Axovant Gene Therapies Receives Orphan Drug Designation from FDA for AXO-AAV-GM1 for the Treatment of GM1 Gangliosidosis

November 18, 2019

NEW YORK and BASEL, Switzerland, Nov. 18, 2019 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (NASDAQ: AXGT), a clinical-stage company developing innovative gene therapies, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for the Company's investigational gene therapy, AXO-AAV-GM1, for the treatment of GM1 gangliosidosis. 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. The Company is planning to complete enrollment of patients in Part A of the AXO-AAV-GM1 registrational program evaluating safety and efficacy and expects to announce 6-month data from Part A in mid-2020.

"We are pleased to receive orphan drug designation for our GM1 gangliosidosis program as we advance the development of this potentially transformative, one-time gene therapy," said Dr. Gavin Corcoran, chief R&D officer at Axovant. "There are currently no FDA approved therapies for patients with GM1 gangliosidosis, and the orphan drug designation underscores the significant unmet medical need. We look forward to increased interaction with the FDA to facilitate the development of AXO-AAV-GM1 and address this unmet need."

FDA orphan drug designation is granted to investigational therapies which are intended for the safe and effective treatment, diagnosis or prevention of rare diseases or conditions that affect fewer than 200,000 people in the United States. Orphan drug designation provides several benefits to drug developers, which in addition to others, include increased FDA interaction, eligibility for 7-year market exclusivity, potential for tax credits towards the cost of clinical trials, and prescription drug user fee waivers at the time of filing a biologics license application (BLA). For more information about orphan drug designation, please visit the FDA website at www.fda.gov.

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, 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.

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.

About Roivant

Roivant aims to improve health by rapidly delivering innovative medicines and technologies to patients. Roivant does this by building Vants – nimble, entrepreneurial biotech and healthcare companies with a unique approach to sourcing talent, aligning incentives, and deploying technology to drive greater efficiency in R&D and commercialization. Roivant today is comprised of a central technology-enabled platform and 20 Vants with over 45 investigational medicines in clinical and preclinical development and multiple healthcare technologies. 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 its preclinical programs, clinical trials, and research and development programs; cash to be used in operating activities; 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; its ability to develop and manufacture its products and successfully transition manufacturing processes; its ability to perform under existing collaborations with, among others, Oxford BioMedica and the University of Massachusetts Medical School, and to add new programs to its pipeline; its ability to enter into new partnerships or collaborations; its ability to retain and successfully integrate its leadership and personnel; and the timing or likelihood of its regulatory filings and approvals 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 its small molecule and gene therapy product candidates and platforms; 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.

Contacts:

Media and Investors

Parag Meswani
Axovant Gene Therapies
(212) 547-2523
investors@axovant.com
media@axovant.com

Source: Axovant Sciences Ltd.