Axsome Therapeutics Reports First Quarter 2020 Financial Results and Provides Business Update

May 8, 2020

Clinical successes highlight Axsome's accelerated evolution into a leading CNS company

Positive pivotal Phase 2/3 results for AXS-05 in Alzheimer's disease agitation further deepen innovative pipeline

Positive efficacy results in 5 significant CNS indications with 4 candidates advance broad late-stage pipeline

Two NDA submissions, for AXS-05 in MDD and AXS-07 in migraine, on track for 4Q 2020

Pre-commercialization activities underway for potentially first-in-class or best-in-class CNS therapies

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

NEW YORK, May 08, 2020 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ: AXSM), a biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, today reported financial results for the first quarter ended March 31, 2020.

"The recent clinical successes in our late-stage pipeline, including positive efficacy data in depression, Alzheimer's disease agitation, migraine, narcolepsy, and fibromyalgia, highlight Axsome's accelerated evolution into a leading, innovative CNS company," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "The ability to tackle unmet medical needs in these serious and difficult-to-treat CNS diseases, underscores the importance of our novel investigational medicines. As we move towards the submission of two NDAs in the fourth quarter, one for AXS-05 in depression and one for AXS-07 in migraine, our commercial team is focused on launch-readiness activities to ensure successful commercial execution. In parallel, we look to continue the momentum in our other late-stage development programs, including AXS-05 in Alzheimer's disease agitation, for which we intend to meet with the FDA after the recently announced positive ADVANCE-1 pivotal trial results in this indication, and AXS-12 in narcolepsy, for which we remain on track to initiate Phase 3 trials later this year."

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 disorders, 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: Axsome remains on track to submit a New Drug Application (NDA) for AXS-05 in MDD to the FDA in the fourth quarter of 2020. The NDA is supported by positive efficacy results from the ASCEND and GEMINI trials. A Phase 3, open-label, long-term safety extension study of AXS-05 in patients with MDD and TRD is ongoing to further support the NDA filing. To date, more than 800 patients have been dosed in this trial.

  In March 2020, Axsome announced results from the Phase 3 STRIDE-1 study, a randomized, double-blind, active-controlled, multicenter, U.S. trial, in patients with confirmed TRD. In this study, AXS-05 met key secondary endpoints by rapidly and statistically significantly improving symptoms of depression as compared to the active comparator bupropion. A second Phase 3 trial of AXS-05 in TRD is planned for the third quarter of 2020.

  AD Agitation: In April 2020, Axsome announced positive results from the Phase 2/3 ADVANCE-1 study, a randomized, double-blind, controlled, multicenter, U.S. trial in patients with AD agitation. In this study, AXS-05 met the primary endpoint by rapidly, substantially, and statistically significantly improving agitation in patients with AD as compared to placebo. Axsome intends to meet with the FDA to discuss these results and next steps in this development program.

  Smoking Cessation: Axsome plans to meet with the FDA in the second half of 2020 to discuss the continued clinical development of AXS-05 as an aid to smoking cessation treatment. Axsome previously announced positive results from a Phase 2 trial of AXS-05 for smoking cessation treatment conducted under a research collaboration between Axsome and Duke University.

- **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: Axsome remains on track to submit an NDA for AXS-07 in the acute treatment of migraine to the FDA in the fourth quarter of 2020. The NDA is supported by positive efficacy results from the MOMENTUM and INTERCEPT trials. A Phase 3, open-label, long-term safety extension study of AXS-07 is ongoing to further support the NDA filing. To date, more than 700 patients have been dosed in this trial.

In April 2020, Axsome announced positive results from the Phase 3 INTERCEPT study, a randomized, double-blind, placebo-controlled, multicenter, U.S. trial, in the early treatment of migraine. In this study, AXS-07 met the two 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 substantially and significantly prevented progression of migraine pain intensity.

- **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: Axsome is on track to initiate Phase 3 trials of AXS-12 in the treatment of narcolepsy in the second half of 2020. Axsome previously announced positive results from the Phase 2 CONCERT study, in which AXS-12 significantly reduced the number of cataplexy attacks and excessive daytime sleepiness as compared to placebo in patients with narcolepsy.

