Axsome Therapeutics Initiates Phase 2/3 Trial of AXS-05 for Alzheimer's Disease Agitation

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First patient enrolled in the ADVANCE-1 study

Agitation reported in nearly 50% of patients with Alzheimer's disease

FDA Fast Track designation previously received for AXS-05 for Alzheimer's disease agitation

Second indication for AXS-05 in late-stage clinical trials

NEW YORK, July 17, 2017 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ:AXSM), a clinical-stage biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, enrolled the first patient in the ADVANCE-1 (Addressing Dementia Via Agitation-Centered Evaluation 1) study, a Phase 2/3 trial evaluating the efficacy and safety of AXS-05 for the treatment of Alzheimer's disease (AD) agitation. AXS-05 is a combination of dextromethorphan (an NMDA receptor antagonist, sigma-1 receptor agonist, and serotonin and norepinephrine reuptake inhibitor) and bupropion (a norepinephrine and dopamine reuptake inhibitor, which also increases the bioavailability of dextromethorphan).

"Agitation is one of the most distressing and difficult-to-treat behavioral symptoms in patients with Alzheimer's disease," said Jeffrey Cummings, M.D., Sc.D., Professor of Neurology, and Director of the Center for Neurodegeneration and Translational Neuroscience, at the Cleveland Clinic Lerner College of Medicine. "It is common, being reported in about half of patients, is one of the primary reasons for early nursing home placement, and is associated with increased mortality. NMDA receptor antagonism, sigma-1 receptor agonism, and serotonin and norepinephrine reuptake inhibition may be relevant mechanisms in this condition. New treatments are needed for agitation and progress in this area is a welcome advance."

"Agitation places a heavy burden on patients with Alzheimer's disease and their caregivers," said Marc Agronin, M.D., Vice President of Behavioral Health and Clinical Research at Miami Jewish Health, and Affiliate Associate Professor of Psychiatry and Neurology at University of Miami Miller School of Medicine. "Unfortunately there is currently no FDA-approved medication for this condition. The mechanisms of action of AXS-05 may hold promise in treating Alzheimer's agitation. We look forward to learning more about AXS-05 and its potential to relieve the symptoms of agitation through the ADVANCE-1 trial."

"The initiation of the ADVANCE-1 trial reflects Axsome's continued commitment to developing treatments for serious CNS disorders for which there are limited treatment options," said Herriot Tabuteau, M.D., Chief Executive Officer of Axsome. "The unique pharmacology of AXS-05 lends itself to the potential treatment of a variety of CNS disorders. With the STRIDE-1 trial in treatment resistant depression also underway, AXS-05 is now being evaluated in late-stage clinical trials in two separate CNS indications."

About the ADVANCE-1 Study

ADVANCE-1 (Addressing Dementia Via Agitation-Centered Evaluation 1) is a Phase 2/3 multicenter, randomized, double-blind, controlled trial to evaluate the efficacy and safety of AXS-05 in patients with agitation associated with Alzheimer's disease. Approximately 435 patients will be randomized in a 1:1:1 ratio to receive AXS-05, bupropion, or placebo for 5 weeks. The primary efficacy measure is the Cohen-Mansfield Agitation Inventory (CMAI). This trial incorporates a planned interim analysis by an independent data monitoring committee to assess the assumptions used to determine the sample size of the trial.

About Alzheimer's Disease (AD) Agitation

Alzheimer's disease (AD) is a progressive neurodegenerative disorder that manifests initially as forgetfulness advancing to severe cognitive impairment and memory loss. It afflicts an estimated 5 million individuals in the United States, a number that is anticipated to increase to approximately 14 million by 2050. In addition to cognitive decline, individuals diagnosed with AD typically experience behavioral and psychological symptoms including agitation which is reported in approximately 45% of patients. Agitation is characterized by emotional distress, aggressive behaviors, disruptive irritability, and disinhibition. Agitation in patients with AD has been associated with increased caregiver burden, decreased functioning, earlier nursing home placement, and increased mortality. There are currently no therapies approved by the FDA for the treatment of agitation in patients with AD.

About AXS-05

AXS-05 is a novel, oral, investigational drug product under development for the treatment of central nervous system (CNS) disorders. AXS-05 utilizes Axsome's technology of combining bupropion and dextromethorphan. Dextromethorphan is an NMDA receptor antagonist, sigma-1 receptor agonist, and inhibitor of the serotonin and norepinephrine transporters. Bupropion serves to increase the bioavailability of dextromethorphan, and is a norepinephrine and dopamine reuptake inhibitor, and a nicotinic acetylcholine receptor antagonist. AXS-05 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 product candidate portfolio includes two late-stage candidates, AXS-05 and AXS-02. AXS-05 is currently in a Phase 3 trial in treatment resistant depression (TRD) and a Phase 2/3 trial in agitation in patients with Alzheimer's disease (AD). AXS-02 is currently in Phase 3 trials in complex regional pain syndrome (CRPS) and knee osteoarthritis (OA) associated with bone marrow lesions (BMLs) with an additional Phase 3 trial planned in chronic low back pain (CLBP) associated with Modic changes (MCs). AXS-05 and AXS-02 are investigational drug products not approved by the FDA. For more information, please visit the company website at www.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, interim analyses and completion of the trials; the timing of and our ability to obtain and maintain U.S. Food and Drug Administration or other regulatory authority approval of, or other action with respect to, our product candidates; 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.

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