Axsome Therapeutics Reports Fourth Quarter and Full Year 2016 Financial Results and Provides Business Update

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Interim analysis results from two Phase 3 trials of AXS-02 in separate indications anticipated in 2H 2017

Top-line results from the Phase 3 STRIDE-1 trial of AXS-05 in treatment resistant depression expected in 1Q 2018

Phase 2/3 trial of AXS-05 in Alzheimer's disease agitation to begin in 2Q 2017

NEW YORK, March 07, 2017 (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, 2016.

"In 2016 we advanced our late-stage portfolio resulting in three ongoing pivotal trials in three indications. We anticipate launching a potentially pivotal trial in a fourth indication, Alzheimer's disease agitation, in the second quarter of 2017," said Herriot Tabuteau, M.D., Chief Executive Officer of Axsome.

"The next 12 months will be significant for Axsome as we expect results from all of our ongoing Phase 3 trials during this period. Prudent cash management has allowed us to extend our cash runway through these important clinical milestones which include an interim analysis for efficacy from the CREATE-1 trial in complex regional pain syndrome, and top-line results from the STRIDE-1 trial in treatment resistant depression. An interim analysis to assess sample size assumptions is now planned for the COAST-1 trial in knee osteoarthritis associated with bone marrow lesions, with results expected in the third quarter of 2017. We look forward to these near-term clinical results which will shed light on the potential of AXS-02 and AXS-05 in multiple indications."

Pipeline Update

Axsome is developing a portfolio of differentiated, patent-protected, CNS product candidates. CNS disorders are distressing, difficult-to-treat, and underserved with many having no approved or satisfactory treatments. Axsome accelerates the development of new CNS medicines in a cost-efficient manner, by utilizing novel mechanisms of action and novel delivery approaches of well-characterized molecules, combined with human proof-of-concept data and innovative clinical trial designs. Our pipeline includes two late-stage product candidates in Phase 3 development and preclinical candidates.

- **AXS-05:** Axsome is developing AXS-05 in two separate Phase 3 clinical programs for treatment resistant depression (TRD) and Alzheimer's disease (AD) agitation. AXS-05 utilizes Axsome's technology of combining bupropion and dextromethorphan (DM) to increase the bioavailability of DM and unlock its therapeutic potential. DM and bupropion target multiple monoaminergic and glutamatergic neurotransmitter systems that are implicated in numerous CNS disorders. Bupropion also serves to increase the bioavailability of DM.

  TRD: In February 2017, the U.S. Food and Drug Administration (FDA) granted Axsome Fast Track designation for AXS-05 for the treatment of TRD. Axsome is evaluating AXS-05 in the STRIDE-1 trial, a Phase 3, multicenter, randomized, double-blind, active-controlled 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. TRD is a serious and difficult-to-treat condition with potentially devastating consequences. STRIDE-1 is a two-period trial in which all subjects are initially being treated with bupropion in Period 1. Those failing to respond in Period 1 are then randomized to treatment with AXS-05 or bupropion in Period 2.

  AD Agitation: In January 2017, Axsome received Investigational New Drug Application (IND) clearance from the FDA to proceed with a Phase 2/3 trial of AXS-05 in the treatment of AD agitation. Agitation is reported in as many

as 40% of patients with AD and has been associated with increased caregiver burden, decreased functioning, earlier nursing home placement, and death. The planned Phase 2/3 trial is a multicenter, randomized, double-blind, placebo-controlled study to examine the efficacy and safety of AXS-05 in AD patients with agitation. Eligible subjects will be randomly assigned to treatment with AXS-05, placebo, or bupropion. There are currently no therapies approved by the FDA for the treatment of agitation in patients with AD.

- **AXS-02:** Axsome is developing AXS-02 in three separate Phase 3 clinical programs: complex regional pain syndrome (CRPS), knee osteoarthritis (OA) associated with bone marrow lesions (BMLs), and chronic low back pain (CLBP) associated with Modic changes (MCs). AXS-02 (disodium zoledronate tetrahydrate) is a potent osteoclast inhibitor being developed as an oral, targeted, non-opioid, potentially first-in-class therapeutic for chronic pain. AXS-02 is dosed once per week for 6 weeks and thereafter may have a duration of effect measured in months.

