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Axsome receives FDA Orphan Drug designation for narcolepsy drug

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AXS-12 is an oral, highly selective and potent norepinephrine reuptake inhibitor. The Company plans to initiate a Phase 2 trial of AXS-12 in Q4 with top-line results anticipated in H1 2019.

Singapore - Axsome Therapeutics, a clinical-stage biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, received on Tuesday, October 16, Orphan Drug Designation from the U.S. Food and Drug Administration (FDA) for AXS-12 for the treatment of narcolepsy. Narcolepsy is a serious, debilitating, neurological condition characterized by excessive daytime sleepiness and cataplexy, which is a sudden reduction or loss of muscle tone while a patient is awake. AXS-12 is a novel, oral, highly selective and potent norepinephrine reuptake inhibitor. Yesterday morning, Axsome announced that it plans to initiate a Phase 2 trial of AXS-12 for the treatment of the symptoms of narcolepsy in the fourth quarter of this year with top-line results anticipated in the first half of 2019.

“We are very pleased to have received Orphan Drug Designation from the FDA for AXS-12 on the heels of our recent announcement of this new CNS product candidate for the treatment of narcolepsy,” said Herriot Tabuteau, M.D., Chief Executive Officer of Axsome. “The designation is an important regulatory milestone in the development of AXS-12 for this debilitating condition. We look forward to starting our planned Phase 2 trial of AXS-12 in patients with narcolepsy this quarter.”

Orphan Drug Designation is granted by the FDA Office of Orphan Drug Products to promising drugs intended for the safe and effective treatment of rare diseases, defined as those affecting fewer than 200,000 people in the U.S. This designation may entitle Axsome to a period of seven years of marketing exclusivity in the U.S. upon FDA approval. Orphan Drug Designation also confers special incentives to Axsome including tax credits towards the cost of clinical trials and a waiver of the Company’s obligation to pay the FDA application user fees for the product as required by the Prescription Drug User Fee Act.