Axsome Therapeutics Announces Positive Outcome of Interim Analysis of STRIDE-1 Phase 3 Trial of AXS-05 in Treatment Resistant Depression

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Independent Data Monitoring Committee recommends trial continuation

Second interim analysis anticipated second half of 2018 for efficacy

NEW YORK, April 26, 2018 (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 a positive outcome of the interim futility analysis for the STRIDE-1 Phase 3 trial of AXS-05 in treatment resistant depression. An independent data monitoring committee (IDMC) conducted the unblinded, pre-specified interim analysis. Based on the results of the analysis, the IDMC recommended that the trial continue. The IDMC also reviewed the available safety information from the study and indicated that, based on the interim results, AXS-05 appeared safe and well-tolerated.

AXS-05 is a novel, oral, fixed-dose combination of dextromethorphan and bupropion. AXS-05 combines glutamatergic and monoaminergic mechanisms of action, which have been associated with antidepressant effects, and Axsome's metabolic inhibition technology. Pharmacokinetic data with AXS-05 and clinical observations with the dextromethorphan component indicate that AXS-05 increases dextromethorphan concentrations into a potentially therapeutic range. AXS-05 has been granted U.S. Food and Drug Administration (FDA) Fast Track designation for the treatment of treatment resistant depression.

"The positive outcome of this interim futility analysis combined with the multiple mechanisms of action of AXS-05 support its continued development for treatment resistant depression," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "We look forward to the next and final interim analysis of the STRIDE-1 trial, anticipated in the second half of this year, which will be conducted to assess efficacy."

The STRIDE-1 interim futility analysis was performed on the first approximately 40% of the target number of subjects. A second interim analysis will be performed on the first approximately 60% of the target number of subjects to assess efficacy.

"The IDMC's recommendation for continuation of the STRIDE-1 trial and its findings of an overall favorable clinical safety profile for AXS-05 are encouraging," said Cedric O'Gorman, MD, Senior Vice President of Clinical Development and Medical Affairs of Axsome. "A significant proportion of patients with major depressive disorder are treatment resistant, having previously failed two or more therapies. There are limited available treatment options for these patients. AXS-05's multiple mechanisms, targeting glutamatergic, monoaminergic and anti-inflammatory pathways, may offer a unique therapeutic approach for this serious condition."

The Company recently held a research and development (R&D) day focusing on AXS-05. Of relevance to treatment resistant depression were presentations by key opinion leaders Stephen M. Stahl, MD, PhD, DSc (Adjunct Professor of Psychiatry, University of California San Diego), and Maurizio Fava, MD (Executive Vice Chair of the Department of Psychiatry, Massachusetts General Hospital). Dr. Stahl discussed the psychopharmacology of AXS-05 and its potential clinical implications. Dr. Fava discussed approaches that target multiple mechanisms of action to address treatment resistant depression, and the potential utility of AXS-05 for this condition. An archived webcast of this event, with slides, can be accessed on the investor page of Axsome's website at www.axsome.com.

The R&D day also featured presentations from key opinion leaders Marc Agronin, MD (Vice President of Behavioral Health and Clinical Research at Miami Jewish Health), who discussed the potential of AXS-05 for the treatment of agitation associated with Alzheimer's disease, and James Davis, MD (Medical Director of the Duke Center for Smoking Cessation, Duke University School of Medicine), who discussed unmet needs in smoking cessation and the potential for AXS-05.

About the STRIDE-1 Study

STRIDE-1 (Symptom Treatment in Resistant Depression 1) is a Phase 3, randomized, double-blind, active controlled trial to assess the efficacy and safety of AXS-05 in the treatment of treatment resistant depression (TRD). Patients with major depressive disorder (MDD) who have previously failed one or two antidepressant treatments are treated in an open-label fashion with bupropion during a 6-week lead-in period. Patients who fail to respond to bupropion during this lead-in period are randomly assigned in a 1:1 ratio to receive bupropion or AXS-05 in a double-blind fashion for 6 weeks. The primary endpoint is the change in the Montgomery-Åsberg Depression Rating Scale (MADRS) after 6 weeks of treatment.

About Treatment Resistant Depression (TRD)

Patients diagnosed with major depressive disorder (MDD) are defined as having TRD if they have failed two or more antidepressant therapies. MDD is a serious condition characterized by depressed mood or a loss of interest or pleasure in daily activities consistently for at least a two-week period, and which impairs social, occupational, educational, or other important functioning. According to the National Institute of Health, an estimated 6.7% of U.S. adults experience MDD each year. Nearly two-thirds of diagnosed and treated patients do not experience adequate treatment response with first-line therapy, and the majority of these initial failures also fail second-line treatment.

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 consists of bupropion and dextromethorphan and utilizes Axsome's metabolic inhibition technology. Dextromethorphan is an NMDA receptor antagonist, sigma-1 receptor agonist, nicotinic acetylcholine receptor antagonist, 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. The safety and efficacy of AXS-05 have not yet been established.

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 five clinical-stage candidates, AXS-02, AXS-05, AXS-06, AXS-07, and AXS-09. AXS-05 is currently in a Phase 3 trial in treatment resistant depression (TRD), a Phase 2/3 trial in agitation associated with Alzheimer's disease (AD), and a Phase 2 trial in smoking cessation. AXS-02 is currently in a Phase 3 trial in 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-07 is being developed for the acute treatment of migraine. AXS-06 is being developed for the treatment of osteoarthritis and rheumatoid arthritis and for the reduction of the risk of NSAID-associated gastric ulcers. AXS-02, AXS-05, AXS-06, AXS-07, and AXS-09 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, futility analyses and receipt of interim results, which are not necessarily indicative of the final results of our ongoing clinical trials; 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 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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