Axsome Therapeutics Reports Second Quarter 2017 Financial Results

August 9, 2017

NEW YORK, Aug. 09, 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 June 30, 2017.

“We continued our forward momentum in the second quarter with the expansion of our clinical programs as well as our team,” said Herriot Tabuteau, M.D., Chief Executive Officer of Axsome. “Our clinical pipeline now consists of three late-stage product candidates: AXS-02, AXS-05, and AXS-06. We look forward to the remainder of the year as we approach results from our ongoing Phase 3 clinical trials.”

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 product candidates in Phase 3 development, one candidate which we believe to be Phase 3-ready, and preclinical candidates.

- **AXS-05:** Axsome is developing 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.

  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. Approximately 435 patients will be randomized in a 1:1:1 ratio to receive AXS-05, bupropion, or placebo for 5 weeks. The primary efficacy measure is the Cohen-Mansfield Agitation Inventory (CMAI). ADVANCE-1 incorporates a planned interim analysis to assess the assumptions used to determine the sample size of the study.

- **AXS-02:** Axsome is developing 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).

  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 conducted on the first approximately 95 enrolled subjects.

  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 will be conducted 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 new subjects in this trial is paused pending the results of the interim analysis.

- **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. Meloxicam is a long-acting NSAID with COX-2 preferential inhibition and potent pain relieving efficacy. However standard meloxicam has an extended time to maximum plasma concentration (T_{max}) which delays its onset of action. AXS-06 utilizes Axsome’s proprietary MoSEIC™ (Molecular Solubility Enhanced Inclusion Complex) technology to substantially

increase the solubility and speed the absorption of meloxicam while maintaining durability of action. AXS-06 also incorporates esomeprazole, a proton pump inhibitor, to reduce the risk of NSAID associated gastrointestinal ulcers which can occur with chronic NSAID use.

Phase 1 Trial Results: In July 2017, Axsome announced positive topline clinical trial results from a Phase 1 pharmacokinetic study of AXS-06. The study compared the pharmacokinetics of meloxicam and esomeprazole after oral administration of AXS-06 tablets (meloxicam 15 mg, esomeprazole 40 mg), and commercially available Mobic® tablets (15 mg meloxicam) and Nexium® capsules (40 mg esomeprazole) in healthy volunteers. The median T_{max} for meloxicam, the trial's primary endpoint, was 9 times faster for AXS-06 as compared to Mobic® (0.5 hour versus 4.5 hours for AXS-06 and Mobic®, respectively, $p<0.0001$). AXS-06 also demonstrated higher mean maximum plasma concentration (C_{max}) ($p=0.0018$), faster time to therapeutic plasma concentration ($p<0.0001$), and time to half-maximal plasma concentration ($p<0.0001$) as compared to Mobic®. Terminal half-lives for meloxicam were similar for AXS-06 and Mobic®. Plasma concentrations and terminal half-lives of esomeprazole after AXS-06 and Nexium® administration were comparable.

Pre-IND Guidance: In July 2017, Axsome received, from the FDA, Pre-Investigational New Drug Application (Pre-IND) written guidance on a proposed clinical developmental program for AXS-06 for the relief of the signs and symptoms of OA and RA, and the reduction in the risk of developing upper gastrointestinal ulcers in patients at risk of developing NSAID associated upper gastrointestinal ulcers. Based on this guidance, Axsome believes that AXS-06 is Phase 3-ready.

Corporate Update

- 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.

- In June 2017, Myrtle Potter was appointed to Axsome's Board of Directors. Ms. Potter is the Chief Executive Officer and Founder of Myrtle Potter & Company, a healthcare and life science advisory firm. Ms. Potter previously served as the President, Commercial Operations and Chief Operating Officer of Genentech, and held executive operating positions at Bristol-Myers Squibb and Merck.

Anticipated Near-Term Clinical Milestones

- **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)

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- **Research and development (R&D) expenses:** R&D expenses were $5.0 million for the quarter ended June 30, 2017 compared to $5.3 million for the comparable period in 2016. The decrease in R&D expenses 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 with AXS-05 and the conduct of the AXS-06 Phase 1 trial.

- **General and administrative (G&A) expenses:** G&A expenses were $1.7 million for the quarter ended June 30, 2017 compared to $1.5 million for the comparable period in 2016. The increase in G&A expenses was primarily due to higher intellectual property and stock compensation expenses.

- **Net loss:** Net loss was $7.1 million, or $(0.30) per share, for the quarter ended June 30, 2017 compared to a net loss of $6.8 million, or $(0.36) per share, for the quarter ended June 30, 2016.

- **Cash:** As of June 30, 2017, Axsome had $38.0 million of cash compared to $36.6 million of cash as of December 31, 2016.

- **Shares outstanding:** As of June 30, 2017, Axsome had 23,608,084 shares of common stock outstanding.

- **Financial guidance:** Axsome believes that its cash as of June 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 June 30,		Six Months Ended June 30,	
	2017	**2016**	**2017**	**2016**
Operating expenses:				
Research and development	$ 5,007,361	$ 5,298,060	$ 10,992,580	$ 9,824,312
General and administrative	1,743,377	1,529,220	3,430,191	2,885,833
Total operating expenses	6,750,738	6,827,280	14,422,771	12,710,145
Loss from operations	(6,750,738)	(6,827,280)	(14,422,771)	(12,710,145)
Interest and amortization of debt discount/premium (expense) income	(333,578)	15,090	(656,584)	32,014
Net loss	$ (7,084,316)	$ (6,812,190)	$(15,079,355)	$(12,678,131)
Net loss per common share – basic and diluted	$ (0.30)	$ (0.36)	$ (0.70)	$ (0.66)
Weighted average common shares outstanding – basic and diluted	23,595,702	19,149,417	21,578,011	19,149,417

Balance Sheet Information:

	June 30, 2017	**December 31, 2016***
	(unaudited)	
Cash	$ 37,994,449	$ 36,618,497
Total assets	39,042,861	38,212,608
Loan payable, current and long-term	9,972,858	9,739,607
Accumulated deficit	(62,720,806)	(47,641,451)
Stockholders' equity	$ 22,754,608	$ 21,571,451

*Condensed from audited financial statements.

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