NEWS RELEASE

Marinus Pharmaceuticals Provides Strategic Update at Virtual R&D Event

10/5/2021

RADNOR, Pa.--(BUSINESS WIRE)-- **Marinus Pharmaceuticals, Inc**. (Nasdaq: MRNS), a pharmaceutical company dedicated to the development of innovative therapeutics to treat seizure disorders, will be presenting an overview and update of its pipeline and commercial plans across its programs in CDKL5 deficiency disorder (CDD), tuberous sclerosis complex (TSC) and status epilepticus (SE), as well as second generation product development and perspectives on unmet needs in epilepsy during today's virtual R&D event for investors and analysts. The live webcast begins at 9am Eastern Time today and can be accessed **here**.

"Today's presentation by our leadership shows focus on advancing our current clinical trials while pressing forward to diversify our portfolio through next generation product development to leverage ganaxolone's unique characteristics and position the company for long-term growth," said Scott Braunstein, M.D., Chief Executive Officer of Marinus Pharmaceuticals. "We will also discuss our plans for the commercial launch of Ztalmy®, the proposed brand name for oral ganaxolone should it be approved by the FDA for the treatment of CDD, as well as highlight our corporate milestones and potential triggers to enhance our financial position."

Pipeline Updates

CDKL5 Deficiency Disorder (CDD)

- Validation of marketing authorization application (MAA) by European Medicines Agency (EMA) expected by end of October; confirms that the application is sufficiently complete to begin the formal review process.
- EMA's Committee for Medicinal Products for Human Use (CHMP) opinion on the MAA is expected in Q2 2022; the European Commission (EC) decision is anticipated in early Q3 2022.

    - In August, Marinus announced that it received accelerated assessment from the EMA for ganaxolone in CDD; accelerated assessment is granted by the CHMP when a medicinal product is expected to be of major public health interest and therapeutic innovation.

- As previously announced, the U.S. Food and Drug Administration (FDA) PDUFA target action date is set for the CDD New Drug Application (NDA) for March 20, 2022; FDA indicated that it is not currently planning to hold an advisory committee meeting for the application.

CDD Expanded Access Program (EAP)

- European expansion of the CDD EAP reflects Marinus' commitment to patients and families.
- Last year, the company established an EAP (**NCT04678479**) for CDD patients in the U.S. Additional information about Marinus' U.S. CDD EAP is available **here**.

Tuberous Sclerosis Complex (TSC)

- Oral ganaxolone clinical development strategy continues to progress, including plans for a global Phase 3 randomized, double blind, placebo-controlled trial, the TrustTSC trial.
- The TSC Investigational New Drug (IND) application has been submitted to the FDA with the final Phase 3 protocol.
- As a result of the recent changes to the Phase 3 protocol design, first-patient-in (FPI) now expected in Q1 2022 and trial read out in 1H 2024.
- Received CHMP positive opinion for the request for orphan drug designation; formal EC decision expected in Q4 2021.
    - U.S. FDA orphan drug designation was previously announced in August.

Status Epilepticus (SE)

- Marinus' U.S. patent application was granted for ganaxolone dosing and method of treatment for status epilepticus, with a 2040 expiry date.
- After slower site initiation in Q2, 10 sites in the RAISE trial have since been activated for a total of 39 sites activated.
- RAISE II initiation and FPI for RESET (Researching Established Status Epilepticus Treatment) on track for Q1 2022.
- Additional case reports of super refractory status epilepticus (SRSE) patients treated with ganaxolone to be presented at the American Epilepsy Society Meeting in December.

Next Generation Product Development – Second Generation Oral Formulations & Prodrug

- Five formulations have been selected, out of which two candidates anticipated to be chosen for clinical and regulatory development.
- First candidate in clinic Q1 2022; second candidate in clinic by mid-2022.
- Sustained release formulation development to begin in 2022.
- Prodrug program continues to progress with candidate selection targeted for mid-2022.

Oral Ganaxolone Proposed Trade Name

ZTALMY® (Za-tal-mee)

- Proposed brand name has been established and trademarked, subject to final FDA approval.
- Branding concept designed to represent new hope for children and families experiencing seizures in CDD. The colors were chosen for their calming and supportive nature, while the open circles signify a community rallying around the child and family.

R&D Event Agenda

Time	Topic	Marinus Speaker
9:00 am - 9:05 am	Welcome & Introduction	Sasha Damouni Ellis VP, Corp Affairs & IR
9:05 am - 9:25 am	Marinus Overview	Dr. Scott Braunstein Chief Executive Officer
9:25 am - 9:30 am	Status Epilepticus Patient Video	
9:30 am - 9:50 am	Clinical Update: IV Franchise	Dr. Joseph Hulihan Chief Medical Officer
9:50 am - 10:15 am	Clinical Update: Oral Franchise	Dr. Alex Aimetti VP, Scientific Affairs
10:15 am - 10:35 am	Unmet Need	Dr. Ian Miller VP, Clinical Development
10:35 am - 10:55 am	Second Generation Product Development	Dr. Mark Paternoster SVP, Development
10:55 am – 11:00 am	Break	
11:00 am – 11:10 am	Financial Overview	Steven Pfanstiel Chief Financial Officer
11:10 am - 11:30 am	Commercial Strategy	Christy Shafer Chief Commercial Officer
11:30 am - 12:00 pm	Q&A and Concluding Remarks	

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 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. 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”, “target”, “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; our expected clinical development plans, enrollment in our clinical trials, trial design, and regulatory communications and submissions for ganaxolone, and the timing thereof; our expectations and beliefs regarding the FDA and the EMA 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; and our expectations regarding our next generation product candidates. 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 our marketing authorization application (MAA) is not validated by European Medicines Agency (EMA) by the end of October; the risk that the timing of the FDA’s review of the CDD NDA or the EMA’s review of the CDD MAA will be delayed; the potential that the FDA or the EMA may not grant or may delay approval for our product

candidate; uncertainties regarding our interactions with FDA and EMA, including reviews and inspections, the timing related thereto and the outcome thereof; 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 regulatory agencies 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 successfully develop next generation formulations or prodrugs of ganaxolone; 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 our product candidates, and our ability to service those markets; our cash and cash equivalents may not be sufficient to support our operating plan for as long as anticipated; our expectations, projections and estimates regarding expenses, future revenue, capital requirements, and the availability of and the need for additional financing; our ability to obtain additional funding to support our clinical development programs; our ability to develop sales and marketing capabilities, whether alone or with potential future collaborators; the rate and degree of market acceptance of our product candidates; 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, led 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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