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Marinus Announces Positive Preliminary Data From Children With CDKL5 Genetic Disorder

Plans to Apply for Orphan Drug Designation

RADNOR, Pa., Jan. 23, 2017 (GLOBE NEWSWIRE) -- [Marinus Pharmaceuticals, Inc.](#) (Nasdaq:MRNS), today announced positive preliminary data from the initial CDKL5 patients enrolled in its ongoing Phase 2 open-label study evaluating its CNS-selective GABA_A modulator, ganaxolone, as a treatment for orphan, genetic disorders. CDKL5 is a severe, rare genetic disorder that results in early-onset, difficult-to-control seizures, and neuro-developmental impairment. Enrollment is continuing in the study with top-line data expected in mid-2017.

Four patients have been enrolled in this cohort of the study and received up to 1800 mg/kg of ganaxolone per day for an average treatment duration of five-months. Three of the four patients experienced a notable reduction in seizure frequency compared to baseline ranging from 52% to 88%. All responders continue to receive treatment, two of whom have completed six-months of treatment and have elected to participate in the study extension. One patient discontinued the study after four-months of treatment due to lack of efficacy. Safety data to date are consistent with earlier studies where ganaxolone has shown to be generally safe and well-tolerated.

"We are encouraged by the results in these difficult-to-treat pediatric patients," commented Dr. Jaakko Lappalainen, Vice President of Clinical Development of Marinus Pharmaceuticals. "Concurrent with completing this study, we will be evaluating the potential for breakthrough therapy and applying for orphan drug designation with the United States Food and Drug Administration. CDKL5 pediatric epilepsy may prove to be an attractive and efficient path for ganaxolone and we look forward to evaluating results from the final patients enrolled in this cohort of the study."

Michael G. Chez, MD, Director of Pediatric Neurology Research and Pediatric Epilepsy, Sutter Neuroscience Institute in Sacramento, CA commented, "I am impressed with the responder rate and magnitude of seizure control seen with ganaxolone in the initial CDKL5 patients. The CGIs (clinical global impression scales) are consistent with seizure control, with responders showing 'much improved' under this scale. I look forward to further evaluating these children and seeing the final results."

At the annual meeting of the American Epilepsy Society, Dr. Chez presented EEG data from one CDKL5 and two PCDH19 patients that he treated with ganaxolone in the on-going Phase 2 open-label study. The patients received up to 1800 mg/day of ganaxolone. EEG measurements were taken at baseline and followed-up at 8-12 weeks of treatment.

The CDKL5 patient showed a >67% seizure reduction and EEG changes consistent with clinical improvement (50% reduction in awake slow-spike wave discharges). The two patients with PCDH19 showed an 80% and 75% reduction in seizure frequency, respectively, and EEG improvement in slow-spike and wave frequency of >90% and 80% on awake and asleep EEG.

This Phase 2 open-label trial is currently accepting patients at five sites in the United States and one in Italy. The multi-cohort study is designed to enroll up to 10 patients with each of CDKL5 disorder, Lennox Gastaut Syndrome (LGS) and PCDH19 pediatric epilepsy. The study is actively recruiting CDKL5 and LGS patients. The PCDH19 cohort of the study is currently closed for enrollment, however, there are still children receiving ganaxolone in the study extension. For more information about the study visit [clinicaltrials.gov](#).

About CDKL5 Disorder

CDKL5 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 proteins 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. Currently, there are no approved therapies for CDKL5 disorder. No previous formal clinical trials have been conducted in this population.

About Ganaxolone

Ganaxolone is a CNS-selective GABA_A modulator 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. Ganaxolone acts on a well-characterized synaptic and extrasynaptic GABA_A target known for its anti-seizure and anti-anxiety activity. Ganaxolone has been studied in more than 1,400 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, with the most commonly reported adverse events of 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 CNS-selective GABA_A modulator 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 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.

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.

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