NEWS RELEASE

# Marinus Pharmaceuticals Provides Business Update and Reports First Quarter 2020 Financial Results

5/4/2020

Constructive End of Phase 2 Meeting with FDA for Status Epilepticus Completed in March; Pivotal Phase 3 Clinical Trial Expected to Begin Next Quarter

Topline Data on Track for Q3 2020 from Pivotal Phase 3 Marigold Study in CDKL5 Deficiency Disorder and Preparations Continue for Potential NDA Filing

First Patients to be Screened this Quarter for Phase 2 Tuberous Sclerosis Complex Clinical Trial

RADNOR, Pa., May 04, 2020 (GLOBE NEWSWIRE) -- **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 development activities and reported its financial results for the first quarter ended March 31, 2020.

“We are proud to have made real progress across all of our clinical programs prior to and during the unprecedented and challenging global impact of the COVID-19 pandemic,” said Scott Braunstein, M.D., Chief Executive Officer of Marinus. “We remain confident that our key milestones are still on track, including the topline data from our pivotal Phase 3 clinical trial in CDKL5 deficiency disorder in Q3 2020. With positive topline data, we intend to continue preparations for both our first NDA submission and the commercial launch of ganaxolone.”

Dr. Braunstein added, “We had a highly constructive end-of-Phase 2 meeting with the FDA for our status epilepticus program and anticipate enrolling the first patient in the Phase 3 trial later this year. In addition, we have made significant headway in expanding our biomarker-informed trial for tuberous sclerosis complex, as our first site has

been activated for patient enrollment. This will be a year of noteworthy milestones for the organization, and we are confident that we are just beginning to unlock ganaxolone's potential to improve the lives of patients and families affected by rare and severe epilepsies."

Pipeline Update:

Status Epilepticus (SE)

- Marinus is moving forward with plans for its Phase 3 pivotal clinical trial in SE after a constructive end-of-Phase 2 meeting with the FDA. Based upon feedback from the FDA, the Company anticipates the following trial design and timeline:
    - Co-primary endpoints that focus on status cessation within 30-minutes and suppression of status for at least 24 hours.
    - Ganaxolone administered intravenously for 48 hours, the first 12 hours of which is expected to target a 500 ng/ml serum concentration.
    - Enrollment of approximately 125 patients; providing greater than 90 percent power to detect a 30 percent efficacy difference between ganaxolone and placebo.
    - Patients enrolled will have previously failed a benzodiazepine and at least two second-line antiepileptic drugs.
    - Patient enrollment to begin in Q3 2020 and trial sites have already identified and are in process of being readying.
    - Topline data expected in the first half of 2022.

CDKL5 Deficiency Disorder (CDD)

- Enrollment has been completed in the pivotal Phase 3 Marigold Study, which is evaluating the use of oral ganaxolone in children and young adults with CDD. The global, double-blind, placebo-controlled, clinical trial has enrolled 101 patients between the ages of 2 and 21 with a confirmed disease-related CDKL5 gene variant.
- Patients randomized to ganaxolone receive up to 600 mg (oral liquid suspension) three times a day.
- The primary endpoint of the trial is percent change in 28-day seizure frequency. The discontinuation rate in this trial is in-line with expectations (less than 10 percent), and enrollment in the open label extension part of the trial continues to be high.
- Marinus remains on-track to report top-line data from the trial in Q3 2020 with no expected material delays due to COVID-19. The Company has begun preparations for a potential NDA filing and the development of its commercial strategy.
- Phase 1 supportive clinical trials have experienced delays in enrollment due to COVID-19, which, as of today, are not expected to impact timing for a potential NDA filing.

Tuberous Sclerosis Complex (TSC)

- Marinus is conducting a Phase 2 open-label trial to evaluate the safety and tolerability of adjunctive ganaxolone treatment in patients with TSC. The trial is expected to enroll approximately 30 patients ages 2 to 65.
- Patients will undergo a four-week baseline period followed by a 12-week treatment period where they will receive up to 600 mg of ganaxolone (oral liquid suspension) three times a day. Patients who meet eligibility criteria may continue ganaxolone treatment in a 24-week extension to the trial.
- The primary endpoint for the trial is percent change in 28-day primary seizure frequency for the treatment period relative to baseline. The Company plans to analyze allopregnanolone sulfate levels as part of the trial efficacy analysis.
- Marinus intends to start screening patients this quarter. As of today, the Company does not anticipate COVID-19 will have a material impact on enrollment or trial progress and expects to report top line data in Q1 2021.

PCDH19 Related Epilepsy (PCDH19-RE)

- After careful consideration, Marinus will transition the ongoing Phase 3 Violet Study to a proof-of-concept (POC) trial evaluating allopregnanolone sulfate as a biomarker in the patients currently enrolled with a confirmed PCDH19 mutation. Marinus has decided to limit trial enrollment due to several factors, including the significant requirements needed to enroll a global trial (including COVID-19 impact), the episodic nature of seizures in PCDH19 patients, and potential commercial challenges. The Company now expects to complete the double-blind portion of the trial with approximately 15-20 patients and announce results of this POC trial in the first half of 2021.
- The scale down to a POC trial is part of the Company's strategic plan to focus capital resources on indications with high unmet needs and a meaningful percentage of patients being underserved by available treatment options.
- Marinus plans to continue to evaluate if the allopregnanolone sulfate biomarker hypothesis could have broader utility in other targeted indications.
- The POC biomarker trial will stratify patients into one of two biomarker groups based on baseline allopregnanolone sulfate levels and randomized (ganaxolone or placebo) within each stratum. The trial will consist of a 12-week prospective baseline period to collect seizure data, followed by a 17-week double-blind treatment phase. Patients randomized to ganaxolone will titrate over four weeks to a dose of up to 600 mg of ganaxolone (oral liquid suspension) three times a day and maintain that dose for the following 13-weeks.
- Marinus intends to provide access to ganaxolone for PCDH19-RE patients who saw benefits in the Violet Study.

