Axovant Announces Partnership with Invitae to Increase Access to Genetic Testing and Accelerate Diagnoses of GM1 and GM2 Gangliosidosis

April 15, 2020

- *Partnership to reduce barriers to genetic diagnosis through sponsored testing for lysosomal storage diseases*
- *Initiative supports identification of patients with GM1 and GM2 gangliosidosis who are currently misdiagnosed or undiagnosed*

NEW YORK and BASEL, Switzerland, April 15, 2020 (GLOBE NEWSWIRE) -- Axovant Gene Therapies Ltd., a clinical-stage company developing innovative gene therapies for neurological diseases, today announced its collaboration with Invitae, a leading medical genetics company, in the Detect Lysosomal Storage Diseases ("Detect") program to facilitate faster diagnoses for children with lysosomal storage disorders ("LSDs"), including GM1 gangliosidosis and GM2 gangliosidosis, also known as Tay-Sachs/Sandhoff disease. Invitae offers genetic testing and counseling at no charge to patients suspected of having an LSD.

"Axovant is committed to developing novel gene therapies for those living with rapidly progressive neurodegenerative diseases. We are hopeful that our collaboration with Invitae will provide families with easier access to genetic testing and bring us one step closer to identifying patients who may benefit from potential therapies," said Parag Meswani, PharmD., Axovant's SVP of Commercial Strategy & Operations. "Our AXO-AAV-GM1 clinical program targeting GM1 gangliosidosis is currently enrolling at the National Institutes of Health, and we are seeking IND clearance for the AXO-AAV-GM2 clinical trial targeting Tay-Sachs and Sandhoff diseases. Early intervention is ideal with potentially disease-modifying genetic therapies, and our diagnostics partnership with Invitae should allow us to identify and enroll children at even earlier stages of disease progression."

LSDs are progressive, multi-system, inherited metabolic diseases associated with premature death, and genetic testing is a crucial first step to arriving at a diagnosis. LSDs are misdiagnosed or undiagnosed in the majority of patients. The Detect program includes a specific LSD testing panel of 53 genes designed to provide patients and families accurate information quickly to preserve valuable treatment time.

"Genetic testing can expedite an accurate diagnosis, facilitate earlier interventions, allow genetic counseling of family members, and support clinical research for LSDs such as GM1 and GM2 gangliosidosis," said Robert Nussbaum, M.D., chief medical officer of Invitae. "We're pleased Axovant has joined the Detect program to help offer no-charge, sponsored genetic testing for those patients suspected of having the disease."

Research has shown no-charge testing programs with large well-designed panels help increase utilization of genetic testing, which can shorten the time to diagnosis by as much as 2 years in some conditions. Accurate diagnoses enable clinicians to focus on providing disease-specific care sooner, helping reduce costs and improve outcomes.[1]

Additional details, as well as terms and conditions of the program, can be found at https://www.invitae.com/en/detectLSDs/.

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.

About Invitae

Invitae Corporation (NYSE: NVTA) is a leading medical genetics company, whose mission is to bring comprehensive genetic information into mainstream medicine to improve healthcare for billions of people. Invitae's goal is to aggregate the world's genetic tests into a single service with higher quality, faster turnaround time, and lower prices. For more information, visit the company's website at invitae.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 "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 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.

1. Miller, Nicole, et al, "Behind the Seizure: A No-Cost 125-gene Epilepsy Panel for Pediatric Seizure Onset Between 2–4 Years". Presented at the American Society of Human Genetics Meeting: October 16–20, 2018, San Diego, CA.

Media Contact:

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

Source: Axovant Sciences Ltd.