



Marinus Pharmaceuticals Provides Business Update and 2017 Financial Results

March 6, 2018

RADNOR, Pa., March 06, 2018 (GLOBE NEWSWIRE) -- [Marinus Pharmaceuticals, Inc.](#) (Nasdaq:MRNS), a biopharmaceutical company dedicated to the development of innovative therapeutics to treat epilepsy and neuropsychiatric disorders, today provided a business update on its clinical development activities and reported its financial results for the year ended December 31, 2017.

Near-term Clinical Value Catalysts

- Initiate Phase 3 pivotal study with oral ganaxolone in children with CDD (CDKL5 Deficiency Disorder) mid-2018
- Report top-line intravenous (IV) ganaxolone data from Phase 2 Magnolia study in women with severe postpartum depression (PPD) third quarter 2018.
- Report top-line oral ganaxolone data from Amaryllis study in women with moderate PPD fourth quarter 2018.

"2017 was a significant year for Marinus, and one that has positioned us for important data readouts and advancing ganaxolone into late stage development in 2018," said Christopher M. Cashman, chief executive officer of Marinus Pharmaceuticals. "The data obtained last September from our study in children with CDKL5 deficiency disorder were impressive and drove our decision to advance this program into a global, pivotal study which we will initiate this year. We, along with the CDKL5 community of caregivers, physicians and investigators, are excited to participate in this first ever late-stage clinical trial for these children suffering from this rare and debilitating epilepsy with no approved treatments or even any reasonable standard of care. Similarly, our two phase 2 studies in women suffering from severe and moderate PPD are expected to generate data with IV and oral regimens of ganaxolone this year to support the design and initiation of pivotal Phase 3 studies next year. We remain focused on our goal of being able to treat underserved patient populations with patient-convenient, setting appropriate, effective and safe treatment regimens."

CDKL5 Deficiency Disorder (CDD)

- In November, the Company presented the successful results from its Phase 2 study evaluating the safety and efficacy of ganaxolone in children with CDD at the annual CDKL5 Forum in Boston. Marinus was recognized as the leader in clinical research in CDD. The data presented showed that ganaxolone provided substantial and durable anti-seizure efficacy in children with CDD and was generally safe and well-tolerated with no serious adverse events.
- The last patient enrolled into the Phase 2 study, recently completed the six-month treatment period, and based on good seizure control, entered the one-year extension to the study. The majority of CDD patients from the Phase 2 study entered the one-year study extension and continue to receive ganaxolone.
- The Company is engaging in successful, collaborative discussions with regulatory agencies and expects to initiate a global, pivotal study in mid-2018.

Postpartum Depression (PPD)

- Marinus is enrolling patients into the Magnolia Study, a Phase 2 double-blind, placebo-controlled, dose-optimization clinical trial to evaluate the safety, efficacy and pharmacokinetics of ganaxolone in women diagnosed with severe PPD (Hamilton Depression Rating Scale (HAM-D17) score ≥ 26). Patients randomized into the first part of the study will undergo an infusion of either ganaxolone or placebo and will be followed for 30 days. The goal of the first part of the study is to evaluate multiple regimens of intravenous (IV) ganaxolone, which will inform dosing for the second part of the study. Patients enrolled into the second part of the study could receive IV ganaxolone of various infusion lengths followed by administration of oral ganaxolone. The goal of the second part of the study is to identify an optimized dose or doses for further testing in phase 3. Based upon the effect size shown in a recent study for a compound with similar mechanism of action, the Company has increased targeted enrollment in this study. This increase in study scope and the corresponding forecast for patient recruitment have extended our expected timing for completion of the first part to the third quarter of 2018.
- The Company is enrolling patients into its Amaryllis Study, a Phase 2 double-blind, placebo-controlled clinical trial to evaluate the safety, tolerability and efficacy of oral ganaxolone in women with moderate PPD (HAM-D17 score > 20 and < 26). The study is designed to enroll approximately 50 women at 20 sites within the US. Data from this study are expected fourth quarter of 2018.

