NTSAD Community News

Research, Collaboration, and Community

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

February

February 2024

In this Issue

In Loving Memory: Judy Gottlieb

This month's Community News is dedicated to Judy Gottlieb, who passed away in late January. Judy, a beloved, long-time member of our community, through her extraordinary generosity helped many healthy siblings of children and adults affected by Tay-Sachs, Canavan, GM1, and Sandhoff diseases pursue their dreams.

After experiencing both love and loss, Judy Gottlieb, a mother and grandmother from New Jersey, chose to move forward in her life by giving to families like hers. In May 2005, she established a special memorial college fund at NTSAD to honor her youngest son, Jeffrey Alan Gottlieb, who passed away in 1975 from Tay-Sachs disease, and her husband, Stanley N. Gottlieb, who passed away in 2001. It was Judy's intent that the monies be distributed for college education, i.e., tuition, books, room, and board.

During the last 18 years, Judy had generously awarded scholarships to 56 young adults, some of whom have received multiple scholarships throughout their education. Since 2005, the Jeffrey Alan and Stanley Gottlieb Memorial Scholarship Fund has awarded nearly $100,000 in scholarships.

Judy will be remembered for her compassion and for the lasting impact she has made. Judy's family plans to continue the Gottlieb scholarship funding, and Judy's mission to help healthy siblings further their education.

Make a gift in Judy's memory to the Gottlieb Scholarship Fund.

We Need You: Support the GM2 Community at Meeting with FDA

Join members of the GM2 Community for the first-ever, Externally Led GM2 Patient-Focused Drug Development (EL-PFDD) Meeting with the U.S. Food and Drug Administration (FDA) happening this Thursday, February 15, 2024, at 9:30 a.m. ET.

The live, virtual meeting will feature adults and families affected by GM2 (Tay-Sachs and Sandhoff diseases), who will share their experiences and perspectives to inform members of the FDA and key stakeholders in drug development about the

impact of GM2 on their lives, their experiences with disease management, and their hopes for accelerated drug development.

Jennifer, Mom to Maddie who has juvenile Sandhoff disease, shared what the meeting means to her, ***"For my family it's important to sign up so that we, as a community, can together raise awareness of GM2 in the hope of prompting further research and to help pave the pathway for a cure. Together our voices are louder. Each of our experiences allows us to demonstrate how we are impacted individually as well as globally."***

This important meeting is free to attend and open to everyone — individuals and families affected by GM2, including bereaved family members, clinicians, researchers, investors, industry partners, and rare allies. The EL-PFDD Meeting will feature moderated conversations and clinical overviews of the three onset levels of GM2, statements from individuals and family members.

The more people who attend, the greater the impact we will have!

Join us on Thursday, February 15, at 9:30 a.m. ET on NTSAD's website, www.ntsad.org. Registration is required. Please note to attend the meeting you also must fill out a brief access form before the start of the meeting.

Register for the PFDD Meeting.

If you are or have been affected by GM2, consider submitting a comment now.

Check out the meeting schedule.

Learn More and Register for GM2 EL-PFDD Meeting!

World Rare Disease Day: February 29

This year, World Rare Disease Day will be held on the rarest day of the year—February 29th!

All month long, you can join NTSAD and the greater rare community, to raise rare awareness.

- Spread awareness and submit a "rare is" statement with a photo to **sdimond@ntsad.org** to be featured on NTSAD's social media.
- Follow, like, and share NTSAD's Rare Awareness posts all month long!
- Support the GM2 Community by participating in the virtual GM2 PFDD Meeting with the FDA and key stakeholders on February 15, 9:30 a.m. ET hosted by NTSAD. **Learn more and register here.** (Watch on www.ntsad.org.)

More 2024 Rare Disease Day Activities:

- Tune in to NIH's Rare Disease Chat on February 21 from 12–12:45 p.m. on Facebook Live. **Learn more.**
- Attend Rare Disease Day at NIH 2024! The event is happening both virtually and in person all

day on February 29! **Learn more.**

- **Register now** for the FDA's Rare Disease Day 2024 Virtual Public Meeting on Friday, March 1 from 9 a.m. - 4:30 p.m. ET.
- #BeRareAware by taking the first steps towards finding out your carrier status! Learn more in the JScreen segment below.

