Axsome Therapeutics Receives Pre-IND Guidance from FDA for AXS-05 for Agitation in Patients with Alzheimer's Disease

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Phase 2/3 Trial Planned in Agitation in Alzheimer's Disease

IND Submission Expected in 2016

NEW YORK, April 20, 2016 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ:AXSM), a clinical-stage biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, received, from the U.S. Food and Drug Administration (FDA), Pre-Investigational New Drug Application (Pre-IND) written guidance on Axsome's proposed clinical developmental plan for AXS-05 in the treatment of agitation in patients with Alzheimer's disease (AD).

The FDA's feedback reflects specific guidance and general agreement on Axsome's regulatory approach, including the design of a proposed Phase 2/3 trial. Based on this feedback, Axsome plans to file an IND by the end of 2016 for the conduct of a Phase 2/3 trial of AXS-05 in the treatment of agitation in patients with AD.

Agitation is seen in a significant percentage of patients with AD and is associated with serious consequences including decreased functioning, institutionalization, 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 an innovative oral therapeutic, consisting of bupropion and dextromethorphan, which is being developed for the treatment of central nervous system (CNS) disorders. Dextromethorphan is an NMDA receptor antagonist, sigma-1 receptor agonist, and inhibitor of the serotonin and norepinephrine transporters. Bupropion is a norepinephrine and dopamine reuptake inhibitor, and nicotinic acetylcholine receptor antagonist. In addition to its CNS activity, bupropion also serves to increase the bioavailability of dextromethorphan. A Phase 3 trial is underway with AXS-05 in treatment resistant depression (TRD). AXS-05 is an investigational product candidate not approved by the FDA.

About Agitation in Patients with 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 is a common form of dementia and 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 and aggression. These symptoms are seen in a high percentage of AD sufferers with agitation being 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 death.

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, including pain, for which there are limited existing treatment options. Axsome's product candidate portfolio includes two late-stage candidates, AXS-02 and AXS-05. 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 is currently in a Phase 3 trial in treatment resistant depression (TRD). A Phase 2/3 trial in agitation in patients with Alzheimer's disease (AD) is planned. AXS-02 and AXS-05 are investigational product candidates 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.

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