Axsome Therapeutics Announces AXS-12 for the Treatment of Narcolepsy

October 16, 2018

Narcolepsy is a debilitating, orphan neurological condition with limited treatment options

Phase 2 trial set to start in 4Q 2018 with results anticipated in 1H 2019

Conference call and webcast scheduled for today at 8:00 AM Eastern Time

NEW YORK, Oct. 16, 2018 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ: AXSM), a clinical-stage biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, today announced its next CNS product candidate, AXS-12 (reboxetine), which the Company is developing for the treatment of narcolepsy. Narcolepsy is a serious, debilitating, orphan neurological condition characterized by excessive daytime sleepiness and cataplexy. Cataplexy is a sudden reduction or loss of muscle tone while a patient is awake, which can affect specific parts of the body or the entire body. AXS-12 is a highly selective and potent norepinephrine reuptake inhibitor that has the potential to address the key symptoms of narcolepsy. Initiation of a Phase 2 trial of AXS-12 in patients with narcolepsy is planned for the fourth quarter of 2018, with topline results anticipated in the first half of 2019.

“AXS-12 is another example of Axsome’s ability to internally generate novel CNS product candidates that have the potential to improve the lives of patients and create significant value for shareholders. AXS-12 broadens our already strong late-stage CNS pipeline which includes AXS-05 for depression, Alzheimer’s disease agitation and smoking cessation, and AXS-07 for migraine,” said Herriot Tabuteau, M.D., Chief Executive Officer of Axsome. “We look forward to upcoming clinical trial results for AXS-05 and the Phase 3 trial initiation for AXS-07. AXS-12 adds another significant near-term clinical milestone with the anticipated top-line results from the planned Phase 2 trial in narcolepsy in the first half of 2019. Importantly, our recently completed financing extends our cash runway into the first quarter of 2020, which is beyond anticipated results from clinical trials with AXS-05, AXS-07 and AXS-12.”

“The debilitating effects of narcolepsy are far reaching for the estimated nearly 200,000 patients living with this disorder in the United States. Narcolepsy interferes with mental and social functioning, increases work and driving related accidents, and results in a nearly two-fold higher mortality rate,” said Dr. Michael J. Thorpy, Professor of Neurology at Albert Einstein College of Medicine. “Unfortunately, currently approved treatments are few for this under-diagnosed orphan condition, and are limited by variability in efficacy from patient to patient, tolerability issues and the need for DEA scheduling. If borne out in clinical trials, the potential of AXS-12 to treat the symptoms of narcolepsy along with its lack of DEA scheduling would represent a significant benefit to patients living with this condition.”

The potential utility of AXS-12 in narcolepsy is supported by positive pre-clinical and preliminary clinical results in narcolepsy, and an extensive positive clinical safety record. Reboxetine, the active agent in AXS-12, significantly and dose-dependently reduced narcoleptic episodes in hypocretin (orexin)-deficient mice, a well-established genetic animal model of narcolepsy.[1] In an open-label pilot trial, reboxetine treatment improved excessive daytime sleepiness and reduced cataplexy in patients with narcolepsy.[2] Reboxetine has an extensive safety record in Europe and in over 40 countries where it is approved for the treatment of depression. Depression is reported in up to 57% of narcolepsy patients.[3] Reboxetine has not been approved in the U.S. for any indication.

Conference Call and Webcast

Axsome will host a conference call and webcast with slides, today, October 16, 2018, at 8:00 AM Eastern with Company management and key opinion leader (KOL) Dr. Michael Thorpy to discuss AXS-12 and unmet needs in the treatment of narcolepsy. To participate in the live conference call, please dial (844) 698-4029 (toll-free domestic) or (647) 253-8660 (international), and use the passcode 2427487. A live and archived webcast of the call, with slides, can be accessed on the "Webcasts & Presentations" page of the "Investors" section of the Company's website at axsome.com. The agenda for the call and Dr. Thorpy’s credentials are being provided in a separate related press release.

About Narcolepsy

Narcolepsy is a serious and debilitating neurological condition that causes dysregulation of the sleep-wake cycle and is characterized clinically by excessive daytime sleepiness, cataplexy, hypnagogic hallucinations, sleep paralysis, and disrupted nocturnal sleep. Narcolepsy afflicts an estimated 185,000 individuals in the U.S. Cataplexy is seen in an estimated 70% of narcolepsy patients and is a sudden reduction or loss of muscle tone while a patient is awake, typically triggered by strong emotions such as laughter, fear, anger, stress, or excitement. Narcolepsy interferes with cognitive, psychological, and social functioning, increases the risk of work- and driving-related accidents, and is associated with a 1.5 fold higher mortality rate. Depression is reported in up to 57% of patients.

