Axsome Therapeutics Presents Positive AXS-07 Phase 1 Trial Results at the 19th Congress of the International Headache Society

September 5, 2019

Dual mechanisms, rapid absorption and long half-life support therapeutic potential of AXS-07 in migraine

AXS-07 MOMENTUM Phase 3 trial ongoing in migraine patients with history of inadequate response to prior acute treatments

NEW YORK, Sept. 05, 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, will present positive results from a Phase 1 pharmacokinetic trial of AXS-07 (MoSEIC™ meloxicam and rizatriptan) at the 19th Congress of the International Headache Society (IHC) in Dublin on September 6, 2019. AXS-07 is Axsome's novel, oral, investigational medicine with distinct dual mechanisms of action being developed for the acute treatment of migraine. Results of the Phase 1 trial demonstrated that AXS-07 is rapidly absorbed after oral administration, with the MoSEIC meloxicam component also displaying an extended plasma half-life, suggesting the potential for enhanced and sustained efficacy. These results support the ongoing MOMENTUM (Maximizing Outcomes in Treating Acute Migraine) Phase 3 trial of AXS-07 in migraine patients with a history of inadequate response to prior acute treatments.

The Phase 1 trial examined the pharmacokinetics of AXS-07 tablets (20 mg MoSEIC meloxicam/10 mg rizatriptan), and 10 mg standard rizatriptan in healthy volunteers. After oral administration of AXS-07, therapeutic plasma concentrations of MoSEIC meloxicam were attained in a median time of 17 minutes, the trial's pre-specified primary endpoint, while maximum plasma concentrations of the rizatriptan component were reached in a median time of 38 minutes. These results suggest the potential for enhanced onset of action for AXS-07. In addition, the terminal half-life of MoSEIC meloxicam was 18 hours indicating the potential for sustained effect. With the administration of AXS-07, the absorption of rizatriptan was numerically faster, and the resulting plasma concentrations numerically greater, as compared to standard rizatriptan. AXS-07 was well tolerated with no relevant differences in safety profile across the two treatment arms. There were no serious adverse events in the study.

The efficacy of AXS-07 in the acute treatment of migraine is currently being evaluated in the ongoing MOMENTUM Phase 3 trial, in which patients are randomized to treatment with AXS-07, rizatriptan, MoSEIC meloxicam, or placebo. MOMENTUM is enrolling only patients with a history of inadequate response to prior acute treatments, assessed using the Migraine Treatment Optimization Questionnaire (mTOQ-4), and the majority of patients randomized to date also report associated allodynia (pain from a normally non-painful stimulus), reflecting a more treatment-resistant population.

The presentation at the IHC includes the Phase 1 trial results as well as information on the design and targeted patient population of the ongoing MOMENTUM Phase 3 trial. Below are the details of the poster presentation:

Title: AXS-07 (MoSEIC™ Meloxicam/Rizatriptan): Novel Oral Therapeutic in Clinical Development for the Acute Treatment of Migraine

Poster Number: IHC-PO-124

Date and Time: Friday, September 6, 2019, 11AM-12PM IST.

A copy of the poster presentation is available on the "Publications" page of the "Science" section of Axsome's website at www.axsome.com.

Phase 1 Trial Design

The study was a randomized, parallel group trial to evaluate the pharmacokinetics and safety of AXS-07 tablets (20 mg MoSEIC™ meloxicam/10 mg rizatriptan), and 10 mg standard rizatriptan, after single-dose, oral administration in healthy volunteers. A total of 20 healthy volunteers were randomly assigned in a 1:1 ratio to treatment with AXS-07 or standard rizatriptan, under fasting conditions. The primary endpoint was the time to therapeutic concentration of meloxicam.

About AXS-07

AXS-07 is a novel, oral, investigational medicine with distinct dual mechanisms of action under development for the acute treatment of migraine. AXS-07 consists of MoSEIC™ meloxicam and rizatriptan. Meloxicam is a new molecular entity for migraine enabled by Axsome's MoSEIC (Molecular Solubility Enhanced Inclusion Complex) technology, which results in rapid absorption of meloxicam while maintaining a long plasma half-life. Meloxicam is a COX-2 preferential non-steroidal anti-inflammatory drug and rizatriptan is a 5-$HT_{1B/D}$ agonist. AXS-07 is designed to provide rapid, enhanced and consistent relief of migraine, with reduced symptom recurrence. AXS-07 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. AXS-05, 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, pace of enrollment and completion of the trials (including our ability to fully fund our disclosed clinical trials, which assumes no material changes to our currently projected expenses), 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, including the Company's anticipated cash runway and the Company's current expectations regarding its plans for future equity financings prior to the readout from its Phase 3 trials; 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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