Axsome Therapeutics Provides Year End 2018 Clinical Update

December 27, 2018

FDA clearance of IND received for Phase 2 trial of AXS-12 in narcolepsy

Phase 2 results of AXS-05 in major depressive disorder on track for early January 2019

Phase 3 results of AXS-05 in treatment resistant depression on track for 1Q 2019

Phase 3 trial of AXS-07 in acute migraine on track to start in 1Q 2019

NEW YORK, Dec. 27, 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 provided the following clinical update for its robust CNS pipeline:

AXS-05

Axsome is evaluating AXS-05 (dextromethorphan/bupropion) in the following indications: treatment resistant depression, major depressive disorder, Alzheimer's disease agitation, and smoking cessation.

Treatment Resistant Disorder

To date, just under 90% of the target number of subjects have been randomized in the Phase 3 STRIDE-1 trial in treatment resistant depression (TRD), with topline results anticipated in the first quarter of 2019. STRIDE-1 is a multicenter, randomized, double-blind, controlled trial in which subjects with TRD are randomized to treatment with AXS-05 or bupropion. A positive interim futility analysis was previously announced for the STRIDE-1 trial. The interim analysis was conducted by an independent data monitoring committee (IDMC) which recommended continuation of the study. Axsome has received U.S. Food and Drug Administration (FDA) Fast Track designation for AXS-05 for the treatment of TRD.

Major Depressive Disorder

Axsome has completed enrollment in the Phase 2 ASCEND study in major depressive disorder (MDD), and is on track to announce topline results in early January 2019, as previously disclosed. ASCEND is a multicenter, randomized, double-blind, controlled trial in which subjects with MDD are randomized to treatment with AXS-05 or bupropion.

Alzheimer's Disease Agitation

In December 2018, Axsome announced positive results of an interim futility analysis for the Phase 2/3 ADVANCE-1 trial of AXS-05 in Alzheimer's disease (AD) agitation. ADVANCE-1 is a multicenter, randomized, double-blind, controlled trial in which subjects with agitation associated with Alzheimer's disease are randomized to treatment with AXS-05, bupropion or placebo. The interim analysis was conducted by an IDMC which recommended continuation of the AXS-05 treatment arm and no further randomization of subjects to the bupropion treatment arm. Axsome intends to follow the IDMC's recommendation. Axsome has received FDA Fast Track designation for AXS-05 for the treatment of AD agitation.

Smoking Cessation

AXS-05 is being evaluated in a Phase 2, randomized, double-blind, controlled trial for smoking cessation treatment. In the trial, smokers are randomized to treatment with AXS-05 or bupropion. The trial is being conducted under a research collaboration between Duke University and Axsome. Top-line results are anticipated in the first quarter of 2019, as previously disclosed.

AXS-07

Axsome is developing AXS-07 (MoSEIC meloxicam/rizatriptan) for the acute treatment of migraine.

Axsome anticipates initiation of its planned Phase 3 trial of AXS-07 in patients with migraine in the first quarter of 2019, as previously disclosed, with topline results expected within approximately one year from trial initiation. The Phase 3 trial will be a multicenter, randomized, double-blind, controlled trial in which subjects with migraine are randomized to treatment with AXS-07, meloxicam, rizatriptan, or placebo.

AXS-12

Axsome is developing AXS-12 (reboxetine) for the treatment of narcolepsy.

In December 2018, Axsome received Investigational New Drug Application (IND) clearance to proceed with its planned Phase 2 trial of AXS-12

(reboxetine) in narcolepsy from the FDA. The planned Phase 2 trial is a multicenter, randomized, double-blind, placebo-controlled, crossover study in patients with narcolepsy. Axsome anticipates initiation of this study in January 2019, with topline results in the first half of 2019. Axsome has received FDA Orphan Drug Designation for AXS-12 for the treatment of narcolepsy.

Anticipated Clinical Trial Readouts

- Phase 2 ASCEND trial of AXS-05 in MDD, topline data (early January 2019)
- Phase 3 STRIDE-1 trial of AXS-05 in TRD, topline data (1Q 2019)
- Phase 2 trial of AXS-05 in smoking cessation, topline data (1Q 2019)
- Phase 2 trial of AXS-12 in narcolepsy, topline data (1H 2019)
- Phase 2/3 ADVANCE-1 trial of AXS-05 in AD agitation, interim efficacy analysis (2019)
- Phase 2/3 ADVANCE-1 trial of AXS-05 in AD agitation, topline data (2H 2019 – 1H 2020)
- Phase 3 trial of AXS-07 in the acute treatment of migraine, topline data (2019)

About AXS-05

AXS-05 is a novel, oral, investigational medicine 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 AXS-07

AXS-07 is a novel, oral, rapidly absorbed, investigational medicine consisting of MoSEIC™ meloxicam and rizatriptan. AXS-07 utilizes Axsome's proprietary MoSEIC™ (Molecular Solubility Enhanced Inclusion Complex) technology to substantially increase the solubility and speed the absorption of meloxicam while maintaining durability of action.

About AXS-12

AXS-12 (reboxetine) is a novel, oral, investigational medicine in development for the treatment of the symptoms of narcolepsy. AXS-12 is a highly selective and potent norepinephrine reuptake inhibitor. AXS-12 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 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 2/3 trial in agitation associated with Alzheimer's disease (AD), a Phase 2 trial in Major Depressive Disorder (MDD), and a Phase 2 trial in smoking cessation. AXS-07 is being developed for the acute treatment of migraine. AXS-12 is being developed for the treatment of the symptoms of 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.

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 ("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 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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