Axsome Therapeutics Announces Planned NDA Submission for AXS-14 for the Management of Fibromyalgia

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Submission anticipated in 4Q 2022

NEW YORK, June 15, 2021 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ: AXSM), a biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, today announced its plan to submit a New Drug Application (NDA) for AXS-14 for the management of fibromyalgia following a pre-NDA meeting with the U.S. Food and Drug Administration (FDA). AXS-14 (esreboxetine) is a novel, oral, potent, and highly selective norepinephrine reuptake inhibitor. The NDA submission is currently anticipated in the fourth quarter of 2022 pending successful completion of manufacturing and other activities related to the product candidate. Acceptance of the NDA will be subject to the FDA's review of the complete filing.

"Fibromyalgia is a debilitating central nervous system condition with limited treatment options. Results from two placebo-controlled trials demonstrate the potential for AXS-14 to significantly improve the symptoms of this serious condition," said Herriot Tabuteau, MD, Chief Executive Officer of Axsome. "We are excited by the potential of AXS-14 to expand the treatment options available to patients living with fibromyalgia, and look forward to completing manufacturing and other activities in anticipation of a planned NDA submission in the fourth quarter of next year."

AXS-14 has completed two positive placebo-controlled trials for the management of fibromyalgia, a Phase 3 and a Phase 2 trial, which will be included in the planned NDA submission:

- In the Phase 3 trial, 1,122 patients with fibromyalgia were treated with AXS-14 (esreboxetine) or placebo for 14 weeks. The study met the co-primary endpoints demonstrating statistically significant improvements compared to placebo in the weekly mean pain score ($p<0.001$, $p<0.001$, and $p=0.025$, for the 4 mg, 8 mg and 10 mg daily doses, respectively), and the Fibromyalgia Impact Questionnaire (FIQ) total score ($p<0.001$, $p<0.001$, and $p=0.023$, for the 4 mg, 8 mg and 10 mg doses, respectively). AXS-14 also resulted in statistically significant improvements as compared to placebo on the Patient's Global Impression of Change (PGI-C) scale ($p=0.002$, $p=0.001$, and $p=0.007$, for the 4 mg, 8 mg and 10 mg doses, respectively), and in fatigue as measured using the Global Fatigue Index ($p=0.001$ and $p=0.001$, for the 4 mg and 8 mg daily doses, respectively).

- In the Phase 2 trial, 267 patients with fibromyalgia were treated with AXS-14 (esreboxetine) (dose escalated to 8 mg/day) or placebo for 8 weeks. The study met its primary endpoint demonstrating statistically significant improvements compared to placebo in the weekly mean pain score ($p=0.006$). The study also demonstrated statistically significant improvements in additional efficacy outcomes including the FIQ total score ($p<0.001$), the PGIC scale ($p<0.001$), and fatigue as measured using the Multidimensional Assessment of Fatigue scale ($p<0.001$).

About Fibromyalgia

Fibromyalgia is a chronic debilitating disorder characterized by widespread pain, fatigue, disturbed sleep, depression, and cognitive impairment. Other symptoms of this disorder can include tingling in the hands and feet and headaches. Fibromyalgia has considerable detrimental effects on physical, emotional, social, and day-to-day functioning. Fibromyalgia is considered to be mediated mainly in the central nervous system. Approximately 5 million Americans, 90% of whom are women, are estimated to suffer from fibromyalgia. Treatment options for fibromyalgia are limited with only three pharmacologic treatments currently approved by the FDA.

About AXS-14

AXS-14 (esreboxetine) is a highly selective and potent norepinephrine reuptake inhibitor for the management of fibromyalgia and other conditions. Esreboxetine, the SS-enantiomer of reboxetine, is more potent and selective than racemic reboxetine. AXS-14 is an investigational drug product not approved by the FDA.

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.

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