Axsome Therapeutics, Inc. Logo

Axsome Therapeutics Reports Second Quarter 2018 Financial Results and Provides Business Update

August 8, 2018

Interim analysis results from two Phase 3 trials and topline results from one Phase 2 trial of AXS-05 anticipated in 4Q 2018

Phase 3 trial of AXS-07 in acute migraine anticipated to start in 4Q 2018

Phase 2 results of AXS-05 in smoking cessation anticipated in 1Q 2019

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

NEW YORK, Aug. 08, 2018 (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 second quarter ended June 30, 2018.

“In the second quarter, we advanced our pivotal trials, expanded our pipeline with the initiation of trials in new indications, and highlighted our science at several medical conferences. We expect to continue this momentum during the rest of this year with the anticipated initiation of a Phase 3 trial of AXS-07 in acute migraine, and results from several clinical trials for AXS-05,” said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. “Specifically, we are on track to report results of the interim efficacy analysis of the Phase 3 STRIDE-1 trial of AXS-05 in treatment resistant depression, the interim futility analysis of the Phase 2/3 ADVANCE-1 trial of AXS-05 in Alzheimer’s disease agitation, and topline results from the Phase 2 ASCEND trial of AXS-05 in major depressive disorder, all in the fourth quarter. We now also expect topline results from the Phase 2 trial of AXS-05 in smoking cessation in the first quarter of 2019.”

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 by utilizing proprietary medicinal chemistry and formulation technologies, novel mechanisms of action, and well-characterized molecules, 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 pipeline includes five clinical-stage product candidates.

- **AXS-05:** Axsome is evaluating AXS-05 in four separate indications: treatment resistant depression (TRD), Alzheimer’s disease (AD) agitation, major depressive disorder (MDD), and smoking cessation. AXS-05 is a novel, oral, investigational medicine consisting 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. 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.

  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. To date, more than 50% of the target number of subjects have been randomized.

  In April 2018, an interim futility analysis of the STRIDE-1 study was conducted by an independent data monitoring committee (IDMC) resulting in a positive outcome. Based on the results of the analysis, the IDMC recommended that the trial continue. The IDMC also reviewed the available safety information from the study and indicated that, based on the interim results, AXS-05 appeared safe and well-tolerated.

  A second planned interim analysis will be performed on the first approximately 60% of the target number of subjects to assess efficacy. Results of this next and final interim analysis are expected in the fourth quarter of 2018.

  AD Agitation: Axsome is enrolling 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.

  ADVANCE-1 incorporates two interim analyses to be performed by an IDMC. The first interim analysis will be performed on the first approximately 30% of the target number of subjects to assess the assumptions used to determine the sample size of the study. The second interim analysis will be performed on the first approximately 60% of the target number of subjects to assess efficacy. Results of the first interim analysis are expected in the fourth quarter of 2018.

  MDD: In June 2018, the first patient was enrolled in the ASCEND study, a Phase 2, randomized, double-blind, active-controlled trial of AXS-05 in patients with MDD. Approximately 74 patients will be randomized in a 1:1 ratio to receive

AXS-05 or bupropion for 6 weeks. Assessments that will be conducted throughout the study include safety parameters, the Montgomery-Åsberg Depression Rating Scale (MADRS), other clinician-rated scales, as well as patient-reported outcome measures. Top-line results are anticipated in the fourth quarter of 2018.

Smoking Cessation: In April 2018, the first patient was enrolled in a Phase 2, randomized, double-blind, controlled trial of AXS-05 for smoking cessation treatment. Approximately 60 smokers interested in quitting will be randomized in a 1:1 ratio to receive either AXS-05 or bupropion for 4 weeks. The primary outcome measure is the change in smoking intensity. The trial is being conducted under a research collaboration between Duke University and Axsome Therapeutics. Top-line results are anticipated in first 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, is expected to result in rapid, superior and consistent relief of migraine pain, with lower symptom recurrence, as compared to currently available therapies. Axsome anticipates starting a Phase 3 trial of AXS-07 for the acute treatment of migraine in the fourth quarter of 2018.

- **Other Product Candidates**

AXS-02: AXS-02 (disodium zoledronate tetrahydrate) is a potent osteoclast inhibitor being developed as an oral, non-opioid, targeted, potentially first-in-class therapeutic for chronic pain. AXS-02 is being evaluated in the COAST-1 Phase 3 trial for the treatment of the pain of knee OA associated with BMLs, and has received FDA IND clearance for a Phase 3 trial in chronic low back pain (CLBP) associated with Modic changes (MCs). Patient screening and enrollment in the COAST-1 trial are anticipated to resume after the final readout from the STRIDE-1 trial, as previously disclosed.

AXS-06: AXS-06 (MoSEIC meloxicam and esomeprazole) is a Phase 3-ready, oral, non-opioid, rapidly-absorbed, once-daily, COX-2 preferential pain therapeutic with a gastroprotectant. It is being developed 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-09: AXS-09 is a novel, oral medicine consisting of chirally pure esbupropion and dextromethorphan. Axsome plans to develop AXS-09 for the treatment of CNS disorders.

