NTSAD

NTSAD Lifeline

Community and Connections for Families and Individuals

November2020

In this Issue

Giving Thanks For You, Our Rare Community

While 2020 has brought more uncertainly than ever, and with the holiday season looking a little different for most of us this year, we at NTSAD 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.

SIO Gene Therapies Clinical Trial News

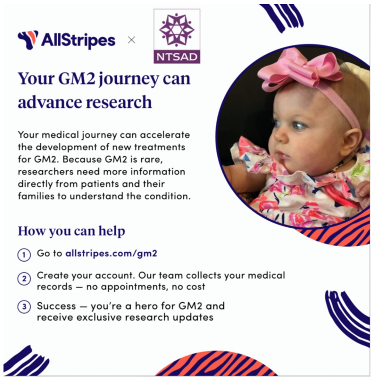
SiO GENE THERAPIES

Axovant Gene Therapies, recently renamed Sio Gene Therapies, prepares for first clinical trial for gene therapy for Tay-Sachs and Sandoff diseases after receiving Investigational New Drug (IND) clearance from the U.S. Food and Drug Administration (FDA) for AXO-AAV-GM2.

The study will enroll both infantile and juvenile patients with GM2 gangliosidosis. The two-part trial will be led by Terence R. Flotte, M.D., Professor of Pediatrics and Dean at the University of Massachusetts Medical School, who will serve as principal investigator of the clinical trial. Stay tuned for more information as it becomes available.

The company also received Rare Pediatric Disease Designation from the FDA for AXO-AAV-GM1, an AAV9-based gene therapy delivered via a single intravenous administration that is in Phase 1/2 development for GM1 gangliosidosis. AXO-AAV-GM1 is **the**

only gene therapy in clinical development for both infantile (Type I) and juvenile (Type II) GM1 gangliosidosis. AXO-AAV-GM1 has Orphan Drug designation as well.



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

Help accelerate drug development for GM2

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

By partnering with AllStripes, formerly RDMD, 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 participate. **Join and register here**.

Imagine & Believe, What an Evening it Was!

Imagine & Believe

NTSAD's Imagine & Believe was held virtually last Tuesday to honor patients and families. Together, our community members shared their stories, experiences, and hopes for the future. NTSAD families and community members Ryan Miller, Lorelei Sandoval, Justin Ungerleider along with a dozen more families participated in the live event. With over 200 attendees tuned in to the evening's program **the 2020 Imagine & Believe program raised over $105,000** for NTSAD's vital programs and services.

The inspiring evening also included NTSAD's Board President Staci Kallish and Executive Director Sue Kahn who highlighted NTSAD's recent accomplishments, advances in research, and our direction for the new year. In addition, Dr. Guangping Gao, a gene therapy pioneer and researcher, Dr. Florian Eicheler NTSAD Advisory Board member and respected clinician, and Sherri Sigel, NTSAD Mom discussed the journey to a clinical trial. Watch the full event here on our site.

Support NTSAD Families with a Limited Edition Imagine & Believe T-Shirt!

There's still time to Imagine & Believe along with us. You can continue to support NTSAD's mission by purchasing a limited edition Imagine & Believe t-shirt for yourself or to give as an inspiring gift. Get your t-shirts, here!

For an extra special holiday shirt, you can find both Christmas and Hanukkah colors at these links, **Christmas**, **Hanukkah**!

Congratulations!

A congratulations are in order for NTSAD sibling, Cassie Bebout on her recent engagement to Evan Abbey.

Welcome To The World!

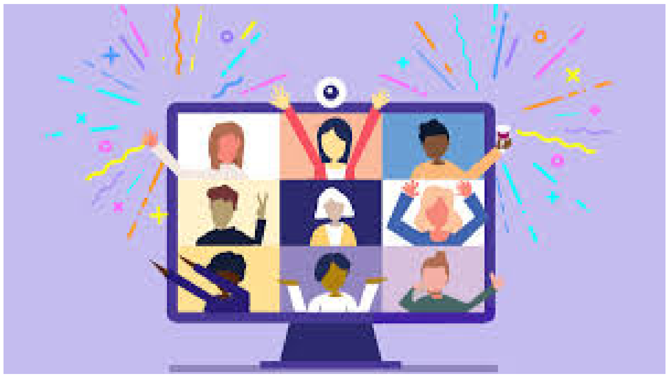
Charlotte Lawrence, 11/12/2020
Healthy daughter of Jon Lawrence and
Ashlee Suran

November is National Caregivers Month

It's no secret that being a caregiver takes a lot of work. Even in the best of times, we can find ourselves mentally, emotionally and physically drained. Rare Mamas Creator and Founder, Nikki McIntosh shares on her blog at **raremamas.com**, **Fifteen 15-Minute Rare Mama Resets** for those in a caregiving role.

We encourage all caregivers to take fifteen minutes practice self-care, because we all know that you can't pour from an empty cup. What will you do to refill your cup today?

Take the @ntsad #15minutechallenge and share a photo on social media of how you take fifteen minutes to practice self-care.



NTSAD December Family Connections "Come As You Are" Holiday Party!

Please join us for our monthly family Zoom chat...with a twist! In December we'll be hosting a "Come As You Are" holiday party. Whether that means in your NTSAD t-shirts, ugly sweaters, or even your black ties, we are gathering via Zoom for an evening of fun and connection. Bring your family and favorite holiday drink and let's get together.

This month we're asking that everyone pre-register to attend the holiday party...because **we have an added surprise for our first ten registered attendees**. Register to join the party, **here**!

NTSAD Community Holiday Gift Exchange

The holidays can feel a little more trying than merry at times, and with COVID preventing many from gathering, everyone could use a little more connection this year. We know our rare community is is one of inclusiveness and and support. What's better than a memento or word of encouragement from someone who understands our groups' unique challenges firsthand - each other! We're pleased to offer this Holiday Gift Exchange for the very first time to help end 2020 on a high note.

For more information and to register for the gift exchange, please click here.

Say Their Names

Carson Morrisette
February 26, 2019 - November 16, 2020
Daughter of Mark and Genna Morrisette

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

The Compassionate Friends
Supporting Family After a Child Dies

Worldwide Candle Lighting Memorial Service

As The Compassionate Friends notes, "The Compassionate Friends Worldwide Candle Lighting on the 2nd Sunday in December unites family and friends around the globe in lighting candles for one hour to honor the memories of the sons, daughters, brothers, sisters, and grandchildren who left too soon. As candles are lit on December 13th, 2020 at 7:00 pm local time, hundreds of thousands of people commemorate and honor the memory of all children gone too soon."

Find more information on The Compassionate Friends, and how to participate, 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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