Axsome Therapeutics Reports Third Quarter 2019 Financial Results and Provides Business Update

November 7, 2019

GEMINI Phase 3 placebo-controlled trial of AXS-05 in MDD completed enrollment and on track for readout of topline results by year-end 2019

STRIDE-1 Phase 3 trial of AXS-05 in TRD to conclude patient screening in November 2019 with topline results expected in 1Q 2020

MOMENTUM Phase 3 trial of AXS-07 in migraine completed enrollment and on track for readout of topline results by year-end 2019

CONCERT Phase 2 trial of AXS-12 in narcolepsy completed enrollment and on track for readout of topline results by year-end 2019

Company to host conference call today at 8:00 AM Eastern

NEW YORK, Nov. 07, 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, today reported financial results for the third quarter ended September 30, 2019.

"So far this year, we have significantly advanced our pipeline of differentiated investigational medicines that have the potential to transform the lives of many of the millions of people living with difficult-to-treat CNS disorders. Our three CNS product candidates in active clinical development are being evaluated in six efficacy trials, including five Phase 3 trials and one Phase 2 trial, across five different indications," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "It is an exciting time for Axsome and a potentially important time for patients and clinicians as all these studies are fast approaching completion. Specifically, topline readouts from the GEMINI Phase 3 trial of AXS-05 in major depressive disorder, the MOMENTUM Phase 3 trial of AXS-07 in the acute treatment of migraine, and the CONCERT Phase 2 trial of AXS-12 in narcolepsy are all expected before year end. We are also concluding patient screening in the Phase 3 STRIDE-1 trial of AXS-05 in treatment resistant depression at the end of this month, allowing for a meaningful proportion of patients from this trial to be included in the long-term safety database which is required for an NDA filing. As a result, topline data from this trial are now expected in the first quarter of 2020. Also anticipated next year are topline results from the Phase 2/3 ADVANCE-1 trial of AXS-05 in Alzheimer's disease agitation in the first half of 2020 and, importantly, potentially two NDA filings, for AXS-05 in major depressive disorder and for AXS-07 in the acute treatment of migraine, in the second half of 2020."

CNS Pipeline Update

Axsome is developing a portfolio of differentiated, patent-protected, central nervous system (CNS) product candidates. CNS disorders are distressing for patients, difficult to treat, and underserved. Axsome accelerates the development of new CNS medicines by utilizing proprietary technologies and novel mechanisms of action, combined with human proof-of-concept data and innovative clinical trial designs. Axsome's technologies include metabolic inhibition, MoSEIC™ delivery, chiral chemistry and formulation, and proprietary chemical synthesis and analysis. The Company's CNS pipeline includes three differentiated product candidates in active clinical development.

- **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: Enrollment in the GEMINI Phase 3 placebo-controlled trial of AXS-05 in MDD has been completed. Axsome remains on track to report topline results from GEMINI in the fourth quarter of 2019.

  Patient screening in the STRIDE-1 Phase 3 active-controlled trial of AXS-05 in TRD will conclude at the end of November, with topline results from this trial now expected in the first quarter of 2020, versus previous guidance of the fourth quarter of 2019. Screening in STRIDE-1 was continued beyond the target enrollment number in order to build the safety database of MDD and TRD patients required for an NDA filing. To date, approximately 300 patients have been randomized into the STRIDE-1 trial.

  Based on the outcome of Axsome's FDA Breakthrough Therapy meeting held earlier this year, positive results from either GEMINI or STRIDE-1 would be sufficient, along with the previously completed ASCEND trial of AXS-05 in MDD, to support an NDA filing for AXS-05 for the treatment of MDD, which is targeted for the second half of 2020.

Axsome is also enrolling a Phase 3 open-label, long-term safety extension study of AXS-05 in order to build the safety database of MDD and TRD patients required for a potential NDA filing. This open-label safety study is enrolling patients exiting both the GEMINI and STRIDE-1 trials.

