



## Axovant Announces Upcoming Presentations at the Annual Meeting of the American Society of Gene and Cell Therapy

May 7, 2020

NEW YORK and BASEL, Switzerland, May 07, 2020 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd. (NASDAQ: AXGT), a clinical-stage company developing innovative gene therapies for neurological diseases, today announced upcoming presentations regarding its investigational gene therapy programs, AXO-Lenti-PD and AXO-AAV-GM2 at the 23<sup>rd</sup> Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT), held virtually on May 12<sup>th</sup> to May 15<sup>th</sup>, 2020.

Oral Presentation Details:

**Oral Presentation:** First-In-Human Gene Therapy for Tay-Sachs Disease: Report of Two Infants Treated on an Expanded Access Clinical Trial of rAAVrh8-HexA/HexB (AXO-AAV-GM2)

**Abstract Number:** 1299

**Presenter:** Terence Flotte, MD, Provost, Executive Deputy Chancellor and Dean, School of Medicine, University of Massachusetts Medical School and Principal Investigator

**Session:** Clinical Trials Spotlight Symposium

**Date and Time:** May 15, 2020, 8:30-8:45am

**Invited Lecture:** AXO-Lenti-PD: A Trans-Atlantic Collaboration on Gene Therapy for Parkinson's Disease

**Presenter:** Gregory R Stewart, PhD, SVP, Scientific Affairs

**Session:** Gene and Cell Therapeutic Strategies to Treat Disorders of the CNS – An International Perspective

**Date and Time:** May 15, 2020, 8:35-9:10am

Poster Presentation Details:

**Title:** Surgical Technique for Bilateral Intrathalamic Infusion of rAAVrh8-HEXA/HEXB Gene Therapy in Infant with Tay-Sachs Disease

**Abstract Number:** 666

**Presenter:** Dr. Oguz Cataltepe, MD, Professor of Neurological Surgery and Pediatrics, University of Massachusetts Medical School; Director of Pediatric Neurosurgery, UMass Memorial Medical Center

**Session:** Synthetic/Molecular Conjugates and Physical Methods for Delivery

**Date and Time:** May 13, 2020, 5:30-6:30pm

Additional information on the meeting can be found on the ASGCT website: <http://www.asgct.org>

### 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](http://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. A three-way Cooperative Research and Development Agreement (CRADA) among Axovant, the NHGRI, and the University of Massachusetts Medical School 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-Estevés, 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](http://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," "might," "will," "would," "should," "expect," "believe," "estimate," 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. 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 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 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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Source: Axovant Sciences Ltd.