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

November 5, 2020

COMET Phase 3 long-term safety trial of AXS-05 in MDD, and MOVEMENT Phase 3 long-term safety trial of AXS-07 in migraine completed

NDA submissions for AXS-05 in depression expected in January 2021, and for AXS-07 in migraine expected in 1Q 2021

Launch readiness activities progressing with buildout of Digital-Centric Commercialization (DCC™) platform

Efficacy results from three Phase 2 open-label efficacy trials of AXS-05 in TRD, antidepressant unresponsive MDD, and suicidal ideation, on track for 4Q 2020

Efficacy results from MOVEMENT Phase 3 open-label trial of AXS-07 in migraine expected in 4Q 2020

Phase 3 trial of AXS-05 in Alzheimer's disease agitation on track for initiation in 4Q 2020

Phase 3 trial of AXS-12 in narcolepsy on track for initiation in 1Q 2021

FDA meeting for AXS-14 in fibromyalgia scheduled for 1Q 2021

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

NEW YORK, Nov. 05, 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 third quarter ended September 30, 2020.

"Over the past several months, we continued to advance our AXS-05 and AXS-07 product candidates towards NDA submissions in major depressive disorder and migraine, and intensified our commercial launch readiness activities," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "We also advanced the rest of our late-stage pipeline holding two positive FDA Breakthrough Therapy designation meetings, one for AXS-05 in Alzheimer's disease agitation, and one for AXS-12 in narcolepsy, resulting in expedited development paths for both programs. Non-dilutive committed capital from our new term loan facility combined with our cash provide financial resources approaching $400 million, positioning us well for our two potential product launches starting next year. Our intellectual property portfolio continues to grow with recently issued and allowed patents for AXS-05 in depression which now provide protection out to 2040. We anticipate an active next few months as we complete our NDA submissions for AXS-05 and AXS-07, release topline efficacy data from open-label trials with these product candidates, initiate Phase 3 trials for AXS-05 in Alzheimer's disease agitation and for AXS-12 in narcolepsy, and meet with FDA on AXS-14 for the treatment of fibromyalgia."

CNS Pipeline Update

For the many people living with serious CNS disorders, Axsome accelerates the invention and adoption of life-changing medicines. The Company is developing a portfolio of differentiated, patent-protected, central nervous system (CNS) product candidates with four 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), Alzheimer's disease (AD) agitation, and smoking cessation. AXS-05 has been granted U.S. Food and Drug Administration (FDA) Breakthrough Therapy designations for MDD, and for AD agitation; as well as Fast Track designations for treatment resistant MDD (TRD), and for AD agitation.

  Depression: Pre-submission activities for Axsome's New Drug Application (NDA) to the FDA for AXS-05 for the treatment of MDD are nearing completion. Due to a COVID-related logistical delay from one vendor, the Company now expects to submit the NDA in January instead of by year-end.

  Axsome has completed the COMET (Clinical Outcomes with NMDA-based Depression Treatment) Phase 3 open-label, long-term safety trial to support the planned NDA filing of AXS-05 in MDD. The three Phase 2 open-label efficacy sub-studies of the COMET trial have also been completed. These sub-studies are evaluating the efficacy and safety of AXS-05 in three clinically pertinent MDD patient populations: the COMET-TRD trial in treatment resistant MDD (TRD), the COMET-AU trial in antidepressant unresponsive MDD, and the COMET-SI trial in MDD with suicidal ideation. Efficacy results from these studies are on track to be reported in the fourth quarter of 2020.

  Axsome is conducting the MERIT (Mechanistic Evaluation of Response in TRD) trial, a Phase 2, double-blind, placebo-controlled, randomized withdrawal study in patients with TRD. Results from the MERIT trial are on track to be reported in the first half of 2021.

  AD Agitation: In June 2020, Axsome received FDA Breakthrough Therapy designation for AXS-05 for the treatment of AD agitation. The designation was supported by the positive results from the pivotal ADVANCE-1 study.

In August 2020, Axsome announced the results of an FDA Breakthrough Therapy meeting for AXS-05 for the treatment of AD agitation. Results of the meeting confirmed the pivotal status of the ADVANCE-1 trial, and the establishment of the superiority of AXS-05 over its components (component contribution) in the treatment of AD agitation. Consequently, only one additional Phase 3 efficacy trial is needed to support the filing of an NDA and only a placebo control will be required for this trial. This additional Phase 3 efficacy trial will be conducted using a randomized-withdrawal design, in which all patients are first treated with open-label AXS-05, with the patients experiencing a treatment response being subsequently randomized in a double-blind fashion to continued treatment with AXS-05 or to switch to placebo. Axsome is on track to initiate this Phase 3 trial this quarter.

Smoking Cessation: Axsome is scheduled to meet with the FDA in the first quarter of 2021 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: Pre-submission activities for the Company's NDA for AXS-07 in the acute treatment of migraine are progressing with major NDA-related items on track for completion by year-end. Axsome now plans to submit the NDA to the FDA in the first quarter of 2021, versus previous guidance of the fourth quarter of 2020, to allow for inclusion of supplemental manufacturing information to ensure a robust submission package.

  Axsome has completed the MOVEMENT (Multimechanistic Treatment Over Time of Migraine Symptoms) Phase 3 open-label, long-term safety trial to support the planned NDA filing of AXS-07 in the acute treatment of migraine. Axsome expects to announce efficacy results from this trial this quarter.

- **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 FDA Breakthrough Therapy designation for the treatment of cataplexy in patients with narcolepsy and Orphan Drug Designation for the treatment of narcolepsy.