Scientific Meeting Presentations

- **Alzheimer's Association International Conference (AAIC):** In July 2018, Axsome delivered a poster presentation, at AAIC 2018, held in Chicago, IL. The presentation highlighted data which demonstrated a correlation between AXS-05 drug levels from Phase 1 trials and neurotransmitter receptor activation and improvements in agitation symptoms in patients with Alzheimer's disease.

- **American Society of Clinical Psychopharmacology (ASCP):** In May 2018, Axsome delivered a poster presentation, at the 2018 ASCP Annual Meeting, held in Miami, FL. Analyses were presented demonstrating similarities between AXS-05 and the potent and fast-acting antidepressant ketamine, based on mechanisms of action and functional animal data. Data from a Phase 1 pharmacokinetic trial of AXS-05 was also presented along with analyses showing attainment of drug levels that target CNS receptors relevant to neuropsychiatric disorders.

- **American Psychiatric Association (APA):** In May 2018, Axsome delivered a poster presentation, at the 2018 Annual Meeting of the APA, held in New York, NY. The presentation highlighted the anti-inflammatory properties of AXS-05, the potential role of inflammation in depressive disorders, and pharmacokinetic and pharmacodynamic analyses supporting the development of AXS-05 in treatment resistant depression.

Anticipated Clinical Milestones

- **Clinical Trial Initiations:**
    - Phase 3 clinical trial of AXS-07 in migraine (4Q 2018)
- **Clinical Trial Readouts:**
    - Phase 3 STRIDE-1 trial of AXS-05 in TRD, interim efficacy analysis (4Q 2018)
    - Phase 2/3 ADVANCE-1 trial of AXS-05 in AD agitation, interim analysis (4Q 2018)

- Phase 2 ASCEND trial of AXS-05 in MDD, top-line data (4Q 2018)
- Phase 3 STRIDE-1 trial of AXS-05 in TRD, top-line data (1H 2019)
- Phase 2/3 ADVANCE-1 trial of AXS-05 in AD agitation, interim efficacy analysis (2019)
- Phase 2 trial of AXS-05 in smoking cessation, top-line data (1Q 2019)
- Phase 2/3 ADVANCE-1 trial of AXS-05 in AD agitation, top-line data (2H 2019 – 1H 2020)

Second Quarter 2018 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $5.6 million for the quarter ended June 30, 2018 and $5.0 million for the comparable period in 2017. The increase was primarily due to our STRIDE-1 and ADVANCE-1 studies, and nonclinical work on AXS-05 and AXS-07, which was partially offset by a reduction in the costs of our previously initiated clinical trials and nonclinical work on AXS-02 and AXS-06.
- **General and administrative (G&A) expenses:** G&A expenses were $2.4 million for the quarter ended June 30, 2018 and $1.7 million for the comparable period in 2017. The increase in G&A expenses was primarily due to higher intellectual property 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 $8.3 million, or $(0.32) per share for the quarter ended June 30, 2018, compared to a net loss of $7.1 million, or $(0.30) per share for the comparable period in 2017.
- **Cash:** At June 30, 2018, Axsome had $20.4 million of cash compared to $26.6 million of cash at March 31, 2018.
- **Shares outstanding:** At June 30, 2018, Axsome had 26,252,562 shares of common stock outstanding.
- **Financial guidance:** Axsome believes that its cash at June 30, 2018 will be sufficient to fund the company's anticipated operations, based on its current operating plans, into the third quarter of 2019.

Conference Call Information

Axsome will host a conference call and webcast today at 8:00 AM Eastern to discuss second quarter 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 passcode 6188054. 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 product candidate portfolio includes five clinical-stage candidates, AXS-02, AXS-05, AXS-06, AXS-07, and AXS-09. 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), a Phase 2 trial in Major Depressive Disorder (MDD), and a Phase 2 trial in smoking cessation. AXS-02 is currently in a Phase 3 trial in 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-07 is being developed for the acute treatment of migraine. AXS-06 is being developed for the treatment of osteoarthritis and rheumatoid arthritis and for the reduction of the risk of NSAID-associated gastric ulcers. AXS-02, AXS-05, AXS-06, AXS-07, and AXS-09 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, 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:

	Three months ended June 30,	
	2018	**2017**
Operating expenses:		
Research and development	$ 5,550,532	$ 5,007,361
General and administrative	2,439,061	1,743,377
Total operating expenses	7,989,593	6,750,738
Loss from operations	(7,989,593)	(6,750,738)
Interest and amortization of debt discount (expense)	(292,323)	(333,578)
Change in fair value of warrant liability	1,000	—
Net loss	$ (8,280,916)	$ (7,084,316)
Net loss per common share, basic and diluted	$ (0.32)	$ (0.30)
Weighted average common shares outstanding, basic and diluted	25,791,177	23,595,702

Balance Sheet Information:

	June 30, 2018	**December 31, 2017**
Cash	$ 20,351,443	$ 34,021,123
Total assets	21,725,395	35,555,564
Loan payable, current and long-term	8,470,687	9,932,351
Accumulated deficit	(89,671,318)	(76,584,843)
Stockholders' equity	$ 7,008,899	$ 16,717,223

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