Axsome Therapeutics Initiates SYMPHONY Phase 3 Trial of AXS-12 in Narcolepsy

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NEW YORK, Sept. 16, 2021 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ: AXSM), a biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, enrolled the first patient in SYMPHONY (Study Evaluating a Mechanistic Approach to Treating Narcolepsy), a Phase 3, randomized, double-blind, placebo-controlled trial of AXS-12 in patients with narcolepsy. AXS-12 is a novel, oral, potent, and highly selective norepinephrine reuptake inhibitor. Topline results from the SYMPHONY trial are anticipated in the first half of 2023.

"The advancement of AXS-12 into Phase 3 testing for narcolepsy demonstrates Axsome's commitment to developing important new medicines for patients living with serious CNS conditions," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "Narcolepsy impairs almost every aspect of a patient's life including cognitive, psychological, social, and emotional functioning. If successfully developed, AXS-12 may address multiple symptoms of this debilitating condition."

AXS-12 has been granted Orphan Drug Designation by the U.S. Food and Drug Administration for the treatment of narcolepsy.

About the SYMPHONY Trial

SYMPHONY (Study Evaluating a Mechanistic Approach to Treating Narcolepsy) is a Phase 3, randomized, double-blind, multicenter, placebo-controlled trial to assess the efficacy and safety of AXS-12 in patients with narcolepsy. Approximately 90 patients will be randomized in a 1:1 ratio to treatment with AXS-12 or placebo for 5 weeks. The primary endpoint will be the frequency of cataplexy attacks. Other symptoms of narcolepsy as well as safety will be assessed throughout the study.

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.

About AXS-12

AXS-12 (reboxetine) is a highly selective and potent norepinephrine reuptake inhibitor under development for the treatment of narcolepsy. AXS-12 modulates noradrenergic activity to promote wakefulness, maintain muscle tone and enhance cognition. AXS-12 has been granted U.S. Food and Drug Administration (FDA) Orphan Drug Designation for the treatment of narcolepsy. AXS-12 is an investigational drug product not approved by the FDA.

About Axsome Therapeutics, Inc.

Axsome Therapeutics, Inc. is a biopharmaceutical company developing novel therapies for central nervous system (CNS) conditions that have limited treatment options. Through development of therapeutic options with novel mechanisms of action, we are transforming the approach to treating CNS conditions. At Axsome, we are intensely committed to developing products that meaningfully improve the lives of patients and provide additional therapeutic options for physicians. 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". We 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 our ongoing clinical trials and anticipated clinical trials for our current product candidates, including statements regarding the timing of initiation, pace of enrollment and completion of the trials (including our ability to fully fund our disclosed clinical trials, which assumes no material changes to our currently projected expenses), futility analyses and receipt of interim results, which are not necessarily indicative of the final results of our ongoing clinical trials, and the number or type of studies or nature of results necessary to support the filing of a new drug application ("NDA") for any of our current product candidates; our ability to fund additional clinical trials to continue the advancement of our product candidates; the timing of and our ability to obtain and maintain U.S. Food and Drug Administration ("FDA") or other regulatory authority approval of, or other action with respect to, our product candidates (including, but not limited to, whether potential filing issues or issues identified by FDA during the substantive review may impact the potential approvability of the Company's NDA submission for AXS-05 in MDD

or the timing of such approval, and whether the FDA will agree with the Company's discontinuation of the bupropion treatment arm of the ADVANCE study in accordance with the independent data monitoring committee's recommendations); whether issues identified by FDA during the substantive review may impact the potential approvability of the Company's NDA for AXS-07 for the acute treatment of migraine in adults with or without aura, pursuant to our special protocol assessment for the MOMENTUM clinical trial; the potential for the ASCEND clinical trial, combined with the GEMINI clinical trial results, to provide a basis for approval of AXS-05 for the treatment of major depressive disorder and accelerate its development timeline and commercial path to patients; 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; the Company's anticipated capital requirements, including the amount of capital required for the Company's commercial launch of its product candidates, and the potential impact on the Company's anticipated cash runway; unforeseen circumstances or other disruptions to normal business operations arising from or related to COVID-19; 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.

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