NTSAD Community News

Research, Collaboration, and Community

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

August

August 2021

In this Issue

Aspa Enrolling for Canavan Clinical Trial

Aspa Therapeutics' CANaspire gene therapy trial is now enrolling Canavan patients in the U.S. This exciting news is a major milestone for the NTSAD Community— **now there are clinical trials for individuals and families affected by Canavan, GM1, Tay-Sachs, and Sandhoff diseases.**

Learn more about CANaspire.

Learn more about the clinical trial requirements.

Sio Reports on GM1 and GM2 Clinical Trials

Sio Gene Therapies continues to drive the company's clinical trials for GM1 and GM2. Recently the company completed its enrollment and dosing of eight, late infantile and juvenile-onset patients in Stage 1 of its GM1 gene therapy clinical trial.

The company recently presented findings of demonstrated Cerebrospinal fluid (CSF) GM1 ganglioside in 4 out of 5 children treated with the lowest dose of AXO-AAV-GM1 at six-month follow up, and **the company will soon share 12-month results and meet with the FDA to discuss registrational pathway in the first half of 2022.**

The company also has dosed the first two Tay-Sachs and Sandhoff patients for its gene therapy, AXO-AAV-GM2, in Phase 1/2 trial and plans to continue patient identification, screening, and enrollment in Stage 1 of the dose-ranging trial.

Read more.

IntraBio's Report on Drug Development for GM2 Patients

IntraBio, Inc. recently reported that the company's IB 1001 demonstrated a statistically significant and clinically meaningful improvement in symptoms, functioning, and quality of life in both the primary and secondary endpoints for child and adults participating in the multinational GM2 (Tay-Sachs and Sandhoff) clinical trial.

This is promising news for the NTSAD Community. We look forward to learning more about the results of the trial and next steps for IB 1001.

Read the press release.

GM1 Guide for Gene Therapy Created

The American Society for Gene and Cell Therapy (ASGCT) created a guide about gene therapy specifically for GM1 patients considering participation in clinical trials. The guide will be updated with information and news from companies conducting trials.

View the guide.

Imagine & Believe Sponsorship Opportunities

This year, we are cautiously optimistic that we will gather safely and in-person for NTSAD's annual **Imagine & Believe reception on October 28 at the Royal Sonesta Boston.** For NTSAD Community members living outside the Boston area, **we also are hosting a virtual Imagine & Believe event on November 3 from 7-8 p.m. EDT.**

At both events we will be honoring our long-time Executive Director Sue Kahn who steps down this fall.

Our goal is to provide opportunities for members of our NTSAD Community living near and far to engage, connect, bid Sue farewell, and above all else — support families. NTSAD relies on industry members, researchers, and rare allies to invest in our families' futures through partnership, philanthropy, and support of our events. Consider sponsoring the event or making a gift in honor of Sue.

Learn more about Imagine & Believe sponsorship opportunities here.

To sponsor Imagine & Believe, please contact Susan Keliher, Director of Development and Communications at **skeliher@ntsad.org** by September 16 to be listed among sponsors on the event invitation.

September Is National Tay-Sachs and Leukodystrophy Awareness Month

As September marks the National Awareness Month for both Tay-Sachs and Leukodystrophy, there are many opportunities to spread awareness, raise funds for research, and advocate for rare.

Read and share NTSAD's social media campaign to build awareness about Tay-Sachs, Canavan, GM1, and Sandhoff diseases with new posts every Wednesday throughout September on **Facebook,** **Twitter,** **Instagram,** and **Linkedin.**

Share information on carrier screening or get tested yourself with **JScreen.**

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

11th Annual Day of Hope

Join the more than 20 NTSAD Community members who are hosting events during NTSAD's 11th Annual Day of Hope to raise rare awareness and critical funds for research. Consider hosting an event such as a virtual or in-person walk/run, trivia night, or other activity at your company or neighborhood.

This year's Day of Hope is September 18, 2021, and we will be recognizing our collective efforts and achievements in social media. However, any day can be a Day of Hope, and activities typically begin in July and continue through October. It's never too late to bring hope!

