NTSAD Community News

Research, Collaboration, and Community

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

May

May 2024

In this Issue

Learn about the Latest in Research

Last month at NTSAD's 46th Annual Family Conference, researchers and industry members shared updates on their research and clinical trials in Tay-Sachs, Canavan, GM1, and Sandhoff diseases during Friday's Research Day breakout sessions.

Read the summaries:

- **Canavan**
- **Infantile and Juvenile GM1 and GM2 (Tay-Sachs and Sandhoff)**
- **Late Onset GM2**

ASGCT Highlights New Research

Last week NTSAD's CEO Kathy Flynn and Research Director Valerie Greger, PhD, attended the American Society of Gene & Cell Therapy (ASGCT) Meeting in Baltimore, Maryland. They left inspired and informed by the latest rare disease research involving gene therapy, gene editing, and other therapeutic approaches.

Sanofi's AMETHIST GM2 Trial Discontinued

At the end of April, Sanofi announced that the AMETHIST phase 3 study of venglustat for the treatment of GM2 gangliosidosis was discontinued based on the absence of positive trends on clinical endpoints.

This is incredibly disappointing news as we all hoped it could potentially have provided a much-needed treatment option for our community. NTSAD is working with our advocacy partners Cure and Action for Tay-Sachs (CATS) Foundation and the Cure GM1 Foundation to explore next steps, and we will provide more details when they are available.

Please know that this will not stop the work we are doing to support our communities, and we will continue to explore potential future treatments.

The full press release from Sanofi can be read here.

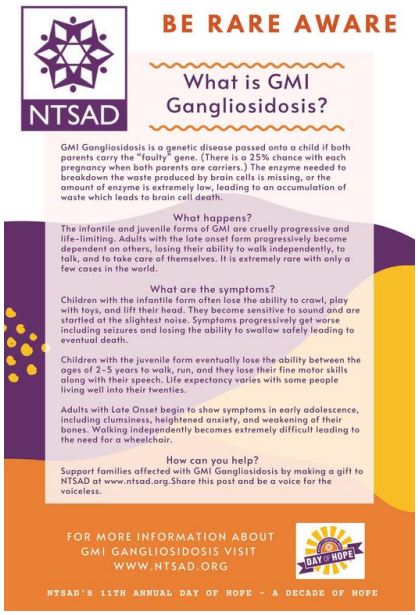
If you have any questions, please email Diana Jussila, Director of Family Services.

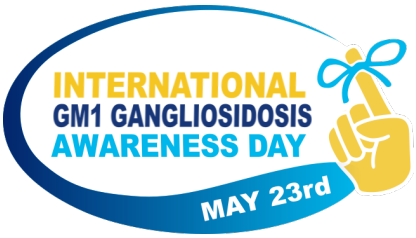
Help Raise GM1 Awareness

Join us on May 23rd for GM1 Awareness Day to support families and raise funds for research leading to effective treatments of GM1 gangliosidosis.

Here are three ways you can help:

- Share your family's experience with GM1 or lift the voices of one of our rare community members! Read Jessie Jackson's story, "Living with Juvenile GM1 Gangliosidosis: Squeezing Every Last Drop Out of Life."
- Educate your friends by sharing NTSAD's GM1 Be Rare Aware infographic on your social media. Right click the graphic to download it.
- Make a gift to NTSAD to support family services, advocacy, and research.





School Community Supports Rare Family

Maddie Suarez, a resilient 12-year-old, loves to give big hugs, make friendship bracelets, and hang out with her family. Maddie does her very best to remain independent despite her diagnosis of Juvenile Sandhoff disease. Beloved by her teachers and classmates, Maddie's school, the Morgan Road Middle School, stepped up to show their support for Maddie by initiating an awareness and fundraising campaign for Sandhoff disease.

One of Maddie's teachers, Mr. Ned, offered to shave his head if the school community reached their fundraising goal of $2,000! This month, they reached their goal, and Maddie had the honor of shaving Mr. Ned's head! A portion of funds were donated to NTSAD.

Maddie's mom Jen shared, ***"Maddie is a warrior, and she has an amazing support system within her community. The first organization that came to mind for this money was NTSAD. My family is so grateful to finally be able to give something back to the organization that has helped us since diagnosis!***

Thank you to Maddie, Mr. Ned, Principal White, Maddie's TA Marybeth Makhlouf, and the entire Morgan Road Middle School Community for raising awareness and for supporting Maddie and other families like hers.

