NEWS RELEASE

Marinus Pharmaceuticals Provides Business Reports Update and 2019 Financial Results

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RADNOR, Pa., March 16, 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 a business update on its clinical development activities and reported its financial results for the year ended December 31, 2019.

Key 2020 Planned Milestones:

- Initiate a pivotal Phase 3 clinical trial in patients with status epilepticus (SE) – mid-2020
- Initiate a Phase 2 clinical trial in patients with tuberous sclerosis complex (TSC) – Q2 2020
- Report top-line data from a global, pivotal Phase 3 clinical trial in children with CDKL5 deficiency disorder (CDD) – Q3 2020

“We have started 2020 in a strong position, with recent accomplishments in the clinic that support our strategy to develop ganaxolone in mechanistically relevant disease states where we have the potential to significantly improve patient outcomes,” said Scott Braunstein, M.D., Chief Executive Officer of Marinus. “We have recently announced enrollment completion for our Phase 3 trial in CDD and remain on track for topline data later this year. Preparations are underway for our first potential NDA filing and commercial launch with oral ganaxolone. Our team continues to build momentum in the status epilepticus program and we are on track for the initiation later this year of our pivotal Phase 3 trial. Taken together, we believe we have developed thoughtful, data-driven clinical programs designed to provide new treatments to patients suffering from severe and rare seizure disorders.”

Clinical Development Overview and Highlights

Marinus is developing oral and intravenous (IV) ganaxolone formulations to treat adults and children suffering from acute and chronic rare seizure disorders where there is a mechanistic rationale for ganaxolone to provide a therapeutic benefit to patients with unmet or underserved medical needs.

Status Epilepticus (SE)

- Marinus is making preparations for an End of Phase 2 meeting with the U.S. FDA in Q1 2020, with the goal of commencing a Phase 3 registration study by the middle of 2020.
- In September 2019, the Company announced positive top-line results in its open-label, dose-finding Phase 2 trial evaluating IV ganaxolone in patients with refractory SE. Ganaxolone met the primary endpoint in the trial with 100% of patients (n=17) not progressing to IV anesthetics within 24 hours of treatment initiation. Additionally, in the target dose group (713 mg/day), no patients (n=8) progressed to additional IV anti-epileptic drugs (AEDs) or IV anesthetics for status relapse at any time through the end of the 24-hour post treatment follow-up period. Ganaxolone had an acceptable safety and tolerability profile for the refractory SE patient population in all dose groups.
- Additional long-term data presented at the American Epilepsy Society (AES) Annual Meeting demonstrated that all patients in the target dose cohort did not experience status relapse during the four-week follow up period (n=6). An independent retrospective central review of seizure EEG data using objective criteria demonstrated a clear dose effect with the target dose level providing sustained reductions in seizure burden (greater than 80%) throughout the entire analysis window.

CDKL5 Deficiency Disorder (CDD)

- Enrollment has been completed in the pivotal Phase 3 clinical trial (Marigold Study) evaluating the use of oral ganaxolone in children and young adults with CDD. The Marigold Study is a global, double-blind, placebo-controlled, clinical trial that has enrolled approximately 100 patients between the ages of 2 and 21 with a confirmed disease-related CDKL5 gene variant and allopregnanolone sulfate levels below a pre-specified limit.
- The trial's primary efficacy endpoint is percent change in 28-day seizure frequency. Secondary outcome measures will include non-seizure-related endpoints to capture certain changes in behavior and sleep.
- The Company plans to announce top-line data from the trial in Q3 2020. If the Phase 3 Marigold Study is successful, this could be the first approved indication for ganaxolone and the first approved treatment for CDD.

PCDH19-related Epilepsy (PCDH19-RE)

- Enrollment is ongoing in a pivotal Phase 3 clinical trial (Violet Study) evaluating the use of oral ganaxolone in children and young adults with PCDH19-RE. The Violet Study will enroll up to 70 patients between the ages of

1 and 17 with a confirmed PCDH19 mutation. Patients enrolled in the trial are stratified into one of two biomarker groups based on baseline allopregnanolone sulfate levels and randomized (ganaxolone or placebo) within each stratum. The trial consists of a 12-week prospective baseline period to collect seizure data, followed by a 17-week double-blind treatment phase. Patients randomized to ganaxolone are titrated over four weeks to a dose of up to 600 mg of oral liquid suspension three times a day and maintain that dose for the following 13-weeks. After the double-blind period, all patients who meet certain eligibility criteria will have the opportunity to receive ganaxolone in an open label phase of the trial.

