Axsome Therapeutics Reports Third Quarter 2017 Financial Results

November 8, 2017

NEW YORK, Nov. 08, 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 quarter ended September 30, 2017.

“In the third quarter we launched our Phase 2/3 ADVANCE-1 trial of AXS-05 in Alzheimer’s disease agitation and reported positive Phase 1 trial results with our new clinical-stage product candidate, AXS-06,” said Herriot Tabuteau, M.D., Chief Executive Officer of Axsome. “We are very excited to be advancing a broad portfolio of differentiated product candidates for important clinical indications. Specifically, we are nearing the interim efficacy results of the CREATE-1 trial of AXS-02 in complex regional pain syndrome, as well as the interim assessment of sample size assumptions of the COAST-1 trial of AXS-02 in knee osteoarthritis associated with bone marrow lesions. With the addition of AXS-06, our portfolio now includes two non-opioid candidates for pain.”

Pipeline Update

Axsome is developing a portfolio of differentiated, patent-protected, central nervous system (CNS) product candidates. CNS disorders are distressing and difficult-to-treat. The patients with them are often underserved with many having no approved or satisfactory treatment options. 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 product candidates in Phase 3 development, one candidate which we believe to be Phase 3-ready, and additional preclinical candidates.

- **AXS-05:** Axsome is evaluating AXS-05 (bupropion and dextromethorphan fixed-dose combination) in two separate Phase 3 clinical programs for treatment resistant depression (TRD) and Alzheimer’s disease (AD) agitation. AXS-05 is a novel fixed-dose combination 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), under development for the treatment of CNS disorders.

TRD: Axsome is enrolling the STRIDE-1 study, 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.

AD Agitation: In July 2017, Axsome announced enrollment of the first patient in the ADVANCE-1 study, a Phase 2/3, multicenter, randomized, double-blind, controlled trial to evaluate the efficacy and safety of AXS-05 in patients with agitation associated with AD.

- **AXS-02:** Axsome is evaluating AXS-02 (disodium zoledronate tetrahydrate) 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 is a potent osteoclast inhibitor being developed as an oral, non-opioid, targeted, potentially first-in-class therapeutic for chronic pain.

CRPS: Axsome is enrolling the CREATE-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 pain in patients with CRPS. CREATE-1 incorporates an interim analysis for efficacy which will be performed by an independent data monitoring committee (IDMC) and results of the analysis are expected late December 2017 to early January 2018. Approaching the interim analysis, approximately 85 subjects have been randomized to date. Enrollment of subjects in the trial is ongoing and will continue through the interim analysis.

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. An interim analysis to assess the sample size of the study will be performed by an IDMC on the first approximately 60 subjects enrolled in the trial. The interim analysis will be performed by the same IDMC and at the same meeting as the interim analysis for the CREATE-1 trial.

- **AXS-06:** Axsome is developing AXS-06 (MoSEIC™ meloxicam and esomeprazole) for the relief of the signs and symptoms of OA and Rheumatoid Arthritis (RA), and the reduction in the risk of developing upper gastrointestinal ulcers in patients at risk of developing nonsteroidal anti-inflammatory drug (NSAID) associated upper gastrointestinal ulcers. AXS-06 is an oral, non-opioid, rapidly-absorbed, once-daily, COX-2 preferential pain therapeutic with a gastroprotectant.

In July 2017, Axsome announced positive topline results from a Phase 1 pharmacokinetic study of AXS-06 which demonstrated, for the first time, rapid achievement of peak plasma levels of meloxicam after oral administration. The median T_{max} for meloxicam, the trial’s primary endpoint, was 9 times faster for AXS-06 as compared to commercially available Mobic® (meloxicam) tablets. Axsome also received, from the FDA, Pre-Investigational New Drug Application (Pre-IND) written guidance on a proposed clinical developmental program for AXS-06. Based on this guidance, Axsome believes that AXS-06 is Phase 3-ready.

