national tay-sachs & allied diseases association

RESEARCH REVIEW | Special Edition 3-11-19

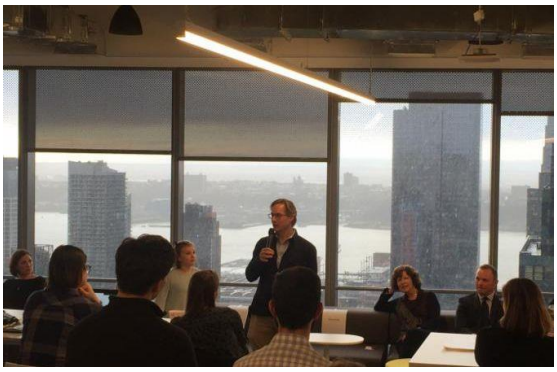
Significant news from Axovant

Axovant issued a press release on March 11, 2019 with an update on a patient that was treated last fall with the AXO-AAV-GM2 gene therapy. In summary, the news is encouraging.

- The treatment was well-tolerated by the 30 month old patient.
- The patient is stable with no sign of deterioration.
- HexA enzyme activity increased enough for a clinically important effect.

It is the first time a gene therapy has been administered to a child with Tay-Sachs disease, and Axovant is encouraged by these early clinical results, which will help inform the design of our future clinical study for AXO-AAV-GM2.

Read the full press release here, and stay tuned to NTSAD's Research Reviews, our Facebook page and website for updates and announcements in the months ahead.



NTSAD dad and past president, Tim Lord, sharing his experience with the team at Axovant in January 2019.

Axovant will be at the NTSAD 41st Annual Family Conference in Raleigh, NC sharing updates, meeting families and learning more about the family experience with GM1 Gangliosidosis and GM2 Tay-Sachs and Sandhoff.

To register for the conference, email Becky, Conference Coordinator, **here**.

Auburn University Live Stream - Tuesday, March 12th

Building a Brighter Future for Rare Brain Disease Patients

There will be talks by families, researchers and biotech who are part of the NTSAD family. See agenda and links below. Anyone can watch part of the program on live-stream video.

Watch the event live-stream video on Tuesday, March 12th from 10 a.m. until noon.

10:00 - 10:15 a.m.
Welcome and Introduction
Auburn College of Veterinary Medicine Dean Calvin Johnson

10:15 - 10:45 a.m.
Family stories from
Alexis Buryk and Katie Buryk
Rojan Vakili
Sara and Michael Heatherly

10:45 - 11:15 a.m.
Research Presentations
Doug Martin, “Gene Therapy for GM1 gangliosidosis

Heather Gray-Edwards, “Gene Therapy for GM2 gangliosidosis (Tay-Sachs and Sandhoff diseases)”

Miguel Sena-Esteves, “Clinical trials of gene therapy for gangliosidosis”

11:15 - 11:45 a.m.
Panel Discussion moderated by Dr. Paul Korner, “Challenges and triumphs of developing novel gene therapies for rare diseases”

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