Axovant Gene Therapies Receives Rare Pediatric Disease Designation for AXO-AAV-GM2 for Tay-Sachs and Sandhoff Disease

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NEW YORK, Oct. 13, 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 received Rare Pediatric Disease Designation from the U.S. Food and Drug Administration (FDA) for AXO-AAV-GM2, a one-time gene therapy delivered directly to the central nervous system that is in development for GM2 gangliosidosis, also known as Tay-Sachs and Sandhoff disease. In addition to the Rare Pediatric Disease designation, AXO-AAV-GM2 has Orphan Drug Designation (ODD) and is the first gene therapy that has been administered to children with Tay-Sachs disease.

“We are thrilled to bring AXO-AAV-GM2 one step closer to patients in need through this Rare Pediatric Disease designation. AXO-AAV-GM2 has the potential to be the first treatment approved for Tay-Sachs and Sandhoff disease, rare and fatal pediatric diseases with no current treatment options,” said Sean O’Bryan, Senior Vice President, Regulatory Affairs & Quality.

Axovant expects to evaluate AXO-AAV-GM2 in a registrational clinical trial which consists of a Stage 1 dose-ranging study and a Stage 2 efficacy study. Previously, Axovant reported the first evidence for potential disease modification in Tay-Sachs disease from an expanded access study administering investigational AXO-AAV-GM2 gene therapy in two patients with infantile (Type I) Tay-Sachs disease. AXO-AAV-GM2 was successfully administered in both patients and has been generally well-tolerated to date, with no serious adverse events or clinically relevant laboratory abnormalities related to therapy.

GM2 gangliosidosis, also known as Tay-Sachs and Sandhoff disease, is a rare and fatal pediatric neurodegenerative lysosomal storage disorder (LSD) resulting from deficiencies in beta-hexosaminidase, a key enzyme in the lysosome. These genetic defects lead to the toxic accumulation of gangliosides, resulting in neurodegeneration and life expectancy shortened to just two to four years of age.

The FDA defines a rare pediatric disease as a serious or life-threatening disease in which the disease manifestations primarily affect individuals aged from birth to 18 years. Pediatric diseases recognized as “rare” affect under 200,000 people in the United States.

About AXO-AAV-GM2

AXO-AAV-GM2 is an investigational gene therapy for Tay-Sachs and Sandhoff disease, which rare and fatal pediatric neurodegenerative genetic disorders within the GM2 gangliosidosis family, caused by defects in the *HEXA* (leading to Tay-Sachs disease) or *HEXB* (leading to Sandhoff disease) genes that encode the two subunits of the β-hexosaminidase A (HexA) enzyme. Both forms of GM2 gangliosidosis are caused by overwhelming storage of GM2 ganglioside within neurons throughout the central nervous system), which is normally degraded in the lysosome by the isozyme HexA. These genetic defects lead to progressive neurodegeneration and shortened life expectancy. AXO-AAV-GM2 aims to restore HexA levels by introducing a functional copy of the *HEXA* and *HEXB* genes via delivery of two co-administered AAVrh8 vectors.

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 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 (also known as 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.

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 “intended”, "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 costs associated with its operating activities 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 impact of the Covid-19 pandemic on our operations, the initiation and conduct of preclinical studies and clinical trials; the availability of data from clinical trials; the scaling up of manufacturing, the expectations for regulatory submissions and approvals; the continued development of our 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 August 11, 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.

Contacts:

Media & Investors

Josephine Belluardo, Ph.D.
LifeSci Communications
646-751-4361
jo@lifescicomms.com
media@axovant.com

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