



March 13, 2017

## **Marinus Pharmaceuticals Provides Business Update and 2016 Financial Results**

RADNOR, Pa., March 13, 2017 (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, 2016.

### **Recent Clinical & Corporate Highlights**

- | The new intravenous (IV) formulation of ganaxolone was successfully administered to healthy volunteers and was shown to be generally safe and well tolerated, and reached dose levels targeted for Phase 2 studies in postpartum depression (PPD), status epilepticus (SE) and other indications.
- | Ganaxolone reduced seizure frequency and was generally safe and well tolerated in an on-going Phase 2 study in children with orphan, genetic disorders, including the initial treated children with CDKL5 disorder and the eleven children with PCDH19 pediatric epilepsy.
- | Ganaxolone improved anxiety and hyperactivity across multiple anxiety scales in a Phase 2 clinical study in Fragile X Syndrome (FXS) children with high baseline anxiety. The FDA has granted orphan drug designation for ganaxolone to treat FXS.
- | Two seasoned biotech executives were appointed to the board of directors.

"We enter 2017 focused on delivering clinical data to support advancing multiple formulations of ganaxolone in indications with few or no approved treatment options," said Christopher M. Cashman, chief executive officer of Marinus Pharmaceuticals. "We are in the process of initiating our Phase 2 clinical trial in women who suffer from postpartum depression, a mood disorder that occurs within weeks of childbirth. For status epilepticus, we have received regulatory feedback from FDA on the study protocol and are making preparations to initiate our Phase 2 clinical trial. With several near-term milestones on the horizon, 2017 has the potential to be a significant year for Marinus."

### **Near-term Clinical Value Catalysts**

- | Initiate Phase 2 study in women with PPD in 1H 2017
- | Report top-line data from patients with orphan, genetic disorders in mid-2017
- | Initiate Phase 2 study in patients with SE in 2H 2017
- | Report data from initial PPD patient cohort(s) in 2H 2017

### **Financial Update**

At December 31, 2016, the Company had cash, cash equivalents and investments of \$30.1 million, compared to \$57.7 million at December 31, 2015. We believe that our cash, cash equivalents and investments as of December 31, 2016 will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2018.

Research and development expenses increased \$3.1 million, to \$22.0 million, for the year ended December 31, 2016, compared to the same period of 2015. The increase was primarily due to an increase of \$2.7 million associated with preclinical and clinical activities in our IV program, and \$1.4 million associated with increases in salaries, benefits and noncash stock-based compensation, mostly attributable to increased headcount. This increase was partially offset by a decrease of \$1.3 million in costs associated with our drug-resistant focal onset seizure program, which discontinued in June 2016.

General and administrative expenses increased \$0.7 million, to \$6.2 million, for the year ended December 31, 2016, compared to the same period of 2015. 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 \$28.6 million and \$24.9 million for the years ended December 31, 2016 and 2015, respectively. Cash used in operating activities was \$24.8 million for the year ended December 31, 2016 compared to \$20.1 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, 2016 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.

## 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 antiseizure and antianxiety 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 orphan pediatric indications for the treatment of genetic seizure and behavior disorders, and preparing to initiate Phase 2 studies in status epilepticus, an orphan indication, and postpartum depression. For more information visit [www.marinuspharma.com](http://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 interpretation of clinical and preclinical studies, assessment of positive nature and notability of preliminary data, 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, and other matters, including the development of formulations of ganaxolone, 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.

## Marinus Pharmaceuticals, Inc.

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

	<b>December 31, December 31,</b>	
	<b>2016</b>	<b>2015</b>
<b>ASSETS</b>		
Cash and cash equivalents	\$ 26,178	\$ 51,722
Investments	3,922	5,962
Other assets	1,347	1,964
Total assets	<u>\$ 31,447</u>	<u>\$ 59,648</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Total current liabilities	\$ 8,084	\$ 7,435
Notes payable, long-term portion	1,743	5,236
Other long term liabilities	141	56
Total liabilities	<u>9,968</u>	<u>12,727</u>
Total stockholders' equity	<u>21,479</u>	<u>46,921</u>
Total liabilities and stockholders' equity	<u>\$ 31,447</u>	<u>\$ 59,648</u>

<b>Year Ended December 31,</b>	
<b>2016</b>	<b>2015</b>

Expenses:

Research and development	\$ 22,005	\$ 18,916
General and administrative	6,237	5,516
Loss from operations	<u>(28,242)</u>	<u>(24,432)</u>
Interest income	128	64
Interest expense	(464)	(475)
Other expense	(65)	(7)
Net loss	<u>\$ (28,643)</u>	<u>\$ (24,850)</u>

Per share information:

Net loss per share of common stock—basic and diluted	<u>\$ (1.47)</u>	<u>\$ (1.67)</u>
Basic and diluted weighted average shares outstanding	<u>19,498,143</u>	<u>14,919,783</u>

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