



# NTSAD Community News

Research, Community and Collaboration



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



December 2020

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## Strength in Community

No matter who you are or where you live, 2020 has been a very hard year.

NTSAD families know hard times—receiving tough news, isolating for months on end, caregiving without a break, and experiencing loss and grief.

Through it all, NTSAD families know the importance of keeping loved ones safe and finding strength in community. They are resilient and hopeful during the advent of clinical trials. Recently a few NTSAD families shared their perspectives which are universal and particularly reflective of the times in which we live.



*"Do the best you can each day." --Cindy Erickson*

*"Just connecting with someone you know who has had a similar experience makes you feel less alone." -- Ruth Feldman*

***"You take the little things day by day. The smiles, the good things. And you try not to look forward too much because that is tough. When you realize that's all you can do. Anyway, that's when you can enjoy things so much more. There is a lot of hope... Still there's a lot of unknown, but I think there is so much hope." -- Kristen Frederic***

From our families to yours....

Wishing you a safe, happy, and hopeful 2021,

Sue Kahn  
Executive Director

Support Families. Advance Research. Give Today.

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## Sio's GM1 Early Stage Clinical Trial Reports Positive Data

Sio Gene Therapies reports positive six-month, follow-up data of the low-dose cohort of the company's AXO-AAV-GM1 gene therapy for children with GM1 gangliosidosis. In Phase 1/2 of the study five patients in the low-dose cohort demonstrated that AXO-AAV-GM1 was generally well tolerated.

**Read More.**

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## Passage Bio Receives Approval to Start GM1 Gene Therapy Clinical Trial

Passage Bio's clinical trial for providing gene therapy for children impacted with GM1 was approved by the United Kingdom's (UK) Medicines Healthcare Products Regulatory Agency (MHRA). This is the first regulatory authorization for the global PBGM01, gene therapy clinical trial. Patient enrollment for the UK is anticipated for the second quarter of 2021.

**Read More.**

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## NTSAD to Hire Research Director

NTSAD is seeking the organization's first Director of Clinical and Research Initiatives to engage key stakeholders regarding the discovery and development of treatments, diagnostics, and technologies for Tay-Sachs, Canavan, GM1 gangliosidosis, and Sandhoff diseases. Consider joining NTSAD-- leading the fight since 1957.

Learn More and Apply **Here.**

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## 2021 Annual Family Conference Virtual and Safe for All

Please save the dates of April 22-25, 2021 for NTSAD's 43rd Annual Family Conference. Last year, the event had more than 400 registrants due to the access and affordability a virtual conference provides. The four-day event will offer comprehensive programming for affected individuals, families, and caregivers, including the latest in research and strategies for navigating clinical trials and caregiving, as well as a special commemoration ceremony to honor members of our community.

In 2020 the conference provided a wealth of information from industry leaders and researchers as well as opportunities for personal connections and conversations. The decision to hold the conference virtually was made to ensure the safety of all, particularly vulnerable patients and their families until vaccinations are widespread. Stay tuned for more details in future newsletters.

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## Diana Pangonis Marks 25 Years of Service with NTSAD

NTSAD's Director of Family Services is known for her kindness, sincerity, and commitment to families above all else. For more than 20 years, Diana has provided compassionate, personal care to nearly 500 families each year. Diana began her career at NTSAD in 1995 and she has worn almost every hat—working in development, managing communications, and even serving as interim Executive Director, but her true calling is serving families.



In 2020, Diana was recognized for her dedication to families receiving the Moments Award for her tireless service from Brian and Sherri Manning. The award is given in tribute to their son Dylan and to honor individuals who are selfless and go above and beyond in their work.

***“Diana treats every family as the only and most special family in the entire world,”*** said Brian Manning, NTSAD Board member.

NTSAD Executive Director Sue Kahn concurs, *“Diana supports families with her whole heart every day and advocates for patients like they are a member of her own family. In addition, Diana is the consummate professional, partner, and colleague to the entire NTSAD Community.”*

To mark her 25 years of service on the anniversary of her first day, December 8, there was a small, socially-distanced celebration among the NTSAD team and a beautiful outpouring of love from families and members of the NTSAD community. Here is a small sampling of the many tributes.

*“Blessed indeed to have Diana working on our behalf. Diana was the first person I talked to after getting Mollie and Madelyn’s diagnosis. Just her sincere, quiet voice supported us on that day.”*  
-- Mandy Ronaldson

*“Thank you for being there for all the families who are going through this journey. I cannot thank you enough for always being there!!!”*– Erin Hobbs

*“Diana has always been the heart and soul of Family Services. I do not know how many families I have reached out to when the first thing they ALWAYS say is “Miss Diana was so kind and welcoming.” Whether these were families here in the US, Pakistan, Brazil, Argentina or even Saudi Arabia, their first contact with Diana is what continues to make them feel they are not so alone.”*  
-- Monica Gettleman, NTSAD Board Member

*“I remember Diana’s smiling face welcoming families and investigators to the Annual Family Conference as far back as the White Plains years...I was a young clinician with a lot to learn. Diana introduced me to so many lovely families who told their stories and shared their beautiful children. Much of what I have learned about our ‘diseases’ I learned from families Diana introduced me to over these many years. Diana is the ‘face’ and the compassion of NTSAD; always there for families and for the rest of us.”*  
-- Cynthia J. Tiff, MD, PhD

*“Thank you so much for 25 years of dedication, empathy, warmth, and your wonderful ability to listen. Think of all of the families you have helped over these years. We are so appreciative of you, and we congratulate you on this awesome accomplishment.”* -- David Rintell, Patient Advocacy, and the Aspa Therapeutics team

*“It’s always about the families.” If there was one statement that most accurately captures your mindset and perspective, it’s this one. How far the community has come in your 25 years at NTSAD, and I’m excited to see what the next 25 years will bring! On behalf of Sio Gene Therapies, a heartfelt thank you for your dedication and commitment to meeting the needs of all NTSAD families, always serving as a resource, as a friend, and as a trusted partner.* -- Parag V. Meswani, Chief Commercial Officer, Sio Gene Therapies

*“Seldom do you see such dedication and selflessness in an individual. The NTSAD community is blessed to have you providing guidance, expertise, leadership and, most significantly, compassion. Sometimes in life, we are faced with situations where it feels there is no place or no one to turn to. In both the darkest and brightest of moments, you are there. Ready to help. Shining the light not on*

*yourself, but on the way forward.*" -- Jonathan Katz, NTSAD Board Member and Partner Acom Healthcare.

Congratulations, Diana! Honor Diana and her 25 years of supporting families by making a **donation**.

**Honor Diana by Making a Gift Today**

## Imagine & Believe All Year-Long

It's never too late to purchase an NTSAD Imagine & Believe t-shirt for family, friends, or yourself!



**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

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