Axsome Therapeutics Reports Fourth Quarter and Full Year 2019 Financial Results and Provides Business Update

March 12, 2020

NDA filing for Breakthrough Therapy designated AXS-05 in MDD expected in 4Q 2020

NDA filing for AXS-07 in migraine expected in 4Q 2020

Topline results for STRIDE-1 Phase 3 trial of AXS-05 in TRD on track for 1Q 2020

Topline results for INTERCEPT Phase 3 trial of AXS-07 in treatment of migraine on track for 1Q 2020

Topline results for ADVANCE Phase 2/3 trial of AXS-05 in Alzheimer's disease agitation anticipated in 3Q 2020

Initiation of Phase 3 trial of AXS-12 in narcolepsy anticipated in 2020

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

NEW YORK, March 12, 2020 (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, 2019.

"Axsome delivered on several transformative achievements in 2019, including positive NDA-enabling clinical trial readouts for AXS-05 in depression and for AXS-07 in migraine, propelling Axsome potentially towards commercial stage as early as 2021," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "In addition, our AXS-12 product candidate for narcolepsy achieved positive Phase 2 results and is progressing to Phase 3, and through our agreement with Pfizer earlier this year, we expanded our Phase 3 pipeline with the addition of AXS-14 for the treatment of fibromyalgia."

Dr. Tabuteau said, "We expect continued operational momentum in 2020 with the anticipated filing of the two NDAs for AXS-05 in depression and for AXS-07 in migraine, which we expect to occur in the fourth quarter. Furthermore, we are on track for readouts from our STRIDE-1 Phase 3 trial of AXS-05 in treatment resistant depression and our INTERCEPT Phase 3 trial of AXS-07 in early treatment of migraine, both by the end of this month. We expect readouts from our ADVANCE-1 Phase 2/3 trial of AXS-05 in Alzheimer's disease agitation in the third quarter. By providing new mechanisms of action, and potentially faster, greater and broader efficacy as compared to currently available treatments, our investigational medicines have the potential to change the current standard of care for difficult-to-treat CNS disorders and transform the lives of patients living with these conditions."

CNS Pipeline Update

Axsome is developing a portfolio of differentiated, patent-protected, central nervous system (CNS) product candidates. For the many people facing unsatisfactory treatments for CNS disease, Axsome accelerates the invention and adoption of life-changing medicines. The Company's CNS pipeline includes four 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: major depressive disorder (MDD), treatment resistant depression (TRD), 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: In December 2019, Axsome announced positive results from the Phase 3 GEMINI study, a randomized, double-blind, placebo-controlled, multicenter, U.S. trial, in patients with confirmed moderate to severe MDD. In this study, AXS-05 met the primary endpoint by rapidly, substantially, and statistically significantly improving symptoms of depression as compared to placebo. The positive results from the GEMINI trial, along with the previously completed ASCEND trial of AXS-05 in MDD, support an NDA filing for AXS-05 in the treatment of MDD, which is anticipated in the fourth quarter of 2020.

  Randomization into the STRIDE-1 Phase 3 active-controlled trial of AXS-05 in TRD has been completed. Axsome remains on track to report topline results from STRIDE-1 in the first quarter of 2020.

To support the planned NDA filing of AXS-05 in MDD, a Phase 3, open-label, long-term safety extension study of AXS-05 in patients with MDD and TRD is ongoing.

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, more than 80% of the target number of subjects have been randomized in this trial. Based on current enrollment, we expect topline results from this trial in the third quarter of 2020.

Smoking Cessation: Axsome plans to meet with the FDA in 2020 to discuss the continued clinical development of AXS-05 as an aid to smoking cessation treatment.

- **AXS-07:** AXS-07 (MoSEIC™ meloxicam/rizatriptan) is Axsome's novel, oral, rapidly absorbed, multi-mechanistic, investigational medicine for the acute treatment of migraine.