AD Agitation: Axsome is enrolling the ADVANCE-1 study, a Phase 2/3, randomized, double-blind, controlled, multicenter trial to evaluate the efficacy and safety of AXS-05 in patients with Alzheimer's disease agitation. To date, approximately 70% of the target number of subjects have been randomized in this trial. Topline results are anticipated in the first half of 2020.

Smoking Cessation: Axsome plans to request a meeting with the FDA in the first quarter of 2020 to discuss the continued clinical development of AXS-05 as an aid to smoking cessation treatment. Earlier in 2019, Axsome reported positive topline results from a randomized, double-blind, active-controlled trial of AXS-05 for smoking cessation treatment. The study was conducted under a research collaboration between Axsome and Duke University.

- **AXS-07:** AXS-07 (MoSEIC™ meloxicam/rizatriptan) is Axsome's novel, oral, investigational medicine with distinct dual mechanisms of action for the acute treatment of migraine. The distinct mechanisms and rapid absorption of MoSEIC™ meloxicam, combined with the known efficacy of rizatriptan, are designed to enable rapid, superior, and consistent relief of migraine pain, with lower symptom recurrence, as compared to currently available therapies.

  Migraine: Axsome has completed enrollment in the MOMENTUM study, a Phase 3, randomized, double-blind, placebo- and active-controlled, multicenter trial to evaluate the efficacy and safety of AXS-07 in the acute treatment of migraine. 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. Axsome remains on track to report topline results from the MOMENTUM trial in the fourth quarter of 2019.

  MOMENTUM 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, which is targeted for the second half of 2020.

  In October 2019, Axsome announced results of the MINDSET physician survey, which demonstrated a significant unmet need for more efficacious acute migraine treatments and a high willingness of physicians to prescribe AXS-07 should it meet the objectives of the ongoing MOMENTUM Phase 3 trial in patients with difficult-to-treat migraines.

  In October 2019, Axsome initiated the INTERCEPT trial, a Phase 3, randomized, double-blind, multicenter, placebo-controlled study evaluating the early treatment of migraine with AXS-07. In the INTERCEPT study, approximately 300 patients will be randomized in a 1:1 ratio to treatment with AXS-07 or placebo. In contrast to the MOMENTUM trial, in which patients with a history of inadequate response treat migraine attacks once they have become of moderate or severe intensity, patients in the INTERCEPT trial are to administer AXS-07 at the earliest sign of migraine pain. INTERCEPT is designed to enhance the market readiness of AXS-07 by generating additional information on its potential real-world use. Topline results from INTERCEPT are anticipated in the first quarter of 2020.

  Axsome is also enrolling a Phase 3 open-label, long-term safety extension study of AXS-07 in order to build the safety database required for a potential NDA filing. This open-label safety study is enrolling patients exiting both the MOMENTUM and INTERCEPT trials.

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

  Narcolepsy: Axsome has completed enrollment in the CONCERT study, a Phase 2, randomized, double-blind, placebo-controlled, crossover, multicenter trial of AXS-12 in patients with narcolepsy. Eligible patients are randomized to receive either AXS-12 followed by placebo, or placebo followed by AXS-12. Efficacy assessments include the frequency of cataplexy attacks and measures of other symptoms of narcolepsy. Axsome remains on track to report topline results from CONCERT in the fourth quarter of 2019.

Scientific Meeting Presentations

- **International Headache Society (IHC)**: In September 2019, Axsome presented results from a Phase 1 pharmacokinetic trial of AXS-07 at the 19th Congress of the International Headache Society, held in Dublin, Ireland. The poster presentation also included information on the design and targeted patient population of the ongoing MOMENTUM Phase 3 trial.

Corporate Update

- In August 2019, Axsome announced the appointment of Dave Marek as Chief Commercial Officer, effective August 31, 2019. Prior to joining Axsome, Mr. Marek was Vice President, and General Manager of Amgen's Neuroscience business unit. Previously, he held executive positions at WebMD and at Saatchi & Saatchi Healthcare Advertising. Mr. Marek began his career at Eli Lilly and Company, followed by AstraZeneca.