Connecting with Families

While visiting her daughter in Los Angeles, California, NTSAD's Director of Family Services Diana Jussila visited with members of the NTSAD family, too. Diana spent a day with Jaqueline and Aaron Wright, whose daughter Annabella passed away last year from Juvenile Tay-Sachs disease. Together they visited Annabella's favorite place, the L.A. Arboretum, to see the tree dedicated in her honor. Diana also visited with Eric Pastor and his family, Kate Gomez and her son Felix, Wendy Tapia Ruiz and her son Victor, and Larry and Terry Beauchamp.

Diana shared she hopes to return to California soon. " *My heart is full seeing some of my NTSAD family! I wish I had more time to visit with everyone including driving down to San Diego to see our friends there, but there is always next time! I'll be back!"*

NTSAD Fall Family Meet Up – New Jersey

Diana together with Becky Benson, NTSAD's Family Services Manager, plan to connect in person with families at the next Family Meet Up in Short Hills, New Jersey on September 21, 2024. If you live in the greater New Jersey, New York City, or Philadelphia area, save the date! More information to come.

Day of Hope 2024

Join us as we Move a Mile for Hope, September 21, 2024! On this day each year, the NTSAD Community unites to raise awareness for Tay-Sachs, Canavan, GM1, and Sandhoff diseases and funds for NTSAD. Last year, rare individuals and families along with friends took a walk, a stroll, and some rolled a mile in their community, including everyone who attended the Regional Family Meet Up in Washington State last year. Plans are in the works to do the same at the New Jersey Family Meetup coincidentally held again on Day of Hope!

You too can Move a Mile for Hope or hold your own Day of Hope event in your community. From June to November each year, rare families host walks, poker runs, trivia nights, golf tournaments, Facebook fundraisers, t-shirt campaigns, and more in support of NTSAD families.

Since the first Day of Hope held in September 2011, the NTSAD Community has raised more than $740,000 for NTSAD's Research Initiative and family services. Since 2002 NTSAD's Research Initiative has awarded more than 72 grants and provided more than $4.7 million in funding.

To learn more about **Day of Hope**, planning your own event, or raising money for Day of Hope, contact Susan Keliher, Director of Development and Communications, at 617-277-4463 or email her at **skeliher@ntsad.org.**

Sydnie Dimond Leaving NTSAD Role

NTSAD's Development and Communications Manager, Sydnie Dimond, has raised thousands of dollars and immeasurable awareness during her four years at NTSAD. But what stands out most is her dedication and genuine love for rare families. She is often the first point of contact for newly diagnosed adults and parents of children offering them compassion and connection to the Family Services Team and resources.

Building on her work at NTSAD, Sydnie is taking on a new opportunity, joining Boston Children's Hospital Trust to raise funds as a Corporate Initiatives Officer.

"I know how lucky Children's Hospital is to have Sydnie on their team. She is a talented, hard-working, and caring person and colleague. She's simply sensational and will be deeply missed. Please join me in wishing Sydnie well in the next step of her career." - Susan Keliher, Director of Development and Communications

"The NTSAD community has brought so much light and love into my life. Thank you all for welcoming me, sharing your stories, and becoming cherished friends. ***Although I am leaving for a new career opportunity, a piece of my heart will always remain with NTSAD. It's been an honor to work here.*** *I hope to volunteer at future Imagine & Believe events and conferences, so this isn't the last you'll see me!"* - Sydnie Dimond

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NTSAD's Research Team Seeks Summer Intern

Are you or do you know a genetic counseling or biology student looking for a paid, part-time summer internship? NTSAD is looking for an intern to help with various research projects. For more information, email Research Director **Valerie Greger, PhD.**

Mark Your Calendars - *Imagine & Believe* and 47th Annual Family Conference

Save your place for NTSAD's signature fundraising event, ***Imagine & Believe,* at the Le Méridien Boston Cambridge on November 7, from 6-9 pm ET!** This year we will honor the Buryk Family for their advocacy, raising funds, and driving research. Join us for a reception, silent auction, and inspiring speaking program as we raise critical funds for family services and programs, advocacy, and research. Sponsorship opportunities available. Buy your tickets now. **Learn more here.**

NTSAD's 47th Annual Family Conference will take place on April 24-27, 2025, in Dallas, Texas! Yee-haw! More details to come. Save the date!!

NTSAD leads the worldwide fight to treat and cure Tay-Sachs, Canavan, GM1, and Sandhoff diseases by driving research, forging collaboration, and fostering community. Supporting families is the center of everything we do.

Donate

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