  Migraine: In December 2019, Axsome announced positive results from the Phase 3 MOMENTUM study, a randomized, double-blind, placebo- and active-controlled, multicenter, U.S. trial, in migraine patients with a history of inadequate response to prior acute treatments. The study incorporated the potent active comparator rizatriptan. In this study, AXS-07 met the two regulatory co-primary endpoints resulting in significantly greater rates of freedom from migraine pain and most bothersome migraine-associated symptoms as compared to placebo. AXS-07 also met the key secondary endpoint, demonstrating statistically significant superiority to the active comparator rizatriptan on sustained freedom from migraine pain. MOMENTUM was conducted pursuant to an FDA Special Protocol Assessment (SPA). The positive results from the MOMENTUM trial support an NDA filing for AXS-07 in the acute treatment of migraine, which is anticipated in the fourth quarter of 2020.

  Enrollment in the Phase 3 INTERCEPT study, a placebo-controlled trial of AXS-07 in the early treatment of migraine has been completed. Axsome remains on track to report topline results from INTERCEPT in the first quarter of 2020.

  To support the planned NDA filing of AXS-07 in the acute treatment of migraine, enrollment in a Phase 3 open-label, long-term safety extension study of AXS-07 is ongoing.

- **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: In December 2019, Axsome announced positive results from the Phase 2 CONCERT study, a randomized, double-blind, placebo-controlled, crossover, multicenter, U.S. trial, of AXS-12 in patients with narcolepsy. In this study, AXS-12 met the prespecified primary endpoint and significantly reduced the number of cataplexy attacks as compared to placebo. AXS-12 also significantly reduced excessive daytime sleepiness (EDS), and improved cognitive function, sleep quality and sleep-related symptoms as compared to placebo.

  Axsome plans to initiate Phase 3 trials of AXS-12 in the treatment of narcolepsy in 2020.

- **AXS-14:** AXS-14 (esreboxetine) is Axsome's novel, oral, potent, and highly selective norepinephrine reuptake inhibitor for the treatment of fibromyalgia. Esreboxetine, the SS-enantiomer of reboxetine, is more potent and selective than racemic reboxetine.

  Fibromyalgia: In January 2020, Axsome obtained exclusive U.S. rights from Pfizer, Inc. for the development and commercialization of AXS-14 (esreboxetine), which has demonstrated positive and statistically significant results in a Phase 3 and a Phase 2 trial in the treatment of fibromyalgia. In a Phase 3 trial conducted by Pfizer in 1,122 patients with fibromyalgia treated with esreboxetine or placebo for 14 weeks, esreboxetine met the two primary endpoints demonstrating statistically significant improvements compared to placebo in the weekly mean pain score ($p<0.001$), and the Fibromyalgia Impact Questionnaire (FIQ) total score ($p<0.001$). Esreboxetine also resulted in statistically significant improvements as compared to placebo in fatigue as measured using the Global Fatigue Index ($p=0.001$).

  Axsome plans to meet with the FDA in 2020 to discuss the further clinical development of AXS-14 for the treatment of fibromyalgia.

Corporate Update

- In December 2019, Axsome was added to the NASDAQ Biotechnology Index®.

- In December 2019, Axsome completed an underwritten public offering of 2,300,000 shares of its common stock, including the full exercise of the underwriters' option to purchase additional shares, at a public offering price of $87.00 per share. The aggregate gross proceeds to Axsome, before deducting underwriting discounts and commissions and other estimated offering expenses, were $200.1 million. SVB Leerink and Morgan Stanley acted as joint bookrunning managers for the offering.

- In January 2020 Axsome entered into an agreement with Pfizer Inc. for an exclusive U.S. license to Pfizer's clinical and nonclinical data, and intellectual property for reboxetine, the active pharmaceutical ingredient in AXS-12, and for exclusive rights to develop and commercialize AXS-14 (esreboxetine), a new product candidate, in the U.S. for the treatment of fibromyalgia. The agreement accelerates the ongoing clinical development of AXS-12 in narcolepsy and expands Axsome's pipeline of late-stage CNS product candidates.

- In March 2020, Axsome announced the appointment of Mark Jacobson as Chief Operating Officer, effective February 28, 2020. Mr. Jacobson has been a member of the Axsome team since April 2014, and has served as Senior Vice President, Operations since September 2017.

