Axsome Therapeutics Receives FDA Breakthrough Therapy Designation for AXS-12 for the Treatment of Narcolepsy

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Designation offers potential for expedited development and review

FDA Orphan Drug Designation previously granted to Axsome for AXS-12 in narcolepsy

Third FDA Breakthrough Therapy designation received by Axsome

NEW YORK, Aug. 05, 2020 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ: AXSM), a biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for AXS-12 for the treatment of cataplexy in patients with narcolepsy. AXS-12 is a novel, oral, highly selective and potent norepinephrine reuptake inhibitor. Axsome previously received Orphan Drug Designation from the FDA for AXS-12 for the treatment of narcolepsy. Narcolepsy is a debilitating, neurological condition characterized by excessive daytime sleepiness (EDS) and cataplexy, a sudden loss of muscle tone triggered by strong emotions.

A Breakthrough Therapy designation is granted to potentially expedite development and review timelines for a promising investigational medicine when preliminary clinical evidence indicates it may demonstrate substantial improvement on one or more clinically significant endpoints over available therapies for a serious or life-threatening condition. The Breakthrough Therapy designation for AXS-12 for the treatment of cataplexy in narcolepsy was supported by the positive results from the Phase 2 CONCERT study, a randomized, double-blind, placebo-controlled, crossover, multicenter U.S. trial. In the trial, 21 patients with a diagnosis of narcolepsy with cataplexy were treated for 2 weeks with AXS-12 or with placebo, followed by a crossover to the other treatment after a 1-week down-titration and washout period. AXS-12 met the primary endpoint demonstrating a highly statistically significant reduction from baseline in the mean weekly number of cataplexy attacks, averaged for the 2-week treatment period (overall treatment effect), as compared to placebo ($p<0.001$), and at the end of the 2-week treatment period ($p=0.002$). AXS-12 also significantly improved EDS compared to placebo, as measured by the Epworth Sleepiness Scale (ESS) and by the frequency of inadvertent naps ($p=0.003$ and $p<0.001$, respectively). In addition, AXS-12 significantly improved cognitive function compared to placebo over the 2-week treatment period as measured by the Ability to Concentrate item of the Narcolepsy Symptom Assessment Questionnaire ($p<0.001$). AXS-12 was well tolerated in this trial with the most commonly reported adverse events being anxiety, constipation, and insomnia.

“This FDA Breakthrough Therapy designation for AXS-12 for cataplexy in narcolepsy highlights its potential, should it be successfully developed, to provide meaningful benefit and substantial improvement over currently available treatment options for patients living with this debilitating condition. We are excited by the burgeoning profile of AXS-12 which has also received Orphan Drug designation for the treatment of narcolepsy,” said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. “Axsome has now received three FDA Breakthrough Therapy designations, including designations for AXS-05 in major depressive disorder and for AXS-05 in Alzheimer’s disease agitation. These designations exemplify our commitment to developing potentially life-changing medicines for patients with difficult-to-treat CNS conditions, our innovative approach to clinical development, and our resulting differentiated and broad late-stage CNS pipeline. We look forward to meeting with the FDA as soon as possible to discuss the continued development of AXS-12 in light of this significant milestone.”

About FDA Breakthrough Therapy Designation

Breakthrough Therapy designation is granted by the FDA in order to expedite the development and review of drugs for serious or life-threatening conditions. In order to receive Breakthrough Therapy designation, a drug must demonstrate preliminary clinical evidence that the drug may have substantial improvement on at least one clinically significant endpoint over available therapy. Breakthrough Therapy designation provides an organizational commitment involving senior managers from the FDA, more intensive FDA guidance on an efficient drug development program, and greater access to and more frequent communication with the FDA throughout the entire drug development and review process. It also provides the opportunity to submit sections of a New Drug Application (NDA) on a rolling basis, where the FDA may review portions of the NDA as they are received instead of waiting for the entire NDA submission. In addition, Breakthrough Therapy designated products are eligible for Priority Review, where the FDA has a goal to take action on an application within six months, as opposed to ten months under standard review. Breakthrough Therapy designation does not change the standards for approval.

About Narcolepsy

Narcolepsy can be 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 highly selective and potent norepinephrine reuptake inhibitor 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) Breakthrough Therapy designation and 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 the management of central nervous system (CNS) disorders for which there are limited treatment options. For the many people facing unsatisfactory treatments for CNS disorders, Axsome accelerates the invention and adoption of life-changing medicines. Axsome's core CNS product candidate portfolio includes five clinical-stage candidates, AXS-05, AXS-07, AXS-09, AXS-12, and AXS-14. AXS-05 is being developed for major depressive disorder (MDD), treatment resistant depression (TRD), Alzheimer's disease (AD) agitation, and as treatment for smoking cessation. AXS-07 is being developed for the acute treatment of migraine. AXS-12 is being developed for the treatment of narcolepsy. AXS-14 is being developed for fibromyalgia. AXS-05, AXS-07, AXS-09, AXS-12, and AXS-14 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". 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, FDA's agreement with the Company's discontinuation of the bupropion treatment arm of the ADVANCE-1 study in accordance with the independent data monitoring committee's recommendations); the potential for the MOMENTUM clinical trial to provide a basis for approval of AXS-07 for the acute treatment of migraine in adults with or without aura, pursuant to our special protocol assessment; 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 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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