NTSAD Lifeline

Community and Connections for Families and Individuals

December 2020

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A Holiday Message For Our Rare Community

As we round out the 2020 holiday season and look forward to a brighter 2021, we at NTSAD want you to know that no matter what this season, or the new year brings, we are thankful to be part of your family. We may be rare, but together we are mighty. We are so thankful for each and every one of our rare community members. Knowing that we are never alone is something both truly special and uplifting.

Your NTSAD Family Services Team certainly understands the challenges our community members are facing. We are here for you this holiday season and always. Please feel free to check in on our **Family Support Group page** over the next several weeks to connect with one another and for some extra support.

Additionally, while the holidays often offer a time of connections, missing those family gatherings this year can be an added hardship on top of our already stressful lives. The CDC offers some proactive ways to cope with these factors here.

2021 Annual Family Conference Announcement

After much consideration **we're announcing our decision to hold the 43rd Annual Family Conference virtually**. Due to the COVID-19 Crisis and the ongoing concern for the health and safety of our families, as well as the continued meeting restrictions we felt this to be the only viable option. We did not make this decision lightly. We know the conference means so much to you. We share in the disappointment of not being together in-person once again, but it is clear that at this point in time it is the best decision for all involved at this point in time.

We are building on the success of last year's Virtual Family Conference to develop an online program that's bigger, better, and even more interactive for all our attendees. **Please save the dates of April 22-25, 2021 for our virtual Family Conference.** We will continue sharing information with you as our program develops.

Clinical Trial Approval for Taysha Gene Therapies

Congratulations to Taysha Gene Therapies on receiving Clinical Trial Application (CTA) approval from Health Canada for its investigator-initiated Phase 1/2 of TSHA-101, for GM2 gangliosidosis.

Read more of the full press release from Taysha Gene Therapies, **here**.

SIO Gene Therapies Clinical Trial News

Sio Gene Therapies Announces Positive Six-Month Follow-Up Data from Low-Dose Cohort of Phase 1/2 Trial of AXO-AAV-GM1 for GM1 Gangliosidosis.

"We are excited to report encouraging safety, tolerability, biomarker, and preliminary efficacy data for AXO-AAV-GM1, the first gene therapy evaluated in a clinical trial for GM1 gangliosidosis, a life-limiting disease caused by mutations in the GLB1 gene that impair beta-galactosidase enzyme activity."

View the full Sio press release, here.

Help accelerate drug development for GM2 with AllStripes

AllStripes × NTSAD

Your GM2 journey can advance research

Your medical journey can accelerate the development of new treatments for GM2. Because GM2 is rare, researchers need more information directly from patients and their families to understand the condition.

How you can help

① Go to allstripes.com/gm2

② Create your account. Our team collects your medical records — no appointments, no cost

③ Success — you're a hero for GM2 and receive exclusive research updates

Sharing your experience with GM2 is vital to helping researchers advance treatments for our community.

By partnering with AllStripes patients and patients' families allow their de-identified medical records to be shared with multiple research studies for GM2 easily and from the comfort of your own home. AllStripes does the work in collecting medical information, and patients receive all of their medical records in one secure account, at no cost. The goal is to have at least 30 patients, across all variations of GM2 diagnoses represented.

As a thank you for your participation NTSAD is offering a $25.00 Grubhub gift card to every family who registers with AllStripes by January 15, 2021. Simply email Diana and share your registration confirmation with her to receive your free gift card.

Join and register here.

Family Zoom Connections

December's Holiday Party was a fun and connective event featuring holiday sweaters, festive drinks, and great communication among our rare community.

January's Zoom event will feature a a guest speaker from AllStripes joining us to explain more about the work they do, and how the information they collect from patients helps researchers advance potential treatments. We invite all families and individuals in our rare community to join us for this collaborative event on **January 19, 2021 at 7:00pm EST**.

NTSAD Family Equipment Exchange

Families passing along equipment that may be helpful to others has long been a hallmark of our rare community's supportive connection to each other. We created a Google spreadsheet to catalog the available products and equipment to make them available upon request to all our families. Additionally, NTSAD can help in facilitating the collection and/or shipping of items between families thanks to NTSAD's Benton's Family Assistance Fund.

To view the available items, please click **here**. If you have items you'd like to make available to others, please email **Becky** or **Diana**.

