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

November 8, 2021

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

NEW YORK, Nov. 08, 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 third quarter ended September 30, 2021.

“Over the past several months we have continued to advance our differentiated late-stage CNS product candidates aimed at meaningfully improving the lives of patients. FDA review of our NDA for AXS-05 in depression continues, and the NDA for AXS-07 in migraine was accepted, positioning Axsome to potentially commercialize two new treatments in the near to intermediate term for patients living with these serious CNS disorders,” said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. “Our commercial launch preparations are essentially complete and we remain focused on ensuring timely and successful launches, assuming approvals. The rest of our rich pipeline continues to progress, highlighted by our ongoing Phase 3 ACCORD trial of AXS-05 in Alzheimer’s disease agitation, the recent initiation of our Phase 3 SYMPHONY trial of AXS-12 in narcolepsy, and the ongoing manufacturing work to support the planned NDA filing of AXS-14 for fibromyalgia. In addition, based on FDA Pre-IND meeting guidance, we plan to proceed to a Phase 2/3 trial of AXS-05 in smoking cessation and expect to provide timing on initiation of that trial next year.”

Business Update

Axsome is committed to developing products that meaningfully improve the lives of patients. 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 August 20, 2021, the FDA informed the Company in a teleconference that its review of the NDA would not be completed by the Prescription Drug User Fee Act (PDUFA) target action date of August 22, 2021. The Company was recently informed of two deficiencies related to analytical methods in the Chemistry, Manufacturing, and Controls (CMC) section of the NDA, which must be addressed prior to the FDA taking action on the NDA. The Company believes these deficiencies are addressable and is confirming the details of the request with the FDA.

- **Alzheimer’s Disease 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. Based on current enrollment trends, Axsome now anticipates completion of the trial in the first half of 2023.

- **Smoking Cessation:** Axsome has received from the FDA positive Pre-Investigational New Drug Application (Pre-IND) meeting written guidance from the FDA on a proposed clinical developmental plan for dextromethorphan-bupropion as an aid to smoking cessation. Based on this feedback, Axsome plans to proceed to a pivotal Phase 2/3 trial in this indication. The Company intends to provide information on the timing of initiation of this study in 2022.

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’s NDA for AXS-07 for the acute treatment of migraine was accepted for review by the FDA. The FDA has set a PDUFA target action date for the NDA of April 30, 2022. The FDA notified the Company that, due to COVID-19 pandemic-related travel restrictions, they may be unable to complete a required inspection of a contract manufacturing facility, located in the United States, prior to the PDUFA date, and that they will continue to monitor the public health

situation as well as travel restrictions.

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:** In September 2021, Axsome initiated the SYMPHONY study, a Phase 3 randomized, multicenter, double-blind, placebo-controlled, parallel-group trial of AXS-12 in the treatment of narcolepsy. Enrollment in the trial is progressing and topline results are anticipated in the first half of 2023.

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:** Manufacturing and other activities related to the planned submission of an NDA for AXS-14 for the management of fibromyalgia are ongoing. Based on the status of these activities, the Company now expects to submit the NDA in 2023. 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 is finalizing preparations for a commercial launch of AXS-05 for the treatment of MDD, if approved, and continues with preparations for a launch of AXS-07 for the acute treatment of migraine, if approved:

- Axsome's Digital Centric Commercialization™ (DCC) platform design and technology implementation are complete. To ensure a smooth execution at launch, testing of the integrated technology and tools is on-going.
- With the field leadership team on board, field force activities have been focused on recruitment. At this time, the field force team build is essentially complete with all signed offers contingent upon approval. 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 the product profiles of both AXS-05 and AXS-07.
- All marketing materials and patient support services for AXS-05 are ready for execution in anticipation of a potential approval.
- Commercial activities related to AXS-07 are on-going and progressing accordingly.

