Marinus Pharmaceuticals Initiates Pivotal Phase 3 Trial in Children with CDKL5 Deficiency Disorder

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Marigold Study targets rare form of pediatric epilepsy with no approved therapies

RADNOR, Pa., June 19, 2018 (GLOBE NEWSWIRE) -- Marinus Pharmaceuticals, Inc. (Nasdaq:MRNS) ("Marinus" or "Company"), a biopharmaceutical company dedicated to the development of innovative therapeutics to treat epilepsy and neuropsychiatric disorders, today announced the initiation of a pivotal Phase 3 clinical trial (Marigold Study) evaluating the use of oral ganaxolone in children and young adults with CDKL5 Deficiency Disorder (CDD).

"We are excited to offer the CDKL5 patient community, for the first time, participation in a pivotal study of ganaxolone that may have the potential to reduce the seizure burden of the disorder," said Christopher M. Cashman, Chief Executive Officer of Marinus. "After seeing the long-term results from our Phase 2 trial showing that ganaxolone provides a meaningful reduction in the number of seizures for children with CDD, as well as an impressive increase in seizure-free days, we focused our efforts to expedite the initiation of this registration study. CDKL5 patients are in need of new treatment options and we look forward to working with the entire community in the conduct of this study."

The Marigold Study is a global, double-blind, placebo-controlled, Phase 3 clinical trial that will enroll approximately 70 patients between the ages of 2 and 21 with a confirmed disease-related CDKL5 gene variant. Patients will undergo a baseline period before being randomized to receive either ganaxolone (up to 1,800 mg/day) or placebo for 17 weeks, in addition to their existing anti-seizure treatment. Following the treatment period, all patients that meet certain eligibility requirements will have the opportunity to receive ganaxolone in the open label phase of the study. The study's primary efficacy endpoint is percent reduction in seizures. Secondary outcome measures will include non-seizure-related endpoints to capture certain changes in behavioral and sleep disturbances that were seen as improvements in previous clinical studies with ganaxolone.

CDD is caused by a mutation of the cyclin-dependent kinase-like 5 (CDKL5) gene, located on the X chromosome. The CDKL5 gene encodes proteins essential for normal brain function. CDD predominantly affects girls and is characterized by early-onset, difficult-to-control seizures and severe neurodevelopmental impairment. Most children affected by CDD cannot walk, talk, or feed themselves, and many are confined to wheelchairs, dependent on others for their care.

Currently, there are no approved therapies for CDD worldwide. Existing anti-epilepsy medications do not work well in CDD, and at best, any efficacy is short-lived.

Ganaxolone is a neurosteroid designed to provide anti-seizure activity by calming the brain and restoring its electrical balance. Ganaxolone's method of action is different from existing epilepsy medications, binding to unique $GABA_A$ receptors and has been shown in animal models to quickly inhibit seizure spread and prevent new seizures from occurring.

Last September, Marinus reported top-line data from a Phase 2 open-label study in patients with CDD. Oral ganaxolone, in addition to baseline treatment, showed a sizable and durable seizure-frequency reduction with a large increase in the number of seizure-free days in the majority of patients and with some reporting behavioral benefits. Ganaxolone has been administered in more than 200 children, as young as four months old and dosed for more than four years. Ganaxolone is generally safe and well-tolerated with no related serious adverse events reported to date in this population. Marinus has received Orphan Drug Designation from the FDA for ganaxolone in CDD.

More information on the Marigold Study will be available at www.themarigoldstudy.com or www.clinicaltrials.gov.

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 anti-seizure, anti-depressant 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 has initiated the first ever pivotal study in children with CDKL5 deficiency disorder, a rare form of epilepsy, and is 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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