NTSAD Community News

Research, Collaboration, and Community

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

August

August 2022

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Day of Hope: September 17, 2022

Each year, affected individuals, families, corporate partners, researchers, and rare allies participate in NTSAD's Day of Hope to raise awareness and critical funds for research, leading to potential treatments for Tay-Sachs, Canavan, GM1, and Sandhoff diseases.

Since the first Day of Hope in 2011, Our Community has raised more than $600,000 towards NTSAD's Research Initiative, and for the first time there are clinical trials across all four diseases.

Thank you to Craig and Sherri Gropp for hosting the first Day of Hope event this year, in honor of their son Brodryck, who had GM1 gangliosidosis.

"We are so thankful that Brodryck was born into such a loving and caring group of extended family and friends, who continue to honour him so much and are always willing to help fundraise for a cure." -Craig, Sherri, and daughter, Honor Gropp

The Gropp Family hosted their annual beanbag (cornhole) tournament, their first since COVID, raising $2,500 Canadian dollars.

You can host your own event this summer or fall, and participate in Day of Hope in a myriad of ways.

Find more information on Day of Hope here. Have more questions on hosting your own event? Email Family Services

Manager Becky Benson at **becky@ntsad.org** or Development and Communications Manager Sydnie Dimond at **sdimond@ntsad.org.**

8 Ways to Participate in NTSAD's Day of Hope!

- **Walk a Mile for Hope on Saturday, September 17** in your neighborhood or at your gym, school, place of worship or work, etc. Invite your friends, family, and colleagues to join you while or walk, or maybe dance, roll, or stroll a mile. Mark your Mile for Hope, post a photo or video on Facebook, tag the NTSAD Facebook page, and use #NTSADDOH22.

- **Join Rare Families and Friends for a virtual Day of Hope Afterparty on September 17** at 6 p.m. ET/5 p.m. CT/4 p.m. PT. All are welcome to connect with families, put your feet up, and raise a glass to our shared hope for future successful treatments. **Register for virtual Day of Hope Afterparty!**

- Raise awareness and funds for research with your own Day of Hope fundraising page that you can share in social media or via email or text. It's easy. In a few clicks, you can **create your own Day of Hope fundraising page here!** Need help? Easy step-by-step instructions can be found **here.**

- **Create your own custom or team Day of Hope t-shirt here!** Net proceeds go to Day of Hope and research.

- **Order NTSAD's 2022 Day of Hope t-shirt here.**

- **Support Rare families and make a gift to Day of Hope.**

- **Add the Day of Hope 2022 frame to your Facebook profile picture.**

- Follow NTSAD's Day of Hope activities on social media! Stay connected with NTSAD on **Facebook,** **Instagram,** **LinkedIn,** and **Twitter.**

Imagine & Believe - Thursday, November 10, 2022

On Thursday, November 10, 2022, the NTSAD Community will gather at the Royal Sonesta in Boston for the first in-person *Imagine & Believe* event in three years. We will also commemorate NTSAD's 65th Anniversary.

At the event we will honor research pioneer Guangping Gao, PhD, for his life's work in identifying the Canavan gene, revitalizing gene therapy, and his many accomplishments leading to potential treatments for Canavan disease and many rare diseases.

The NTSAD Community of rare allies -- clinicians, researchers, industry members, and supporters -- join our families in imagining a world with effective treatments for Tay-Sachs, Canavan, GM1, and Sandhoff diseases. ***Imagine & Believe*** **includes a reception, silent auction, and speaking program, and raises funds for programs and services for Rare families.**

Admission begins at $200 per person, and sponsorships range from $750-$25,000.

Learn more about *Imagine & Believe* sponsorship opportunities here.

Sponsor or Reserve Your Spot for *Imagine & Believe*!

Items for our silent auction are needed. To donate an item for the auction, email Development and Communications Manager Sydnie Dimond at **sdimond@ntsad.org.**

Thank you to our sponsors of *Imagine & Believe* 2022! To become a sponsor or learn more about sponsoring *Imagine & Believe*, contact Director of Development and Communications Susan Keliher at **skeliher@ntsad.org**.

(Sponsor listing as of August 30, 2022.)

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*NTSAD Board Member

GM1 Community Leaders Meet with FDA

Cure GM1, along with other GM1 community leaders from around the world, are meeting with the Food and Drug Administration (FDA) on October 14th in an Externally-Led Patient-Focused Drug Development (EL-PFDD) meeting in an effort to advance treatment.

