Axsome Therapeutics Announces Positive Efficacy and Safety Results from the Phase 3 MOVEMENT Long-Term Trial of AXS-07 in the Acute Treatment of Migraine

December 31, 2020

Over 21,000 migraine attacks treated with AXS-07

Achieved migraine pain relief in approximately 70% of patients, and pain freedom in approximately 40% of patients, at 2 hours

Achieved durable relief, with approximately 85% of patients free from rescue medication use over 48 hours

Long-term safety profile consistent with previously completed controlled trials

NDA on track for submission in 1Q 2021

NEW YORK, Dec. 31, 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 positive results from the long-term, open-label Phase 3 MOVEMENT trial of AXS-07, Axsome's novel, oral, multi-mechanistic investigational medicine in the acute treatment of migraine. Treatment with AXS-07, rapidly, substantially, and durably relieved migraine pain and associated symptoms in this trial. AXS-07 was well tolerated over long-term treatment with a safety profile consistent with that observed in the previously reported controlled trials. Axsome remains on track to submit an NDA for AXS-07 in the acute treatment of migraine in the first quarter of 2021.

"The results of the open-label, Phase 3 MOVEMENT trial confirm in a real-world setting the strong efficacy of AXS-07 observed in our previous controlled trials, and demonstrate a favorable long-term safety profile," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "The rapid and substantial efficacy of AXS-07 now observed in three separate trials indicates that AXS-07 may provide unique benefits to patients with migraine and help address the current unmet need for more effective treatments. These data further support our planned NDA filing of AXS-07 in the acute treatment of migraine in the first quarter."

The MOVEMENT (Multimechanistic Treatment over Time of Migraine Symptoms) trial evaluated the long-term safety of AXS-07 (20 mg MoSEIC™ meloxicam/10 mg rizatriptan), dosed for up to 12 months, in patients with migraine attacks. The study enrolled patients who had completed the previous pivotal studies of AXS-07: the MOMENTUM and INTERCEPT trials. Enrolled patients were allowed to treat up to 10 migraine attacks per month during the up to 12-month period, with one dose of AXS-07 for each migraine that occurred. The safety and efficacy of AXS-07 was assessed during the trial. A total of 706 patients were enrolled. The trial was concluded once at least 300 patients had treated at least 2 migraines a month for 6 months, and approximately 100 patients had treated at least 2 migraines a month for 12 months, as pre-specified. At the time of study conclusion, 515 patients had reached at least 6 months, and 155 patients had reached at least 12 months of treatment. Over 21,000 migraine attacks were treated with AXS-07 during the trial.

In the MOVEMENT trial, administration of AXS-07 resulted in rapid, and substantial relief of migraine pain and associated symptoms. Within 1 hour after dosing, 39% (range: 37-41%) of patients achieved relief of migraine pain, demonstrating the rapid onset of AXS-07. Two hours after administration of AXS-07, relief of migraine pain was achieved by 68% (range: 65-71%) of patients and pain freedom by 38% (range: 37-40%) of patients. Freedom from most bothersome symptom (photophobia, phonophobia, nausea) was achieved by 47% (range: 46-49%) of patients within 2 hours after dosing.

AXS-07 durably relieved migraine pain with 85% (range: 84-86%) of patients free from rescue medication use through 24 hours, and 83% (range: 82-85%) of patients free from rescue medication use through 48 hours after a single administration of AXS-07. Rates of sustained pain relief from 2 to 24 hours and from 2 to 48 hours were 60% (range: 59-62%) and 59% (58-60%), respectively. Rates of sustained pain freedom from 2 to 24 hours and from 2 to 48 hours were 33% (range: 33-35%) and 32% (range: 32-34%), respectively.

AXS-07 was well tolerated with long-term dosing. The safety profile of AXS-07 over the 12-month treatment period was consistent with that previously reported in short-term controlled trials. The most commonly reported adverse events (≥3%) were nausea, dizziness, and vomiting. During the 12-month trial, 1.6% of patients discontinued due to adverse events.

AXS-07 is a novel, oral, rapidly absorbed, multi-mechanistic investigational medicine for the acute treatment of migraine, consisting of MoSEIC™ meloxicam and rizatriptan. AXS-07 is thought to act by inhibiting CGRP release, reversing CGRP-mediated vasodilation, and inhibiting neuro-inflammation, pain signal transmission, and central sensitization. Axsome's MoSEIC™ technology significantly increases the speed of absorption of the meloxicam component after oral administration while maintaining a long plasma half-life. AXS-07 is covered by more than 80 issued U.S. and international patents providing protection out to 2036, and Axsome maintains worldwide rights.

About the MOVEMENT Trial

MOVEMENT (Multimechanistic Treatment over Time of Migraine Symptoms) was a Phase 3, open-label trial to evaluate the long-term safety of

AXS-07 (20 mg MoSEIC™ meloxicam/10 mg rizatriptan), dosed for up to 12 months, in patients with migraine attacks. The study enrolled patients who had completed the previous pivotal studies of AXS-07: the MOMENTUM and INTERCEPT trials. Enrolled patients were allowed to treat up to 10 migraine attacks per month during the up to 12-month period, with one dose of AXS-07 for each migraine that occurred. The safety and efficacy of AXS-07 was assessed during the trial. Efficacy measures included relief of migraine pain and most bothersome symptom (photophobia, phonophobia, nausea), and use of rescue medication.

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, rapidly absorbed, multi-mechanistic investigational medicine for the acute treatment of migraine, consisting 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 covered by more than 80 issued U.S. and international patents which provide protection out to 2036. 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), Alzheimer's disease (AD) agitation, and as a 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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2. Smelt AF, Louter MA, Kies DA, Blom JW, Terwindt GM, van der Heijden GJ, De Gucht V, Ferrari MD, Assendelft WJ. What do patients consider to be the most important outcomes for effectiveness studies on migraine treatment? Results of a Delphi study. PLoS One. 2014 Jun 16;9(6):e98933.
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 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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