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

August 9, 2021

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

NEW YORK, Aug. 09, 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 second quarter ended June 30, 2021.

"As part of the ongoing review of our NDA for AXS-05, the FDA recently notified us that they have identified deficiencies that preclude labeling discussions at this time. We are attempting to learn the nature of these deficiencies with the goal of addressing them, however, this development may lead to a delay in the potential approval of AXS-05. We will keep you informed as we learn more," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "Our other programs continue to advance. We successfully filed our NDA for AXS-07 for the acute treatment of migraine in the second quarter, and we remain on track to initiate the planned Phase 3 trial of AXS-12 for the treatment of narcolepsy this quarter. The buildout of our team and infrastructure is also continuing as we work towards our goal of delivering potentially life-changing medicines to people living with serious CNS 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 and is currently under review by the FDA. On July 30, 2021, the Company received a letter from the FDA stating that it has identified deficiencies that preclude discussion of labeling and post-marketing requirements/commitments at this time. The letter stated further that the notification does not reflect a final decision on the information under review. The letter did not state what the deficiencies are. In response to the Company's inquiries regarding the nature of the deficiencies since receipt of the letter, the FDA has informed the Company that their review is ongoing, but have not yet communicated any details regarding the nature of the deficiencies. The Prescription Drug User Fee Act (PDUFA) target action date for the NDA is August 22, 2021.

  Axsome has completed the MERIT trial, a Phase 2, randomized, double-blind, placebo-controlled, relapse prevention trial of AXS-05 in treatment resistant depression (TRD) patients. In a separate release issued this morning, Axsome announced that AXS-05 met the primary and key secondary endpoint in the trial. AXS-05 substantially and significantly delayed the time to relapse of depression (p=0.002, primary endpoint), and prevented relapse of depression (p=0.004, key secondary endpoint), compared to placebo.

- **AD Agitation:** Axsome is conducting 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. Axsome anticipates completion of the trial in the fourth quarter of 2022.

- **Smoking Cessation:** Axsome is scheduled to meet with the FDA in the third quarter 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 submitted an NDA for AXS-07 for the acute treatment of migraine in the second quarter. The Company intends to announce the FDA's decision regarding its acceptance of the NDA filing in the third 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 Orphan Drug designation for the treatment of narcolepsy.

- **Narcolepsy:** Axsome is on track to initiate a Phase 3 trial of AXS-12 in the treatment of narcolepsy in the third quarter. 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 management of fibromyalgia. Esreboxetine, the SS-enantiomer of reboxetine, is more potent and selective than racemic reboxetine.

- **Fibromyalgia:** In June 2021, Axsome announced it had completed a pre-NDA meeting with the FDA for AXS-14. Axsome anticipates submitting an NDA for AXS-14 for the management of fibromyalgia in the fourth quarter of 2022, pending successful completion of manufacturing and other activities related to the product candidate. 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 management of fibromyalgia.

Commercial and Launch-Readiness Activities

Axsome continues with preparations for a potential 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:

- Axsome's Digital Centric Commercialization™ (DCC) platform design and implementation is now complete and testing of the platform for execution at launch is underway.
- Axsome's field leadership team is now fully staffed and field force representative hiring has commenced. The Company anticipates having all field representatives on-board by launch.
- 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.

2021 Anticipated Milestones

- **Regulatory and Commercial:**
  - AXS-05 for MDD, PDUFA target action date (August 22, 2021)
  - AXS-07 for migraine, NDA acceptance decision (3Q 2021)
  - AXS-05 for smoking cessation, FDA meeting (3Q 2021)
  - AXS-05 for MDD, commercial launch, if approved (4Q 2021)
- **Clinical Trial Initiations:**
  - Phase 3 trial of AXS-12 in the treatment of narcolepsy (3Q 2021)

Upcoming Scientific Conferences

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

- International Headache Congress (IHC), September 8-12, 2021

Second Quarter 2021 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $14.5 million for the three months ended June 30, 2021 and $10.5 million for the comparable period in 2020. The increase was driven by costs to support the NDA filings and personnel expense which includes an increase in headcount along with an increase in stock compensation expense.
- **General and administrative (G&A) expenses:** G&A expenses were $16.3 million for the three months ended June 30, 2021 and $7.2 million for the comparable period in 2020. The increase was primarily due to pre-commercial activities and

personnel expense which includes an increase in headcount along with an increase in stock compensation expense.

- **Net loss:** Net loss was $32.3 million, or $(0.86) per share, for the three months ended June 30, 2021 compared to a net loss of $18.3 million, or $(0.49) per share, for the comparable period in 2020.
- **Cash:** At June 30, 2021, Axsome had $141.2 million of cash compared to $183.9 million at December 31, 2020.
- **Shares outstanding:** At June 30, 2021, Axsome had 37,648,948 shares of common stock outstanding.

Financial Guidance

- Axsome believes that its cash at June 30, 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 potential 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 second 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 2545435. 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 central nervous system (CNS) conditions that have limited treatment options. Through development of therapeutic options with novel mechanisms of action, we are transforming the approach to treating CNS conditions. At Axsome, we are intensely committed to developing products that meaningfully improve the lives of patients and provide additional therapeutic options for physicians. 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; 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 June 30,		Six Months Ended June 30,	
	2021	**2020**	**2021**	**2020**
Operating expenses:				

Research and development	$ 14,503,326	$ 10,542,957	$ 31,099,014	$ 38,064,357
General and administrative	$ 16,344,361	$ 7,235,877	$ 27,592,734	$ 12,205,934
Total operating expenses	30,847,687	17,778,834	58,691,748	50,270,291
Loss from operations	(30,847,687)	(17,778,834)	(58,691,748)	(50,270,291)
Interest and amortization of debt discount (expense) income	(1,436,522)	(548,158)	(2,852,431)	(540,847)
Net loss	$ (32,284,209)	$ (18,326,992)	$ (61,544,179)	$ (50,811,138)
Net loss per common share, basic and diluted	$ (0.86)	$ (0.49)	$ (1.64)	$ (1.37)
Weighted average common shares outstanding, basic and diluted	37,595,069	37,100,770	37,512,716	37,081,064

Balance Sheet Information:

	June 30, 2021	December 31, 2020
Cash and cash equivalents	$ 141,219,090	$ 183,876,453
Total assets	143,264,750	186,134,323
Loan payable, current and long-term	48,882,599	48,321,848
Accumulated deficit	(340,340,272)	(278,796,093)
Stockholders' equity	$ 71,436,620	$ 113,792,909

Axsome Contact:
Mark Jacobson
Chief Operating Officer
Axsome Therapeutics, Inc.
22 Cortlandt Street, 16th Floor
New York, NY 10007
Tel: 212-332-3243
Email: mjacobson@axsome.com
www.axsome.com

Source: Axsome Therapeutics, Inc.