NTSAD Lifeline

Community and Connections for Families and Individuals

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

AUGUST 2020

In this Issue

NTSAD's 10th Annual Day of Hope

This year marks the 10th Anniversary of Day of Hope, and with so many clinical trials on the horizon we are truly in a Season of Hope!

To date, more than **$500,000** has been raised since our First Annual Day of Hope on September 18, 2011. Now more than ever, we are searching for connection and community. **Day of Hope events unite people in our shared cause to find effective treatments for individuals affected by Tay-Sachs, Canavan, GM1, and Sandhoff.**

Join us by hosting an event to strengthen our rare and mighty community during our Season of Hope.

Questions? Contact Becky at **becky@ntsad.org**.

Our thanks to the Cornett, Watson, Karp, Ronaldson, Stidham families for hosting events in your communities, and all the families holding t-shirt campaigns!

Navigating the System: Applying for Social Security Disability

Good news! GM1 Gangliosidosis Type I and Type II have been officially added to the Compassionate Allowances (CAL) list of rare diseases that allows an application for Social Security Disability (SSDI) to be fast-tracked!

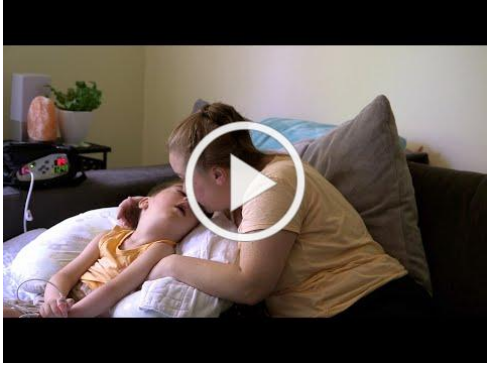
Currently, Canavan, GM1 Gangliosidosis (infantile and juvenile), Sandhoff and infantile Tay-Sachs are on the CAL list. We continue to advocate for the inclusion of juvenile Tay-Sachs, and we will share the news when it's officially added. Learn more **here**.

Caregiving: Advocating for Your Child and Trusting Yourself

As parents and caregivers we want what is best for our children and loved ones. We hope we make the right decisions and try not to doubt ourselves.

Watch this Courageous Parents Network video featuring Mona, Owen's mom, as she talks about decision-making about Owen's care including planning for what the future may hold.

Learn more from other parents who understand life-limiting illnesses and visit Courageous Parents Network **here**.



Attention Late Onset Community

SANOFI GENZYME

!!! Sanofi Genzyme Update !!!

The first patient has been screened and fully enrolled in the Sanofi Genzyme Venglustat trial at New York University Medical Center, and a second patient is in the middle of the screening process at Mass General Hospital (Boston).

New York University Medical Center (NYU) and Mass General (MGH) are officially recruiting adults with Late Onset Sandhoff or Tay-Sachs. NIH and UCLA are expected to begin enrolling soon.

Learn more about the study **here**. If you have questions or concerns about the trial, traveling during COVID, or any other issues, please contact Diana **here**.

Stay Connected with Late Onset Community Zoom Chats

Scheduled for the third Wednesday of every month at 8 pm (ET) / 6 pm (CT) / 5 pm (PT)

Join us on Wednesday, September 16th. Use **this link** to enter the room.

Canavan Families - A Special Message from Aspa

Aspa a bridgebio company

Aspa Therapeutics would like to invite you a webinar, "An Introduction to Gene Therapy for Families Living with Canavan Disease."

Aspa Therapeutics is a biotechnology company focused on developing a gene therapy for Canavan disease.

Join Professor Guangping Gao, a pioneer in gene therapy who developed Aspa's investigational gene therapy program, and an expert panel of speakers who will give an introduction to Aspa, provide background about gene therapy, and describe Aspa's clinical development program for Canavan disease. There also will be a general discussion about clinical trials and an opportunity to ask questions of the panelists.

The webinar will take place on **Wednesday, September 16, at 8 pm Eastern time, 5 pm Pacific**. When you register, you will receive a link that will enable you to join the webinar.

If you would like to join the webinar, please click on this link: **Aspa Canavan Disease Webinar**

If you have any questions, please contact our advocacy partners, or email patientadvocacy@aspatx.com

GM1 Families - Join a Virtual Natural History Study

casimir

This GM1 natural history study, conducted by Casimir and sponsored by Lysogene, is an opportunity to share through video how your child functions at home to further illustrate the impact of the disease and

further advance clinical trials.

Email **GM1@casimirtrials.com** to learn more and enroll in the trial.

Coping with Loss: An Essay about Loss and Anticipation

Carla Steckman, NTSAD Board member and Talia's mom, wrote an essay about her experience with anticipatory grief following Talia's diagnosis, and its familiarity with the current state of the world and the ongoing pandemic. As she writes, "We've been through worse...and we will make it through this as well."

Read her full essay **here** on *Modern Loss*.

Jeffrey & Stanley Gottlieb Sibling Scholarships: EXTENDED deadline

Now accepting 2020 Sibling Scholarship applications. Deadline extended: Sept 11, 2020.

The Jeffrey Alan Gottlieb and Stanley N. Gottlieb Memorial Scholarship Funds awards monetary grants to healthy siblings in rare families attending college. Siblings of children and adults who are or were affected by Tay-Sachs, Canavan, GM1, Sandhoff, or an allied disease may apply for financial support.

After experiencing both love and loss, Judy Gottlieb has chosen to move forward in her life by demonstrating support and generosity to other rare families by investing in their bright futures. Judy's intent is that the monies be distributed for use toward college education, i.e., tuition, books, room, and board. Download the application **here**.

Download application

Summer Memories Shared

Thank you to the families who shared their beautiful children:

Kaydence	Maximus
Brooklyn	Tobin
Cooper	Pax
Mollie & Madelyn	Keaton
Prince	Isaac
Jessie	Jorgen
Pauly & HarleeGrace	Finnegan
Kensley	Cenzy
Ava D.	Novalee
Ava R.	Sage
Levi	

Say Their Names

Lilah Yang
September 26, 2012 - July 4, 2020
Daughter to Connie Yang and Blk Xiong
Little sister to Pac Xiong

*If your loved one's name is missing, our most deepest apologies.
Please send Diana the right information and dates to make those corrections.*

Welcome to the World

Penelope Lane Cornett Bayer
Born on August 11, 2020
Daughter of Ashley Cornett
Little sister of Jase and Tripp

Wesley Davis Miller
Born on August 6, 2020
Son of Shannon and Ryan Miller
Little brother of James

Mathias Maximus Orloff
Born on May 12, 2020
Son of Briana and David
Brother of Maximus

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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