Substantial and Durable Anti-Seizure Efficacy Shown in Patients Suffering From Severe,

Rare Genetic Epilepsy

Marinus to Host Conference Call and Webcast Today at 9:00 am EDT

RADNOR, Pa., Sept. 11, 2017 (GLOBE NEWSWIRE) -- Marinus Pharmaceuticals, Inc. (Nasdaq:MRNS), today announced that top-line data from the Phase 2 open-label study in patients with CDKL5 disorder support advancing ganaxolone into a definitive late-stage clinical trial. Oral ganaxolone, in addition to baseline treatment, showed a sizable and durable seizure-frequency reduction in the majority of patients, with some achieving an increase in the number of seizure-free days and reporting behavioral benefits. CDKL5 disorder is a severe, rare genetic epilepsy that results in early-onset, treatment-refractory seizures, pervasive neuro-developmental delay and disabling behavioral issues. There are no approved or effective available treatment options. Marinus plans to meet with regulatory agencies to obtain agreement on the clinical development plan that would be needed for approval of ganaxolone for CDKL5 disorder.

Orrin Devinsky, MD, Director of the NYU Langone Medical Center's Comprehensive Epilepsy Center and Principal Investigator in the current study, commented, "I am impressed with the magnitude of seizure reduction and gain in seizure-free days seen with ganaxolone treatment in children with this highly refractory epilepsy. The durable anti-epileptic effect seen in several children distinguishes ganaxolone's efficacy from the more than 20 currently available anti-epileptic drugs that provide limited seizure control lasting a few weeks to months. When treating children with the severest forms of epilepsy such as CDKL5 disorder, there is a desperate need for new drugs that are well-tolerated, efficacious and easy to administer with no need for special monitoring. With no other treatment showing this degree of promise, I am excited to lead the effort to advance ganaxolone into potentially the first, late-stage clinical trial in children with CDKL5 disorder."

Top-line Data:

- The median change in 28-day seizure frequency from baseline in the ITT (intent-to-treat) population (primary endpoint) was a decrease of 43% (n=7).
- The median change from baseline in seizure-free days in the ITT population (key secondary endpoint) was an increase of 78% (n=5; two subjects cannot be calculated due to 0 baseline seizure-free days).
- Five of the seven children experienced a meaningful seizure reduction compared to baseline; median reduction of 65% (range 24% - 85%).
- Three children have so far met the criteria to enter the one year study extension (completed 26-weeks of treatment with excellent seizure control) and continue to experience a median seizure reduction of 70% and median increase in seizure-free days of 75%.
- The Clinical Global Impression Scale rated by Investigators (CGI-I) and Caregivers (CGI-P) were consistent with seizure control for all the children. Children with a 43% or higher seizure reduction were rated as "much improved" or "very much improved" by the Investigators and

Caregivers.

- Investigators and Caregivers reported improvements in attention, mood, behavior and sleep via investigator narratives.
- Ganaxolone was generally safe and well-tolerated with no serious adverse events. To date, there have been no adverse event reports of somnolence or dizziness.
- One child has not yet reached the 26-week visit and two children discontinued prior to completing the 26-week treatment due to lack of efficacy.

Nicola Specchio, MD, PhD, Principal Investigator at the Ospedale Pediatrico Bambino Gesù in Rome, commented, "I am very impressed with the improvement my patients experienced while on ganaxolone. They showed a sizeable decrease in seizure frequency and increase in attention associated with a calmer demeanor, which exceeded my expectations. Ganaxolone's safety profile and effect on seizure and non-seizure related comorbidities, common in children with CDKL5 disorder, makes it an exciting potential therapeutic for this rare patient population that critically needs an effective and lasting treatment option."

Marinus is planning to submit the full CDKL5 disorder data set for publication or presentation at a medical conference. Earlier this year, Marinus received Orphan Drug Designation from the U.S. Food and Drug Administration (FDA) to develop ganaxolone for the treatment of CDKL5 Disorder.

"We believe that the efficacy and safety profile of ganaxolone in this study provide compelling evidence that a definitive study in patients with CDKL5 disorder should be undertaken as soon as possible," commented Dr. Lorianne Masuoka, Chief Medical Officer of Marinus Pharmaceuticals. "The tolerability and complete lack of dose regimen-limiting safety concerns, as compared to the most widely used anti-epileptic drugs for this condition, could make ganaxolone an exceptional option for patients with CDKL5 disorder."

The study enrolled seven children between the ages of 2 and 16 at three sites within the U.S. and Italy. Following screening and baseline evaluations, children received up to 1,800 mg/day of oral ganaxolone in addition to their current treatment regimen for up to 26-weeks. The primary efficacy measure was percent change from baseline in the 28-day seizure frequency over 26-weeks. The key secondary outcome measure was percent change from baseline in seizure-free days. Safety and tolerability were secondary objectives of the study. Patients responding to therapy were given the option to enroll into a 52-week extension program.

In addition to the CDKL5 disorder cohort of patients, Marinus evaluated a cohort of Lennox-Gastaut syndrome (LGS) patients. Based upon these robust CDKL5 disorder clinical results and etiologic fit with ganaxolone's mechanism of action, Marinus has prioritized CDKL5 disorder as its lead pediatric orphan program for advancement into later stage clinical development.

Conference Call and Webcast Details

Marinus will host a conference call today at 9:00 a.m. EDT. Stockholders and other interested parties may participate in the call by dialing 844-277-9448 (domestic) or 336-525-7135 (international) and referencing conference ID number 79335646. The live webcast can be accessed on the investor page of Marinus' website at http://ir.marinuspharma.com/events.cfm. A replay will be available on Marinus'

website approximately two hours after completion of the event and will be archived for up to 30 days.

About CDKL5 Disorder

CDKL5 disorder is a serious and rare genetic disorder that is caused by a mutation of the cyclin-dependent kinase-like 5 (CDKL5) gene, located on the X chromosome. It predominantly affects girls and is characterized by early-onset, difficult-to-control seizures and severe neuro-developmental impairment. The CDKL5 gene encodes a protein essential for normal brain function. Most children affected by CDKL5 cannot walk, talk, or care for themselves. Many also suffer from scoliosis, visual impairment, gastrointestinal difficulties, and sleeping disorders. CDKL5 disorder is among the epileptic encephalopathies that are most refractory to treatment. Currently, there are no approved therapies for CDKL5 Disorder.

About Ganaxolone

Ganaxolone, a positive allosteric modulator of GABAA, is being developed in three different dose forms (intravenous, capsule, and liquid) intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Unlike benzodiazepines, ganaxolone exhibits anti-seizure and anti-anxiety activity via its effects on synaptic and extrasynaptic GABAA receptors. Ganaxolone has been studied in more than 1,500 subjects, both pediatric and adult, at therapeutically relevant dose levels and treatment regimens for up to two years. In these studies, ganaxolone was generally safe and well-tolerated. The most commonly reported adverse events were somnolence, dizziness and fatigue.

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 GABAA 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 in orphan pediatric indications for the treatment of genetic seizure and behavior disorders, and preparing to initiate a Phase 2 study in status epilepticus, an orphan indication. 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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