Axsome Therapeutics Reports Fourth Quarter and Full Year 2018 Financial Results and Provides Business Update

March 14, 2019

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

NEW YORK, March 14, 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 fourth quarter and year ended December 31, 2018.

"In 2018, we advanced our clinical programs and significantly expanded our CNS pipeline through the addition of new internally generated product candidates, and the launch of clinical trials in new indications," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "We have continued this momentum into 2019, reporting positive results from the Phase 2 ASCEND trial of AXS-05 in major depressive disorder, and initiating the Phase 3 MOMENTUM trial of AXS-07 in migraine and the Phase 2 CONCERT trial of AXS-12 in narcolepsy. All in all, we are currently conducting registration or mid-stage clinical trials with our potentially first- or best-in-class product candidates in five different important CNS indications. In addition, our recently completed financings allow us to advance our deep pipeline well beyond data readouts for all ongoing clinical trials. Over the next several quarters, we look forward to topline results from the Phase 3 STRIDE-1 trial of AXS-05 in treatment resistant depression, the Phase 2 trial of AXS-05 in smoking cessation, the Phase 2 CONCERT trial of AXS-12 in narcolepsy, the Phase 3 MOMENTUM trial of AXS-07 in migraine, and the Phase 2/3 ADVANCE-1 trial of AXS-05 in Alzheimer's disease agitation."

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), Alzheimer's disease (AD) agitation, major depressive disorder (MDD), 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) Fast Track designations for the treatment of TRD and for the treatment of AD agitation.

  Depression: Axsome is enrolling a Phase 3 trial in TRD (the STRIDE-1 study), and has completed a Phase 2 trial in MDD (the ASCEND study). The Phase 3 STRIDE-1 study is a randomized, double-blind, active-controlled, multicenter trial to assess the efficacy and safety of AXS-05 in TRD, defined as major depressive disorder which has failed to respond to two or more antidepressant treatments. To date, approximately 95% of the target number of subjects have been randomized. Topline results are anticipated in the second quarter of 2019.

  In January 2019, Axsome announced positive results from the Phase 2 ASCEND study, a randomized, double-blind, active-controlled, multicenter, U.S. trial, in patients with confirmed moderate to severe MDD. In this study, AXS-05 met the prespecified primary endpoint by rapidly, substantially, and statistically significantly reducing depressive symptoms, measured using the Montgomery-Åsberg Depression Rating Scale (MADRS) total score, as compared to the active comparator bupropion. Further details of the study results are expected to be presented at upcoming scientific meetings. Axsome anticipates meeting with the FDA in the second quarter of 2019 to discuss the potential regulatory path for developing AXS-05 for the broader MDD indication.

  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. In December 2018, Axsome announced positive results of an interim futility analysis for the ADVANCE-1 trial. The interim analysis was conducted by an independent data monitoring committee (IDMC) which recommended continuation of the AXS-05 treatment arm and no further randomization of subjects to the bupropion treatment arm. The IDMC did not indicate that there were any safety concerns in the study. Axsome has followed the IDMC's recommendation. The previously planned second interim analysis will no longer be performed in order to preserve statistical power for the final analysis. To date, just over 40% of the target number of subjects have been randomized in this trial. Topline results are anticipated in the first half of 2020.

Smoking Cessation: AXS-05 is being evaluated in a Phase 2, randomized, double-blind, active-controlled trial for smoking cessation treatment in smokers interested in quitting. The change in smoking intensity will be measured using behavioral and biochemical assessments. The trial is being conducted under a research collaboration between Duke University and Axsome. To date, approximately 95% of the target number of subjects have been randomized in this trial. Topline results are anticipated in the second quarter of 2019.

- **AXS-07:** Axsome is developing AXS-07 for the acute treatment of migraine. AXS-07 is a novel, oral, rapidly absorbed, investigational medicine consisting 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 February 2019, Axsome reached agreement with the FDA under a Special Protocol Assessment (SPA) for the design, endpoints, and statistical approach of the MOMENTUM study, a Phase 3, randomized, double-blind, controlled, multicenter trial assessing the efficacy and safety of AXS-07 in the acute treatment of migraine. In March 2019, Axsome enrolled the first patient in this trial. The study will include approximately 875 patients, with a history of inadequate response to prior migraine treatments, who will be randomized in a 2:2:2:1 ratio to treatment with AXS-07, rizatriptan, meloxicam, or placebo. The two co-primary endpoints of the trial are the proportion of patients who are free from headache pain two hours after dosing, and the proportion of patients who no longer suffer from their most bothersome migraine-associated symptom (nausea, photophobia, phonophobia) two hours after dosing. Topline results from this trial are expected in the first quarter of 2020.

