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

August 8, 2019

Both STRIDE-1 Phase 3 trial in TRD and GEMINI Phase 3 trial in MDD for AXS-05 on track for readout of topline results in 2H 2019

MOMENTUM Phase 3 trial of AXS-07 in migraine on track for readout of topline results in 2H 2019

FDA End-of-Phase 2 meeting for AXS-07 held; MOMENTUM is only efficacy trial required to support NDA filing of AXS-07 for migraine

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

Cash runway extended into 4Q 2021

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

NEW YORK, Aug. 08, 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 second quarter ended June 30, 2019.

"Over the past four months, we achieved several important clinical and regulatory milestones which significantly accelerated the clinical development of our potentially first-in-class or best-in-class CNS product candidates, with the aim of improving the lives of millions of patients living with difficult-to-treat CNS disorders," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "Following a successful FDA Breakthrough Therapy meeting, we launched two new clinical trials: the placebo-controlled GEMINI Phase 3 trial of AXS-05 in major depressive disorder, and a Phase 3 open-label long-term safety trial of AXS-05 in patients with major depressive disorder and treatment resistant depression. We also held a successful End-of-Phase 2 meeting with the FDA for AXS-07 in the acute treatment of migraine. Based on this meeting, the ongoing Phase 3 MOMENTUM trial, if successful, will be the only efficacy trial required to support an NDA filing for AXS-07. These developments position us to file potentially two NDAs in the second half of next year, one for AXS-05 in depression and one for AXS-07 in migraine."

"The next several months are expected to be highly active and potentially transformative for Axsome as we look forward to readouts from the Phase 3 STRIDE-1 trial of AXS-05 in treatment resistant depression, the GEMINI Phase 3 trial of AXS-05 in major depressive disorder, the Phase 3 MOMENTUM trial of AXS-07 in the acute treatment of migraine, and the Phase 2 CONCERT trial of AXS-12 in narcolepsy, with all four anticipated before the end of this year," continued Dr. Tabuteau. "In addition, recent financing activities have enabled us to fully fund all ongoing clinical trials while further extending our cash runway into the fourth quarter of 2021, well beyond topline data readouts for all ongoing efficacy trials."

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 often underserved, with many having no approved or satisfactory treatment options. Axsome accelerates the development of new CNS medicines by utilizing proprietary medicinal chemistry and formulation 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. Our CNS pipeline includes three differentiated product candidates in active clinical development.

- **AXS-05:** AXS-05 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 consists of a proprietary formulation of dextromethorphan and bupropion and utilizes Axsome's metabolic inhibition technology. 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: In June 2019, Axsome initiated the GEMINI study, a Phase 3, randomized, double-blind, multicenter, placebo-controlled trial of AXS-05 in patients with MDD. In the GEMINI study, approximately 300 patients with a confirmed diagnosis of moderate to severe MDD will be randomized in a 1:1 ratio to treatment with AXS-05 or placebo. To date, approximately 30% of the targeted number of patients have been randomized into this trial. Based on the results of Axsome's recent Breakthrough Therapy meeting, the previously completed ASCEND trial of AXS-05 in MDD is considered pivotal and sufficient with either the GEMINI trial of AXS-05 in MDD or the STRIDE-1 trial of AXS-05 in TRD, if positive, to

support an NDA filing for AXS-05 for the treatment of MDD, as previously reported. Axsome continues to expect topline efficacy results from both the STRIDE-1 and GEMINI trials in the second half of 2019, with an NDA filing anticipated in the second half of 2020.

In July 2019, Axsome initiated 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 the Phase 3 GEMINI trial, as well as patients exiting the Phase 3 STRIDE-1 trial.

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 agitation associated with AD. To date, just under 60% 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 intends to meet with the FDA later this year to obtain advice on the continued clinical development of AXS-05 as an aid to smoking cessation treatment. Axsome previously 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 is Axsome's novel, oral, investigational medicine with distinct dual mechanisms of action being developed for the acute treatment of migraine. AXS-07 consists of MoSEIC™ meloxicam and rizatriptan. The distinct mechanism of action 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 is enrolling 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. The trial is being conducted pursuant to an FDA Special Protocol Assessment (SPA), and is enrolling only patients with a history of inadequate response to prior acute migraine treatments. The historical inadequate response in this difficult-to-treat population has been associated with a significantly increased risk of new-onset chronic migraine, which may be prevented by improving acute treatment outcomes [1]. In the MOMENTUM trial, patients are randomized to treatment with AXS-07, rizatriptan, meloxicam, or placebo. Rizatriptan, the active comparator in the trial, is considered to be one of the most efficacious oral medications currently available for the acute treatment of migraine [2]. Superiority of AXS-07 to the rizatriptan and meloxicam arms would be established based on sustained freedom from headache pain from two to 24 hours after dosing. The MOMENTUM study continues to enroll ahead of expectations and, to date, more than 70% of the target number of subjects in the trial have been randomized. Axsome continues to expect topline results from this trial in the second half of 2019.

  In May 2019, Axsome held an End-of-Phase 2 meeting with the FDA to discuss the development status and plan for AXS-07 for the acute treatment of migraine. Based on the results of this meeting, the ongoing 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. A safety database including at least 300 patients treated with AXS-07 for at least six months and at least 100 patients treated for one year is required for the NDA filing. Axsome anticipates an NDA filing for AXS-07 in the acute treatment of migraine in the second half of 2020.

  In July 2019, Axsome initiated 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 the Phase 3 MOMENTUM trial.

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

  Narcolepsy: Axsome is enrolling 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 will include the frequency of cataplexy attacks and measures of other symptoms of narcolepsy. To date, randomization in this trial is approaching 50% of the target number of patients. Axsome continues to expect topline results from this trial in the second half of 2019.

