Axsome Therapeutics Reports First Quarter 2021 Financial Results and Provides Business Update

May 10, 2021

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

NEW YORK, May 10, 2021 (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, 2021.

"We continued to make significant strides in the quarter toward becoming a premier biopharmaceutical company focused on delivering potentially life-changing medicines to people living with serious CNS conditions. We successfully filed our NDA for AXS-05 for the treatment of MDD with the FDA, which was granted Priority Review, and are on track to file our NDA for AXS-07 for the acute treatment of migraine this quarter," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "Pre-commercial activities are intensifying as planned to ensure launch readiness assuming FDA approval. The rest of our pipeline continues to progress with the anticipated initiation of the planned Phase 3 trial for AXS-12 for the treatment of narcolepsy mid-year."

"Axsome is proud to observe May is Mental Health Month and is partnering with advocacy groups to help raise awareness and support for people living with depression and other mental health conditions," continued Dr. Tabuteau. "Recent data from the Centers for Disease Control indicate a more than four-fold increase in the prevalence of depression symptoms in U.S. adults compared to prior to the pandemic. The need for effective mental health treatments therefore has never been greater, and Axsome is committed to developing innovative treatments for those living with the debilitating effects of depression and other mental health conditions."

Business Update

For the many people facing unsatisfactory treatments for CNS disorders, Axsome accelerates the invention and adoption of life-changing medicines. The Company is developing a portfolio of differentiated, patent-protected, CNS product candidates with four in active clinical development.

AXS-05

AXS-05 (dextromethorphan-bupropion) 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.

- **Depression:** Axsome's New Drug Application (NDA) for AXS-05 for the treatment of MDD was granted Priority Review by the FDA. The FDA has set a Prescription Drug User Fee Act (PDUFA) target action date for the NDA of August 22, 2021.
- **AD Agitation:** Axsome initiated the ACCORD study, a Phase 3, double-blind, placebo-controlled, multicenter, randomized withdrawal trial to evaluate the efficacy and safety of AXS-05 in the treatment of Alzheimer's disease (AD) agitation, in December 2020. Enrollment in the trial is progressing.
- **Smoking Cessation:** Axsome is scheduled to meet with the FDA in the third 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:** Axsome is compiling the NDA for AXS-07 for the acute treatment of migraine, which is on track for submission to the FDA in the second quarter of 2021. Axsome plans to announce the FDA's decision regarding its acceptance of the NDA filing in the third quarter of 2021.

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 Orphan Drug designation for the treatment of narcolepsy as well as Breakthrough Therapy designation for the treatment of

cataplexy in patients with narcolepsy.

- **Narcolepsy:** Axsome is planning to initiate a Phase 3 trial of AXS-12 in the treatment of narcolepsy early in the third quarter of 2021. The planned Phase 3 trial will be a randomized, double-blind, placebo-controlled, parallel-group study.

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 met with the FDA in the second quarter to discuss the further clinical development of AXS-14 for the treatment of fibromyalgia, and is awaiting written feedback from the meeting. AXS-14 has previously met the primary endpoints and demonstrated positive and statistically significant results in a Phase 3 and in a Phase 2 trial for the treatment of fibromyalgia.

Commercial and Launch-Readiness Activities

Axsome continues with preparations for a potential 2H 2021 commercial launch of AXS-05 for the treatment of MDD, if approved, and for a subsequent launch of AXS-07 for the acute treatment of migraine, if approved, including technology implementation and team build:

- The foundational technology infrastructure for our Digital Centric Commercialization™ (DCC) platform is now fully implemented, and we continue to build-out our digital capabilities.
- Functional senior leadership across marketing, market access, commercial operations, and sales are in place, and field force hiring at both the manager and representative level has commenced.
- The market access team continues to engage in permitted ongoing discussion with payers, ensuring awareness of Axsome and of AXS-05's product profile, and is actively setting up comprehensive patient support services.

Corporate

- Axsome recently announced that it is joining Mental Health America (MHA) and other advocacy organizations in observing May is Mental Health Month, by raising awareness of and supporting people living with depression and other mental health conditions. MHA is the nation's leading community-based nonprofit organization dedicated to addressing the needs of those living with mental illness.