2024 Annual Family Conference

The 46th Annual Family Conference in Chicago, Illinois is just two months away! The conference brings together rare individuals and families, researchers, patient advocates, industry members, and more for four days of connection, collaboration, and support. We hope to see you in the windy city from April 11 to 14!

When asked what the conference meant to her, recently bereaved Rare Mom Kim shared,

"The conferences provide us with such a relief, a place where we can be ourselves and not feel judged. A place where people talk about Greyson and don't shy away from his name.

I can't imagine not seeing the kiddos and families who have become such a support to us throughout this journey. The conference is something we look forward to from the minute the previous conference ended. ***Meeting families in person is life changing. I've made lifelong friends, and I would be lost without them. Plus, seeing the amazing kids helps with our grief of losing Grey."***

No matter if you're recently diagnosed, currently caregiving, or bereaved, the conference is a place to find support and companionship. Consider joining the NTSAD Community for the 2024 Conference.

Register for the Annual Family Conference

Don't forget to reserve your hotel room at the Conference.

If you are an affected individual or family who requires financial assistance to attend the event, learn more about applying for a Helping Hand Grant.

Apply for a Helping Hand Grant

Sponsorship makes it possible for NTSAD to hold an unforgettable experience for our rare community. **Learn more about the sponsorship opportunities here.**

Sponsor the Conference

THANK YOU to our 2024 sponsors! Every year, you make it possible for the NTSAD Community to gather for this memorable experience.

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Sponsor listing as of print date.
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You can be a sponsor. Learn more.

2023 Impact Report: Bringing Our Community Together

You, your engagement, and financial contributions support children, adults, and families coping with a diagnosis and loss from Tay-Sachs, Canavan, GM1, and Sandhoff diseases. We continue to fight until these devastating diseases are no longer fatal or debilitating.
NTSAD has a 66-year history of responding to our community's needs by creating new programs and resources, expanding advocacy initiatives, and broadening and driving research efforts.

Thank you for supporting families and accelerating research leading to potential treatments. Together, we have made important strides. You make our work possible.

Learn about our shared accomplishments in the 2023 Impact Report.

Thank you to NTSAD Board Member Jonathan Katz, and the entire team at **ACOM Healthcare** for designing this annual impact report *pro bono* again this year.

Free Expanded Carrier Screening for NTSAD Families

Jewish Genetic Screening Awareness Week is February 4 to 10 and serves as a reminder leading to Rare Disease Day that EVERYONE, regardless of heritage, should speak with their doctor about genetic counseling and their risk of being a carrier for rare disease.

Expanded Carrier Screening (ECS) gives families the information they need to safely plan and expand their families. #BeRareAware by taking the first steps towards finding out your carrier status.

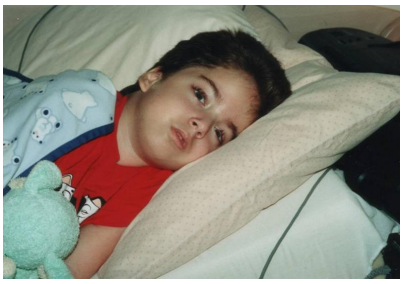
NTSAD strives to empower families by offering free comprehensive carrier screening services to family members of a child or adult affected by Tay-Sachs, Canavan, GM1, or Sandhoff disease. Go to JScreen.org and use code NTSAD24 for a ReproGen test at no cost to you.

Learn more.

Mathew Forbes Romer Foundation's "Sweetness and Laughter" Celebration

Each year at the Mathew Forbes Romer Foundation (MFRF)'s "Sweetness and Laughter" Celebration, an individual is honored for their impact on the rare community. **This year, MFRF has chosen to honor NTSAD's Chief Executive Officer, Kathy Flynn, at the event on Sunday, February 25.**

Learn more about the MFRF's "Sweetness and Laughter" Celebration.



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.

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