Corporate Update

• In September 2017, Cedric O'Gorman, M.D. was appointed Senior Vice President, Clinical Development and Medical Affairs. Dr. O'Gorman was previously Vice President of Medical Affairs at Intra-Cellular Therapies; U.S. medical lead for psychiatry at Genentech/Roche; and Medical Director, U.S. Medical Affairs at Pfizer.

• In July 2017, Axsome announced the appointment of John Golubieski as Chief Financial Officer (CFO) effective August 4, 2017. Mr. Golubieski was previously CFO of Osmotica Holdings; CFO of Fougera Pharmaceuticals, the former U.S. business of Nycomed; Senior Vice President, Financial Planning & Analysis of King Pharmaceuticals; and Senior Director, Strategic Analysis in the Worldwide Medicines Group at Bristol-Myers Squibb.

Anticipated Near-Term Clinical Milestones

• Clinical Trial Readouts:

- Phase 3 CREATE-1 trial of AXS-02 in CRPS, interim efficacy analysis (late December 2017 to early January 2018)
- Phase 3 COAST-1 trial of AXS-02 in knee OA associated with BMLs, interim analysis (late December 2017 to early January 2018)
- Phase 3 STRIDE-1 trial of AXS-05 in TRD, top-line data (1H 2018)

Third Quarter 2017 Financial Results

• **Research and development (R&D) expenses:** R&D expenses were $4.5 million for the quarter ended September 30, 2017 compared to $5.6 million for the comparable period in 2016. The decrease in R&D expense was primarily due to a reduction in the costs of our previously initiated clinical trials, which was partially offset by the initiation of our ADVANCE-1 study, pre-clinical costs for AXS-06, and manufacturing costs associated with our product candidates.

• **General and administrative (G&A) expenses:** G&A expenses were $1.8 million for the quarter ended September 30, 2017 compared to $1.6 million for the comparable period in 2016. The increase in G&A expenses was primarily due to higher intellectual property expenses.

• **Net loss:** Net loss was $6.4 million, or $(0.27) per share, for the quarter ended September 30, 2017 compared to a net loss of $7.2 million, or $(0.38) per share, for the quarter ended September 30, 2016.

• **Cash:** As of September 30, 2017, Axsome had $31.7 million of cash compared to $36.6 million of cash as of December 31, 2016.

• **Shares outstanding:** As of September 30, 2017, Axsome had 23,665,532 shares of common stock outstanding.

• **Financial guidance:** Axsome believes that its cash as of September 30, 2017 will be sufficient to fund the company's anticipated operations, based on its current operating plans, into the first quarter of 2019.

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 three clinical-stage candidates, AXS-02, AXS-05, and AXS-06. 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). 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). A Phase 1 trial of AXS-06 has been completed. AXS-02, AXS-05, and AXS-06 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, interim analyses and completion of the trials; 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 (unaudited):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Operating expenses:				
Research and development	$ 4,471,126	$ 5,568,777	$ 15,463,706	$ 15,393,089
General and administrative	1,826,290	1,639,026	5,256,481	4,524,859
Total operating expenses	6,297,416	7,207,803	20,720,187	19,917,948
Loss from operations	(6,297,416)	(7,207,803)	(20,720,187)	(19,917,948)
Interest and amortization of debt discount/premium (expense) income	(343,234)	13,219	(999,818)	45,233
Tax credit	207,114	0	207,144	0
Net loss	$ (6,433,536)	$ (7,194,584)	$ (21,512,891)	$(19,872,715)
Net loss per common share – basic and diluted	$ (0.27)	$ (0.38)	$ (0.97)	$ (1.04)
Weighted average common shares outstanding – basic and diluted	23,634,040	19,149,906	22,270,885	19,149,579

Balance Sheet Information:

		September 30, 2017 (unaudited)		December 31, 2016*
Cash	$	31,673,383	$	36,618,497
Total assets		32,618,112		38,212,608
Loan payable, current and long-term		10,092,481		9,739,607
Accumulated deficit		(69,154,342)		(47,641,451)
Stockholders' equity	$	16,839,057	$	21,571,451

*Condensed from audited financial statements.

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