  CRPS: Axsome is evaluating AXS-02 in the CREATE-1 study, a Phase 3, global, randomized, double-blind, placebo-controlled trial to assess the efficacy and safety of AXS-02 in the treatment of pain in patients with CRPS. CREATE-1 incorporates an interim analysis for efficacy that will be conducted on the first approximately 95 enrolled subjects. Axsome previously received positive scientific advice from the European Medicines Agency (EMA). Based on this advice, Axsome believes the CREATE-1 trial, if successful, will be sufficient to support a marketing authorization application (MAA) to the EMA seeking European approval of AXS-02 for the treatment of pain associated with CRPS.

  In October 2016, Axsome announced the publication of data showing that AXS-02 inhibits pain in a well-validated animal model of CRPS. CRPS is characterized by severe, continuous, disabling limb pain, is widely considered one of the most painful conditions, and is not considered responsive to standard pain medications. There is currently no drug approved by the FDA or the EMA for this condition. AXS-02 has received Fast Track designation from the FDA, and orphan drug designation from the FDA and EMA for the treatment of CRPS.

  Knee OA associated with BMLs: Axsome is evaluating AXS-02 in the COAST-1 study, a global, randomized, double-blind, placebo-controlled Phase 3 clinical trial to assess the efficacy and safety of AXS-02 in the treatment of the pain of knee OA associated with BMLs. COAST-1 is being conducted pursuant to an FDA Special Protocol Assessment (SPA), and this program has received Fast Track designation from the FDA.

  An interim analysis will now be performed by an independent data monitoring committee on the first approximately 60 subjects enrolled in the trial to assess the assumptions used to determine the sample size of the study. Screening of subjects in this trial will be paused pending results of the interim analysis, and will resume after readouts from Axsome's other ongoing Phase 3 trials in CRPS and TRD. The pause in screening is expected to have a positive effect on Axsome's cash runway.

  CLBP associated with MCs: In February 2017, Axsome received IND clearance from the FDA to proceed with a Phase 3 trial of AXS-02 in the treatment of CLBP associated with MCs. The planned Phase 3 trial is a multicenter, randomized, double-blind, placebo-controlled study in patients with CLBP and type 1, or mixed type 1 and type 2 MCs. The initiation of this clinical trial is contingent upon the availability of resources. The IND clearance builds upon previously reported positive results from the randomized, double-blind, placebo-controlled Phase 2 trial conducted with intravenous zoledronic acid showing a statistically significant reduction in low back pain intensity and NSAID usage as compared to placebo in patients with CLBP and MCs. AXS-02 is a proprietary oral formulation of zoledronic acid.

- **Other Programs:** Axsome is currently evaluating preclinical product candidates, including AXS-06, that it intends to develop for CNS disorders, including chronic pain. Formulation work for AXS-06 is ongoing.

Corporate Update

- **NASDAQ Global Market tier transfer:** In March 2017, Axsome's common stock listing was transferred to the NASDAQ Global Market from the NASDAQ Capital Market. Listing on the NASDAQ Global Market is one of several requirements for inclusion in the NASDAQ Biotechnology Index. This transfer did not impact Axsome's trading symbol.

- **Term loan agreement with Silicon Valley Bank:** In November 2016, Axsome entered into a $20 million term loan agreement with Silicon Valley Bank. Axsome has drawn $10 million of this amount and has the option to draw the remaining $10 million subject to the achievement of certain clinical and financial milestones.

