NTSAD Community News

Research, Collaboration, and Community

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

July 2021

In this Issue

Aspa's Canavan Clinical Trial

The FDA has approved Aspa Therapeutics' Investigational New Drug application (IND) for its gene therapy for patients affected with Canavan disease. This is the first multicenter, international, clinical trial for Canavan disease. While this is a major step forward, there are a few more steps to be completed before enrollment starts for CANaspire, the company's clinical trial.

With Aspa announcing its IND application all four diseases represented by NTSAD—Canavan, GM1, Tay-Sachs, and Sandhoff—now have pathways to potential treatments.

Learn More

Lysogene GM1 Gene Therapy Update

Lysogene received the FDA's Fast Track Designation for LYS-GM101 the company's gene therapy for patients with GM1 Gangliosidosis. The Fast Track Designation speeds the review process enabling more interactions with the FDA in an effort to expedite the identification of potential treatment options for patients.

This is great news for GM1 patients and their families as now there are a total of three gene therapy companies engaged in clinical trials—Lysogene, Passage Bio, and Sio Gene Therapies.

Learn More

For information on clinical trials impacting the NTSAD Community go to **NTSAD's Clinical Trial Tracker.**

NIH, NTSAD, and Buryk Fund Collaborate on Late Onset Think Tank

In 2019, NTSAD through the Katie & Allie Buryk Research Fund awarded a two-year grant to partially fund a project coordinator for the Late Onset Tay-Sachs and Sandhoff (LOTSS) Think Tank, supporting two post-baccalaureate students through the NIH Intramural Research Training Award (IRTA) Program.

The first Think Tank was held in 2018 in an effort to accelerate the road to clinical trials and to expedite effective treatments for LOTSS patients and was the inspiration of Alexis Buryk, mother of twins Katie and Allie, both of whom have LOTSS. **The goal of the LOTSS Think Tank is to address the key gaps in understanding through collaboration. The 4th Annual LOTSS Think Tank is scheduled for October 2021.**

During the last year, Mosufa Zainab held the part-time position of LOTSS Think Tank Coordinator and kept its busy investigators “on track” to maintain project momentum. Partnering with moderators Drs. Cynthia Tifft and Steve Walkley, Mosufa planned the largest Annual Think Tank Meeting to date with a total of 41 participants, including participants from industry, academia, and government, who convened virtually.

Mosufa also led the compilation of a mini-review of GM2 gangliosidosis for a special issue on lysosomal disorders for *Neuroscience Letters,* with Steve Walkley, PhD, as the guest editor. Leveraging her recent experience, Mosufa will begin medical school at Virginia Tech. She plans to be a physician-scientist when she completes her medical training.

NTSAD Seeks Next Executive Director

With Sue Kahn departing after 14 incredible years, NTSAD is searching for its next Executive Director. We seek a passionate, experienced, and inspiring leader in this unprecedented time for the NTSAD Community. The research landscape continues to evolve with a growing number of promising clinical trials and drug development activity underway—all as NTSAD remains committed to supporting families.

Learn more and apply!

NTSAD’s 11th Annual Day of Hope

Join NTSAD in a Day of Hope as we raise awareness of rare families and critical funds for research. Consider hosting an event in your community or at your company.

You also can participate by purchasing your own Day of Hope t-shirt or design a TEAM shirt for your family or company. A portion of t-shirt proceeds go toward research, and you'll inspire others to be allies and raise money at the same time!

Purchase and create t-shirts 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.

Imagine & Believe 2021

Join us as we once again **Imagine & Believe--** and for the first time in two years, gather in person!

At the event we will recognize Sue Kahn for her years of service and many achievements. Come say thank you and wish her well!

Save the Date! Imagine & Believe, October 28, 6 p.m. at the Royal Sonesta in Cambridge, Mass.

All are welcome—NTSAD families, researchers, healthcare professionals, industry leaders, and friends. More details to come.

For sponsorship info, email development@ntsad.org.

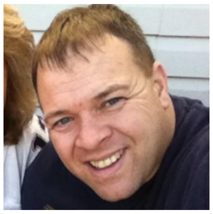
Thank You to Departing NTSAD Board Members

Every June, NTSAD reviews and renews its Board leadership, acknowledging departing members for their service, and at the same time welcoming the next generation of leaders. **We are grateful to Merle Adelman, Risa Asnen, Edna Kaplan, Brian Manning, Kevin Romer, and Shari Ungerleider—who together have served nearly 100 years on the Board and as volunteers.** NTSAD is a better organization and a stronger Community thanks to you!

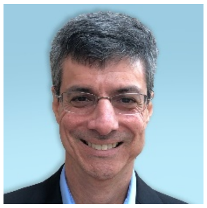
As a fundraising professional, **Merle Adelman** leveraged her expertise—quietly and humbly contributing to NTSAD as an extension of our small staff. Merle took on several projects, making a significant impact during her many years of service, even before joining the Board. Since becoming involved at NTSAD, Merle has served as a coach, sounding board, and strategic partner to the development team. Merle worked closely with our team on several appeals, created annual reports, and served as project manager for the website launch in 2011. Merle also led the search to find both current members of the development and communications team. Merle has helped NTSAD grow into the strong and healthy organization it is today, and even though she is leaving the Board, she will always be a part of NTSAD's team. Merle served on the Board of Directors from 2009-2021.

Risa Asnen has been part of the NTSAD Community for more than 40 years, serving as a volunteer and then as a Board member. For decades, she was the VP of conference planning creating a place for NTSAD families to make lasting connections. She managed everything for the conference: finding accessible locations and hotels, planning meals, creating sessions with the conference committee, and making sure each family received what they needed. Risa has been a true, life-long volunteer, and a cherished member of the NTSAD Community. We sincerely thank Risa for all of her hard work and unrelenting dedication to families.