Corporate Update:

- In April, the Company announced the formation of Scientific Advisory Board with six globally recognized seizure disorder experts.
- In March, Sasha Damouni Ellis was appointed Vice President, Investor Relations and Corporate Communications.

Financial Update:

On March 31, 2020, the Company had cash and cash equivalents and investments totaling $77.8 million compared to $91.7 million on December 31, 2019. Marinus believes that its cash, cash equivalents and investments as of March 31, 2020 will enable the Company to fund operating expenses and capital expenditure requirements into the third quarter of 2021.

Research and development expenses increased to $15.0 million for the three months ended March 31, 2020, as compared to $8.9 million for the three months ended March 31, 2019. The primary drivers for the increase to our research and development expenditures are clinical and manufacturing activities in support of our Phase 3 trial in CDD and preparations for a Phase 3 trial in SE, partially offset by decreased costs for non-seizure disorder indications.

General and administrative expenses were $3.9 million for the three months ended March 31, 2020 as compared to $3.7 million in the prior year. The primary drivers of the increase were professional fees and other costs associated with an increased scale of operations.

The Company reported a net loss of $18.7 million for the three months ended March 31, 2020, compared to $12.5 million for the three months ended March 31, 2019. Cash used in operating activities increased to $14.0 million for the three months ended March 31, 2020 compared to $11.7 million for the three months ended March 31, 2019.

Readers are referred to, and encouraged to read in its entirety, the Company's Quarterly Report on Form 10-Q for the quarter ended March 31, 2020 to be filed with the Securities and Exchange Commission, which includes further detail on the Company's business plans, operations, financial condition and results of operations.

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

| | March 31, 2020 | December 31, 2019 |
|---|---|---|
| Assets: | | |
| Cash and cash equivalents | $70,326 | $90,943 |
| Investments | 7,460 | 739 |
| Other assets | 6,772 | 7,160 |
| Total assets | $84,558 | $98,842 |
| Liabilities and stockholders' equity: | | |
| Accounts payable and accrued expenses | $10,325 | $8,031 |
| Other liabilities | 2,921 | 3,042 |
| Total liabilities | 13,245 | 11,073 |
| Series A preferred stock | 28,200 | 28,200 |
| Total stockholders' equity | 43,113 | 59,569 |
| Total liabilities and stockholders' equity | $84,558 | $98,842 |

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| | Three Months Ended March 31, 2020 | 2019 |
|---|---|---|
| Expenses: | | |
| Research and development | $15,004 | $8,872 |
| General and administrative | 3,850 | 3,667 |
| Loss from operations | (18,854) | (12,539) |
| Interest income | 222 | 96 |
| Other expense | (40) | (40) |
| Net loss | (18,672) | (12,483) |
| Deemed dividends on convertible preferred stock | (8,880) | — |
| Net loss applicable to common shareholders | $(27,552) | $(12,483) |
| Per share information: | | |
| Net loss per share of common stock—basic and diluted | $(0.32) | $(0.24) |
| Basic and diluted weighted average shares outstanding | 86,661,845 | 52,465,207 |

## About Marinus Pharmaceuticals

Marinus Pharmaceuticals, Inc. is a pharmaceutical company dedicated to the development of innovative therapeutics to treat rare 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, anti-depressant, and anti-anxiety effects. Ganaxolone is being developed in IV and oral dose forms intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Marinus is conducting the first ever Phase 3 pivotal trial in children with CDKL5 deficiency disorder, along with a Phase 2 trial in Tuberous Sclerosis Complex, and a Phase 2 biomarker driven proof of concept trial in PCDH19-related epilepsy. The Company intends to initiate a Phase 3 trial in 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 clinical development plans for ganaxolone, expected dosing in our clinical trials, the clinical development schedule and milestones, our expected timing to begin and complete enrollment in our clinical trials, the expected protocols for our clinical trials, interpretation of scientific basis for ganaxolone use, timing for availability and release of data, the potential safety and efficacy of ganaxolone, the therapeutic potential of ganaxolone, our expectations regarding the effect of the COVID-19 pandemic on our business and clinical development plans, and our expectations regarding the sufficiency of our working capital. Forward-looking statements in this 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, uncertainties and delays relating to the design, enrollment, completion, and results of clinical trials; unanticipated costs and expenses; clinical trial results may not support further development in a specified indication or at all; actions or advice of the U.S. Food and Drug Administration 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; delays, interruptions or failures in the manufacture and supply of our product candidate; our ability to raise additional capital; the effect of the COVID-19 pandemic on our business, the medical community 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. Marinus undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of the Company in general, see filings Marinus has made with the Securities and Exchange Commission.

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