Anticipated Near-Term Clinical Milestones

- **Clinical Trial Initiations:**

  -- Phase 2/3 clinical trial of AXS-05 in AD agitation (2Q 2017)

- **Clinical Trial Readouts:**

  -- Phase 3 COAST-1 trial of AXS-02 in knee OA associated with BMLs, interim analysis (3Q 2017)

  -- Phase 3 CREATE-1 trial of AXS-02 in CRPS, interim efficacy analysis (4Q 2017)

  -- Phase 3 STRIDE-1 trial of AXS-05 in TRD, top-line data (1Q 2018)

Fourth Quarter and Full Year 2016 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $5.8 million for the quarter ended December 31, 2016 and $21.2 million for the year ended December 31, 2016 compared to $2.2 million and $6.8 million for the comparable periods in 2015. The increase in R&D expenses was primarily due to the conduct of the CREATE-1, STRIDE-1, and COAST-1 Phase 3 clinical trials.

- **General and administrative (G&A) expenses:** G&A expenses were $1.8 million for the quarter ended December 31, 2016 and $6.3 million for the year ended December 31, 2016 compared to $0.9 million and $2.4 million for the comparable periods in 2015. The increase in G&A expenses was primarily due to external fees associated with operating as a public company, as well as an increase in personnel costs and stock compensation expense.

- **Net loss:** Net loss was $7.3 million, or $(0.38) per share for the quarter ended December 31, 2016, compared to a net loss of $3.4 million, or $(0.23) per share for the comparable period in 2015. Net loss for the year ended December 31, 2016 was $27.2 million, or $(1.42) per share, compared to a net loss of $12.2 million, or $(1.02) per share for the comparable period in 2015.

- **Cash:** At December 31, 2016, Axsome had $36.6 million of cash compared to $48.0 million of cash at December 31, 2015.

- **Shares outstanding:** At December 31, 2016, Axsome had 19,158,417 shares of common stock outstanding.

- **Financial guidance:** Axsome believes that its cash at December 31, 2016 will be sufficient to fund the company's anticipated operations, based on its current operating plans, through the first quarter of 2018.

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 product candidate portfolio includes two late-stage candidates, AXS-05 and AXS-02. AXS-05 is currently in a Phase 3 trial in treatment resistant depression (TRD), and a Phase 2/3 trial in agitation in patients with Alzheimer's disease (AD) is planned. AXS-02 is currently in Phase 3 trials in complex regional pain syndrome (CRPS) and knee osteoarthritis (OA) associated with bone marrow lesions (BMLs) with an additional Phase 3 trial planned in chronic low back pain (CLBP) associated with Modic changes (MCs). AXS-05 and AXS-02 are investigational drug products not approved by the FDA. For more information, please visit the company website at www.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, interim analyses and receipt of interim results; the timing of and our ability to obtain and maintain U.S. Food and Drug Administration or other regulatory authority approval of, or other action with respect to, our product candidates; 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, (unaudited)		Year Ended December 31,	
	2016	2015	2016	2015
Operating expenses:				
Research and development	$ 5,806,771	$ 2,207,915	$ 21,199,860	$ 6,776,987
General and administrative	1,818,789	946,618	6,343,648	2,419,289
Total operating expenses	7,625,560	3,154,533	27,543,508	9,196,276
Loss from operations	(7,625,560)	(3,154,533)	(27,543,508)	(9,196,276)

Interest and amortization of debt discount/premium expense	(177,657)	(116,403)	(132,424)	(736,048)
Tax credit	474,279	—	474,279	—
Change in fair value of warrant liability	—	(93,976)	—	(108,539)
Change in fair value of embedded derivative liabilities	—	—	—	274,800
Loss on extinguishment of debt	—	—	—	(2,444,516)
Net loss	$ (7,328,938)	$ (3,364,912)	$ (27,201,653)	$ (12,210,579)
Net loss per common share – basic and diluted	$ (0.38)	$ (0.23)	$ (1.42)	$ (1.02)
Weighted average common shares outstanding – basic and diluted	19,153,993	14,429,540	19,150,690	11,945,318

Balance Sheet Information:

	December 31, 2016	December 31, 2015
Cash	$ 36,618,497	$ 48,036,260
Total assets	38,212,608	49,076,156
Accumulated deficit	(47,641,451)	(20,439,798)
Stockholders' equity	$ 21,571,451	$ 46,444,261

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