Here's a quick and easy way to show you care for rare by purchasing a Day of Hope t-shirt, where a portion of proceeds go toward NTSAD's Research Initiative. You also can design your own TEAM shirt for your family or company.

Purchase a Day of Hope t-shirt.

Create your own TEAM t-shirts here.

For families, allies, and companies hosting events NTSAD has great swag to share with your guests and to use as prizes and awards, including t-shirts, car decals, and reusable grocery cooler bags. Contact Sydnie at **sdimond@ntsad.org.**

Learn more ways you can participate in Day of Hope, including event ideas here!

For assistance on hosting a family event email Becky at **becky@ntsad.org.**

For assistance on holding a corporate activity email Susan at **skeliher@ntsad.org.**

44th Annual Family Conference

NTSAD ANNUAL FAMILY CONFERENCE

Mark Your Calendars: July 7-10, 2022

Renaissance Denver Central Park Hotel

Denver, Colorado

For the first time in three years NTSAD's Annual Family Conference will be held in person in Denver, Colorado from July 7-10, 2022!

NTSAD's past two conferences, held virtually due to COVID, have allowed for new connections, inclusiveness and greater accessibility at the same time alleviating some feelings of isolation due to ongoing quarantining. We look forward to seeing those connections grow stronger in Denver.

The conference provides the latest updates in research, resources, and support for families affected by Canavan, GM1, Tay-Sachs and Sandhoff. All are welcome.

Two Disabled Dudes Podcast Features NTSAD Community Members

In case you missed this year's Annual Family Conference, Sean Baumstark and Kyle Bryant, the hosts of Two Disabled Dudes podcast hosted an inspiring, panel discussion about how to find hope following a rare diagnosis, during caregiving, and after loss.

Listen to the inspiring conversation between NTSAD Board President Staci Kallish and fellow Board members and parents, Kevin Romer and Sara Scaparotti on the latest episode of the Two Disabled Dudes' podcast out now that includes additional insight from rare mom Becky Benson, NTSAD's Family Services and Conference Coordinator.

Listen here.

Jack Chesler's Legacy Honors His Parents

Jack Chesler never forgot how much his parents gained from NTSAD. To honor his parents Leon and Lillian, who lost a child to Tay-Sachs, Jack Chesler made a six-figure gift to NTSAD. Sadly, Mr. Chesler (pronounced Kesler) passed away a year ago, but he made a lasting impact on this world, leaving his entire estate to charity.

Mr. Chesler also made gifts to Make-A-Wish Foundation, Shriner's Hospital for Children and eight other charities, including his beloved David Posnack Jewish Community Center in Davie, Florida, where he exercised everyday. A former engineer, Mr. Chesler was born and raised in Brooklyn, New York and moved to Florida in 2018. he was known for his love of sports, passion for talking politics, and ever-present smile.

While he lived frugally, he was bigger than life and extremely generous. He was always willing to help anyone who needed support in his community.

He leaves behind three children and two siblings, and he is predeceased by his parents and one sibling.

Mr. Chesler's parents had connections to the early years of NTSAD, which was founded in 1957 by Evelyn and Leonard Sussman along with two other families who had children affected with Tay-

Sachs and were looking for answers. NTSAD is among the first patient advocacy groups established in the country. In 2019, Michael Sussman, along with his wife, Renee Licht, founded the Evelyn and Leonard Sussman Legacy Circle to honor his parents and help recognize every individual and family in the NTSAD Community who has included NTSAD in their will, estate, or long-term charitable giving.

Mr. Chesler is a part of the Sussman Legacy Circle, and NTSAD remains grateful to Mr. Chesler for his impactful gift as well as to the Sussman Family for their enduring legacy in creating the NTSAD Community and advancing research.

If you are considering make a legacy gift and would like more information, please contact Susan Keliher, Director of Development and Communications at skeliher@ntsad.org or call 617-277-4463.

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

STAFF
Sue Kahn, Executive Director
Becky Benson, Family Services and Conference Coordinator
Sydnie Dimond, Development and Communications Associate
Susan Keliher, Director of Development and Communications
Diana Pangonis, Director of Family Services

NTSAD
2001 Beacon Street
Suite 204
Boston, MA 02135

info@ntsad.org
www.NTSAD.org