- **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: Axsome plans to meet with the FDA in the second half of 2020 to discuss the further clinical development of AXS-14 for the treatment of fibromyalgia. AXS-14 has previously met the primary endpoints and demonstrated positive and statistically significant results in a Phase 3 and a Phase 2 trial in the treatment of fibromyalgia.

Anticipated Milestones

- **NDA Submissions:**
  - AXS-05 in the treatment of MDD (4Q 2020)
  - AXS-07 in the acute treatment of migraine (4Q 2020)
- **FDA Meetings:**
  - AXS-14 for fibromyalgia (2H 2020)
  - AXS-05 for smoking cessation (2H 2020)
- **Clinical Trial Initiations:**
  - Phase 3 trial of AXS-05 in TRD (3Q 2020)
  - Phase 3 trials of AXS-12 in narcolepsy (2H 2020)
  - Phase 3 trial of AXS-05 in Alzheimer's disease agitation (2H 2020)

First Quarter 2020 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $27.5 million for the quarter ended March 31, 2020 and $7.6 million for the comparable period in 2019. R&D expense in the quarter included a one-time charge of $10.2 million for the Pfizer license agreement, of which $7.2 million was non-cash related. The remaining increase was due primarily to ongoing spend for our active clinical trials in the quarter which included the STRIDE-1, ADVANCE-1, and INTERCEPT trials, close-out costs for our previously completed GEMINI, MOMENTUM, and CONCERT trials, along with costs associated with the AXS-05 and AXS-07 open-label safety studies.

- **General and administrative (G&A) expenses:** G&A expenses were $5.0 million for the quarter ended March 31, 2020 and $2.8 million for the comparable period in 2019. 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 $32.5 million, or $(0.88) per share for the quarter ended March 31, 2020, compared to a net loss of $10.6 million, or $(0.32) per share for the comparable period in 2019.
- **Cash:** At March 31, 2020, Axsome had $197.3 million of cash compared to $220.0 million of cash at December 31, 2019.
- **Shares outstanding:** At March 31, 2020, Axsome had 37,075,422 shares of common stock outstanding.
- **Financial guidance:** Axsome believes that its cash at March 31, 2020 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 with slides today at 8:00 AM Eastern to discuss first quarter 2020 financial results as well as to provide a corporate update. To participate in the live conference call, please dial (833) 579-0911 (toll-free domestic) or (778) 560-2804 (international), and use the conference ID 4088615. 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 biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders for which there are limited treatment options. For the many people facing unsatisfactory treatments for CNS disorders, Axsome accelerates the invention and adoption of life-changing medicines. 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 being developed for major depressive disorder (MDD), treatment resistant depression (TRD), Alzheimer's disease (AD) agitation, and as treatment for smoking cessation. AXS-07 is being developed for the acute treatment of migraine. AXS-12 is being developed for the treatment of narcolepsy. AXS-14 is being developed for 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 discontinuation of 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; unforeseen circumstances or other disruptions to normal business operations arising from or related to COVID-19; 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 March 31, 2020	2019
Operating expenses:		
Research and development	$ 27,521,400	$ 7,603,081
General and administrative	4,970,057	2,818,392
Total operating expenses	32,491,457	10,421,473
Loss from operations	(32,491,457)	(10,421,473)
Interest income (expense)	7,311	(218,903)

Net loss	$ (32,484,146)	$ (10,640,376)
Net loss per common share, basic and diluted	$ (0.88)	$ (0.32)
Weighted average common shares outstanding, basic and diluted	37,061,356	33,052,468

Balance Sheet Information:

	March 31, 2020	December 31, 2019
Cash and cash equivalents	$ 197,313,408	$ 219,966,167
Total assets	197,800,871	220,549,760
Loan payable, current and long-term	20,112,570	19,934,918
Accumulated deficit	(208,379,639)	(175,895,493)
Stockholders' equity	$ 156,156,866	$ 178,722,389

Axsome Contact:
Mark Jacobson
Chief Operating Officer
Axsome Therapeutics, Inc.
200 Broadway, 3rd Floor
New York, NY 10038
Tel: 212-332-3243
Email: mjacobson@axsome.com
www.axsome.com

Source: Axsome Therapeutics, Inc.