- The Company plans to announce top-line data from the Violet Study in 2H 2021.

Tuberous Sclerosis Complex (TSC)

- Marinus is planning to initiate a Phase 2 open-label trial to evaluate the safety and tolerability of adjunctive ganaxolone treatment in patients with TSC in 2Q 2020.
- The trial will be conducted at approximately 6 sites in the U.S. and enroll approximately 30 patients ages 2 to 65. Patients will undergo a four-week baseline period followed by a 12-week treatment period. All patients who meet certain eligibility criteria will have the opportunity to receive ganaxolone in a 24-week extension of the trial. The primary endpoint for the trial is percent change in 28-day primary seizure frequency through the end of the 12-week treatment period relative to the 4-week baseline period.
- Efficacy results will be evaluated for correlation to baseline endogenous allopregnanolone sulfate levels.

Financial Update

At December 31, 2019, we had cash and cash equivalents of $90.9 million compared to $67.7 million at December 31, 2018. We believe that our cash, cash equivalents and investments as of December 31, 2019 will enable us to fund our operating expenses and capital expenditure requirements into the third quarter of 2021.

Research and development expenses increased to $43.0 million for the year ended December 31, 2019, as compared to $28.4 million in the year ended December 31, 2018. The primary drivers for the increase to our research and development expenditures were clinical and manufacturing activities in support of our Phase 3 trials in CDD and PCDH19-RE, partially offset by decreased costs for non-seizure disorder indications.

General and administrative expenses were $11.5 million for the year ended December 31, 2019 as compared to $8.8 million in the prior year. The primary drivers of this increase were $1.3 million in severance expenses related to the departure of former executive officers ($0.4 million of which was non-cash equity compensation expense), and approximately $1.2 million in professional fees and other costs associated with an increased scale of operations.

The Company reported a net loss of $54.1 million for the year ended December 31, 2019, compared to $36.7 million

for the year ended December 31, 2018. Cash used in operating activities increased to $48.6 million for the year ended December 31, 2019 compared to $27.8 million for the year ended December 31, 2018.

Readers are referred to, and encouraged to read in its entirety, the Company's Annual Report on Form 10-K for the quarter ended December 31, 2019 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)

	December 31, 2019	2018
Assets:		
Cash and cash equivalents	$ 90,943	$ 67,727
Investments	739	4,998
Other assets	7,160	2,509
Total assets	$ 98,842	$ 75,234
Liabilities and stockholders' equity:		
Accounts payable	$ 2,763	$ 2,472
Accrued expenses	5,268	4,437
Other liabilities	3,042	—
Total liabilities	11,073	6,909
Series A convertible preferred stock	28,200	—
Total stockholders' equity	59,569	68,325
Total liabilities and stockholders' equity	$ 98,842	$ 75,234

	Year Ended December 31, 2019	2018
Operations:		
Research and development	$ 42,966	$ 28,394
General and administrative	11,456	8,785
Loss from operations	(54,422)	(37,179)
Interest income	354	454
Other expense	(53)	(1)
Net loss	$ (54,121)	$ (36,726)
Per share information:		
Net loss per share of common stock—basic and diluted	$ (0.99)	$ (0.90)
Basic and diluted weighted average shares outstanding	54,512,778	40,895,406

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 pivotal trials in children with CDKL5 deficiency disorder and PCDH19-related epilepsy. Later this year, the company intends to initiate a Phase 3 trial in SE and a Phase 2 trial in Tuberous Sclerosis Complex (TSC).For more information visit **www.marinuspharma.com**. Please follow us on Twitter: @MarinusPharma.

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 interpretation of preclinical studies, development plans for our product candidate, including the development of dose forms, the clinical development schedule and milestones, the ability to complete enrollment in our clinical studies, interpretation of scientific basis for ganaxolone use, timing for availability and release of data, the safety, potential efficacy and therapeutic potential of our product candidate and our expectation 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, the uncertainties inherent in the conduct of clinical trials, the timing of clinical trials, enrollment in clinical trials, availability of data from clinical trials, expectations for regulatory approvals, the attainment of clinical trial results that will be supportive of regulatory approvals, and other matters, including the development of formulations of ganaxolone, unanticipated costs and expenses, our ability to raise additional capital and the availability or potential availability of alternative products or treatments for conditions targeted by the Company that could affect the availability or commercial potential of our drug candidates. 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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