Corporate Update

- In May 2019, Axsome raised $20.1 million through the sale of 942,285 shares of its common stock under its existing at-the-market facility with SVB Leerink.

- In July 2019, Axsome was added to the Russell 3000® and Russell 2000® Indexes, as part of the annual Russell indexes reconstitution.

- In July 2019, Axsome amended its existing term loan facility agreement, led by Silicon Valley Bank (SVB), to extend the initial 12-month interest-only payment period as well as the time for drawing down additional funding under the loan facility. Axsome entered into the $24 million loan agreement in March 2019. Under the amendment, the interest-only payment period is extended by a minimum of six months, to 18 months, which may be further extended to 24 months should Axsome elect to draw down an additional $4 million that remains unfunded under the loan agreement.

Anticipated Milestones

- **Clinical Trial Readouts:**

O Phase 3 STRIDE-1 trial of AXS-05 in TRD, topline data (2H 2019)

O Phase 3 GEMINI trial of AXS-05 in MDD, topline data (2H 2019)

O Phase 3 MOMENTUM trial of AXS-07 in migraine, topline data (2H 2019)

O Phase 2 CONCERT trial of AXS-12 in narcolepsy, topline data (2H 2019)

O Phase 2/3 ADVANCE-1 trial of AXS-05 in AD agitation, topline data (1H 2020)

- **NDA Filings:**

O AXS-05 in the treatment of MDD (2H 2020)

O AXS-07 in the acute treatment of migraine (2H 2020)

Upcoming Investor Conferences

Axsome is scheduled to participate in the following five upcoming investor conferences over the next two months:

- BTIG Biotechnology Conference – August 12, 2019, New York, NY (Not webcasted)
- H.C Wainwright Global Investment Conference – September 8-10, 2019, New York, NY (To be webcasted)
- Morgan Stanley Global Healthcare – September 11, 2019, New York, NY (To be webcasted)
- Ladenburg Thalmann Healthcare Conference – September 24, 2019, New York, NY (To be webcasted)
- Cantor Fitzgerald Global Healthcare Conference – October 2-4, 2019, New York, NY (To be webcasted)

The exact timing of these presentations and associated webcast information will be posted in advance under the Webcast and Presentations page on the Company's website at www.axsome.com.

Second Quarter 2019 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $11.0 million for the quarter ended June 30, 2019 compared with $5.6 million for the comparable period in 2018. The increase was primarily due to the significantly faster-than-expected enrollment in the MOMENTUM trial; the preparation for and initiation of the GEMINI trial, the AXS-05 open-label safety study, and the AXS-07 open-label safety study; and continued progress of the STRIDE-1, ADVANCE-1, and CONCERT trials.

- **General and administrative (G&A) expenses:** G&A expenses for the three months ended June 30, 2019 was $2.4 million, which was unchanged compared with the three months ended June 30, 2018.

- **Net loss:** Net loss was $13.8 million, or $(0.41) per share for the quarter ended June 30, 2019, compared with a net loss of $8.3 million, or $(0.32) per share for the comparable period in 2018.

- **Cash:** At June 30, 2019, Axsome had $53.8 million of cash compared with $42.6 million of cash as of March 31, 2019.

- **Shares outstanding:** At June 30, 2019, Axsome had 34,330,441 shares of common stock outstanding.

Financial Guidance

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

- Axsome believes that its cash at June 30, 2019 will be sufficient to fund the company's anticipated operations, based on its

current operating plans, into the fourth quarter of 2021.

- As previously disclosed in June 2019, Axsome currently does not anticipate new equity financings prior to the readout from its Phase 3 trials.

Conference Call Information

Axsome will host a conference call and webcast today at 8:00 AM Eastern to discuss second 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 4184366. 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 a Phase 3 trial for the acute treatment of migraine. AXS-12 is currently in a Phase 2 trial in narcolepsy. The Axsome Pain and Primary Care business unit (Axsome PPC) houses Axsome's pain and primary care assets, including AXS-02 and AXS-06, and intellectual property which covers these and related product candidates and molecules being developed by Axsome and others. AXS-02 is being developed for osteoporosis, the pain of knee osteoarthritis, and chronic low back pain. AXS-06 is being developed for osteoarthritis and rheumatoid arthritis. AXS-02, AXS-05, AXS-06, 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 June 30,	
	2019	**2018**
Operating expenses:		
Research and development	$ 11,003,142	$ 5,550,532
General and administrative	2,445,077	2,439,061
Total operating expenses	13,448,219	7,989,593
Loss from operations	(13,448,219)	(7,989,593)

Interest and amortization of debt discount (expense)	(313,995)	(292,323)
Change in fair value of warrant liability	—	1,000
Net loss	$ (13,762,214)	$ (8,280,916)
Net loss per common share, basic and diluted	$ (0.41)	$ (0.32)
Weighted average common shares outstanding, basic and diluted	33,801,749	25,791,177

Balance Sheet Information:

	June 30, 2019	December 31, 2018
Cash	$ 53,753,137	$ 13,968,742
Total assets	54,777,766	15,379,279
Loan payable, current and long-term	19,579,743	6,910,814
Accumulated deficit	(131,952,897)	(107,550,307)
Stockholders' equity	$ 24,282,945	$ 937,921

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[2] Ferrari MD, Roon KI, Lipton RB, Goadsby PJ. Oral triptans (serotonin 5-HT(1B/1D) agonists) in acute migraine treatment: a meta-analysis of 53 trials. Lancet. 2001 Nov 17;358(9294):1668-75.

AXSOME THERAPEUTICS

Source: Axsome Therapeutics, Inc.