Anticipated Milestones

- **Regulatory and Commercial:**
  - AXS-05 for MDD, PDUFA target action date (August 22, 2021)
  - AXS-07 for migraine, NDA submission (2Q 2021)
  - AXS-05 for smoking cessation, FDA meeting (3Q 2021)
  - AXS-05 for MDD, commercial launch, if approved (2H 2021)
- **Clinical Trial Readouts:**
  - Phase 2 MERIT trial of AXS-05 in TRD, topline data (2H 2021)
- **Clinical Trial Initiations:**
  - Phase 3 trial of AXS-12 in the treatment of narcolepsy (early 3Q 2021)

Upcoming Scientific Conferences

Axsome is scheduled to present data at the following upcoming scientific conferences:

- International Society for Pharmacoeconomics and Outcomes Research (ISPOR) annual meeting, May 17-20, 2021
- American Society for Clinical Psychopharmacology (ASCP) annual meeting, June 1-4, 2021
- American Headache Society (AHS) annual meeting, June 3-6, 2021

Upcoming Investor Conferences

Axsome is scheduled to participate in the following upcoming investor conferences:

- BofA Health Care Conference, May 11, 2021 (to be webcast)
- RBC Capital Markets Global Healthcare Conference, May 18, 2021 (to be webcast)
- UBS Global Healthcare Virtual Conference, May 24, 2021 (to be webcast)
- William Blair Growth Stock Conference, June 3, 2021 (to be webcast)

First Quarter 2021 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $16.6 million for the quarter ended March 31, 2021 and $27.5 million for the comparable period in 2020. The decrease was driven by a one-time charge of $10.2 million for the Pfizer license agreement in the comparable prior period along with conclusion of several clinical trials, which were ongoing in the comparable prior period. The first quarter included a $2.9 million PDUFA fee related to the NDA submission for AXS-05. This fee will be refunded subsequent to the end of the first quarter, as our Small Business Waiver request for the fee was recently granted by the FDA.
- **General and administrative (G&A) expenses:** G&A expenses were $11.2 million for the quarter ended March 31, 2021 and $5.0 million for the comparable period in 2020. The increase was primarily due to pre-commercial activities along with increased stock compensation expense.
- **Net loss:** Net loss was $29.3 million, or $(0.78) per share, for the quarter ended March 31, 2021 compared to a net loss of $32.5 million, or $(0.88) per share, for the comparable period in 2020.
- **Cash:** At March 31, 2021, Axsome had $164.7 million of cash compared to $183.9 million at December 31, 2020.
- **Shares outstanding:** At March 31, 2021, Axsome had 37,563,882 shares of common stock outstanding.

Financial Guidance

- Axsome believes that its cash at March 31, 2021, along with the remaining committed capital from the $225 million term loan facility, is sufficient to fund anticipated operations, based on the current operating plan, which includes costs for the commercial launch of AXS-05 in MDD and AXS-07 in migraine, into at least 2024.
- Axsome expects that its operating expenses will increase year over year as we continue to build out the commercial function and further advance our pipeline.

Conference Call Information

Axsome will host a conference call and webcast today at 8:00 AM Eastern to discuss first quarter 2021 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 conference ID 6591589. 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. 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, whether potential filing issues or issues identified by FDA during the substantive review may impact the potential approvability of the Company's NDA submission for AXS-05 in MDD or the timing of such approval, and whether the FDA will agree with the Company's discontinuation of the bupropion treatment arm of the ADVANCE study in accordance with the independent data monitoring committee's recommendations); the successful submission of and approval by the FDA of an NDA for AXS-07 for the acute treatment of migraine in adults with or without aura, pursuant to our special protocol assessment for the MOMENTUM clinical trial; 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 amount of capital required for the Company's commercial launch of its product candidates, and the potential impact on the Company's anticipated cash runway; as well as 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,	
	2021	**2020**
Operating expenses:		
Research and development	$ 16,595,689	$ 27,521,400
General and administrative	$ 11,248,372	$ 4,970,057
Total operating expenses	27,844,061	32,491,457
Loss from operations	(27,844,061)	(32,491,457)
Interest and amortization of debt discount (expense)	(1,415,909)	7,311
Net loss	$ (29,259,970)	$ (32,484,146)
Net loss per common share, basic and diluted	$ (0.78)	$ (0.88)
Weighted average common shares outstanding, basic and diluted	37,429,450	37,061,356

Balance Sheet Information:

	March 31, 2021	**December 31, 2020**
Cash and cash equivalents	$ 164,660,132	$ 183,876,453
Total assets	166,903,840	186,134,323
Loan payable, current and long-term	48,597,943	48,321,848
Accumulated deficit	(308,056,063)	(278,796,093)
Stockholders' equity	$ 96,293,199	$ 113,792,909

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