Members of the GM1 Community, particularly affected families, are invited to participate in this important event. For more information, please attend an informational webinar on September 7th at 3 p.m. ET/2 p.m. CT/1 p.m. PT.

Register for the September 7th informational webinar.

Navigating Clinical Trials

With the ever-changing landscape of clinical trials, **NTSAD provides families with a current list of opportunities as well as professional staff who can help navigate the process.**

NTSAD partner, Courageous Parents Network (CPN), has a guided pathway about clinical trials with helpful videos addressing what to expect, how to navigate and process the news whether a trial is a fit for your family. **Watch CPN's video about balancing hope and expectations** featuring patient advocate and NTSAD Board member Jamie Ring.

If you have additional questions, please reach out to NTSAD's Family Services team at **care@ntsad.org**

Raise Rare Awareness in September

September marks three National Awareness Month Campaigns -- for Tay-Sachs disease, Leukodystrophy, and Newborn Screening -- providing multiple opportunities for you to *Advocate for Rare*.

Every Wednesday throughout September, NTSAD will post on our social media a "Be Rare Aware" infographic about Tay-Sachs, Canavan, GM1, and Sandhoff diseases. Follow NTSAD on **Facebook,** **Instagram,** **LinkedIn,** and **Twitter** and share our posts!

You also can share information on genetic carrier screening or get tested through JScreen. NTSAD strives to empower families by offering free comprehensive carrier screening services to family members of an affected child or individual. Thanks to the Evan Lee Ungerleider Fund of NTSAD, the NTSAD New York Area Fund, Mathew Forbes Romer Foundation, and the Canavan Foundation for making free screening possible through JScreen and giving families information they need to safely plan and expand their families. **Go to JScreen.org and use code NTSAD22 for a ReproGen test at no cost to you. Insurance information will be required.**

Learn more about the importance of and how to advocate for Newborn Screening. EveryLife Foundation for Rare Diseases is co-hosting a Newborn Screening Bootcamp to Empower Advocates for Life-Saving Screening. The event will educate participants on the best practices to build relationships between stakeholders and all aspects of the newborn screening system. There is a virtual training on October 4, 2022, as well as a concurrent in-person event in Tacoma, Washington. **Learn more.**

Participate and support NTSAD's Day of Hope, which raises awareness and money for research initiatives. **Learn more.**

College Scholarships Available for Heathy Siblings

Applications are now open for the Jeffrey Alan Gottlieb and Stanley N. Gottlieb Memorial Scholarship Funds which provide financial support for college to healthy siblings of children and adults affected by Tay-Sachs, Canavan, GM1, Sandhoff or an allied disease.

After experiencing both love and loss, Judy Gottlieb has chosen to move forward in her life by showing love. From caring and concern for her own family, she has decided to express concern for other children by generously contributing to their futures. In May 2005, this mother and grandmother from Somerset, New Jersey, established two separate memorial college funds at NTSAD to honor

her youngest son, Jeffrey Alan Gottlieb, who succumbed to Tay-Sachs in 1975, and her husband, Stanley N. Gottlieb, who passed away in 2001.

It is Judy's intent that the monies be distributed *"for use in their college education, i.e., tuition, books, room and board."* Siblings who are entering college or are currently in college may apply.

Apply now. The deadline is September 30, 2022.

Note: If you previously received a Gottlieb scholarship, and you are still in college, you may apply again!

NTSAD's 2023 Annual Family Conference

Save the Date for NTSAD's 45th Annual Family Conference, June 1-4, 2023 in Reston, Virginia! The cornerstone of NTSAD's programming -- the Annual Family Conference brings together affected individuals and families, researchers, industry members, and clinicians for four days of connection, community, and support. The event also provides updates on the latest research, tips on caregiving, and opportunities for families to share their experience.

Stay tuned for more information on all things Conference!

TORCH Awards 2022 Honors Blyth Lord

Congratulations to the recipients of Sanofi's US 2022 TORCH Awards, including Blyth Lord, NTSAD's Past Board President and Founder and Executive Director of **Courageous Parents Network (CPN)**. At the TORCH Awards, Blyth was honored for her strength and positive contributions as an advocate for the Rare Disease community. As a parent to Cameron, who passed of Tay-Sachs disease, Blyth became part of the NTSAD Community and was inspired to launch CPN, so parents would have information, resources, and choices regarding their children's care. Jennifer Siedman, CPN's Director of Community Engagement and fellow Rare Mom, was also honored with a TORCH Award for her advocacy.

Courageous Parents Network empowers, supports, and equips families and providers caring for children with serious illness.

Watch the award ceremony here.

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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