Axsome Therapeutics Announces FDA Pre-NDA Meeting Minutes for AXS-05 in Alzheimer's Disease Agitation Supporting NDA Submission

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Supplemental New Drug Application (sNDA) submission anticipated in 3Q 2025

NEW YORK, March 03, 2025 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ: AXSM), a biopharmaceutical company leading a new era in the treatment of central nervous system (CNS) disorders, today announced that it has received formal pre-New Drug Application (NDA) meeting minutes from the U.S. Food and Drug Administration (FDA) supporting a supplemental NDA (sNDA) submission for AXS-05 in Alzheimer's disease agitation. AXS-05 (dextromethorphan-bupropion) is a novel, oral, investigational NMDA receptor antagonist, sigma-1 agonist, and aminoketone CYP2D6 inhibitor. The purpose of the meeting was to reach agreement with the FDA on the proposed content and format of the Company's planned sNDA submission including the clinical and nonclinical requirements.

Based on the feedback from the FDA, the Company's regulatory data package would be sufficient for the submission of an sNDA for AXS-05 for the treatment of Alzheimer's disease agitation. Axsome anticipates submitting the planned sNDA in the third quarter of 2025. Acceptance of the final sNDA will be subject to the FDA's review of the complete filing. AXS-05 was granted Breakthrough Therapy designation for the treatment of Alzheimer's disease agitation in June 2020.

"We are pleased with the pre-NDA meeting feedback we received from the FDA, which confirmed the studies and data to be presented in our planned sNDA submission of AXS-05 for the treatment of Alzheimer's disease agitation," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "Alzheimer's disease agitation is a serious, distressing, and prevalent condition with limited treatment options. If approved, AXS-05 would represent a new mechanism of action for the treatment of Alzheimer's disease agitation with the potential to address this high unmet medical need. As an FDA Breakthrough Therapy designated program, the application for AXS-05 will be eligible for Priority Review. We look to complete the sNDA submission in the third quarter of 2025, with the goal of making this important new potential therapy available to patients living with Alzheimer's disease as quickly as possible."

The comprehensive clinical development program of AXS-05 in Alzheimer's disease agitation includes four completed pivotal, Phase 3, placebo-controlled efficacy trials, and a completed long-term safety trial. AXS-05 demonstrated statistically significant improvement in Alzheimer's disease agitation, assessed using the Cohen-Mansfield Agitation Inventory (CMAI), compared to placebo, in three Phase 3 efficacy trials: the ADVANCE-1 parallel-group trial (p=0.010), the ACCORD-1 randomized withdrawal trial (p=0.014), and the ACCORD-2 randomized withdrawal trial (p=0.001). Additional controlled safety and supportive efficacy results are provided by a fourth trial (ADVANCE-2). The long-term safety and tolerability of AXS-05 was also evaluated in more than 300 patients treated for at least 6 months and more than 100 patients treated for at least 12 months. AXS-05 was well tolerated with long-term dosing. There were no deaths in patients receiving AXS-05 and AXS-05 was not associated with increased risk of falls, sedation, or cognitive decline as assessed by the Mini-Mental State Examination (MMSE).

About Alzheimer's Disease Agitation

Alzheimer's disease (AD) is the most common form of dementia, affecting approximately 7 million people in the United States.[1] Agitation is reported in up to 70% of patients with AD and is characterized by emotional distress, verbal and physical aggressiveness, disruptive irritability, and disinhibition.[1,2] AD agitation has been associated with accelerated cognitive decline, increased caregiver burden, earlier nursing home placement, and increased mortality.[3]

About AXS-05

AXS-05 (dextromethorphan-bupropion) is a novel, oral, investigational N-methyl-D-aspartate (NMDA) receptor antagonist, sigma-1 agonist, and aminoketone CYP2D6 inhibitor under development for the treatment of Alzheimer's disease (AD) agitation and smoking cessation. AXS-05 utilizes a proprietary formulation and dose of dextromethorphan and bupropion, and Axsome's metabolic inhibition technology, to modulate the delivery of the components. The dextromethorphan component of AXS-05 is an uncompetitive NMDA receptor antagonist, also known as a glutamate receptor modulator, and a sigma-1 receptor agonist. The bupropion component of AXS-05 serves to increase the bioavailability of dextromethorphan and is a norepinephrine and dopamine reuptake inhibitor. AXS-05 is covered by a robust patent estate extending out to at least 2043. AXS-05 was granted U.S. FDA Breakthrough Therapy designation for the treatment of Alzheimer's disease agitation in June 2020.

About Axsome Therapeutics

Axsome Therapeutics is a biopharmaceutical company leading a new era in the treatment of central nervous system (CNS) conditions. We deliver scientific breakthroughs by identifying critical gaps in care and develop differentiated products with a focus on novel mechanisms of action that enable meaningful advancements in patient outcomes. Our industry-leading neuroscience portfolio includes FDA-approved treatments for major depressive disorder, excessive daytime sleepiness associated with narcolepsy and obstructive sleep apnea, and migraine, and multiple late-stage development programs addressing a broad range of serious neurological and psychiatric conditions that impact over 150 million people in the United States.

Together, we are on a mission to solve some of the brain's biggest problems so patients and their loved ones can flourish. For more information, please visit the Company's website at www.axsome.com.

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 commercial success of the Company's Sunosi®, Auvelity®, and Symbravo® products and the success of the Company's efforts to obtain any additional indication(s) with respect to solriamfetol and/or AXS-05; the Company's ability to maintain and expand payer coverage; the success, timing and cost of the Company's ongoing clinical trials and anticipated clinical trials for the Company's current product candidates, including statements regarding the timing of initiation, pace of enrollment and completion of the trials (including the Company's ability to fully fund the Company's disclosed clinical trials, which assumes no material changes to the Company's currently projected revenues or expenses), futility analyses and receipt of interim results, which are not necessarily indicative of the final results of the Company's ongoing clinical trials, and/or data readouts, 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 the Company's current product candidates; the Company's ability to fund additional clinical trials to continue the advancement of the Company's product candidates; the timing of and the Company's ability to obtain and maintain U.S. Food and Drug Administration ("FDA") or other regulatory authority approval of, or other action with respect to, the Company's product candidates, including statements regarding the timing of any NDA submission; 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 products and product candidates, if approved; the Company's anticipated capital requirements, including the amount of capital required for the commercialization of Sunosi, Auvelity, and Symbravo and for the Company's commercial launch of its other product candidates, if approved, and the potential impact on the Company's anticipated cash runway; the Company's ability to convert sales to recognized revenue and maintain a favorable gross to net sales; unforeseen circumstances or other disruptions to normal business operations arising from or related to domestic political climate, geo-political conflicts or a global pandemic 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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Source: Axsome Therapeutics, Inc.