



NEWS RELEASE

# Marinus Pharmaceuticals Completes In Vivo M2 Metabolite Study and Provides European Regulatory Update

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In vivo study of M2 metabolite shows no signs of genotoxicity

Marinus' CDKL5 deficiency disorder marketing authorization application to convert to standard review timeline

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 preliminary results from an in vivo study on the M2 metabolite showing that no genotoxicity was found, as measured by formation of micronuclei in the bone marrow or comet morphology in the liver.

In August 2021, Marinus **announced a collaboration with Orion Corporation** for European commercialization of ganaxolone. Under the agreement, Orion paid Marinus €25 million (~\$30 million at the time of announcement) in cash as an upfront fee, subject to a 75% clawback of the upfront payment and a right to terminate the agreement in the event the M2 metabolite study showed genotoxicity. The final study report is expected prior to the end of February 2022.

Marinus also announced its marketing authorization application (MAA) for ganaxolone in CDKL5 deficiency disorder will convert to a standard review and that it had reached an agreement with the European Medicines Agency (EMA) to extend the Day 120 clock stop by three months to allow sufficient time to respond to questions received as part of the review process. The company now expects the EMA's Committee for Medicinal Products for Human Use opinion on the MAA by year end 2022.

## 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 1,900 pediatric and adult subjects across various indications at therapeutically relevant dose levels and treatment regimens for up to more than two years.

## 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. 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 expected clinical development plans, enrollment in our clinical trials, regulatory communications and submissions and product launches for ganaxolone, and the timing thereof; our expectation regarding the M2 metabolite study report and the expected timing thereof; our expectations and beliefs regarding the FDA and EMA with respect to our product candidates, including our expectation that the EMA's Committee for Medicinal Products for Human Use will issue its opinion on the MAA by year-end 2022; our expectations regarding the Orion Corporation collaboration; our expectation regarding the impact of the COVID-19 pandemic on our business and clinical development plans; our financial projections; 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 risk that the FDA will require additional clinical trials or data; any delays in review of the NDA submission by the FDA

for any reason, including the COVID-19 pandemic; the timing of regulatory filings for our product candidates; the potential that regulatory authorities, including the FDA and EMA, may not grant or may delay approval for our product candidate; our ability to respond to the Day 120 questions to the satisfaction of the EMA; the risk that EMA's Committee for Medicinal Products for Human Use does not issue its opinion on the MAA by year end 2022; uncertainties and delays relating to the design, enrollment, completion, and results of clinical trials; unanticipated costs and expenses; early clinical trials may not be indicative of the results in later clinical trials; clinical trial results may not support regulatory approval or further development in a specified indication or at all; actions or advice of the FDA or EMA 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; our ability to obtain, maintain, protect and defend intellectual property for our product candidates; the potential negative impact of third party patents on our or our collaborators' ability to commercialize ganaxolone; delays, interruptions or failures in the manufacture and supply of our product candidate; the size and growth potential of the markets for the company's product candidates, and the company's ability to service those markets; the company's cash and cash equivalents may not be sufficient to support its operating plan for as long as anticipated; the company's expectations, projections and estimates regarding expenses, future revenue, capital requirements, and the availability of and the need for additional financing; the company's ability to obtain additional funding to support its clinical development programs; the company's ability to develop sales and marketing capabilities, whether alone or with potential future collaborators; the rate and degree of market acceptance of the company's product candidates; the potential for Orion to breach the collaboration or terminate the agreement in accordance with its terms; the potential for Orion to recoup a percentage of the upfront fee depending on the additional pre-clinical testing; the effect of the COVID-19 pandemic on our business, the medical community, regulators 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. 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](http://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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