

Axsome Therapeutics Receives FDA Clearance of IND for Phase 2/3 Trial of AXS-05 in Alzheimer's Disease Agitation

January 3, 2017 7:00 AM ET

NEW YORK, Jan. 03, 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, today announced that its Investigational New Drug Application (IND) for AXS-05 in the treatment of agitation in patients with Alzheimer's disease (AD) has been cleared by the U.S. Food and Drug Administration (FDA). The IND clearance permits Axsome to proceed with its planned Phase 2/3 clinical trial of AXS-05 in this indication. Axsome anticipates commencing this trial in the first half of 2017.

"Agitation, including aggression, is seen in a significant percentage of patients with Alzheimer's disease, leads to early nursing home placement, accelerates cognitive decline, and increases the risk of death," said Herriot Tabuteau, M.D., Chief Executive Officer of Axsome Therapeutics. "We look forward to examining the potential of AXS-05 to treat this distressing condition for which there is currently no approved treatment. The successful filing and FDA clearance of this IND is a key milestone for Axsome and marks the second open IND for AXS-05."

The planned Phase 2/3 trial is a multicenter, randomized, double-blind, placebo-controlled study to examine the efficacy and safety of AXS-05 in AD patients with agitation. Approximately 330 subjects will be randomly assigned to treatment with AXS-05, placebo, or bupropion, which is one of the components of AXS-05. The primary endpoint of the trial will be assessed using the Cohen-Mansfield Agitation Inventory.

About Agitation in Alzheimer's Disease

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 as many as 40% 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) is planned. 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 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.

Axsome Contact:

Mark Jacobson
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

Trout Group Contact:

Marcy Beth Nanus
Senior Vice President
The Trout Group LLC
Tel: 646-378-2927
Email: mnanus@troutgroup.com



Axsome Therapeutics, Inc.