About AXS-12

AXS-12 (reboxetine) is a novel, oral, investigational medicine in development for the treatment of the symptoms of narcolepsy. AXS-12 is a highly selective and potent norepinephrine reuptake inhibitor. AXS-12 is an investigational drug product not approved by the FDA.

About Axsome Therapeutics, Inc.

Axsome Therapeutics, Inc. is a clinical-stage biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders for which there are limited treatment options. Axsome's core CNS product candidate portfolio includes four clinical-stage candidates, AXS-05, AXS-07, AXS-09, and AXS-12. AXS-05 is currently in a Phase 3 trial in treatment resistant depression (TRD), a Phase 2/3 trial in agitation associated with Alzheimer's disease (AD), a Phase 2 trial in Major Depressive Disorder (MDD), and a Phase 2 trial in smoking cessation. AXS-07 is being developed for the acute treatment of migraine. AXS-12 is being developed for the treatment of the symptoms of narcolepsy. The Axsome Pain and Primary Care business unit (Axsome PPC) houses Axsome's pain and primary care assets, including AXS-02 and AX-06, and intellectual property which covers these and related product candidates and molecules being developed by Axsome and others. AXS-02 is being developed for osteoporosis, the pain of knee osteoarthritis, and chronic low back pain. AXS-06 is being developed for osteoarthritis and rheumatoid arthritis. AXS-02, AXS-05, AXS-06, AXS-07, AXS-09, and AXS-12 are investigational drug products not approved by the FDA. For more information, please visit the Company's website at axsome.com. The Company may occasionally disseminate material, nonpublic information on the company website.

Forward Looking Statements

Certain matters discussed in this press release are "forward-looking statements". The Company may, in some cases, use terms such as "predicts," "believes," "potential," "continue," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. In particular, the Company's statements regarding trends and potential future results are examples of such forward-looking statements. The forward-looking statements include risks and uncertainties, including, but not limited to, the success, timing and cost of the Company's ongoing clinical trials and anticipated clinical trials for its current product candidates, including statements regarding the timing of initiation, interim analyses and completion of the trials, futility analyses and receipt of interim results, which are not necessarily indicative of the final results of the Company's ongoing clinical trials; the Company's ability to fund additional clinical trials to continue the advancement of its product candidates; the timing of and the Company's ability to obtain and maintain U.S. Food and Drug Administration or other regulatory authority approval of, or other action with respect to, its product candidates; the Company's expected cash runway; the Company's ability to successfully defend its intellectual property or obtain the necessary licenses at a cost acceptable to the Company, if at all; the successful implementation of the Company's research and development programs and collaborations; the success of the Company's license agreements; the acceptance by the market of the Company's product candidates, if approved; and other factors, including general economic conditions and regulatory developments, not within the Company's control. The factors discussed herein could cause actual results and developments to be materially different from those expressed in or implied by such statements. The forward-looking statements are made only as of the date of this press release and the Company undertakes no obligation to publicly update such forward-looking statements to reflect subsequent events or circumstance.

References

1. Schmidt C, Leibiger J, Fendt M. The norepinephrine reuptake inhibitor reboxetine is more potent in treating murine narcoleptic episodes than the serotonin reuptake inhibitor escitalopram. Behav Brain Res. 2016 Jul 15;308:205-10.
2. Larrosa O, de la Llave Y, Bario S, Granizo JJ, Garcia-Borreguero D. Stimulant and anticataplectic effects of reboxetine in patients with narcolepsy: a pilot study. Sleep. 2001 May 1;24(3):282-5.
3. Daniels E., King M.A., Smith I.E., Shneerson J.M. Health-related quality of life in narcolepsy. J. Sleep Res. 2001;10:75–81.

Axsome Contact:
Mark Jacobson
Senior Vice President, Operations
Axsome Therapeutics, Inc.
25 Broadway, 9th Floor
New York, NY 10004
Tel: 212-332-3243
Email: mjacobson@axsome.com
www.axsome.com

Source: Axsome Therapeutics, Inc.