  Narcolepsy: In August 2020, Axsome received FDA Breakthrough Therapy designation for AXS-12 for the treatment of cataplexy in patients with narcolepsy. The designation was supported by the positive results from the Phase 2 CONCERT study.

  In September 2020, Axsome announced the expedited development status and plan for AXS-12 for the treatment of narcolepsy following an FDA Breakthrough Therapy meeting. The expedited development plan includes one Phase 3 efficacy trial, which, along with the previously completed Phase 2 CONCERT trial, will be used to support the filing of an NDA for approval of AXS-12 for the treatment of cataplexy in narcolepsy. The planned Phase 3 trial will be a randomized, double-blind, placebo-controlled, parallel-group study. Axsome is on track to initiate this Phase 3 trial in the first quarter of 2021.

- **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 is scheduled to meet with the FDA in the first quarter of 2021 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 (January 2021)
  - AXS-07 in the acute treatment of migraine (1Q 2021)
- **Clinical Trial Readouts:**
  - Phase 2 COMET-TRD trial of AXS-05 in TRD, topline data (4Q 2020)

- Phase 2 COMET-AU trial of AXS-05 in antidepressant unresponsive MDD, topline data (4Q 2020)
- Phase 2 COMET-SI trial of AXS-05 in MDD with suicidal ideation, topline data (4Q 2020)
- Phase 2 MERIT trial of AXS-05 in TRD, topline data (1H 2021)

- **Clinical Trial Initiations:**
  - Phase 3 trial of AXS-05 in AD agitation (4Q 2020)
  - Phase 3 trial of AXS-12 in narcolepsy (1Q 2021)
- **FDA Meetings:**
  - AXS-05 for smoking cessation (1Q 2021)
  - AXS-14 for fibromyalgia (1Q 2021)

Commercial Update

- In November 2020, Axsome and Veeva Systems announced a strategic partnership to augment the build of Axsome's Digital-Centric Commercialization (DCC™) platform. The partnership with Veeva is part of the continuing build of Axsome's DCC platform in preparation for the potential launches of AXS-05 for depression and AXS-07 for migraine. The Axsome DCC platform is a proprietary approach to commercialization that incorporates specialized digital tools, proprietary data and analytics, integrated systems, and an intelligent operating model. Through this digital platform, Axsome aims to optimize physician and patient engagements, enhance engagement quality, and increase the effectiveness of promotional efforts as compared to traditional approaches.

Corporate Update

- In September 2020, Axsome secured a $225 million term loan facility with Hercules Capital. Under the terms of this facility, $60 million can be drawn at closing; $115 million may be drawn at the Company's option, in three separate tranches, upon approval of AXS-05 in MDD, upon approval of AXS-07 in migraine, and upon certain combined sales criteria for AXS-05 and AXS-07; and an additional $50 million is available, subject to the approval of Hercules Capital, to support future strategic initiatives, including further pipeline advancement or expansion. The committed capital strengthens the Company's balance sheet through the anticipated commercial launches of AXS-05 for MDD and AXS-07 for migraine, and extends its cash runway into at least 2024, based on current operating plans.

Third Quarter 2020 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $14.8 million for the quarter ended September 30, 2020 and $15.8 million for the comparable period in 2019. The decrease of $1.0 million was driven by the completion of several clinical trials which were ongoing in the comparable prior period.
- **General and administrative (G&A) expenses:** G&A expenses were $6.3 million for the quarter ended September 30, 2020 and $3.1 million for the comparable period in 2019. The change was primarily due to an increase in stock compensation expense, along with the build-out of the commercial function.
- **Net loss:** Net loss was $22.9 million, or $(0.61) per share, which includes a $1.3 million one-time charge related to the extinguishment of the previous debt facility, for the quarter ended September 30, 2020, compared to a net loss of $19.1 million, or $(0.56) per share for the comparable period in 2019.
- **Cash:** At September 30, 2020, Axsome had $202.4 million of cash compared to $190.7 million of cash at June 30, 2020.
- **Shares outstanding:** At September 30, 2020, Axsome had 37,344,201 shares of common stock outstanding.
- **Financial guidance:** Axsome believes that its cash at September 30, 2020 along with the committed capital from its $225 million term loan facility will be sufficient to fund the Company's anticipated operations, based on its current operating plans, into at least 2024.

Conference Call Information

Axsome will host a conference call and webcast with slides today at 8:00 AM Eastern to discuss third quarter 2020 financial results as well as to provide a corporate update. To participate in the live conference call, please dial (866) 393-4306 (toll-free domestic) or (734) 385-2616 (international), and use the conference ID 4382529. 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 product candidates, AXS-05, AXS-07, AXS-09, AXS-12, and AXS-14. AXS-05 is being developed for major depressive disorder (MDD), 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 September 30, 2020	2019
Operating expenses:		
Research and development	$ 14,795,493	$ 15,835,573
General and administrative	6,331,308	3,111,662
Total operating expenses	21,126,801	18,947,235
Loss from operations	(21,126,801)	(18,947,235)
Interest income (expense)	(551,002)	(327,825)
Tax Credit	—	139,448
Loss on extinguishment of debt	(1,247,012)	—
Net loss	$ (22,924,815)	$ (19,135,612)
Net loss per common share, basic and diluted	$ (0.61)	$ (0.56)
Weighted average common shares outstanding, basic and diluted	37,311,726	34,445,489

Balance Sheet Information:

	September 30, 2020	December 31, 2019
Cash and cash equivalents	$ 202,360,292	$ 219,966,167
Total assets	203,629,404	220,549,760
Loan payable, current and long-term	48,026,108	19,934,918

Accumulated deficit	(249,631,446)	(175,895,493)
Stockholders' equity	$ 135,261,758	$ 178,722,389

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