Axsome Therapeutics Completes Successful FDA Pre-NDA Meeting for AXS-07 for the Acute Treatment of Migraine

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NDA submission on track for 4Q 2020

NEW YORK, Aug. 20, 2020 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ: AXSM), a biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, today announced that it has completed a successful pre-New Drug Application (NDA) meeting with the U.S. Food and Drug Administration (FDA) for AXS-07 for the acute treatment of migraine. AXS-07 is Axsome's novel, oral, multi-mechanistic investigational medicine for the acute treatment of migraine. The purpose of the meeting was to reach agreement with the FDA on the proposed content and format of the Company's planned NDA submission including the clinical and nonclinical requirements.

Based on the feedback from the FDA, the Company believes its regulatory data package will be sufficient to support an NDA for AXS-07 for the acute treatment of migraine, and Axsome remains on track to submit the planned NDA in the fourth quarter of 2020. Acceptance of the final NDA will be subject to the FDA's review of the complete filing.

"Axsome is pleased with the outcome of our recent pre-NDA meeting with the FDA, which confirmed the studies and data to be presented in our planned NDA submission of AXS-07 for the acute treatment of migraine," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "AXS-07 incorporates multiple mechanisms of action, and rapid absorption after oral administration, to address various migraine processes with the goal of providing enhanced effectiveness. We remain on track to complete the submission in the fourth quarter to make this new potential therapy available to patients living with migraine."

Axsome previously announced positive results from two Phase 3, randomized, controlled trials of AXS-07 in the acute treatment of migraine, the MOMENTUM and INTERCEPT trials, which demonstrated rapid, substantial, and statistically significant elimination of migraine pain and prevention of progression of migraine pain intensity with AXS-07 compared to control.

MOMENTUM, conducted pursuant to an FDA Special Protocol Assessment, randomized 1,594 patients with a history of inadequate response to prior acute migraine treatments to treat a single migraine attack with AXS-07, rizatriptan, MoSEIC™ meloxicam, or placebo. Rizatriptan, an active comparator in the trial, is considered to be the fastest acting oral triptan and one of the most effective medications currently available for the acute treatment of migraine [1]. AXS-07 provided substantially greater and more sustained migraine pain relief compared to placebo and rizatriptan, which translated to a significant reduction in rescue medication use for AXS-07 compared to placebo and rizatriptan. The percentage of patients achieving sustained pain relief from 2 to 24 hours after dosing was 53.3% for AXS-07, compared to 33.5% for placebo and 43.9% for rizatriptan ($p<0.001$, $p=0.006$, respectively versus AXS-07). Rescue medication was used by 23.0% of AXS-07 patients, compared to 43.5% of placebo and 34.7% of rizatriptan patients ($p<0.001$ for each group versus AXS-07). AXS-07 provided rapid relief of migraine pain with the percentage of patients achieving pain relief with AXS-07 being numerically greater than with rizatriptan at every time point measured starting at 15 minutes.

INTERCEPT randomized 302 patients to treat a single migraine attack with AXS-07 or placebo, at the earliest sign of migraine pain, while the pain intensity was mild. In this trial, AXS-07 resulted in a statistically significantly greater percentage of patients as compared to placebo achieving pain freedom (32.6% versus 16.3%, $p=0.002$) and freedom from most bothersome symptom (43.9% versus 26.7%, $p=0.003$), 2 hours after dosing (co-primary endpoints). A single dose of AXS-07 prevented progression of migraine pain beyond mild intensity in 73.5% of patients versus 47.4% of placebo patients ($p<0.001$), with rescue medication being required by only 15.3% of AXS-07 patients versus 42.5% of placebo patients ($p<0.001$) within 24 hours after dosing. AXS-07 rapidly eliminated migraine symptoms, with numerical separation from placebo as early as 30 minutes for migraine pain freedom and most bothersome symptom freedom.

AXS-07 was safe and well tolerated in both trials. The most commonly reported adverse events with AXS-07 in the MOMENTUM trial were nausea, dizziness and somnolence, none of which occurred at a rate greater than placebo or greater than 3%. The most commonly reported adverse events with AXS-07 in the INTERCEPT trial were somnolence, dizziness, and paresthesia, all of which occurred at a rate of less than five percent.

About Migraine

Over 37 million Americans suffer from migraine according to the Centers for Disease Control, and it is the leading cause of disability among neurological disorders in the United States according to the American Migraine Foundation. Migraine is characterized by recurrent attacks of pulsating, often severe and disabling head pain associated with nausea, and sensitivity to light and or sound. It is estimated that migraine accounts for $78 billion in direct (e.g. doctor visits, medications) and indirect (e.g. missed work, lost productivity) costs each year in the United States [2]. Published surveys of migraine sufferers indicate that more than 70% are not fully satisfied with their current treatment, that nearly 80% would try a new therapy, and that they desire treatments that work faster, more consistently, and result in less symptom recurrence [3,4].

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-$HT1_{B/1D}$ 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 biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders for which there are limited treatment options. For the many people facing unsatisfactory treatments for CNS disorders, Axsome accelerates the invention and adoption of life-changing medicines. Axsome's core CNS product candidate portfolio includes five clinical-stage candidates, AXS-05, AXS-07, AXS-09, AXS-12, and AXS-14. AXS-05 is being developed for major depressive disorder (MDD), treatment resistant depression (TRD), Alzheimer's disease (AD) agitation, and as treatment for smoking cessation. AXS-07 is being developed for the acute treatment of migraine. AXS-12 is being developed for the treatment of narcolepsy. AXS-14 is being developed for fibromyalgia. AXS-05, AXS-07, AXS-09, AXS-12, and AXS-14 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

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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 discontinuation of the bupropion treatment arm of the ADVANCE-1 study in accordance with the independent data monitoring committee's recommendations); the potential for the MOMENTUM clinical trial to provide a basis for approval of AXS-07 for the acute treatment of migraine in adults with or without aura, pursuant to our special protocol assessment; the potential for the ASCEND clinical trial, combined with the GEMINI clinical trial results, 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; unforeseen circumstances or other disruptions to normal business operations arising from or related to COVID-19; 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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