Anticipated Milestones

- **NDA Filings:**
  - AXS-05 in the treatment of MDD (4Q 2020)
  - AXS-07 in the acute treatment of migraine (4Q 2020)

- **Clinical Trial Readouts:**
  - 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 (3Q 2020)

- **Clinical Trial Initiations:**
  - Phase 3 trials of AXS-12 in the treatment of narcolepsy (2H 2020)

Upcoming Investor Conferences

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

- William Blair Biotech Focus Conference – April 2, 2020, New York, NY (Not webcasted)
- Needham Healthcare Conference – April 14-15, 2020, New York, NY (Not webcasted)
- SunTrust Robinson Humphrey Life Sciences Summit – May 5-6, 2020, New York, NY (Not webcasted)

Fourth Quarter 2019 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $19.2 million for the quarter ended December 31, 2019 and $7.2 million for the comparable period in 2018. This increase was due to a significant number of new clinical trials that were conducted during the quarter as compared to the prior year period, including the CONCERT, GEMINI, MOMENTUM, INTERCEPT trials, and the AXS-05 and AXS-07 open-label safety studies, in addition to the ongoing progress of the STRIDE-1 and ADVANCE-1 trials.

- **General and administrative (G&A) expenses:** G&A expenses were $5.2 million for the quarter ended December 31, 2019 and $2.3 million for the comparable period in 2018. The change was primarily due to personnel costs, mainly associated with an increase in stock compensation expense, along with the build-out of the commercial function.

- **Net loss:** Net loss was $24.8 million, or $(0.71) per share for the quarter ended December 31, 2019, compared to a net loss of $9.6 million, or $(0.32) per share for the comparable period in 2018. Net loss for the year ended December 31, 2019 was $68.3 million, or $(2.01) per share, compared to a net loss of $31.0 million, or $(1.15) per share per share for the comparable period in 2018.

- **Cash:** At December 31, 2019, Axsome had $220.0 million of cash compared to $14.0 million of cash at December 31,

2018.

- **Shares outstanding:** At December 30, 2019, Axsome had 36,933,217 shares of common stock outstanding.

Financial Guidance

- R&D expenses are anticipated to decrease in subsequent quarters reflecting the conclusion of ongoing trials.
- Axsome believes that its cash at December 31, 2019 will be sufficient to fund the company's anticipated operations, based on its current operating plans, for at least two years.

Conference Call Information

Axsome will host a conference call and webcast today at 8:00 AM Eastern to discuss fourth quarter and full year 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 2067997. 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 five clinical-stage candidates, AXS-05, AXS-07, AXS-09, AXS-12, and AXS-14. 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 is being developed for major depressive disorder (MDD). 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 being developed for the treatment of narcolepsy. AXS-14 is being developed for the treatment of fibromyalgia. AXS-05, AXS-07, AXS-09, AXS-12, and AXS-14 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 MOMENTUM clinical trial to provide a basis for approval of AXS-07 for the acute treatment of migraine in adults with or without aura, pursuant to our special protocol assessment; the potential for the ASCEND clinical trial, combined with the GEMINI clinical trial results, 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 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,	
	2019	**2018**	**2019**	**2018**
Operating expenses:				
Research and development	$ 19,205,271	$ 7,151,232	$ 53,647,067	$ 23,495,055
General and administrative	5,222,899	2,299,083	13,598,030	9,351,522
Total operating expenses	24,428,170	9,450,315	67,245,097	32,846,577
Loss from operations	(24,428,170)	(9,450,315)	(67,245,097)	(32,846,577)
Interest and amortization of debt discount (expense)	(378,814)	(248,700)	(1,239,537)	(1,127,305)

Tax Credit	—	—	139,448	217,418
Change in fair value of warrant liability	—	102.000	—	2,791,000
Net loss	$ (24,806,984)	$ (9,597,015)	$ (68,345,186)	$ (30,965,464)
Net loss per common share, basic and diluted	$ (0.71)	$ (0.32)	$ (2.01)	$ (1.15)
Weighted average common shares outstanding, basic and diluted	34,757,910	29,874,410	34,020,257	26,883,656

Balance Sheet Information:

	December 31, 2019	December 31, 2018
Cash and cash equivalents	$ 219,966,167	$ 13,968,742
Total assets	220,549,760	15,379,279
Loan payable, current and long-term	19,934,918	6,910,814
Accumulated deficit	(175,895,493)	(107,550,307)
Stockholders' equity	$ 178,722,389	$ 937,921

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