Axovant Provides Clinical Program Update for AXO-AAV-GM1, a Novel Investigational Gene Therapy for GM1 Gangliosidosis

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NEW YORK and BASEL, Switzerland, Dec. 19, 2019 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (NASDAQ: AXGT) today announced preliminary findings from an expanded access treatment in which a single patient with GM1 gangliosidosis was administered investigational AXO-AAV-GM1 gene therapy.

GM1 gangliosidosis is a progressive and fatal pediatric lysosomal storage disorder caused by mutations in the *GLB1* gene leading to impaired production of the β-galactosidase enzyme. AXO-AAV-GM1 is an investigational gene therapy that delivers a functional copy of the *GLB1* gene via an adeno-associated viral (AAV9) vector, with the goal of restoring β-galactosidase enzyme activity. The natural history of GM1 gangliosidosis demonstrates progressive neurological decline including worsening of swallowing ability, which impacts nutritional status, and impaired motor function.

"GM1 gangliosidosis is a devastating pediatric disease for which there are no currently approved treatment options," said Dr. Cynthia Tifft, Deputy Clinical Director at the National Human Genome Research Institute (NHGRI), part of the National Institutes of Health (NIH), and a leading expert in ganglioside storage disorders. "These preliminary findings suggest safety, tolerability, and clinical improvement in the first child dosed with gene therapy for GM1 gangliosidosis. I am particularly encouraged that this child has regained her ability to swallow more effectively and has demonstrated meaningful weight gain during the 6 months following gene therapy. We are hoping to complete enrollment in Part A of the ongoing registrational study in early 2020."

According to Dr. Tifft, from baseline gene transfer to 6-month follow-up, the subject was observed to have clinically significant improvements based on neurological exam, the Vineland-3 scale, Clinical Global Impression (CGI) assessments, and nutritional status. The Vineland-3 scale is an individually administered measure of adaptive behavior that is widely used to assess individuals with intellectual, developmental, and other disabilities.

In addition, Dr. Tifft observed that AXO-AAV-GM1 was generally well tolerated with no reports of serious adverse events related to the investigational gene therapy or intravenous (IV) administration of the vector.

A separate registrational study of AXO-AAV-GM1 is also being conducted at the NIH Clinical Center by Dr. Tifft in collaboration with Axovant Gene Therapies. Part A of the registrational study of AXO-AAV-GM1 (n=5) evaluates safety, tolerability, and exploratory measures of efficacy.

AXO-AAV-GM1 was granted orphan drug designation (ODD) by the U.S. Food and Drug Administration (FDA) in November 2019.

About AXO-AAV-GM1

AXO-AAV-GM1 is an investigational gene therapy that 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.

In 2018, Axovant licensed exclusive worldwide rights from the University of Massachusetts Medical School for the development and commercialization of gene therapy programs for GM1 gangliosidosis and GM2 gangliosidosis, including Tay-Sachs and Sandhoff diseases. A three-way Cooperative Research and Development Agreement (CRADA) between Axovant, the NHGRI, and the University of Massachusetts was established earlier this year to support the conduct of the clinical program.

About Axovant Gene Therapies

Axovant Gene Therapies, 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 neurodegenerative diseases. Our current pipeline of gene therapy candidates targets GM1 gangliosidosis, GM2 gangliosidosis (including Tay-Sachs disease and Sandhoff disease), and Parkinson's disease. 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.

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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," "would," "should," "expect," "believe," "estimate," and other similar expressions are intended to identify forward-looking statements. For example, all statements Axovant makes regarding the initiation, timing, progress, and reporting of results of clinical trials and research and development programs; its ability to advance its gene therapy product

candidates into and successfully initiate, enroll, and complete clinical trials; the potential clinical utility of its product candidates; its ability to continue to develop its gene therapy platforms; are forward-looking. 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; the continued development of product candidates; Axovant's scientific approach and general development progress; and the availability or commercial potential of Axovant's 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 filed with the Securities and Exchange Commission on November 8, 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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