Axsome Therapeutics Announces Continued Progress in Clinical Development of AXS-07 for the Acute Treatment of Migraine

October 28, 2019

Enrollment in MOMENTUM Phase 3 migraine trial of AXS-07 in patients with history of inadequate response nearly complete; on track for topline results in 4Q 2019

First patient dosed in new INTERCEPT Phase 3 migraine trial evaluating early treatment with AXS-07; topline results anticipated in 1Q 2020

MINDSET survey of migraine specialists supports rationale for MOMENTUM and INTERCEPT trials of AXS-07

Migraine key opinion leaders conference call planned for November 25, 2019

NEW YORK, Oct. 28, 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, announces the initiation of the INTERCEPT trial, a Phase 3 study evaluating the early treatment of migraine with AXS-07 (MoSEIC™ meloxicam/rizatriptan), the Company's novel, oral, investigational medicine with distinct dual mechanisms of action for the acute treatment of migraine. The Company separately announced results of the MINDSET survey of physicians who treat in aggregate more than 50,000 migraine patients annually. The vast majority of survey physicians (90%) believe it is very important for patients to administer their acute treatment at the earliest sign of migraine pain, supporting the rationale for the INTERCEPT trial which is designed to enhance the market readiness of AXS-07 by generating information on its potential real-world use. The vast majority of respondents (85%) also cite efficacy as the most significant unmet need in the acute treatment of migraine, supporting the rationale for the MOMENTUM Phase 3 trial in patients with difficult-to-treat migraine. Enrollment in MOMENTUM is nearly complete and the study is on track for readout of topline results before year end. Based on FDA feedback, the MOMENTUM trial, if positive, will be the only efficacy trial required to support an NDA filing for AXS-07 for the acute treatment of migraine, as previously disclosed.

"We are pleased with the continued progress in the clinical development of AXS-07 for the acute treatment of migraine. With a potential NDA filing for AXS-07 next year, the newly launched INTERCEPT early treatment trial is designed to further enhance the positioning of AXS-07 and ready it for potential market entry," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "Results of the MINDSET survey of migraine-treating physicians suggest high receptivity to the potential clinical profile of AXS-07. The vast majority of physicians indicate that they are more likely to prescribe AXS-07 over current treatments, as well as over other treatments in development, if AXS-07 meets the objectives of the ongoing MOMENTUM Phase 3 registration trial in patients with difficult-to-treat migraine. Importantly, MOMENTUM remains on track to report topline results before year end."

The Company plans to hold an investor R&D call with migraine key opinion leaders on November 25, 2019 to discuss unmet needs in the acute treatment of migraine and the potential for AXS-07.

AXS-07 Progress Update

MOMENTUM Phase 3 Trial in Patients with History of Inadequate Response

- Enrollment in the MOMENTUM (Maximizing Outcomes in Treating Acute Migraine) trial is nearly complete, and the Company remains on track to report topline results from this study in the fourth quarter of 2019.
- MOMENTUM is enrolling only patients with a history of inadequate response to prior acute migraine treatments, assessed using the Migraine Treatment Optimization Questionnaire (mTOQ-4), and incorporates the potent active comparator rizatriptan. The trial is being conducted pursuant to an FDA Special Protocol Assessment (SPA). Based on FDA feedback, this trial, if positive, will be the only efficacy trial required to support an NDA filing for AXS-07 for the acute treatment of migraine.

INTERCEPT Phase 3 Trial Evaluating Early Treatment of Migraine Pain

- The Company launched the INTERCEPT (Initiating Early Control of Migraine Pain and Associated Symptoms) trial, a Phase 3, randomized, double-blind, placebo-controlled study evaluating the early treatment of migraine with AXS-07.
- In contrast to the ongoing MOMENTUM trial in which patients with a history of inadequate response treat migraine attacks once they have become of moderate or severe intensity, in the INTERCEPT trial, patients are to administer AXS-07 at the earliest sign of migraine pain. Approximately 300 patients will be randomized in this study in a 1:1 ratio to treatment with AXS-07 or placebo. Topline results from the INTERCEPT trial are anticipated in the first quarter of 2020.

