



NEWS RELEASE

# Marinus Launches Expanded Access Program for Ganaxolone Treatment in CDKL5 Deficiency Disorder

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RADNOR, Pa.--(BUSINESS WIRE)-- **Marinus Pharmaceuticals, Inc.** (Nasdaq: MRNS), a pharmaceutical company dedicated to the development of innovative therapeutics to treat rare seizure disorders, today announced the launch of an expanded access program (EAP) that will allow the company to offer ganaxolone to patients with CDKL5 Deficiency Disorder (CDD) who were unable to participate in the company's Marigold Study Phase 3 clinical trial.

"With the positive results in our clinical trial, we felt it was an important measure to make ganaxolone available to patients with CDD as we prepare to submit a new drug application to the FDA," said Scott Braunstein, M.D., Chief Executive Officer of Marinus. "There are currently no approved treatments specifically for patients with CDD, creating a strong need to provide early, compassionate use access to investigational ganaxolone therapy for these children."

Candidates for the EAP must be at least two years of age, with a confirmed diagnosis of CDD, and experiencing uncontrolled seizures. The investigational treatment will initially be available in sites in the United States. It is important to remember that investigational drugs have not yet received regulatory approval; therefore, their potential risks and benefits are not yet established. Doctors and patients should consider all possible benefits and risks when seeking expanded access to an investigational drug.

In the Marigold Study, patients given oral ganaxolone showed a significant 32.2% median reduction in 28-day major motor seizure frequency, compared to a 4.0% reduction for those receiving placebo. In this trial, ganaxolone was generally well tolerated with a safety profile consistent with previous clinical trials, with the most frequent adverse

event being somnolence. Marinus is preparing to meet with the U.S. Food and Drug Administration (FDA) in Q1 2021 regarding its planned mid-2021 NDA submission, as well as a 1H 2021 pre-marketing authorization application (MAA) meeting with the European Medicines Agency (EMA).

For more information on the Marinus Expanded Access Treatment Protocol for ganaxolone, please visit <https://marinuspharma.com/expanded-access-program/>

## 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. Currently, there are no therapies approved specifically for CDD.

## About Ganaxolone

Ganaxolone, a positive allosteric modulator of GABAA 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 antiseizure, antidepressant and anti-anxiety activity via its effects on synaptic and extrasynaptic GABAA receptors. More than 1,600 study participants, both adults and children, have received ganaxolone at therapeutically relevant dose levels and treatment regimens for up to four years.

## About Marinus Pharmaceuticals

Marinus Pharmaceuticals, Inc. is a pharmaceutical company dedicated to the development of innovative therapeutics to treat rare seizure disorders. Ganaxolone is a positive allosteric modulator of GABAA receptors 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 formulations intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Marinus recently completed the first ever Phase 3 pivotal trial in children with CDKL5 deficiency disorder and is conducting a Phase 2 trial in tuberous sclerosis complex, as well as a Phase 2 biomarker-driven proof-of-concept trial in PCDH19-related epilepsy. The company has also initiated a Phase 3 trial in status epilepticus. 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 plans to meet with the U.S. Food and Drug Administration (FDA) in Q1 2021; our plans to submit a new drug application to the FDA in mid- 2021; our plans to meet with the European Medicines Agency (EMA) in first half of 2021 regarding a pre-marketing authorization application (MAA). 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, uncertainties and delays relating to the design, enrollment, completion, and results of clinical trials; unanticipated costs and expenses; clinical trial results may not support further development in a specified indication or at all; actions or advice of the U.S. Food and Drug Administration may affect the design, initiation, timing, continuation and/or progress of clinical trials or result in the need for additional clinical trials; our ability to obtain and maintain regulatory approval for our product candidate; delays, interruptions or failures in the manufacture and supply of our product candidate; our ability to raise additional capital; the effect of the COVID-19 pandemic on our business, the medical community and the global economy; and the availability or potential availability of alternative products or treatments for conditions targeted by us that could affect the availability or commercial potential of our product candidate. 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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