NTSAD Community News

Research, Collaboration, and Community

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

May 2021

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43rd Annual Family Conference
Heart to Heart and Home to Home!

The 43rd Annual Family Conference was NTSAD's largest conference ever with more than **500 attendees, including 190 first-time attendees and more than 150 families and 26 countries represented!**

The Conference had many highlights including the **opening session** hosted by Sean Baumstark and Kyle Bryant, known for their ***Two Disabled Dudes*** podcast. Sean and Kyle kicked off the conference with an honest and inspiring conversation with NTSAD families and Board members Staci Kallish, Kevin Romer, and Sara Scaparotti. They reminded us to live beyond circumstance, to redefine hope, to find purpose and the beauty in community, and to learn and love.

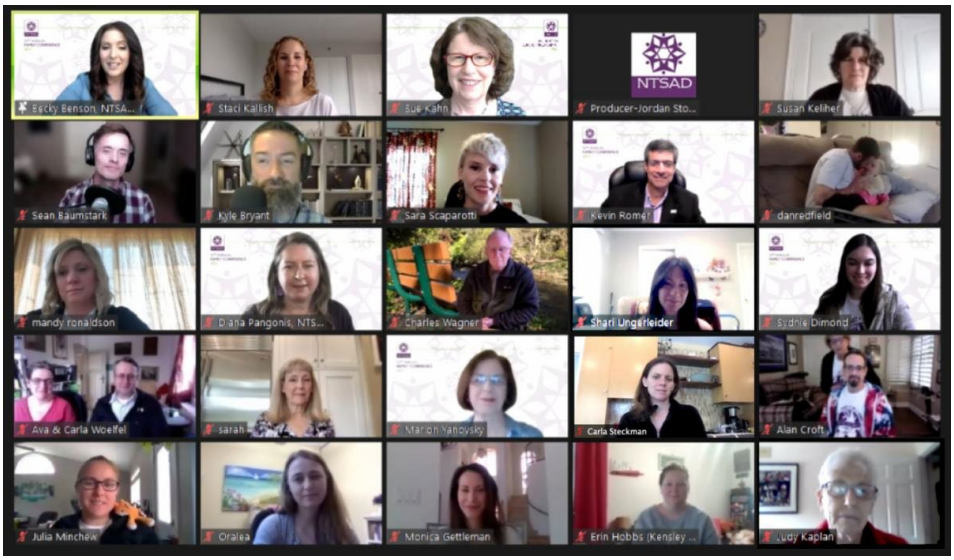
Friday's Research Day had more than 30 researchers and industry members shared the latest advances in research, drug development, and clinical trials for Canavan, GM1, Sandhoff, and Tay-Sachs diseases.

Throughout the conference, valuable information was shared, support was offered and families received unique understanding via peer meetings, health management sessions, and support groups. Overall, the Conference featured more than 70 speakers and facilitators and 30 sessions. **Recorded sessions are now available to watch.**

The NTSAD community remains powerful through a global pandemic and supportive of one another as families navigate care, clinical trials, loss, disappointment, and challenges. We are a resilient community with more rare allies than ever.

Together we can do almost anything. We can find effective treatments and above all else, support rare families.

Thank you to all who attended, presented, sponsored, supported, honored loved ones, and provided care during the conference and all year long. See you next year!



SUPPORTING FAMILIES IS THE CENTER OF EVERYTHING WE DO!

Sio's GM1 Clinical Trial Shows Early Positive Biomarkers

Sio Gene Therapies' Phase 1/2 Clinical Trial shows four out of five children treated with the lowest dose of gene therapy at six-month follow up show direct evidence of positive biochemical effect. **Dr. Cynthia Tifft, Deputy Clinical Director of the National Human Genome Research Institute (NHGRI) and Principal Investigator in the study, is presenting early findings at the 24th Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT) conference on May 13 at 6:15 p.m. EDT.**

For more on the findings, read press release.

For more on Dr. Tifft's ASGCT May 13 presentation

Mathew Forbes Romer Foundation Highlights NTSAD Partnership

The Mathew Forbes Romer Foundation hosts its third and final event in its gala mini-series on Wednesday, May 19 at 7 p.m. EDT. For almost 20 years the Mathew Forbes Romer Foundation has been an affiliate of NTSAD and partner in driving quality genetic testing and advancing research. **NTSAD's Executive Director Sue Kahn will share milestones from the decades-long collaboration during the May event,** which culminates with a “See the Light Awards” presentation to honoree Dr. Joan Keutzer, Former V.P. Global Scientific Affairs, Rare Diseases at Sanofi Genzyme. Joining Dr. Keutzer for an interactive discussion on the future of national newborn screening is Dr. Rodney Howell, Chairman Emeritus of the University of Miami Miller School of Medicine's Department of Pediatrics and the Founding Chair of the U.S. Department of Health and Human Services Secretary's Advisory Committee on Heritable Disorders in Newborns and Children.

Learn more and purchase tickets here.

Update on First Patient Dosed in Sio's GM2 Gene Therapy Clinical Trial

USA Today profiled the first GM2 patient to receive the full dose of gene therapy as part of Sio's clinical trial made possible by NTSAD's partnership and funding of research of Miguel Sena Esteves, Heather Gray-Edwards, **UMass Medical School**, Doug Martin, **Auburn University**, Dr. Florian Eichler, **Massachusetts General Hospital**, and Dr. Cynthia Tifft, **National Institutes of Health (NIH)**, that led to **Sio Gene Therapies**' GM2 clinical trial. NTSAD shares in the family's joy in hearing Alissa's deep belly laughs following the treatment.

Read the *USA TODAY* article.

Landsman Family Raise Money and Participate in Canavan Clinical Trial

When Jennie and Gary Landsman learned that both their sons, Benny and Josh, had Canavan disease, they were stunned that there was no treatment. They went on to fundraise millions of dollars for research leading to a clinical trial for Canavan disease in which their son Benny recently participated. The Landsmans are optimistic that the gene therapy will help their two sons as well as other families with affected children.

Read the article.

Watch the NTSAD Virtual Family Conference 2021 Canavan Research Breakout Session.

Thank You 2021 Annual Family Conference Sponsors

Thank you to our sponsors and supporters for making the Annual Family Conference possible and sustaining our important work!

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Please forgive any omissions or errors.

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.

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STAFF
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

NTSAD
2001 Beacon Street
Suite 204
Boston, MA 02135

info@ntsad.org
www.NTSAD.org