Axovant Completes Enrollment of Low-Dose Cohort in Phase 1/2 Study of AXO-AAV-GM1 and Expands Study to Include Type I (Infantile Onset) Patients with GM1 Gangliosidosis

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- *Six-month data from low-dose cohort expected in Q4 2020*

- *IND amended to include Type I (infantile onset) patients and to evaluate a higher dose*

- *Expect to initiate high-dose cohort in 2H 2020*

NEW YORK and BASEL, Switzerland, June 08, 2020 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (NASDAQ: AXGT), a clinical-stage company developing innovative gene therapies for neurological diseases, today announced that it has completed enrollment in the low-dose cohort of the Phase 1/2 ("Stage 1") study for Type II (late infantile and juvenile onset) GM1 patients evaluating safety, tolerability, and exploratory measures of efficacy at a dose of $1.5x10^{13}$ vg/kg delivered intravenously. Currently, the study is on track to report 6-month data (n=5) from the low-dose cohort of the AXO-AAV-GM1 clinical trial by Q4 2020. Additionally, the investigational new drug (IND) filing has been amended to expand the program to include Type I (infantile) patients and to evaluate a 3-fold higher dose ($4.5x10^{13}$ vg/kg). The Company expects to initiate dosing in the high-dose cohort, which will include both Type I and Type II patients, in the second half of 2020.

"The successful enrollment of the low-dose cohort of the ongoing Phase 1/2 study amidst the COVID-19 pandemic speaks to the dedication of all involved and the significant unmet need that exists for these children," said Dr. Gavin Corcoran, chief R&D officer. "With an expanded study protocol that now includes infantile-onset patients, AXO-AAV-GM1 is the only gene therapy in development to include both Type I and Type II GM1 patients, populations of children who suffer from a deficiency in the same enzyme, β-galactosidase. We are grateful for the collaboration and perseverance of the National Institutes of Health (NIH), patients, and their families to advance efforts towards finding a treatment for this devastating pediatric disease."

This study is being conducted at the NIH under the direction of Dr. Cynthia Tifft, Deputy Clinical Director at the National Human Genome Research Institute (NHGRI) in collaboration with Axovant Gene Therapies. In late 2019, the Company presented an update from the first GM1 Type II child dosed with AXO-AAV-GM1 under an expanded access protocol who was observed to have clinically significant improvements from baseline gene transfer to six-month follow-up based on neurological exam, the Vineland-3 scale, Clinical Global Impression assessments, and nutritional status. In addition, AXO-AAV-GM1 was observed to be generally well-tolerated with no reports of serious adverse events related to the investigational gene therapy or intravenous administration of the vector.

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. There are currently no approved treatments for GM1 gangliosidosis.

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.

About Axovant Gene Therapies

Axovant Gene Therapies 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.

In 2018, Axovant licensed exclusive worldwide rights from the University of Massachusetts Medical School (UMMS) 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) among Axovant, the NHGRI, and the University of Massachusetts was established in 2019 to support the conduct of the clinical program.

About the University of Massachusetts Medical School

The mission of the University of Massachusetts Medical School is to advance the health and well-being of the people of the commonwealth and the world through pioneering education, research, public service and health care delivery.

Research into potential therapies for lysosomal storage diseases such as Tay-Sachs, Sandhoff disease and GM1 gangliosidosis at UMass Medical School and Auburn University has led to significant advances in the field. Miguel Sena-Esteves, PhD, associate professor of neurology at UMass Medical School; Heather Gray-Edwards, PhD, DVM, formerly of Auburn and currently assistant professor of radiology at UMass Medical School; and Douglas Martin, PhD, professor of anatomy, physiology and pharmacology in the College of Veterinary Medicine and the Scott-Ritchey Research

Center at Auburn University, have worked collaboratively for more than a decade on animal models and therapeutic approaches for these and similar disorders. For more information, visit www.umassmed.edu.

Forward-Looking Statements

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," "anticipate," "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 clinical data for its clinical programs, 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 the AXO-AAV-GM1 program and the availability of data for disclosures; Axovant's scientific approach and general development, manufacturing and regulatory progress; Axovant's ability to perform under its existing clinical and business collaborations; and risks of unforeseen operational delays and other uncertainties caused by the COVID-19 pandemic. 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 February 10, 2020, 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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