# Axsome Therapeutics Reports First Quarter 2019 Financial Results and Provides Business Update

May 9, 2019

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

NEW YORK, May 09, 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 first quarter ended March 31, 2019.

"The first five months of 2019 have been highly productive at Axsome as we advanced our mission to develop innovative medicines for patients living with serious and difficult-to-treat CNS disorders. So far this year, we reported positive topline results from the Phase 2 ASCEND trial of AXS-05 in major depressive disorder as well as the Phase 2 trial of AXS-05 in smoking cessation, and launched the Phase 3 MOMENTUM trial of AXS-07 in the acute treatment of migraine and the Phase 2 CONCERT trial of AXS-12 in the treatment of narcolepsy," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "We also received Breakthrough Therapy designation from the FDA for AXS-05 for the treatment of major depressive disorder, and recently announced, based on the Breakthrough Therapy meeting, an expedited clinical development plan for AXS-05 in depression which could result in a potential NDA filing for AXS-05 in 2020. Over the remainder of the year, we look forward to several major clinical trial readouts including the Phase 3 STRIDE-1 trial of AXS-05 in treatment resistant depression, the planned placebo-controlled Phase 3 trial of AXS-05 in major depressive disorder, the Phase 2 CONCERT trial of AXS-12 in narcolepsy, and the Phase 3 MOMENTUM trial of AXS-07 in the acute treatment of migraine."

**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 a novel, oral, investigational NMDA receptor antagonist with multimodal activity, which is being evaluated in four separate indications: treatment resistant depression (TRD), major depressive disorder (MDD), Alzheimer's disease (AD) agitation, and smoking cessation. AXS-05 consists of dextromethorphan (an NMDA receptor antagonist, sigma-1 receptor agonist, and serotonin and norepinephrine reuptake inhibitor) and bupropion (a norepinephrine and dopamine reuptake inhibitor, which also increases the bioavailability of dextromethorphan). 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 March 2019, Axsome received FDA Breakthrough Therapy designation for AXS-05 for the treatment of MDD. The designation was supported by the positive results from the Phase 2 ASCEND study, a randomized, double-blind, active-controlled, multicenter, U.S. trial, in which 80 patients with confirmed moderate to severe MDD were treated with either AXS-05 or the active comparator bupropion. Axsome recently announced the expedited development and pivotal status for AXS-05 for the treatment of MDD and TRD following an FDA Breakthrough Therapy meeting. Based on the results of the Breakthrough Therapy meeting, the previously completed ASCEND trial, now considered pivotal, is sufficient with the ongoing STRIDE-1 trial in TRD, if positive, to support an NDA filing for AXS-05 for the treatment of MDD. Alternatively, Axsome may file an NDA for AXS-05 for the treatment of MDD with the completed ASCEND trial and a placebo-controlled Phase 3 trial of AXS-05 in MDD. Axsome intends to initiate this new placebo-controlled Phase 3 MDD trial in the second quarter of 2019. A safety database of MDD and TRD patients totaling at least 300 patients treated with AXS-05 for at least six months and at least 100 patients treated for one year is required for the NDA filing. Target enrollment in the ongoing STRIDE-1 Phase 3 trial has been reached. Based on the potentially accelerated timeline to NDA filing, patient screening in the STRIDE-1 trial however will continue in order to build the required safety database. Axsome expects topline efficacy results for both the Phase 3 STRIDE-1 trial in TRD, and the planned placebo-controlled Phase 3 trial in MDD, in the second half of 2019, with an NDA filing anticipated in 2020.

  **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 50% of the target number of subjects have been randomized in this trial. Topline results are anticipated in the first half of 2020.

**Smoking Cessation:** In April 2019, Axsome announced that Duke University completed its topline analysis of the Phase 2 trial of AXS-05 for smoking cessation treatment. The Phase 2 study was a randomized, double-blind, active-controlled trial, in which 58 adult smokers were treated either with AXS-05 or the active comparator bupropion, twice daily, and assessed over a 3-week period. Treatment with AXS-05 resulted in a 25% greater reduction in the average number of cigarettes smoked per day over the 3-week period, the prespecified primary endpoint, as compared to bupropion (p=0.0016). Consistent with this finding, a greater proportion of smokers receiving AXS-05 experienced a more than 50% reduction in expired carbon monoxide levels, a biochemical marker of smoking intensity, as compared to those treated with bupropion (52.0% for AXS-05 versus 30.4% for bupropion, p=0.15). The trial was conducted at the Duke Center for Smoking Cessation under a research collaboration between Axsome and Duke University. Axsome is evaluating next steps in the clinical development of AXS-05 for smoking cessation treatment.