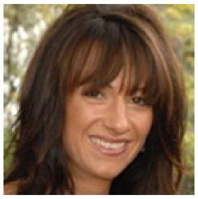
Edna Kaplan's passion for NTSAD grew from her friendship with Judy and Gary Kaplan, and their daughter Linda, who was diagnosed with Late Onset Tay-Sachs. Linda's journey included a 15-year search for a definitive diagnosis, and as the disease took its toll on Linda, Edna was there for the family as a friend and advocate. After volunteering with the Late Onset Tay-Sachs community for a number of years, Edna joined the board of NTSAD in 2011. In her ten years of service, Edna has made a significant contribution. Her work as a public relations professional has helped build awareness of NTSAD, Late Onset Tay-Sachs, and the research and clinical trials currently underway. NTSAD thanks Edna for all the support she has given to our community in the last decade. Edna served on the Board of Directors from 2011-2021.



Long before **Brian Manning** joined the Board, he was well known throughout the NTSAD Community for his warmth, kindness, and genuine caring for NTSAD Families. Each year Brian and his wife Sherri host the Drive FORE Dylan Golf Tournament in memory of their son Dylan. Brian and Sherri recently hosted the 18th annual event raising hundreds of thousands of dollars in support of NTSAD families. When the Board needed a vice president, Brian joined the Board and took on the role without hesitation. He later served as Board President. With extraordinary admiration, NTSAD thanks Brian for his years of service and his unwavering friendship to countless families. Brian served on the Board of Directors from 2013-2021.



In honor of his son Mathew who had Tay-Sachs, **Kevin Romer** and his wife Lisajane launched the Mathew Forbes Romer Foundation with the mission to raise awareness, prevent genetic diseases, and promote nursing education. The partnership that Kevin and Lisajane created between the Mathew Forbes Romer Foundation and NTSAD has led to significant funding in advancing research. Kevin served as Board President and was the founding co-chair for the research committee. He was instrumental in developing NTSAD's Research Initiative program, leading to 64 projects and more than $4 million in grants, which have generated more than $30 million in funding from the NIH and similar institutions. NTSAD thanks Kevin for his vision, leadership, and hard work. Kevin served on the Board of Directors from 2003-2021.



When **Shari Ungerleider's** first child, Evan, was diagnosed with infantile Tay-Sachs, Shari and her husband Jeff, along with her late father, Stan Michelman, created the Evan Lee Ungerleider Fund. As Evan slowly lost his battle with Tay-Sachs, Shari worked tirelessly to turn his short life into a positive force to help educate others about the need for carrier screening. Over many years, hundreds of people were educated and tested at free carrier screening events hosted by the Ungerleider Fund. Shari also was a Co-President of the NTSAD New York Chapter and served as NTSAD's Board President. Shari has boundless energy, bringing her passion for education to her job as Project Coordinator of the Jewish Genetic Disease Consortium. NTSAD honors Shari for all that she has accomplished and for her continued dedication. Shari served on the Board of Directors from 2009-2021.

Welcome New Board Members

Please join us in welcoming new NTSAD Board members Christine Chapman, Martha Kleinman, Abigail Rogers, and Lisajane Romer.

Christine Chapman is the grandmother of Emma Artinian, who passed from Sandhoff disease in 2015 at the age of three. Chris brings extensive professional expertise to the Board from her lengthy career in the healthcare sector, where she was the Vice President of InterSystems, managing a global team of more than 600 employees. In her role, Chris was responsible for design, development, marketing, deployment, and support of the company's International Healthcare systems. ***"I admire the positive impact NTSAD has on families facing one of the most difficult times in their lives. I found great comfort in all of the support my family received, and I want nothing more than to see NTSAD continue to be a strong and viable organization for other families."***

In March 1949, **Martha Kleinman's** younger sister was diagnosed with Tay-Sachs disease and passed away seven months later. Her sister's diagnosis was made solely based on a red spot at the back of her eye. Fortunately, Martha and her husband were tested and were able to have two healthy children, who each now have two healthy children of their own. Martha has been involved with NTSAD's mission for decades, and she previously served on the Board from 2009-2014, bringing her years of professional experience in fundraising and non-profit governance. We are delighted to welcome Martha back to the Board. ***"I am grateful that NTSAD can provide support and information to families affected with these terrible diseases, support which my own parents never had. And of course, the hope of developing treatments and someday a cure is something in which I want to be involved."***

Abby Rogers' experience as the mother of a child with a rare disease greatly informed her time at the March of Dimes and other organizations, where she used her skills professionally to advocate for families. Abby's son Benjamin was diagnosed with Canavan disease in the spring of 2002, and he passed unexpectedly in 2014. Abby works to make impactful changes in public policy, and she understands the need for continuous medical research, access to care, and family support, and she will be an asset to the Board. ***"My family will be forever grateful for the community provided to us, the knowledge shared by those families who came before us to make Benjamin's short, yet meaningful life the best it could be."***

Lisajane Romer joins the Board and Executive Committee taking on the role of Secretary. As a parent of an affected child, co-founder of the Mathew Forbes Romer Foundation (MFRF), and a professional with more than 30 years in product management experience, Lisajane Romer has much to offer the Board during this crucial time in the organization's history. Lisajane can support families on their journey as they care for their loved ones and navigate research and potential treatments. Lisajane and the MFRF have raised more than $2.4 million to fund projects spanning across its mission including research of treatments and cures, education and genetic screening, and care through nursing school educational programs. "***NTSAD provided the support to help me navigate through Mathew's journey as well as my own, and I look forward to giving back to help others."***

Find a full listing of the NTSAD Board 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

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

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