



October 31, 2017

## **Marinus Pharmaceuticals Provides Business Update and Reports Third Quarter 2017 Financial Results**

RADNOR, Pa., Oct. 31, 2017 (GLOBE NEWSWIRE) -- [Marinus Pharmaceuticals, Inc.](#) (Nasdaq:MRNS) (the "Company"), a biopharmaceutical company dedicated to the development of innovative therapeutics to treat epilepsy and neuropsychiatric disorders, today provided a business update and reported its financial results for the quarter ended September 30, 2017.

"We achieved an important milestone this quarter as we announced successful results from our clinical study in children with CDKL5 deficiency disorder, a rare, genetic epilepsy with no approved therapies," said Christopher M. Cashman, Chief Executive Officer of Marinus Pharmaceuticals. "On the heels of this data, we raised capital to advance the development of ganaxolone into CDKL5 patients and extend our cash runway. With a strong balance sheet in place, we are focused on quality execution of our clinical studies in postpartum depression, which will inform our later stage clinical development plans for this important underserved patient population."

### **Clinical Programs Update**

#### CDKL5 Deficiency Disorder (CDD)

- | In September, the Company announced successful results from a Phase 2 open-label study evaluating the safety and efficacy of ganaxolone in children with CDD. The data showed that ganaxolone provided substantial and durable anti-seizure efficacy in children with CDD. Ganaxolone was generally safe and well-tolerated with no serious adverse events. Four patients continue to receive ganaxolone; three of which have entered the one-year extension of the study and one of which is still receiving treatment within the 26-week treatment period. Based on the data, the Company is planning to meet with regulatory agencies to discuss the clinical development plan with the goal of commencing a clinical study in 2018.
- | The Phase 2 data in patients with CDD was presented at the 2017 Child Neurology Society Annual Meeting in October. The Child Neurology Society is the preeminent professional association of pediatric neurologists worldwide devoted to fostering the discipline of child neurology and promoting the optimal care and welfare of children with neurological and neuro developmental disorders.

#### Postpartum Depression (PPD)

- | Enrollment is on-going in the Magnolia Study, a Phase 2 double-blind, placebo-controlled clinical trial to evaluate the safety, efficacy and pharmacokinetics of intravenous (IV) ganaxolone in women diagnosed with severe PPD. The study consists of multiple cohorts of women with a Hamilton Depression Rating Scale (HAM-D17) score  $\geq 26$ . Patients randomized in the initial cohort(s) will undergo an infusion of either ganaxolone or placebo and will be followed for 30 days, with data expected in early 2018. Subsequent cohorts could include shorter- or higher-dose IV regimens alone, or in sequential administration with ganaxolone capsules.
- | The Company is initiating its Amaryllis Study, a Phase 2 clinical trial to evaluate the safety, tolerability and efficacy of oral ganaxolone in women with moderate PPD (HAM-D17 score  $\geq 20$  and  $< 26$ ). The goal of this study is to determine the oral dosing regimen for future studies. Oral ganaxolone has the potential to provide the largest segment of the PPD patient population with access to convenient, oral outpatient therapy. Data from this study is expected in 2018.

#### Status Epilepticus (SE)

- | The Company is initiating its Phase 2 feasibility study with ganaxolone IV in patients with refractory status epilepticus (RSE). The Phase 2 trial is designed to treat patients in the SE treatment paradigm as second-line, when they have active brain function and potential for better outcomes. Data from this feasibility study is expected in 2018.

### **Financial Update**

At September 30, 2017, the Company had cash, cash equivalents and investment balances of \$62.9 million. The Company believes that its cash, cash equivalents and investments as of September 30, 2017 are adequate to fund operations into

2020.

Research and development expenses decreased to \$2.6 million and \$9.0 million for the three and nine months ended September 30, 2017, respectively, as compared to \$4.8 million and \$17.6 million for the same periods in the prior year. The decreases were primarily due to a decrease of \$1.8 million and \$9.3 million for the three- and nine-month periods ended September 30, respectively, associated with our drug-resistant focal onset seizures program, which 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 \$0.9 million for the nine-month period ended September 30, associated with our IV programs in PPD, for which a Phase 2 clinical trial was initiated in June 2017, and SE, for which we are initiating a Phase 2 clinical trial.

General and administrative expenses were \$1.6 million and \$5.1 million for the three and nine months ended September 30, 2017 as compared to similar expense amounts of \$1.5 million and \$4.7 million for the same periods in the prior year.

Cash used in operating activities decreased to \$14.3 million for the nine months ended September 30, 2017 compared to \$18.7 million for the same period a year ago. The decrease was driven primarily by a decrease in our net loss of \$8.5 million, partially offset by a decrease in the change in operating assets and liabilities of \$4.1 million. The net decrease in the change in operating assets and liabilities was primarily due to upfront payments for planned clinical trials in our IV program, payment of corporate insurance premiums and the timing of payment obligations related to our drug supply in 2016.

Readers are referred to, and encouraged to read in its entirety, the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2017 to be filed with the Securities and Exchange Commission, which includes further detail on the Company's business plans and operations, financial condition and results of operations.

### **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<sub>A</sub> 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 currently evaluating ganaxolone in women with postpartum depression and preparing to initiate studies in children with CDKL5 deficiency disorder and patients with status epilepticus, both of which are orphan indications. For more information visit [www.marinuspharma.com](http://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.

### **CONTACT:**

Lisa M. Caperelli

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

	<b>September 30, 2017</b>	<b>December 31, 2016</b>
<b>ASSETS</b>		
Cash and cash equivalents	\$ 37,530	\$ 26,178
Investments	25,373	3,922
Other assets	1,754	1,347
Total assets	\$ 64,657	\$ 31,447
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Total current liabilities	\$ 2,430	\$ 8,084
Notes payable, long-term portion	—	1,743
Other long term liabilities	127	141
Total liabilities	2,557	9,968
Total stockholders' equity	62,100	21,479
Total liabilities and stockholders' equity	\$ 64,657	\$ 31,447

	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>	
	<b>2017</b>	<b>2016</b>	<b>2017</b>	<b>2016</b>
Expenses:				
Research and development	\$ 2,642	\$ 4,840	\$ 9,032	\$ 17,593
General and administrative	1,571	1,529	5,074	4,719
Loss from operations	(4,213)	(6,369)	(14,106)	(22,312)
Interest income	45	36	116	93
Interest expense	(3)	(118)	(159)	(365)
Other income (expense)	1	(13)	(11)	(44)
Net loss	\$ (4,170)	\$ (6,464)	\$ (14,160)	\$ (22,628)
Per share information:				
Net loss per share of common stock—basic and diluted	\$ (0.15)	\$ (0.33)	\$ (0.60)	\$ (1.16)
Basic and diluted weighted average shares outstanding	28,666,656	19,509,220	23,531,745	19,494,424