Caregiving Corner - Holiday Inclusiveness with Your Child

Many families struggle during the holidays with how to find safe and engaging ways to include their affected child in the ongoing traditions and celebrations.

Some suggestions from families who have been through the holiday season with their children include:

- Dabbing powdered sugar, icing, or placing a little cotton candy on your child's tongue
- Taking them outside and letting the snow fall on their cheek
- Wrapping them in extra fuzzy blankets to feel the soft and cozy warmth
- Showing them the twinkling holiday lights on you tree or houses around you
- Playing soft music and dancing along with them in your arms

NTSAD Family Curated Holiday Playlist #GoodMoodMusic

We polled families in our **Facebook Support Group page** to ask what songs help get them through the holiday season or into the spirit of the season. Below you'll find the link to our Spotify playlist with all the songs recommended by our very own rare families.

NTSAD Spotify Holiday Playlist

B Brave Foundation - Building Bridges to a Brighter Future

Congratulations to NTSAD board member, and mom to Joey, Sara Scaparotti and her husband Sam on the launch of the new website for their nonprofit, B Brave Foundation.

"B Brave Foundation supports children, families and healthcare providers. B Brave was born out of love, and inspired by the life of Joey Scaparotti, who created the spark for an organization dedicated to improving the lives of children affected by rare incurable neurological disorders."

Learn more about the B Brave Foundation, **here**.

NTSAD Sibling Spotlight - A Message of Hope from Justin Ungerleider

My name is Justin Ungerleider.

NTSAD is very, near and dear to me and has been a part of my life as long as I can remember.In 1995, my older brother, Evan, was diagnosed with Tay-Sachs disease.

One of the worst memories, forever engrained in my mind, is hearing the doctors tell my parents that there was no hope for Evan. The doctors said they would not see a cure or treatment for children like Evan in my parents' lifetime.

Evan would not live a full life.

No parent should ever have to hear there is no hope.

From that moment on, I was determined to ensure that no other family would experience what mine did.

Several years ago at an NTSAD event, I shared my desire to pursue medicine with Miguel Sena-Esteves, PhD, who is principal investigator in the Tay-Sachs Gene Therapy Consortium. Miguel offered me an internship at UMass Medical School's Gene Therapy Lab.

I was ecstatic...*"I couldn't believe it!* ***18 years after being told there would never be a treatment for this disease--- there I was working on it!*** *It's hard to describe the THRILL of seeing the research progress—****from raising money to working side-by-side*** *with other researchers* ***on the development of a treatment,*** *to* ***holding the hand of one of the first Tay-Sachs patients ever given this gene therapy!!!***

There's always hope, and I have seen it first-hand.

I am grateful to have witnessed how dedicated researchers are to ridding the world of rare diseases. I cannot stress how important it is to support NTSAD today to keep pushing for breakthroughs in research. And to all the families like mine, know that one day there ***WILL BE*** *a world where children like Evan will grow up. Go to school. Make friends. And simply live normal lives.* ***This day is coming."***

Grief Share - Words on Loss

"Grief is the last act of love we can give. Where there is grief there was great love." - Unknown

As we prepare to move into 2021 we know that the anticipation of a new year can be daunting for those who are grieving the loss of a loved one. You are not alone.

We've created an anonymous Google form with a series of prompts for anyone who would like to write down their grief, in as few, or as many words as they'd like to share. We will gather these responses to share collectively in our January issue of Lifeline.

You can view and fill out the Google Form anonymously, here.

NTSAD Bereaved Parents Group on Facebook

We've launched a new group on Facebook specifically for grieving parents. The NTSAD Bereaved Parents Group is offered as a safe, supportive space for bereaved parents to share openly with

each other. This space is to be considered confidential, remain free of judgements, and is intended to be used as a means of support via compassion, comfort, and connection with one another. It will be overseen by the NTSAD Family Services Team.

If you are a bereaved parent you may join the group, here.

Say Their Names

Delilah Beaman
November 7, 2017 - November 30, 2020
Daughter of Stephanie Baker and John Beaman

Scott Hunger
October 15, 1970 - December 21, 2020
Son of Marsha and Sherman Hunger

Finnegan Minchew
May 12, 2019 - December 3, 2020
Son of Julia and Jerry Minchew

Brooke Plumstead
May 9, 2010 - December 16, 2020
Daughter of Elizabeth Sciliano

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

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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