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

  Narcolepsy: In January 2019, Axsome initiated 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 will be 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.

Corporate Update

- In January 2019, Axsome raised gross proceeds of approximately $25.8 million from the sale of shares of its common stock under its at-the-market facility with SVB Leerink, fully utilizing the facility.
- In March 2019, Axsome entered into a $24 million growth capital term loan facility with Silicon Valley Bank (SVB) and WestRiver Innovation Lending Fund. Axsome received $20 million at closing, and can draw the remaining $4 million tranche, at its option, subject to the achievement of positive results from the Company's ongoing Phase 2 trial of AXS-12 in narcolepsy.

Anticipated Clinical Milestones

- **Clinical Trial Readouts:**

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

  — Phase 2 trial of AXS-05 in smoking cessation, topline data (2Q 2019)

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

  — Phase 3 MOMENTUM 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)

Fourth Quarter 2018 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $7.2 million for the quarter ended December 31, 2018 and $23.5 million for the year ended December 31, 2018, compared to $4.5 million and $20.0 million for the comparable periods in 2017. The increase was primarily due to increased costs for our STRIDE-1 and ADVANCE-1

studies, initiation and completion of our ASCEND study, and AXS-07 and AXS-12 study startup and manufacturing costs, which was partially offset by a reduction in the costs of our clinical trials for AXS-02 and AXS-06, and nonclinical work on AXS-05.

- **General and administrative (G&A) expenses:** G&A expenses were $2.3 million for the quarter ended December 31, 2018 and $9.4 million for the year ended December 31, 2018 and $2.0 million and $7.2 million for the comparable periods in 2017. The increase in G&A expenses was primarily due to higher intellectual property costs and legal expenses, external fees associated with operating as a public company as well as an increase in personnel costs.

- **Net loss:** Net loss was $9.6 million, or $(0.32) per share for the quarter ended December 31, 2018, compared to a net loss of $7.4 million, or $(0.31) per share for the comparable period in 2017. Net loss for the year ended December 31, 2018 was $31.0 million, or $(1.15) per share, compared to a net loss of $28.9 million, or $(1.27) per share for the comparable period in 2017.

- **Cash:** At December 31, 2018, Axsome had $14.0 million of cash. Including proceeds from the recently completed at-the-market equity financings and new growth capital term loan, Axsome's pro forma cash balance was $52.6 million, which compares to $34.0 million of cash at December 31, 2017.

- **Shares outstanding:** At December 31, 2018, Axsome had 30,087,213 shares of common stock outstanding.

- **Financial guidance:** Axsome anticipates that its current cash, including proceeds from the January 2019 equity financings and March 2019 term loan, will be sufficient to fund its anticipated operations, based on its current operating plans, into at least the fourth quarter of 2021.

Conference Call Information

Axsome will host a conference call and webcast today at 8:00 AM Eastern to discuss fourth quarter and full year 2018 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 9087075. 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 2/3 trial in agitation associated with Alzheimer's disease (AD), and a Phase 2 trial in smoking cessation. 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 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; 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 December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
Operating expenses:				
Research and development	$ 7,151,232	$ 4,493,910	$ 23,495,055	$ 19,957,616
General and administrative	2,299,083	1,950,210	9,351,522	7,206,691
Total operating expenses	9,450,315	6,444,120	32,846,577	27,164,307
Loss from operations	(9,450,315)	(6,444,120)	(32,846,577)	(27,164,307)
Interest and amortization of debt discount/premium (expense) income	(248,700)	(340,381)	(1,127,305)	(1,340,199)
Tax credit	0	0	217,418	207,114
Change in fair value of warrant liability	102,000	(646,000)	2.791,000	(646,000)
Net loss	$ (9,597,015)	$ (7,430,501)	$ (30,965,464)	$ (28,943,392)
Net loss per common share – basic and diluted	$ (0.32)	$ (0.31)	$ (1.15)	$ (1.27)
Weighted average common shares outstanding – basic and diluted	29,874,410	24,229,652	26,883,656	22,764,606

Balance Sheet Information:

	December 31, 2018	**December 31, 2017**
Cash	$ 13,968,742	$ 34,021,123
Total assets	15,379,279	35,555,564
Loan payable, current and long-term	6,910,814	9,932,351
Accumulated deficit	(107,550,307)	(76,584,843)
Stockholders' equity	$ 937,921	$ 16,717,223

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Source: Axsome Therapeutics, Inc.