Status Epilepticus (SE)

- The Company has initiated its Phase 2 study with ganaxolone IV in patients with refractory status epilepticus (RSE). Data from this proof-of-concept study are expected fourth quarter of 2018.

Financial Update

At December 31, 2017, the Company had cash, cash equivalents and investments of \$58.4 million, compared to \$30.1 million at December 31, 2016. We believe that our cash, cash equivalents and investments as of December 31, 2017 will enable us to fund our current scale of operating expenses and capital expenditure

requirements into 2020.

Research and development expenses decreased to \$12.4 million for the year ended December 31, 2017, as compared to \$22.0 million in the prior year. The decrease was primarily due to a decrease of \$11.0 million associated with our drug-resistant focal onset seizures program, which we discontinued in June 2016. Additionally, we sold \$0.4 million in state research and development tax credits which we used to offset research and development expenses. The decrease was partially offset by an increase of \$2.3 million associated with our IV programs in PPD, for which a Phase 2 clinical trial was initiated in June 2017.

General and administrative expenses increased \$0.4 million, to \$6.7 million, for the year ended December 31, 2017, compared to 2016. The increase in general and administrative expenses was primarily due to an increase in noncash stock-based compensation expense.

The Company reported net losses of \$18.9 million and \$28.6 million for the years ended December 31, 2017 and 2016, respectively. Cash used in operating activities was \$18.8 million for the year ended December 31, 2017 compared to \$24.8 million for the same period a year ago.

Readers are referred to, and encouraged to read in its entirety the Company's Annual Report on Form 10-K for the year ended December 31, 2017 to be filed with the Securities and Exchange Commission, which includes further detail on the above-referenced transactions and the Company's business plans and operations, financial condition and results of operations.

Marinus Pharmaceuticals, Inc.

Selected Financial Data (in thousands, except share and per share amounts) (unaudited)

	December 31, 2017	December 31, 2016
ASSETS		
Cash and cash equivalents	\$33,531	\$26,178
Investments	24,825	3,922
Other assets	2,316	1,347
Total assets	\$60,672	\$31,447
LIABILITIES AND STOCKHOLDERS' EQUITY		
Total current liabilities	\$2,544	\$8,084
Notes payable, long-term portion	—	1,743
Other long term liabilities	120	141
Total liabilities	2,664	9,968
Total stockholders' equity	58,008	21,479
Total liabilities and stockholders' equity	\$60,672	\$31,447

	Year Ended December 31, 2017	2016
Expenses:		
Research and development	\$ 12,376	\$ 22,005
General and administrative	6,667	6,237
Loss from operations	(19,043)	(28,242)
Interest income	324	128
Interest expense	(159)	(464)
Other expense	(20)	(65)
Net loss	\$ (18,898)	\$ (28,643)
Per share information:		
Net loss per share of common stock—basic and diluted	\$ (0.80)	\$ (1.47)
Basic and diluted weighted average shares outstanding	23,540,738	19,498,143

About Marinus Pharmaceuticals

Marinus Pharmaceuticals, Inc. is a biopharmaceutical company dedicated to the development of ganaxolone, which offers a new mechanism of action, demonstrated efficacy and safety, and convenient dosing to improve the lives of patients suffering from epilepsy and neuropsychiatric disorders. Ganaxolone is a positive allosteric modulator of GABA_A that acts on a well-characterized target in the brain known to have both anti-seizure and anti-anxiety effects. Ganaxolone is being developed in three different dose forms (IV, capsule and liquid) intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Marinus is preparing to initiate a pivotal study in children with CDKL5 deficiency disorder, a rare form of epilepsy, and currently conducting studies in patients with postpartum depression and refractory status epilepticus. 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 trial testing schedule and milestones, the ability to complete enrollment in our clinical trials, 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 future clinical trials, the timing of the clinical trials, enrollment in clinical trials, availability of data from ongoing 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, 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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