NTSAD Community News

Research, Collaboration, and Community

Supporting families is the center of everything we do…

January

January 2022

In this Issue

Dear NTSAD Community,

As we begin the New Year, we renew our focus on the fight for effective treatments for Tay-Sachs, Canavan, GM1, and Sandhoff diseases. Thankfully, we have been able to rely on each other and the power of the NTSAD Community while we assess the outcomes of clinical trials. **We remain grateful for your support of NTSAD through your generous donations and for always supporting families.**

This year marks important milestones for NTSAD. We will commemorate our 65th Anniversary by honoring and remembering loved ones and acknowledging our accomplishments and progress thus far. Also, we are proud to announce that after reevaluating our Research Initiative Program and broadening our research focus to include efforts leading to earlier diagnosis and newborn screening, **this month we launched a request for research proposals for our 2022 round of funding.**

Next month, NTSAD plans to attend the 18th Annual WORLD Symposium in San Diego from February 7 to 11. Come visit us at booth PS6. Also at WORLD, Board President Staci Kallish, DO, will be among the featured speakers, and former NTSAD Executive Director Sue Kahn will receive the Patient Advocate Leader (PAL) Award. Congratulations, Sue!

Read more here.

In less than six months, we look forward to hosting the first in-person Annual Family Conference in three years in Denver, Colorado from July 7 to 10. We hope you can join us for the 44th Conference. Due to the ongoing pandemic, to protect the vulnerable families we serve, we will be requiring COVID vaccinations and the wearing of masks. See the specific guidelines in this newsletter.* Our goal remains to keep everyone safe as we come together for much needed connection and support, advocacy, and updates on the latest research.

Finally, after marking your calendars to attend our Annual Family Conference, please share the news of our research funding, and reach out if you are interested in becoming more engaged with NTSAD. For 65 years, community and collaboration have served us well.

With gratitude, anticipation, and hope for a bright 2022,

Kathy

Kathleen Flynn
Chief Executive Officer

NTSAD Launches Next Round of Research Funding: Seeking Proposals

In 2019, NTSAD made the decision to pause the Research Initiative Program and reflect on how our research funds can make the greatest impact in the evolving landscape of clinical trials for therapeutic treatments. After engaging in an extensive strategic planning process, we were ready to re-introduce our Research Initiative Program. Recently, we launched the next round of research funding for projects that involve basic research, translational studies, or clinical studies for Tay-Sachs, Canavan, GM1, and Sandhoff diseases. Projects that cover all aspects of therapeutic discovery and those that focus on understanding the pathophysiology and the role of inflammation, or the process of myelination and how it is affected by the disease are of particular interest. **Cure Tay-Sachs Foundation - CTSF** and **Blu Genes Foundation** are partnering with NTSAD on this initiative to fund research.

Since 2002 NTSAD's Research Initiative has awarded more than $4 million in seed grants that have been leveraged to more than $30 million of additional investments leading to potential new therapies for Tay-Sachs, Canavan, GM1, and Sandhoff diseases. As a result, there have been many advances including eight active clinical trials specifically addressing these diseases.

2022 Research Initiative Program grant amounts are significantly higher than awards made in past years with one-year grants of up to $70,000 or two-year grants of up to $140,000 for two years, inclusive of indirect costs not to exceed 5%. Funding for the second year of a two-year grant is contingent upon adequate progress made in the first year.

Requests for funding from NTSAD’s Research Initiative Program will adhere to a two-step application process: a one-page pre-application letter of intent, followed by a full application. **Please note the opportunity to submit a full application is by invitation only.**

Pre-applications are due Feb. 18, 2022.

Learn more.

Aspa Therapeutics' Upcoming Webinar for Canavan Disease

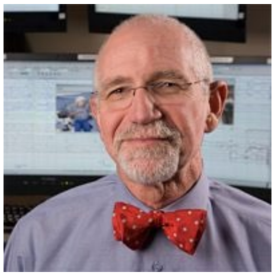
Have questions about Aspa Therapeutics' upcoming clinical trial? **Register now for Aspa's upcoming webinar, Investigational Approach to Gene Therapy for Canavan Disease: A Closer Look at the Science.**

The webinar will be hosted by Aspa on Wednesday, February 2, 2022, 8 pm Eastern/7 pm Central/6 pm Mountain/5 pm Pacific.

To register and submit questions in advance, please complete this form.

