Axsome Therapeutics to Provide Update on Continued Progress at the 2019 Cantor Global Healthcare Conference

October 2, 2019

GEMINI Phase 3 placebo-controlled trial of AXS-05 in MDD on track for readout of topline results in 4Q 2019

STRIDE-1 Phase 3 trial of AXS-05 in TRD on track for readout of topline results in 4Q 2019

MOMENTUM Phase 3 trial of AXS-07 in migraine on track for readout of topline results in 4Q 2019

CONCERT Phase 2 trial of AXS-12 in narcolepsy on track for readout of topline results in 4Q 2019

Cash runway into 4Q 2021

NEW YORK, Oct. 02, 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 participate in the 2019 Cantor Global Healthcare Conference on Thursday, October 3, 2019. Herriot Tabuteau, MD, Axsome's Chief Executive Officer, will participate in a fireside chat at 3:00 PM Eastern Time. The conference will be held at the InterContinental New York Barclay in New York, NY.

Dr. Tabuteau will provide the following update on the Company's continued progress during the presentation:

AXS-05: AXS-05 (dextromethorphan/bupropion modulated delivery tablet) is Axsome's novel, oral, investigational NMDA receptor antagonist with multimodal activity being developed for the following indications: treatment resistant depression (TRD), major depressive disorder (MDD), Alzheimer's disease (AD) agitation, and smoking cessation. AXS-05 has been granted U.S. Food and Drug Administration (FDA) Breakthrough Therapy designation for the treatment of MDD and Fast Track designations for the treatment of TRD and for the treatment of AD agitation.

Depression

- To date, approximately 75% of the targeted number of patients have been randomized into the GEMINI Phase 3 placebo-controlled trial of AXS-05 in MDD. Axsome remains on track to report topline efficacy results from both the GEMINI trial and the STRIDE-1 trial of AXS-05 in TRD in the fourth quarter of 2019.
- An NDA filing for AXS-05 in the treatment of MDD is targeted for the second half of 2020.

Alzheimer's Disease Agitation

- To date, approximately 65% of the targeted number of patients have been randomized into the ADVANCE-1 Phase 2/3 trial of AXS-05 in agitation associated with Alzheimer's disease. Axsome continues to anticipate topline results from the ADVANCE-1 trial in the first half of 2020.

AXS-07: AXS-07 (MoSEIC™ meloxicam/rizatriptan) is Axsome's novel, oral, investigational medicine with distinct dual mechanisms of action being developed for the acute treatment of migraine.

- To date, more than 80% of the targeted number of patients have been randomized into the MOMENTUM Phase 3 trial of AXS-07 in the acute treatment of migraine. MOMENTUM is being conducted pursuant to an FDA Special Protocol Assessment (SPA). Axsome remains on track to report topline results from the MOMENTUM trial in the fourth quarter of 2019.
- An NDA filing for AXS-07 in the acute treatment of migraine is targeted for the second half of 2020.

AXS-12: AXS-12 (reboxetine) is Axsome's novel, oral, potent and highly selective norepinephrine reuptake inhibitor being developed for the treatment of narcolepsy. AXS-12 has been granted Orphan Drug Designation by the FDA for the treatment of narcolepsy.

- To date, 70% of the targeted number of patients have been randomized into the CONCERT Phase 2 trial of AXS-12 in patients with narcolepsy. Axsome remains on track to report topline results from the CONCERT trial in the fourth quarter of 2019.

Financial Update

- Axsome believes that its current cash will be sufficient to fund the Company's anticipated operations, based on its current operating plans, into the fourth quarter of 2021, well beyond the readout of all of the above ongoing clinical trials.
- As previously disclosed, Axsome currently does not anticipate future equity financings prior to the readout from its Phase 3 trials.

A live webcast and archive of the event can be viewed on the "Webcasts & Presentations" page of the "Investors" section of the Company's website at www.axsome.com.

About AXS-05

AXS-05 is a novel, oral, patent-protected, investigational NMDA receptor antagonist with multimodal activity under development for the treatment of major depressive disorder and other central nervous system (CNS) disorders. AXS-05 consists of a proprietary formulation and dose of dextromethorphan and bupropion and utilizes Axsome's metabolic inhibition technology. The dextromethorphan component of AXS-05 is a non-competitive N-methyl-D-aspartate (NMDA) receptor antagonist, also known as a glutamate receptor modulator, which is a novel mechanism of action, meaning it works differently than currently approved therapies for major depressive disorder. The dextromethorphan component of AXS-05 is also a sigma-1 receptor agonist, nicotinic acetylcholine receptor antagonist, and inhibitor of the serotonin and norepinephrine transporters. The bupropion component of AXS-05 serves to increase the bioavailability of dextromethorphan, and is a norepinephrine and dopamine reuptake inhibitor, and a nicotinic acetylcholine receptor antagonist. AXS-05 is covered by more than 36 issued U.S. and international patents which provide protection out to 2034. AXS-05 is not approved by the FDA.

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 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 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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