on overall trial design for the established status epilepticus (ESE) RESET (Researching Established Status Epilepticus Treatment) trial
    - The Phase 2 RESET trial of adjunctive ganaxolone in ESE is planned to begin U.S. enrollment in 1H 2022
    - The RESET trial will examine a shorter dosing paradigm in conjunction with a first line antiepileptic drug (AED) and will target convulsive status epilepticus
- As previously announced, due to COVID-19 priorities in several participating Phase 3 academic centers, including staff turnover and the need for clinical sites to devote significant resources to patients with COVID-19, the Phase 3 RAISE (RAndomized therapy In Status Epilepticus) trial has experienced site initiation and enrollment delays. Given these most recent challenges, Marinus has shifted its top-line data readout to 2H 2022
    - Company expects the vast majority of sites to be open by the end of Q3 2021
- The RAISE trial is a randomized, double-blind, placebo-controlled trial that will evaluate the efficacy and safety of intravenous (IV) ganaxolone in patients with refractory status epilepticus (RSE) who have failed benzodiazepines and two or more second line IV AEDs
- Planning continues for the RAISE II trial to support European registration of IV ganaxolone in RSE and is on track to initiate enrollment in the first half of 2022
    - Overall alignment on the clinical development plan was achieved at an end-of-Phase 2 scientific advice meeting held in March 2021 with the EMA
    - The RAISE II trial differs from the RAISE trial in the U.S., with RAISE II using ganaxolone that can be initiated earlier in the course of RSE. If successful, this trial will provide complementary data to the Phase 3 RAISE trial in the U.S.
- Company continues to field requests from investigators for IV ganaxolone for patients with super refractory status epilepticus under emergency Investigational New Drug Applications

Corporate Update

- Received notice of allowance from the U.S. Patent and Trademark Office for refractory status epilepticus (RSE) dosing and method of treatment patent application
- Signed exclusive collaboration with Orion Corporation for European commercialization of ganaxolone in CDD,

TSC and RSE,generating €25 million (~$30 million) in cash as an upfront payment and up to €97 million (~$115 million) in R&D reimbursement, development and commercialization milestone payments, as well as tiered double-digit royalties on net sales in Europe

- Organization further strengthened through key hires in patient advocacy, marketing, R&D, finance and commercial operations
- Appointment of Santiago Arroyo, M.D., Ph.D. to the Board of Directors
    - Dr. Arroyo brings a distinguished career in academic neurology and clinical research and development, including his leadership in the neurology department at the Johns Hopkins Hospital. In addition, he has extensive expertise in epilepsy drug development contributing to numerous new therapeutic alternatives. Most recently, he served as Chief Medical Officer of Momenta Pharmaceuticals
- Second generation formulation of ganaxolone to be discussed during the Marinus October 5, 2021, R&D Day

Financial Update

- At June 30, 2021, the company had cash, cash equivalents, and investments of $112.5 million, compared to $140.0 million at December 31, 2020. This excludes the ~$30 million upfront payment associated with the recently completed European collaboration with Orion
- Marinus recognized $1.9 million and $3.7 million in Biomedical Advanced Research and Development Authority (BARDA) federal contract revenue for the three and six months ended June 30, 2021, respectively; no federal contract revenue was recognized in the six months ended June 30, 2020
- Research and development expenses were $18.6 million and $37.2 million for the three and six months ended June 30, 2021, respectively, as compared to $11.8 million and $26.8 million, respectively, for the same periods in the prior year; the increase was due primarily to start-up of the RSE Phase 3 trial, regulatory activities associated with the CDD NDA submission, and increased R&D headcount
- General and administrative expenses were $6.8 million and $17.2 million for the three and six months ended June 30, 2021, respectively, as compared to $4.1 million and $8.0 million, respectively, for the same periods in the prior year; the primary drivers of the increase were additional support for scale up of the company's operations as well as preparation for commercialization
- The company reported net losses of $23.8 million and $51.0 million for the three and six months ended June 30, 2021, respectively; cash used in operating activities increased to $39.1 million for the six months ended June 30, 2021, compared to $30.0 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 June 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 total GAAP operating expenses (selling, general & administrative expenses and R&D expenses) to be in the range of $113 to $118 million, of which the company expects stock-based compensation to be approximately $16 million. 2021 BARDA contract revenues are projected to be in the range of $7 to $10 million for the full year. Previously expected BARDA contract revenues for 2021 were in the range of $9 to $12 million. Total GAAP operating expenses and stock-based compensation estimates remain unchanged from the prior guidance

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Marinus Pharmaceuticals, Inc.
Selected Financial Data (in thousands, except share and per share amounts)

	June 30, 2021	December 31, 2020
ASSETS		
Cash and cash equivalents	$112,493	$138,509
Investments	—	1,474
Other assets	12,496	10,479
Total assets	$124,989	$150,462
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities	$16,694	$10,729
Long Term Debt, Net	$10,970	$0
Other long-term liabilities	2,266	2,534
Total liabilities	29,930	13,263
Total stockholders' equity	95,059	137,199
Total liabilities and stockholders' equity	$124,989	$150,462

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Federal contract revenue	$ 1,905	$ —	$ 3,711	$ —
Expenses:				
Research and development	$ 18,562	$ 11,752	$ 37,153	$ 26,756
General and administrative	6,828	4,130	17,204	7,980
Loss from operations	(23,485)	(15,882)	(50,646)	(34,736)
Interest income	16	212	40	398
Interest expense	(351)	—	(351)	—
Other expense, net	(3)	(5)	(7)	(9)
Net loss and comprehensive loss	$ (23,823)	$ (15,675)	$ (50,964)	$ (34,347)
Deemed dividends on convertible preferred stock	—	—	—	(8,880)
Net loss applicable to common shareholders	$ (23,823)	$ (15,675)	$ (50,964)	$ (43,227)
Per share information:				
Net loss per share of common stock—basic and diluted	$ (0.65)	$ (0.63)	$ (1.39)	$ (1.86)
Basic and diluted weighted average shares outstanding	36,659,615	24,942,624	36,629,823	23,295,199

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 1,800 pediatric and adult subjects across various indications at therapeutically relevant dose levels and treatment regimens for up to more than two years.

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.

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 and is conducting a Phase 2 trial in tuberous sclerosis complex and a Phase 3 trial in refractory status epilepticus. 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 collaboration, including the expected benefits of such collaboration and receipt of an upfront fee and potential R&D reimbursement and milestone and royalty payments thereunder; our expectation regarding the impact of the COVID-19 pandemic on our business and clinical development plans; our expectations regarding our reformulation efforts; our expectations regarding the announcement of a European expanded access program; our expectations regarding the Oaktree credit facility; 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 timing of FDA's review of the NDA will be delayed or that the NDA is not accepted for filing by the FDA; the risk that the FDA will require additional clinical trials or data; any delays in acceptance and review of the NDA submission by the FDA for any reason, including the COVID-19 pandemic; the timing of regulatory filings, including the timing of filing the ganaxolone MAA with the EMA; 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 expected to be completed in Q1 2022; 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 the company's 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 the company makes in this press release speak only as of the date of this press release. The company assumes 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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