Migraine Treatment Needs and Physician Receptivity (MINDSET) Survey

- Axsome separately announced today results of the "Migraine Treatment Needs and Physician Receptivity" (MINDSET) survey of 106 neurologists and other migraine-treating physicians, who collectively treat more than 50,000 migraine patients annually. The MINDSET survey was conducted to understand treating physicians' views of the unmet needs in the acute treatment of migraine and their potential receptivity to AXS-07.
- The vast majority of physicians cite efficacy as the most significant unmet need in the acute treatment of migraine, and believe it is very important for patients to administer their acute treatment at the earliest sign of migraine pain (85% and 90% of physicians, respectively).
- If AXS-07 demonstrates superior efficacy over rizatriptan as assessed in the ongoing MOMENTUM trial, 91% of physicians report that they are more likely to prescribe AXS-07 over currently available treatments, with 56% reporting that they are significantly or moderately more likely to prescribe AXS-07.
- If AXS-07 demonstrates superior efficacy over rizatriptan as assessed in the ongoing MOMENTUM trial, 87% of physicians report that they are more likely to prescribe AXS-07 over other drugs in development, including oral CGRPs, that have not demonstrated superior efficacy to current treatments, with 56% reporting that they are significantly or moderately more likely to prescribe AXS-07.

Migraine Key Opinion Leaders (KOLs) Conference Call

- Axsome plans to host an investor R&D conference call and webcast on November 25, 2019 with migraine key opinion leaders (KOLs), focusing on AXS-07 and the unmet needs in the acute treatment of migraine.
- This R&D event will feature presentations from Dr. Stewart J. Tepper, Professor of Neurology at The Geisel School of Medicine at Dartmouth, and from Dr. Richard B. Lipton, Professor and Vice Chair of Neurology, and Director of the Montefiore Headache Center, at the Albert Einstein College of Medicine. Additional event details including the agenda and access information will be provided at a later date.

About the INTERCEPT Trial Evaluating Early Treatment

INTERCEPT (Initiating Early Control of Migraine Pain and Associated Symptoms) is a Phase 3, randomized, double-blind, multicenter, placebo-controlled trial evaluating the early treatment of migraine with AXS-07. Approximately 300 patients will be randomized in a 1:1 ratio to treatment with AXS-07 or placebo. Patients are to administer AXS-07 at the earliest sign of migraine pain. The two co-primary endpoints of the trial are the proportion of patients who are free from headache pain two hours after dosing, and the proportion of patients who no longer suffer from their most bothersome migraine-associated symptom (nausea, photophobia, or phonophobia) two hours after dosing.

About the MOMENTUM Trial in Patients with History of Inadequate Response

MOMENTUM (Maximizing Outcomes in Treating Acute Migraine) is a Phase 3, randomized, double-blind, multicenter, controlled trial to assess the efficacy and safety of AXS-07 in the acute treatment of moderate and severe migraine in patients with a history of inadequate response to prior acute treatments. Approximately 875 eligible patients will be randomized in a 2:2:2:1 ratio to treatment with AXS-07, rizatriptan, MoSEIC™ meloxicam, or placebo. The two co-primary endpoints of the trial are the proportion of patients who are free from headache pain two hours after dosing, and the proportion of patients who no longer suffer from their most bothersome migraine-associated symptom (nausea, photophobia, or phonophobia) two hours after dosing, for AXS-07 as compared to placebo. Superiority of AXS-07 to the rizatriptan and meloxicam arms (component contribution) will be established based on sustained freedom from headache pain from two to 24 hours after dosing. The MOMENTUM study is being conducted pursuant to an FDA Special Protocol Assessment (SPA).

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 [1]. 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 [2,3].

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 two Phase 3 trials 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.

References

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3. Lipton RB, Stewart WF. Acute migraine therapy: do doctors understand what patients with migraine want from therapy? Headache. 1999;39(suppl 2):S20-S26.

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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Source: Axsome Therapeutics, Inc.