Anticipated Milestones

- **Clinical Trial Readouts:**
  - Phase 3 GEMINI trial of AXS-05 in MDD, topline data (4Q 2019)
  - Phase 3 MOMENTUM trial of AXS-07 in migraine, topline data (4Q 2019)
  - Phase 2 CONCERT trial of AXS-12 in narcolepsy, topline data (4Q 2019)
  - Phase 3 STRIDE-1 trial of AXS-05 in TRD, topline data (1Q 2020)
  - Phase 3 INTERCEPT trial of AXS-07 in migraine, topline data (1Q 2020)
  - Phase 2/3 ADVANCE-1 trial of AXS-05 in AD agitation, topline data (1H 2020)
- **NDA Filings:**
  - AXS-05 in the treatment of MDD (2H 2020)
  - AXS-07 in the acute treatment of migraine (2H 2020)

Upcoming Migraine Key Opinion Leaders Conference Call and Webcast

Axsome will 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.

Third Quarter 2019 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $15.8 million for the quarter ended September 30, 2019 and $6.0 million for the comparable period in 2018. The increase was primarily due to trials that were initiated in 2019, which include the GEMINI, MOMENTUM, INTERCEPT, and CONCERT studies, along with the AXS-05 and AXS-07 open-label safety studies.
- **General and administrative (G&A) expenses:** G&A expenses were $3.1 million for the quarter ended September 30, 2019 and $2.2 million for the comparable period in 2018. The increase was primarily due to higher stock compensation expense and personnel costs.
- **Net loss:** Net loss was $19.1 million, or $(0.56) per share for the quarter ended September 30, 2019, compared to a net loss of $8.3 million, or $(0.31) per share for the comparable period in 2018.
- **Cash:** At September 30, 2019, Axsome had $43.6 million of cash compared to $53.8 million of cash at June 30, 2019.
- **Shares outstanding:** At September 30, 2019, Axsome had 34,496,846 shares of common stock outstanding.

Financial Guidance

- R&D expenses are anticipated to decrease in subsequent quarters reflecting the conclusion of ongoing trials and the completion of initiation of new trials.

- Axsome believes that its cash at September 30, 2019 will be sufficient to fund the company's anticipated operations, based on its current operating plans, into the second quarter of 2021.

- Axsome currently does not anticipate new equity financings prior to the readout from its Phase 3 trials, as previously disclosed.

Conference Call Information

Axsome will host a conference call and webcast today at 8:00 AM Eastern to discuss third quarter 2019 financial results as well as to provide a corporate update. To participate in the live conference call, please dial (844) 698-4029 (toll-free domestic) or (647) 253-8660 (international), and use the conference ID 3389043. The live webcast can be accessed on the "Webcasts & Presentations" page of the "Investors" section of the Company's website at axsome.com. A replay of the webcast will be available for approximately 30 days following the live event.

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.

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.

Axsome Therapeutics, Inc.
Selected Consolidated Financial Data

Statements of Operations Information:

	Three months ended September 30,	
	2019	**2018**
Operating expenses:		
Research and development	$ 15,835,573	$ 6,040,780
General and administrative	3,1111,662	2,202,679
Total operating expenses	18,947,235	8,243,459

Loss from operations	(18,947,235)	(8,243,459)
Interest and amortization of debt discount (expense)	(327,825)	(270,933)
Tax Credit	139,448	217,418
Change in fair value of warrant liability	—	15,000
Net loss	$ (19,135,612)	$ (8,281,974)
Net loss per common share, basic and diluted	$ (0.56)	$ (0.31)
Weighted average common shares outstanding, basic and diluted	34,445,489	26,325,904

Balance Sheet Information:

	September 30, 2019	December 31, 2018
Cash	$ 43,641,861	$ 13,968,742
Total assets	44,439,729	15,379,279
Loan payable, current and long-term	19,756,667	6,910,814
Accumulated deficit	(151,088,509)	(107,550,307)
Stockholders' equity	$ 6,822,596	$ 937,921

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