



Axsome Therapeutics Initiates GEMINI Phase 3 Trial of AXS-05 in Major Depressive Disorder

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First patient enrolled in placebo-controlled GEMINI study

Topline results anticipated in 2H 2019

NEW YORK, June 24, 2019 (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 its GEMINI (Glutamatergic and Monoaminergic Modulation in Depression) study, a Phase 3, randomized, double-blind, placebo-controlled trial of AXS-05 in the treatment of major depressive disorder (MDD). AXS-05 is a novel, oral, investigational NMDA receptor antagonist with multimodal activity. Topline results from the GEMINI trial are anticipated in the second half of 2019.

"Initiation of the placebo-controlled GEMINI Phase 3 trial continues the expedited clinical development of AXS-05 in depression. If successfully developed, AXS-05 has the potential to be the first orally administered NMDA receptor antagonist approved for the treatment of this debilitating condition," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "Based on the results of our recent FDA Breakthrough Therapy meeting for AXS-05, we believe that either the GEMINI study in MDD or the ongoing STRIDE-1 trial in treatment resistant depression, if positive, would, in conjunction with our previously completed ASCEND trial in MDD, be sufficient to support the filing of an NDA for AXS-05 for the treatment of MDD. We continue to anticipate topline results from both the STRIDE-1 and GEMINI trials in the second half of 2019."

In March 2019, Axsome received Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) for AXS-05 for the treatment of MDD. A Breakthrough Therapy designation is granted to potentially expedite development and review timelines for a promising investigational medicine when preliminary clinical evidence indicates it may demonstrate substantial improvement on one or more clinically significant endpoints over available therapies for a serious or life-threatening condition. AXS-05 has also been granted FDA Fast Track designations for the treatment of TRD and for the treatment of agitation associated with Alzheimer's disease.

About the GEMINI Trial

GEMINI (Glutamatergic and Monoaminergic Modulation in Depression) is a Phase 3, randomized, double-blind, multicenter, placebo-controlled trial of AXS-05 in patients with major depressive disorder (MDD). Approximately 300 patients with a confirmed diagnosis of moderate to severe MDD will be randomized in a 1:1 ratio to treatment with AXS-05 or placebo for 6 weeks. Assessments including the Montgomery-Åsberg Depression Rating Scale (MADRS), safety parameters, clinician-rated scales, as well as patient-reported outcome measures will be conducted throughout the study.

About Major Depressive Disorder (MDD)

Major depressive disorder (MDD) is a debilitating, chronic, biologically-based disorder characterized by low mood, inability to feel pleasure, feelings of guilt and worthlessness, low energy, and other emotional and physical symptoms, and which impairs social, occupational, educational, or other important functioning. In severe cases, MDD can result in suicide. According to the National Institutes of Health, an estimated 7.1% of U.S. adults, or approximately 17.3 million, experience MDD each year¹. According to the World Health Organization (WHO), depression is the leading cause of disability worldwide, and is a major contributor to the overall global burden of disease². Nearly two-thirds of diagnosed and treated patients do not experience adequate treatment response with currently available first-line therapy³, highlighting the need for additional therapies with new mechanisms of action. The majority of patients who initially fail also fail second-line treatment. Patients diagnosed with MDD are defined as having treatment resistant depression (TRD) if they have failed to respond to two or more antidepressant therapies.

About AXS-05

AXS-05 is a novel, oral, patent-protected, investigational NMDA receptor antagonist with multimodal activity under development for the treatment of major depressive disorder and other central nervous system (CNS) disorders. AXS-05 consists of a proprietary formulation and dose of dextromethorphan and bupropion and utilizes Axsome's metabolic inhibition technology. The dextromethorphan component of AXS-05 is a non-competitive N-methyl-D-aspartate (NMDA) receptor antagonist, also known as a glutamate receptor modulator, which is a novel mechanism of action, meaning it works differently than currently approved therapies for major depressive disorder. The dextromethorphan component of AXS-05 is also a sigma-1 receptor agonist, nicotinic acetylcholine receptor antagonist, and inhibitor of the serotonin and norepinephrine transporters. The bupropion component of AXS-05 serves to increase the bioavailability of dextromethorphan, and is a norepinephrine and dopamine reuptake inhibitor, and a nicotinic acetylcholine receptor antagonist. AXS-05 is covered by more than 36 issued U.S. and international patents which provide protection out to 2034. AXS-05 is 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 core CNS product candidate portfolio includes four clinical-stage candidates, AXS-05, AXS-07, AXS-09, and AXS-12. AXS-05 is currently in a Phase 3 trial in treatment resistant depression (TRD), a Phase 3 trial in major depressive disorder (MDD), and a Phase 2/3 trial in agitation associated with Alzheimer's disease (AD). AXS-05 is also being developed for smoking cessation treatment. AXS-07 is currently in a Phase 3 trial for the acute treatment of migraine. AXS-12 is currently in a Phase 2 trial in narcolepsy. The Axsome Pain and Primary Care business unit (Axsome PPC) houses Axsome's pain and primary care assets, including AXS-02 and AXS-06, and intellectual property which covers these and related product candidates and molecules being developed by Axsome and others. AXS-02 is being developed for osteoporosis, the pain of knee osteoarthritis, and chronic low back pain. AXS-06 is being developed for osteoarthritis and rheumatoid arthritis. AXS-02, AXS-05, AXS-06, AXS-07, AXS-09, and AXS-12 are investigational drug products not approved by the FDA. For more information, please visit the Company's website at axsome.com. The Company may occasionally disseminate material, nonpublic information on the company website.

References

1. National Institute of Mental Health. (2017). Major Depression. Retrieved from <https://www.nimh.nih.gov/health/statistics/major-depression.shtml>.
2. World Health Organization. Fact Sheets: Depression.
3. Rush AJ, et al. (2007) Am J. Psychiatry 163:11, pp. 1905-1917 (STAR*D Study).

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, pace of enrollment 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, 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 our current product candidates; 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 (“FDA”) or other regulatory authority approval of, or other action with respect to, our product candidates (including, but not limited to, FDA’s agreement with the Company’s plan to discontinue the bupropion treatment arm of the ADVANCE-1 study in accordance with the independent data monitoring committee’s recommendations); the potential for the ASCEND clinical trial to provide a basis for approval of AXS-05 for the treatment of major depressive disorder and accelerate its development timeline and commercial path to patients; 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; the Company’s anticipated capital requirements; 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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