Axovant Announces Clinical Update From First Tay-Sachs Disease Patient Dosed With AXO-AAV-GM2 Gene Therapy

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- Generally well-tolerated with no serious adverse events in a 30-month-old patient with advanced infantile Tay-Sachs disease
- Stable clinical condition from baseline to month 3 without deterioration on neurological exam
- Increase in CSF β-Hexosaminidase A enzyme activity from baseline to month 3, surpassing the 0.5% threshold expected for a clinically important effect
- Axovant to present at the Cowen and Company 39th Annual Health Care Conference on March 11th at 12:00 PM (Eastern Time)

NEW YORK and BASEL, Switzerland, March 11, 2019 (GLOBE NEWSWIRE) -- Axovant (Nasdaq: AXGT), a clinical-stage company developing innovative gene therapies, today reported three-month data from an investigator-initiated study administering investigational AXO-AAV-GM2 gene therapy in a patient with advanced infantile Tay-Sachs disease, a rare and fatal pediatric neurodegenerative genetic disorder characterized by impaired β-Hexosaminidase A enzyme production. AXO-AAV-GM2 is an investigational gene therapy designed to restore β-Hexosaminidase A enzyme activity in the central nervous system. Terence R. Flotte, M.D., professor of pediatrics and dean of the School of Medicine at the University of Massachusetts Medical School, is the principal investigator leading the study under an investigator-initiated Investigational New Drug (IND) application that was cleared by the U.S. Food and Drug Administration (FDA).

The study is evaluating a total dose of 1.0x 10^{14} vg of AXO-AAV-GM2 in a 30-month-old child with advanced infantile Tay-Sachs disease. AXO-AAV-GM2 was administered into the cisterna magna and lumbar spinal canal only. Due to the patient's advanced disease, a co-delivered intrathalamic injection of AXO-AAV-GM2 was not administered. Future patients in the program, who are expected to be treated earlier in their disease course, will receive AXO-AAV-GM2 co-delivered into the thalamus bilaterally as well as into the cisterna magna and spinal canal.

AXO-AAV-GM2 was generally well-tolerated and no serious adverse events have been reported as of the 3-month visit. At 3 months, no clinically relevant laboratory abnormalities were observed following AXO-AAV-GM2 administration.

The patient's clinical condition was stable from baseline to month 3 without clinical deterioration observed on neurological exam. Furthermore, there was no significant deterioration in the condition from the pre-treatment magnetic resonance imaging (MRI) of the brain at baseline to the post-treatment MRI at month 3.

β-Hexosaminidase A activity was determined using the 4MUGS assay, the standard assay for assessing activity of the enzyme. At baseline, the patient's enzyme activity in the cerebrospinal fluid (CSF) was 0.46% of normal. At 3 months, there was an apparent increase in enzyme activity in the CSF to 1.44% of normal, an increase surpassing the 0.5% threshold that could represent a clinically important effect. Serum β-Hexosaminidase A enzyme activity was also increased from baseline at all time points measured following administration of AXO-AAV-GM2. Additional independent assays and sampling will be conducted to further evaluate the biological activity of AXO-AAV-GM2.

"Our first priority has been to identify a vector construct and route of delivery that can reconstitute a clinically important level of enzyme activity in a safe manner," said Dr. Flotte. "This first demonstration of the feasibility of human gene therapy for Tay-Sachs disease is a tribute to the tremendous collaborative effort between the research teams at UMass Medical School and Auburn University, and provides a strong foundation for the transition of this program to the team at Axovant."

"This is the first time a gene therapy has been administered to a child with Tay-Sachs disease, and it is remarkable that we have not only seen good safety and tolerability to date, but also evidence of functional β-Hexosaminidase A enzyme activity," said Dr. Gavin Corcoran, Executive Vice President of Research and Development at Axovant. "These encouraging early clinical results suggest that AXO-AAV-GM2 may offer a meaningful treatment option for patients who currently have no approved therapies."

Pavan Cheruvu, M.D., chief executive officer of Axovant, will present at the Cowen and Company 39th Annual Health Care Conference on March 11, 2019 at 12:00 PM (Eastern Time). A copy of the Company's presentation slides and link to a live webcast of the presentation can be found below.

http://investors.axovant.com/investors

About AXO-AAV-GM2

AXO-AAV-GM2 is an investigational gene therapy for GM2 gangliosidosis (also known as Tay-Sachs and Sandhoff diseases), a set of rare and fatal pediatric neurodegenerative genetic disorders caused by defects in the *HEXA* (leading to Tay-Sachs disease) and *HEXB* (leading to Sandhoff disease) genes that encode the two subunits of the β-hexosaminidase A (Hex A) enzyme. These genetic defects lead to neurodegeneration and shortened life expectancy. AXO-AAV-GM2 aims to restore Hex A function by introducing a functional copy of the *HEXA* and *HEXB* genes via delivery of two co-administered AAVrh8 vectors.

About Axovant

Axovant, part of the Roivant family of companies, is a clinical-stage gene therapy company focused on developing a pipeline of innovative product

candidates for debilitating neurological and neuromuscular diseases. The company's current pipeline of gene therapy candidates targets GM1 gangliosidosis, GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), Parkinson's disease, oculopharyngeal muscular dystrophy (OPMD), amyotrophic lateral sclerosis (ALS) and frontotemporal dementia. Axovant is focused on accelerating product candidates into and through clinical trials with a team of experts in gene therapy development and through external partnerships with leading gene therapy organizations. For more information, visit www.axovant.com.

About Roivant

Roivant Sciences aims to improve health by rapidly delivering innovative medicines and technologies to patients. It does this by building Vants – nimble, entrepreneurial biotech and healthcare technology companies with a unique approach to sourcing talent, aligning incentives, and deploying technology to drive greater efficiency in R&D and commercialization. For more information, please visit www.roivant.com.

Forward Looking Statements and Information

This press release contains forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995 and other federal securities laws. The use of words such as "may," "might," "will," "expect," "plan," "anticipate," "believe," "intend," "future," or "continue" and other similar expressions are intended to identify forward-looking statements. For example, all statements Axovant makes regarding the following are forward looking: the success and timing of its ongoing development of AXO-AAV-GM2; the anticipated start dates, durations and completion dates of its ongoing and future clinical trials; the anticipated designs of its future clinical studies; and the success of its interactions with the FDA. In addition, promising interim results or other preliminary analyses do not in any way ensure that later or final results in a clinical trial or in related or similar clinical trials will replicate those interim results. The product candidate discussed is investigational and not approved and there can be no assurance that the clinical programs will be successful in demonstrating safety and/or efficacy, that Axovant will not encounter problems or delays in clinical development, or that the product candidate will ever receive regulatory approval or be successfully commercialized. All forward-looking statements are based on estimates and assumptions by Axovant's management that, although Axovant believes to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that Axovant expected. Such risks and uncertainties include, among others, the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the expectations for regulatory submissions and approvals; and the availability or commercial potential of product candidates. These statements are also subject to a number of material risks and uncertainties that are described in Axovant's most recent Quarterly Report on Form 10-Q for the quarterly period ended December 31, 2018, filed with the Securities and Exchange Commission on February 7, 2019, as updated by its subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Axovant undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise.

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