

# NTSAD Lifeline: News and Updates for Families

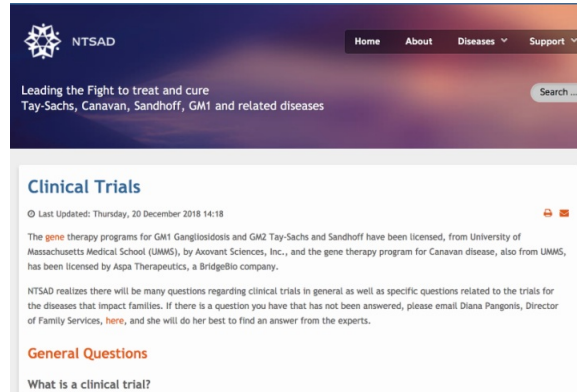
*There are no strangers here;  
only friends you haven't met yet. ~ Albert Camus*

Friday, June 28, 2019

## Clinical Trials & Updates

Since April, two clinical trials in the U.S. that have opened for GM1 and GM2 Tay-Sachs and Sandhoff.\* With the news, we recognize it can be confusing, you may have questions about criteria and how decisions are made. We have created FAQs to help sort out some of the questions and we will continue to expand on it as more questions are asked of us.

*\* We will share news about open trials outside of the U.S. when we receive notice.*



The screenshot shows the NTSAD website header with navigation links: Home, About, Diseases, and Support. Below the header is a search bar and a tagline: "Leading the Fight to treat and cure Tay-Sachs, Canavan, Sandhoff, GM1 and related diseases". The main content area is titled "Clinical Trials" and includes a sub-header "General Questions" with a link "What is a clinical trial?".

[NTSAD Clinical Trials FAQs](#)



**N-Acetyl-L-Leucine trial**  
Sponsor: IntraBio

Same active ingredient as Tanganil but formulated in a different method.

U.S. Trial open to 18+ GM2 Gangliosidosis.

Trial sites at UCLA, Mayo Clinic, and NYU (currently recruiting).

Click [here](#) for full trial listing,



National Human Genome  
Research Institute

**GM1 Gene Therapy**  
Sponsor: NHGRI  
Collaborator: Axovant  
Sciences

Testing gene therapy to potentially treat Type II GM1 Gangliosidosis.

U.S. Trial open to ages 2-12 with Type II GM1 Gangliosidosis.

Trial site at National Institutes of Health Clinical Center

Click [here](#) for full trial listing.



## 9th Annual Day of Hope

The official **Day of Hope** is Saturday, September 21st however any day can be a day of hope. This year we're opening it up so families can choose to raise funds for research or for the annual family conference experience.

Our theme this year is **NTSAD's Day of Hope: Moving Mountains!**

In seven years, we have raised, collectively, over \$430,000! Let's take it over half a million this year! If you want to host an event or share a t-shirt campaign, send Becky Benson a note [here](#).

[Sign me up!](#)



## Canavan Families!

**REMINDER!** Medical Record Retrieval Service is open & available to Canavan Families

Aspa Therapeutics is offering this service free of charge to Canavan Families. Families



## Patient Insight Networks

**Patient Insights Network (PIN)** gives families the opportunity to share clinical information as well as how the family is impacted by the diagnosis. You will be an integral part of building community through your experiences.

Companies developing therapies want to know how many patients are affected by these disease. The anonymous data gathered in the PIN will inform companies, biotech and pharmaceutical, and researchers understand the disease from all angles.

[Learn MORE](#)



## Late Onset Adults

**For our Late Onset community** - we need your help finding adults diagnosed with Late Onset to contact NTSAD. We have heard from companies asking questions about the Late Onset



## Newborn Screening

As we begin to move toward the possibility of treatments, **Newborn Screening** becomes even more relevant. Your voice in promoting this legislation is important and can save lives.

If you have not already done so, please ask your Member of Congress to co-sponsor this critical bill. It takes less than a minute to fill out the online form and your message will automatically be sent to your Member of Congress.

Find and fill out the online form to your representative [here](#).

[Learn MORE](#)



## Zoom Chats

We have started Zoom Chats this summer for the NTSAD family as a way to stay in touch between the conferences.

Following is the schedule for

are free to use the records as they wish. *CAN-Inform*, the updated Canavan Natural History Study, will be opening soon and to enroll in this study, you will need your child's medical records. Depending on the number of healthcare providers your child has seen, the time needed to sign up is approximately 20 to 30 minutes.

If you think you might want to enroll in *CAN-Inform*, we encourage you to take advantage of this retrieval service soon, as it could take up to 2 months to gather the medical records.

[Click here.](#)

community, how many, where are they and if we can be in touch with them.

Please share this request with your neurologist in the event they have other diagnosed patients that may not have been in touch with NTSAD.

And if you know of other Late Onset adults, encourage them to reach out to us.

With potential therapies around the corner, we want to be sure we can reach the broader Late Onset community!

Have them email us below.

[Email NTSAD](#)

July 2019:

### **Parents (Infantile/Juvenile)**

Tuesday, July 2  
7:00pm (East Coast)  
6:00pm (Central)  
4:00pm (West Coast)

### **Bereaved Parents**

Tuesday, July 9  
7:00pm (East coast)  
6:00pm (Central)  
4:00pm (West coast)

### **Late Onset Adults**

Tuesday, July 16  
7:00pm (East Coast)  
6:00pm (Central)  
4:00pm (West Coast)

A virtual meeting room is available to you if you'd like to chat privately with other parents. Email Diana at [diana@ntsad.org](mailto:diana@ntsad.org) if you want some time.



## **Take a moment and say their names.**

*James  
Jaxon  
Ambrose  
Elliot  
Brianna  
Dhasia  
Stanley  
T-Jay*

The NTSAD Family stands with their families as their families learn to carry the weight of their absence in the seconds, minutes, days, weeks and months that lie ahead.

NTSAD | 617.277.4463 | [www.NTSAD.org](http://www.NTSAD.org)

