NTSAD Community News

Research, Collaboration, and Community

Supporting families is the center of everything we do...

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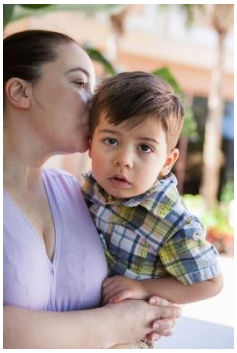
Support the 45th Annual Family Conference



Shortly after her son, Owen, was diagnosed with Tay-Sachs disease, Mona found NTSAD. She spent a year trying to find an answer to what was causing her son's loss of developmental milestones. When she finally had a diagnosis, she was relieved to have resources from NTSAD on the disease and providing care. However, as a single mother she was overwhelmed by the idea of traveling across the country with Owen to attend NTSAD's Annual Family Conference.

But then Diana, NTSAD's Director of Family Services, reached out directly to Mona and offered help. Diana explained how the Conference would be an opportunity for Mona to meet with other parents and learn about the latest research. She could also get a break while trained volunteers cared for Owen at the Conference. Diana also coordinated travel arrangements and NTSAD provided financial aid for Mona and Owen to attend the Conference.

"*Diana from NTSAD set it all up, and thank goodness she did, because I couldn't have,"* says Mona. *"I was in survival mode at that point. All I had to do was get on a plane, show up, and breathe—which was really all I could do at the time."*

At the Conference, Mona made deep connections with other parents and found the Conference to be a safe place where she could finally express all her emotions – fear, anger, and even grief.

This June, the NTSAD Community will gather for the 45th Annual Family Conference in Reston, Virginia. More than 75 families are attending the Conference including 12 families who are attending the Conference for the very first time; some are newly diagnosed, while others are newly bereaved. The Annual Family Conference is a unique, immersive experience where families and adults facing Tay-Sachs, Canavan, GM1 and Sandhoff receive and give support.

Learn more about the Conference.

Give to NTSAD to ensure the Conference is a place where families know they're not alone.

Read the rest of Mona and Owen's story.

Annual Family Conference: See You Soon!

In one week, the NTSAD Community will gather again for the Annual Family Conference. To date, nearly 300 people have registered to join us in Reston, Virginia!

Sara Scaparotti, Mom to Joey who had GM1, as well as NTSAD Board Vice President and Co-Founder of B Brave Foundation, shares what the Annual Family Conference means to her.

"*It wasn't until attending my first NTSAD Conference and being invited into other parents' suffering that I was reminded of the coexistence of joy and pain. Listening to the stories – newly bereaved, newly diagnosed and the perspectives of people who have been traveling this road for many years rekindled hope and joy in my heart. When the world tells you, 'Don't bring your suffering to me,' the community of brokenness says, 'please, you're welcome here.' Their suffering doesn't overwhelm me, and mine doesn't overwhelm them. It's a beautiful thing.* ***To me, Conference is a time to connect with some of the most incredible people that I know, to offer support and to feel supported, to remember and to treasure the here and now, and to bear witness to the most amazing love stories this life has to offer."***

If you're a rare family, it's not too late to register for the Conference.

You can find the schedule and speakers on the new Conference website, designed pro bono by Michael Foley of eLearnza.

Thanks to our **2023 Conference sponsors** who make it possible for affected children and adults and their families to experience four days of connection, community, and support!

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*NTSAD Board Members

Email Director of Development and Communications Susan Keliher, to learn more about sponsorship opportunities.

Aspa Reports Positive Data from Canavan Trial

Aspa Therapeutics, a BridgeBio company, shared more encouraging data from its CANaspire gene therapy trial during a presentation at the 26th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT).

- All trial participants showed a rapid and lasting decrease in levels of NAA, a key chemical marker which is elevated in children with Canavan disease, after dosing with BBP-812.
- Brain MRI scans of participants after dosing demonstrate improvement in myelin, also known as white matter (a type of tissue that is essential for brain function and does not form properly in Canavan disease).
- Positive changes in sitting ability and head control have also been observed using multiple assessments, including the Gross Motor Function Measure-88 (GMFM-88), a clinical outcome measure used by physical therapists to assess movement function in children.

Read the press release.

International GM1 Gangliosidosis Awareness Day

The Governor of Massachusetts, Maura Healey, proclaimed the 23rd day of May as GM1 Gangliosidosis Awareness Day in Massachusetts! NTSAD CEO Kathy Flynn joined some of our partners yesterday at the Massachusetts State House. Eleven other states have also granted this official day of observation: Arizona, California, Colorado, Idaho, Illinois, Iowa, Michigan, North Carolina, Texas, Virginia, and Washington.

ASGCT 26th Annual Meeting

American Society of Gene & Cell Therapy's (ASGCT) conference took place this month in Los Angeles. More than 6,000 attendees came together to discuss the latest advances in gene therapy. A few of the companies whose representatives spoke at ASGCT, including Aspa Therapeutics, UMass Chan Medical School, and Myrtelle, will also be presenting at NTSAD's Annual Family Conference's Research Day on Friday, June 2. **Check out the schedule for NTSAD's research day.**

Read Aspa Therapeutics' press release.

Read Myrtelle's press release.

Read interview with Terry Flotte, UMass Chan Medical, newly elected ASCGT Vice President.

Save the Date: *Imagine & Believe* 2023

NTSAD's signature event *Imagine & Believe* is November 9, 2023, from 6 to 9 pm at the Royal Sonesta Boston. *Imagine & Believe* brings together families, members of industry, clinicians, researchers, and rare allies for a reception, silent auction, and inspiring speaking program to raise critical funds for family services, programs, and research.

Each year we recognize an individual who makes a significant and positive impact on the rare disease community. The 2023 honoree is Jayne Gershkowitz, Chief Patient Advocate at Amicus Therapeutics, a trailblazer in patient advocacy and a remarkable individual who dedicated her career to championing children, adults, and families affected by rare diseases. We hope you will join us in celebrating all Jayne has done for the rare community!

Sponsorship opportunities are available.

Admission begins at $200. Donations and tribute ads are welcome. **Learn more.**

Imagine & Believe

A Resource for Rare Siblings: A Present for My Sister

Rare Revolution Magazine recently highlighted **Cure & Action for Tay-Sachs (CATS) Foundation's** resource for siblings, a children's book titled *A Present for My Sister*. Written by Rosalind Stopps and illustrated by Jessica Fitz-Howard, the book helps siblings understand Tay-Sachs and Sandhoff diseases and ways they support their affected loved ones. **Here, Ralfe Miller, a 10-year-old boy and rare sibling himself, gives his review of the book.**

Download a digital copy of *A Present for My Sister* or request a physical copy here.

NTSAD leads the worldwide fight to treat and cure Tay-Sachs, Canavan, GM1, and Sandhoff diseases by driving research,

Donate

forging collaboration, and fostering community. Supporting families is the center of everything we do.

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