Corporate

- In October 2021, Axsome's term loan facility agreement with Hercules Capital, Inc. was amended. The amendment increased the size of the facility to $300 million, increased the amount immediately available upon FDA approval of AXS-05 for MDD to $100 million, and provides access to an additional $150 million thereafter, at the Company's option. The amendment also extends the maturity and interest-only period of the facility.

Anticipated Milestones

- **Regulatory and Commercial:**
  - AXS-05 for MDD, FDA action on NDA
  - AXS-07 for migraine, FDA action on NDA (PDUFA date April 30, 2022)
  - AXS-05 for MDD, commercial launch, if approved
  - AXS-07 for acute migraine, commercial launch, if approved (2022)
  - AXS-14 for fibromyalgia, NDA submission (2023)
- **Clinical Trial Readouts:**
  - Phase 3 SYMPHONY trial of AXS-12 in narcolepsy, topline data (1H 2023)
  - Phase 3 ACCORD trial of AXS-05 for Alzheimer's disease agitation, topline data (1H 2023)

Upcoming Scientific Conferences

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

- American College of Neuropsychopharmacology (ACNP) Annual Meeting, December 5-8, 2021

Third Quarter 2021 Financial Results

- **Research and development (R&D) expenses:** R&D expenses were $13.2 million for the three months ended September 30, 2021 and $14.8 million for the comparable period in 2020. The decrease was driven by completion of clinical trials that were ongoing in the prior comparable period.
- **General and administrative (G&A) expenses:** G&A expenses were $20.2 million for the three months ended September 30, 2021 and $6.3 million for the comparable period in 2020. The increase was primarily related to pre-commercial activities and personnel expense, along with an increase in non-cash stock compensation expense.
- **Net loss:** Net loss was $34.9 million, or $(0.93) per share, for the three months ended September 30, 2021 compared to a net loss of $22.9 million, or $(0.61) per share, for the comparable period in 2020.
- **Cash:** At September 30, 2021, Axsome had $114.6 million of cash compared to $183.9 million at December 31, 2020.
- **Shares outstanding:** At September 30, 2021, Axsome had 37,687,883 shares of common stock outstanding.

Financial Guidance

- Axsome believes that its cash at September 30, 2021, along with the remaining committed capital from the $300 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 it continues to build out the commercial function and further advance its pipeline.

Conference Call Information

Axsome will host a conference call and webcast today at 8:00 AM Eastern to discuss third 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 6365926. 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 committed to developing products that meaningfully improve the lives of patients and provide new 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); whether issues identified by FDA during the substantive review may impact the potential approvability of the Company's 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 September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
Operating expenses:				
Research and development	$ 13,180,258	$ 14,795,493	$ 44,279,272	$ 52,859,850
General and administrative	$ 20,226,884	$ 6,331,308	$ 47,819,618	$ 18,537,242
Total operating expenses	33,407,142	21,126,801	92,098,890	71,397,092
Loss from operations	(33,407,142)	(21,126,801)	(92,098,890)	(71,397,092)
Interest and amortization of debt discount (expense) income	(1,475,535)	(551,002)	(4,327,966)	(1,091,849)
Loss on extinguishment of debt	—	(1,247,012)	—	(1,247,012)
Net loss	$ (34,882,677)	$ (22,924,815)	$ (96,426,856)	$ (73,735,953)
Net loss per common share, basic and diluted	$ (0.93)	$ (0.61)	$ (2.57)	$ (1.98)
Weighted average common shares outstanding, basic and diluted	37,680,966	37,311,726	37,569,416	37,158,513

Balance Sheet Information:

	September 30, 2021	**December 31, 2020**
Cash and cash equivalents	$ 114,622,893	$ 183,876,453
Total assets	116,598,522	186,134,323
Loan payable, current and long-term	49,173,709	48,321,848
Accumulated deficit	(375,222,949)	(278,796,093)
Stockholders' equity	$ 42,717,844	$ 113,792,909

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