For additional information, visit **www.treatcanavan.com** or for questions, email **patientadvocacy@aspatx.com**.

NTSAD Presents: Recognizing and Understanding Seizure Activity



Join us on Tuesday, February 15 at 6 pm Eastern/5 pm Central/4 pm Mountain/3 pm Pacific for an interactive Zoom meeting with neurologist Jeffrey Buchhalter M.D. Dr. Buchhalter will share his knowledge on the presentation and complications of seizure activity. He also will answer questions from attendees during this virtual event.

Register now for the Zoom event.

Rare Disease Day 2022

Rare Disease Day is Monday February 28, 2022!

Don't miss out on this opportunity to raise rare awareness, support families, and advocate for rare affected loved ones! Through out the month of February, NTSAD will be featuring Rare Disease Day events, stories on **Facebook,** **LinkedIn,** **Twitter,** and **Instagram,** and resources, including a few below.

Share with your friends why you personally #Care4Rare by making a post on social media.

Check out the Rare Disease Day website.

Register for NORD's virtual Rare Disease Day event Monday, February 28, 1:30-2:30pm EST.

Learn how to advocate for legislation that impacts the rare disease community.

NTSAD Parents Highlight the Power of Families in Advocating and Driving Treatments

NTSAD parents and past and current Board members Oralea Marquardt, Kevin Romer **(The Mathew Forbes Romer Foundation)**, and Shari Ungerleider **(The Evan Lee Ungerleider Fund)** co-authored an article recently published in the esteemed *Clinical Pharmacology and Therapeutics Journal for the American Society of Clinical Pharmacology and Therapeutics* (ASCPT), titled *Family Driven Development of Treatments for Rare Pediatric Neurological Diseases*. Oralea, mother of William, Kevin, father of Mathew, and Shari, mother of Evan have all dedicated themselves to promoting prevention and carrier screening, educating health professionals, driving and funding research, and supporting other rare families like theirs in memory of their children. In their article they state, ***"We hope this empowers many families of affected children!"*** NTSAD is a stronger community because of parents, leaders, and changemakers like Oralea, Kevin, and Shari.

Read the article.

NTSAD Mom Shares Her Love and Grief

The Courageous Parent Network recently shared **Myra Sacks' moving letter and tribute to her daughter, *Dear Beauty: This is Love and Grief in Our Home.*** Myra is mom to Havi, who was two years, four months, and sixteen days old when she died from Tay-Sachs disease a year ago.

"Havi taught us that life can be even more beautiful and painful than we ever imagined. And when we live at the edge of that deepest beauty and deepest pain, then everything—our hearts, our world view, our community—will deepen and expand." Myra

Myra's letter has been shared on several other news outlets including Upworthy. **Help us to remember Havi and create safer spaces for those experiencing grief by reading the article now.**

Get Involved with NTSAD

Have you ever thought about becoming more involved in NTSAD's efforts? We are always looking for community member volunteers to join us in our mission! **The NTSAD Board of Directors is seeking new Board members for its 2022 slate of officers, and if you have an interest in getting more formally involved, we want to hear from you!** We are in particular need of individuals with a finance or accounting background. And if volunteering on a committee sounds like something you may enjoy, we are always looking for community and family members to join our efforts – be it helping to plan an event, supporting our research projects, or providing aid with our communication efforts. **NTSAD is here to serve YOU, and we welcome all community members who are interested in exploring ways to volunteer with us!**

NTSAD
National Tay-Sachs & Allied Diseases Association
65th Anniversary

To express interest or find out more about ways to get involved, please contact Kathy at kflynn@ntsad.org.

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*NTSAD's COVID Guidelines

NTSAD ANNUAL
FAMILY
CONFERENCE

Due to the on-going pandemic in 2022, all individuals who participate in NTSAD's 2022 Annual Family Conference must comply with the following COVID vaccination and mask guidelines:

For the safety of the entire NTSAD community, all conference participants who meet age eligibility for the COVID vaccine must be fully vaccinated per CDC guidelines at the time of the conference.

Proof of vaccination will be required at the time of online registration and/or during the check-in process prior to the beginning of the conference. In addition, due to vulnerability of the individuals and families we serve, masks will be required (whenever not actively eating or drinking) for all those over the age of two years who can safely wear a mask. These guidelines may adjust per the CDC, NTSAD leadership, and local state guidelines.

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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