



Axovant Announces Negative Topline Results of Intepirdine Phase 3 MINDSET Trial in Alzheimer's Disease

September 26, 2017

--Conference call today at 8:00 a.m. EDT--

BASEL, Switzerland, Sept. 26, 2017 /PRNewswire/ -- Axovant Sciences (NASDAQ: AXON) today announced that the Phase 3 MINDSET clinical trial of its investigational drug intepirdine in patients with mild to moderate Alzheimer's disease (AD) who were receiving background donepezil therapy did not meet its co-primary efficacy endpoints. At 24 weeks, patients treated with 35 mg of intepirdine did not experience improvement in cognition or in measures of activities of daily living as measured by the Alzheimer's Disease Assessment Scale-Cognitive Subscale (ADAS-Cog) and by the Alzheimer's Disease Cooperative Study-Activities of Daily Living scale (ADCS-ADL), respectively, compared to patients treated with placebo. In the study, intepirdine was generally well tolerated.

After 24 weeks of treatment, change from baseline in cognition was non-significantly improved in the intepirdine arm versus the placebo arm (0.36 ADAS-Cog points; p-value = 0.22). In addition, there was essentially no difference between the intepirdine and placebo arms in change from baseline in activities of daily living (0.09 ADCS-ADL points; p-value = 0.83). Of the endpoints analyzed to date, the only endpoint in which any significant improvement was seen in the intepirdine arm versus the placebo arm was in the first key secondary endpoint, the Clinician Interview-Based Impression of Change plus caregiver interview, or CIBIC+ (0.12 CIBIC+ points; p-value = 0.02). The Company will work with investigators to conclude the MINDSET open-label extension study.

"While we are deeply disappointed by these trial results, we also are saddened for the millions of patients and families impacted by Alzheimer's disease. However, we believe that the fight against Alzheimer's and other important areas of unmet need in neurology is too important to be derailed by this setback," said David Hung, M.D., chief executive officer of Axovant. "We are grateful to the investigators, patients and caregivers who participated in this important trial and supported us in this journey. Moreover, we remain committed to advancing our pipeline, which includes our Phase 2b HEADWAY study of intepirdine, and nelotanserin, our highly selective inverse agonist of the 5-HT_{2A} receptor in Phase 2 development, both of which are being evaluated in patients with dementia with Lewy bodies."

The HEADWAY trial studying intepirdine in patients with dementia with Lewy bodies (DLB) remains on track to report topline results at the end of 2017. This study investigates two doses of intepirdine, 35 mg (the dose used in the MINDSET trial) and 70 mg, a higher dose intended to engage both 5-HT₆ and 5-HT_{2A} receptors. Intepirdine has received Fast Track designation from the U.S. Food and Drug Administration for the treatment of DLB.

About MINDSET

The global, randomized, double-blind, placebo-controlled Phase 3 MINDSET trial evaluated the efficacy, safety and tolerability of intepirdine in patients with mild to moderate AD on donepezil therapy over 24 weeks. The trial compared once-daily oral doses of intepirdine 35 mg to placebo in 1,315 patients ages 50 to 85. The Mini-Mental State Examination (MMSE) score at baseline ranged from 10 to 26. Co-primary efficacy endpoints were ADAS-Cog and the ADCS-ADL.

About Alzheimer's Disease

Alzheimer's disease (AD), the most common form of dementia, is a chronic, progressive neurodegenerative disorder that worsens over time. It is the fifth leading cause of death among people age 65 years and older in the United States.^[i] According to the Alzheimer's Association, about 5.5 million people in the U.S. today are affected by AD,^[ii] and that number could triple by 2050.^[iii] In addition to the suffering and devastating effect on quality of life experienced by both patients and caregivers, AD costs the U.S. approximately \$259 billion annually.^[iv] No new chemical entities for AD have been approved by the FDA since 2003.^[v]

About Intepirdine

Intepirdine is an oral, once-daily, investigational drug in development for the treatment of mild to moderate AD and dementia with Lewy bodies (DLB). A potent antagonist of the 5-HT₆ receptor, intepirdine promotes the release of acetylcholine in the brain. This neurotransmitter is believed to be critical for alertness, memory, thought and judgment -- the key components of cognition and function that are impaired in patients with dementia. At higher doses, intepirdine also blocks the 5-HT_{2A} receptor, which is believed to play a role in the psychosis, REM sleep behavior disorder and motor dysfunction seen in patients with DLB.

Teleconference/Webcast Details

To participate in the live conference call today, September 26, at 8:00 a.m. EDT, please dial (352) 672-9956 from the U.S. and Canada or +1 (844) 842-5660 internationally, and use the passcode 89218918. The live call is being webcast and can be accessed on the "Events and Presentations" page of the "Investors" section of the Company's website at <http://investors.axovant.com>. A replay of the webcast will be available for 30 days following the live event.

About Axovant Sciences

Axovant is a clinical-stage biopharmaceutical company focused on developing and commercializing innovative medicines to broadly address multiple forms of dementia and related neurological disorders. Axovant is developing a pipeline of product candidates that focuses on the cognitive, functional and behavioral aspects of debilitating conditions such as Alzheimer's disease, Lewy body dementia and other neurological disorders. For more information, visit www.axovant.com.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding Axovant's plans for the development of its pipeline and completion of the MINDSET open-label extension study, as well as the HEADWAY study. Forward-looking statements can be identified by the words "believe," "anticipate," "continue," "estimate," "project," "expect," "plan," "potential," "intends," "will," "would," "could," "should" or the negative or plural of these words or other similar expressions that are predictions or indicate future events, trends or prospects. Forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially and reported results should not be considered as an indication of future performance. These risks and uncertainties include, but are not limited to: risks associated with the success, cost and timing of our product development activities and clinical trials, increased regulatory requirements, and interim results or other preliminary analyses do not ensure that later or final results in a clinical trial or in related or similar clinical trials will replicate those interim results. There can be no assurance that any of our product candidates will ever receive regulatory approval or be successfully commercialized.

For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to Axovant's business in general, see the "Risk Factors" section of our quarterly report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on August 7, 2017, and other filings that Axovant makes with the SEC from time to time. These forward-looking statements are based on information available to Axovant as of the date of this press release and speak only as of the date of this release. Axovant disclaims any obligation to update these forward-looking statements, except as may be required by law.

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^[i]https://www.alz.org/documents_custom/2017-facts-and-figures.pdf

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^[j]https://www.alz.org/documents_custom/2017-facts-and-figures.pdf

^[ii]<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4095696/>

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