- **AXS-07:** AXS-07 is a novel, oral, rapidly absorbed, 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:** In March 2019, Axsome initiated 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). 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. The trial is enrolling only patients with a history of inadequate response to prior acute migraine treatments. The majority of patients enrolled to date also report allodynia (pain from normally non-painful stimuli such as brushing hair or wearing glasses), a phenomenon that has been shown to be strongly associated with worse outcomes for pain freedom and pain relief after treatment. To date, approximately 40% of the target number of subjects have been randomized. Axsome recently announced that, based on the faster-than-expected enrollment in this trial, topline results are anticipated in the second half of 2019, versus prior guidance of the first quarter of 2020.

- **AXS-12:** AXS-12 (reboxetine) is a novel, oral, highly selective and potent norepinephrine reuptake inhibitor being developed for the treatment of the symptoms 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. The study will enroll approximately 20 patients, all of whom will be treated with AXS-12 for three weeks and with placebo for three weeks. 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. Topline results from this trial are expected in the second quarter of 2019.

**Anticipated Clinical Milestones**

- **Clinical Trial Initiations:**

  o Phase 3 placebo-controlled trial of AXS-05 in MDD (2Q 2019)

  o Open-label safety trial of AXS-05 in patients with MDD and TRD (2Q 2019)

- **Clinical Trial Readouts:**

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

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

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

**First Quarter 2019 Financial Results**

- **Research and development (R&D) expenses:** R&D expenses were $7.6 million for the quarter ended March 31, 2019 and $4.8 million for the comparable period in 2018. The increase was primarily due to the initiation and rapid progress of the MOMENTUM study, progress of our STRIDE-1, ADVANCE-1 and smoking cessation studies, and manufacturing costs related to our AXS-07 product candidate, which was partially offset by a reduction in the costs of previously completed clinical trials and nonclinical work.

- **General and administrative (G&A) expenses:** G&A expenses were $2.8 million for the quarter ended March 31, 2019 and $2.4 million for the comparable period in 2018. The increase in G&A expenses was primarily due to higher legal expenses, external fees associated with operating as a public company, and personnel costs.

- **Net loss:** Net loss was $10.6 million, or $(0.32) per share for the quarter ended March 31, 2019, compared to a net loss of $4.8 million, or $(0.19) per share for the comparable period in 2018.

- **Cash:** At March 31, 2019, Axsome had $42.6 million of cash compared to $14.0 million of cash as of December 31, 2018.

- **Shares outstanding:** At March 31, 2019, Axsome had 33,304,047 shares of common stock outstanding.

- **Financial guidance:** Axsome believes that its cash at March 31, 2019 will be sufficient to fund the company's anticipated operations, based on its current operating plans, into at least the first quarter of 2021.

**Conference Call Information**

Axsome will host a conference call and webcast today at 8:00 AM Eastern to discuss first 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 3058938. 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) and a Phase 2/3 trial in agitation associated with Alzheimer's disease (AD). AXS-05 is also being developed for major depressive disorder (MDD) and 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, 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; 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 March 31, | |
|---|---|---|
| | **2019** | **2018** |
| Operating expenses: | | |
| Research and development | $ 7,603,081 | $ 4,752,511 |
| General and administrative | 2,818,392 | 2,410,699 |
| Total operating expenses | 10,421,473 | 7,163,210 |
| Loss from operations | (10,421,473) | (7,163,210) |
| Interest and amortization of debt discount (expense) | (218,903) | (315,349) |
| Change in fair value of warrant liability | — | 2,673,000 |
| Net loss | $ (10,640,376) | $ (4,805,559) |
| Net loss per common share, basic and diluted | $ (0.32) | $ (0.19) |
| Weighted average common shares outstanding, basic and diluted | 33,052,468 | 25,501,188 |

**Balance Sheet Information:**

| | **March 31, 2019** | **December 31, 2018** |
|---|---|---|
| Cash and cash equivalents | $ 42,624,487 | $ 13,968,742 |
| Total assets | 43,901,088 | 15,379,279 |
| Loan payable, current and long-term | 19,353,359 | 6,910,814 |
| Accumulated deficit | (118,190,683) | (107,550,307) |
| Stockholders' equity | $ 17,373,699 | $ 937,921 |

**Axsome Contact:**
Mark Jacobson
Senior Vice President, Operations
Axsome Therapeutics, Inc.
200 Broadway, 3rd Floor
New York, NY 10038
Tel: 212-332-3243
Email: mjacobson@axsome.com
www.axsome.com

Source: Axsome Therapeutics, Inc.