**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.

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**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](#).

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**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”