

Marinus Pharmaceuticals Joins the Loulou Foundation in Collaboration on CANDID, a Comprehensive Observational Study 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 seizure disorders, today announced it has joined forces with the **Loulou Foundation**, a private foundation dedicated to advancing research on the causes and treatment of CDKL5 deficiency disorder (CDD), and six other biotech and pharmaceutical organizations, to undertake a key observational CDD clinical study to better understand the natural history and the utility of various clinical assessments. Marinus and the other industry organizations will share in the funding and governance of a three-year observational study in CDD patients – the Clinical Assessment of NeuroDevelopmental measures In CDD (CANDID) study. The Loulou Foundation will serve as the study coordinator, and the study will involve CDD clinical centers worldwide.

“Seizures in CDD are often refractory to treatment, and despite available anti-seizure medications, there remains an unmet need for treatments specific to CDD. At Marinus, we are developing ganaxolone, an investigational drug for the treatment of rare epilepsies, including CDD,” said Alex Aimetti, Ph.D., Vice President, Scientific Affairs at Marinus Pharmaceuticals. “We are pleased to be collaborating on the CANDID study to gain a more complete view of how CDD affects patients and to define relevant endpoints for future clinical trials. We are privileged to be adding to the scientific knowledge about this serious disorder and to be able to support the CDD community by partnering with the Loulou Foundation on this study.”

The CANDID study aims to provide researchers with information on the suitability of clinical assessments across

multiple domains. The study also aims to document the longitudinal trajectories in CDD patients across the various assessments. The results of the CANDID study will ultimately be shared with the entire scientific and medical community to aid in the design of clinical trials and to inform therapeutic development for CDD and related neurodevelopmental disorders, including Rett syndrome and Angelman syndrome.

In September, Marinus' New Drug Application for the use of ganaxolone to treat seizures associated with CDD was accepted for filing with priority review by the U.S. Food and Drug Administration (FDA). The FDA assigned a Prescription Drug User Fee Act (PDUFA) action date of March 20, 2022. Validation of Marinus' marketing authorization application by the European Medicines Agency is expected by the end of October. These applications for commercialization are supported by data from the Marigold trial, a Phase 3, double-blind placebo-controlled study which enrolled 101 patients and demonstrated that patients treated with ganaxolone experienced a 30.7% median reduction in 28-day major motor seizure frequency, compared to a 6.9% reduction for those receiving placebo, achieving the trial's primary endpoint ($p=0.0036$). Patients in the Marigold open label extension treated with ganaxolone for at least 12 months ($n=48$) experienced a median 49.6% reduction in major motor seizure frequency. In the Marigold trial and its open label extension, ganaxolone was generally well tolerated with a safety profile consistent with previous clinical trials, with the most frequent adverse event being somnolence.

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 neurodevelopmental impairment. Currently, there are no therapies approved specifically for CDD.

About Ganaxolone

Ganaxolone, a positive allosteric modulator of GABAA receptors, is an investigational product 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. Ganaxolone exhibits anti-seizure and anti-anxiety activity via its effects on synaptic and extrasynaptic GABAA receptors. Ganaxolone has been studied in more than 1,800 pediatric and adult subjects across various indications at therapeutically relevant dose levels and in treatment regimens for up to more than two years.

About the Loulou Foundation

The Loulou Foundation is a private non-profit organization founded in 2015 to support the development of effective therapeutics and eventual cures for CDD. Through robust grant and directed research programs, the Foundation provides tools and resources to basic and clinical scientists to enable the development of disease-modifying therapeutics for CDD. These programs include support for pre-clinical, translational, and clinical research into basic

CDKL5 biology, CDD disease mechanisms, and the proof-of-concept studies for gene therapy and genome modifying therapeutics.

About Marinus Pharmaceuticals

Marinus Pharmaceuticals, Inc. is a pharmaceutical company dedicated to the development of innovative therapeutics to treat 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, antidepressant 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 completed the first ever Phase 3 pivotal trial in children with CDKL5 deficiency disorder last year, is planning to conduct a Phase 3 trial in tuberous sclerosis complex, and a Phase 3 trial in refractory status epilepticus is ongoing. 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 expectations and beliefs regarding our new drug application submission to the U.S. Food and Drug Administration (FDA) for ganaxolone in CDKL5 deficiency disorder; our marketing authorization application to the European Medicines Agency; our regulatory interactions with respect to our product candidates; and the potential safety and efficacy of ganaxolone, as well as its therapeutic potential in a number of indications.

Forward-looking statements in this press 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 potential that the FDA may not grant or may delay approval for our product candidate; the risk that the marketing authorization application for our product candidate is not validated or ultimately approved by the European Medicines Agency; uncertainties regarding interactions with regulatory authorities, including reviews and inspections. This list is not exhaustive and these and other risks are described in our periodic reports, including the annual report on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K, filed with or furnished to the Securities and Exchange Commission and available at www.sec.gov. Any forward-looking statements that we make in this press release speak only as of the date of this press release. We assume no obligation to update forward-looking statements whether as a result of new information, future events or

otherwise, after the date of this press release.

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