Marinus Pharmaceuticals Completes Targeted Enrollment in Pivotal Phase 3 Study for CDKL5 Deficiency Disorder

Top-line data on-track for Q3 2020

RADNOR, PA, February 25, 2020 -- Marinus Pharmaceuticals, Inc. (Nasdaq: MRNS), a pharmaceutical company dedicated to the development of innovative therapeutics to treat epilepsy and neuropsychiatric disorders, today announced it has reached the 100 patient enrollment target for the Marigold Study. The Marigold Study is a pivotal Phase 3 study evaluating oral ganaxolone in children and young adults with CDLK5 Deficiency Disorder (CDD), a rare refractory form of pediatric epilepsy with no currently approved treatments. The Company will continue enrollment through the end of the month to capture the remaining few patients in the screening phase. Marinus is on-track to report top-line results in the third quarter of 2020.

"Enrolling 100 patients in our registrational, pivotal Phase 3 trial evaluating ganaxolone in children with CDD is a significant milestone for both Marinus and the CDD community," said Joe Hulihan, M.D., Chief Medical Officer of Marinus. "Our ability to identify and rapidly enroll qualified patients into this study is indicative of the significant need for a new therapy to reduce seizure burden and improve patient outcomes. The study has been well conducted and we are encouraged by the limited adverse events, low dropout rates and the vast majority of patients entering the open-label extension. We are appreciative of the patients and broader CDD community for their participation in the Marigold study and we remain committed to addressing the unmet need of these patients who currently have no approved treatment options."

Karen Utley, President of the International Foundation for CDKL5 Research, commented, "We are grateful for Marinus' recognition of the tremendous need of children with CDD and its dedication to advancing the development of ganaxolone as a potential new treatment for these children through the Marigold study. We are proud of our collaborative partnership to lead the way in finding a cure and treatments for children with CDD and are hopeful that ganaxolone may be a novel treatment option that improves outcomes for our children."

The Marigold Study is a global, double-blind, placebo-controlled, Phase 3 clinical trial designed to enroll approximately 100 patients between the ages of 2 and 21 with a confirmed disease-related CDKL5 gene variant. Patients 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 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.

About CDKL5 Deficiency Disorder
CDKL5 deficiency disorder (CDD) 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. CDD is characterized by early-onset, difficult-to-control seizures and severe neuro-developmental impairment. Most children affected by CDD cannot walk, talk, or feed themselves, and many are confined to wheelchairs, dependent on others for everything. Currently, there are no approved therapies for CDD.

About Ganaxolone
Ganaxolone, a positive allosteric modulator of $GABA_A$ receptors, is being developed in intravenous and oral formulations 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 $GABA_A$ receptors. Ganaxolone has been studied in more than 1,600 subjects, both pediatric and adult, at therapeutically relevant dose levels and treatment regimens for up to four 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 pharmaceutical 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 depression. 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 IV and oral dose forms intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Marinus is conducting the first ever pivotal studies in children with CDKL5 deficiency disorder and PCDH19-related epilepsy. Based on results from a recent Phase 2 study in refractory SE and from biomarker analysis research, the Company intends to initiate later this year a Phase 3 study in SE and a Phase 2 study in Tuberous Sclerosis Complex (TSC), respectively. 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 study testing

schedule and milestones, the ability to complete enrollment in our clinical studies, 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 studies, the timing of the clinical studies, enrollment in clinical studies, availability of data from ongoing clinical studies, expectations for regulatory approvals, the attainment of clinical study 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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