



Axovant Announces Dosing of First Patient in Clinical Program for AXO-AAV-GM1, a Novel Gene Therapy for GM1 Gangliosidosis

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- Milestone marks the first patient with GM1 gangliosidosis treated with gene therapy-

-Initial data expected in second half of 2019-

-AXO-AAV-GM1 is Axovant's third investigational gene therapy program to enter the clinic-

BASEL, Switzerland, May 16, 2019 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (Nasdaq: AXGT), a clinical-stage company developing innovative gene therapies, today announced dosing of the first patient in a clinical study of AXO-AAV-GM1 (also known as AAV9-GLB1), an investigational gene therapy for the treatment of GM1 gangliosidosis. To date, the patient has experienced no complications related to the intravenous (IV) administration of the vector and continues to be followed by physicians. GM1 gangliosidosis is a progressive and fatal pediatric lysosomal storage disorder caused by mutations in the *GLB1* gene leading to impaired production of the beta-galactosidase enzyme. There are currently no approved treatments for GM1 gangliosidosis. Axovant expects initial data from the AXO-AAV-GM1 clinical program in the second half of 2019, as well as continued enrollment of patients in this program throughout 2019.

The clinical study of AXO-AAV-GM1 is being conducted at the National Institutes of Health (NIH), by Cynthia Tifft, M.D., Ph.D., Deputy Clinical Director at the National Human Genome Research Institute, and a leading expert in ganglioside storage disorders. A three-way Cooperative Research and Development Agreement (CRADA) between Axovant, the NIH, and the University of Massachusetts (UMass) Medical School has been established to support the conduct of the clinical program.

"GM1 gangliosidosis is a devastating disease in young children, for which there are no currently approved treatment options. The development of a safe and effective gene therapy for these patients would be a welcome advancement in the field of pediatric lysosomal storage disorders affecting the brain," said Dr. Tifft.

The endpoints of the clinical study include safety, biomarker, neurodevelopment, and Magnetic Resonance Imaging (MRI) and Magnetic Resonance Spectroscopy (MRS) measures.

AXO-AAV-GM1 delivers a functional copy of the *GLB1* gene via an adeno-associated viral (AAV) vector, with the goal of restoring β -galactosidase enzyme activity for the treatment of GM1 gangliosidosis. The gene therapy is delivered intravenously, which has the potential to broadly transduce the central nervous system and treat peripheral manifestations of the disease as well. Preclinical studies in murine and a naturally-occurring feline model of GM1 gangliosidosis have supported AXO-AAV-GM1's ability to improve β -galactosidase enzyme activity, reduce GM1 ganglioside accumulation, improve neuromuscular function, and extend survival.

About Axovant Gene Therapies

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," "will," "expect," "would," "intend," "future," and other similar expressions are intended to identify forward-looking statements. 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. In addition, Axovant's business is subject to additional risks and uncertainties, including among others, the initiation and conduct of preclinical studies and clinical trials; the timing and availability of data from clinical trials; the expectations for regulatory submissions and approvals; the potential safety concerns or profile of Axovant's product candidates; 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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