NTSAD Community News

Research, Collaboration, and Community

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

September

September 2021

In this Issue

Imagine & Believe October 28, 2021

Join us for an inspirational evening where we Imagine & Believe in a world with effective treatments for Tay-Sachs, Canavan, GM1 and Sandhoff diseases.

We will honor and thank NTSAD's longtime Executive Director Sue Kahn for her 14 years of service and support of rare families and leadership in advancing research toward multiple clinical trials for our Community.

Imagine & Believe will be held virtually on Thursday, October 28 from 7-8 p.m. EDT. The event is free, easily accessible, and open to all.

In addition to paying tribute to Sue, we will hear from families and patients who will share their perspective and hopes for the future.

This year, we had hoped to bring our Community of families, researchers, clinicians, industry partners and rare allies together for an in-person Imagine & Believe reception. However due to COVID, the rise of the Delta variant, and in an abundance of caution, particularly for the vulnerable population we serve, we are canceling the in-person reception. As such, we will be hosting the virtual event on October 28 instead of November 3 as originally planned.

- Day of Hope 2021
- IntroBio's GM2 Clinical Trial
- Lysogene Doses GM1 Patient
- Sio's GM1 Clinical Trial
- ASGCT's GM2 Guide
- September Awareness
- NTSAD Calendar

For more information on participating, registering, and supporting the event including making a donation in honor of Sue, please visit www.ntsad.org.

Thank you to Aspa Therapeutics, a Bridgebio Company, and all of our Imagine & Believe sponsors for their generous support and partnership.

Register Now for the Virtual Event

Make a Gift in Honor of Sue Kahn

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Sponsor listing as of print date. Please forgive any omissions or errors.

To sponsor Imagine & Believe, please contact Susan Keliher, Director of Development and Communications at **skeliher@ntsad.org.**

Sanofi Genzyme Honors Dr. Edwin Kolodny and Makes a Gift to NTSAD

Sanofi Genzyme honored rare disease pioneer Dr. Edwin Kolodny during the 30th Anniversary celebration of the company's Rare Humanitarian Program. The Rare Humanitarian Program was created by former Genzyme CEO Henri Termeer to ensure treatments for rare diseases are made available globally, and so that no patient is ever left behind.

Through the Rare Humanitarian Program, Dr. Kolodny provided care to individuals and trained healthcare professionals around the world. **At the celebration Sanofi Genzyme made an extraordinary $30,0000 gift to NTSAD in honor of Dr. Kolodny's lifetime of service to the rare community.**

Thank you Dr. Kolodny for your enduring legacy and selfless care of the rare community, and thank you Sanofi Genzyme for your contribution, support, and partnership.

Sue Kahn helped pay tribute to Dr. Kolodny speaking at the event:

"Dr. Kolodny, it is an incredible honor to be here to recognize you and represent the countless NTSAD families you have served for more than 50 years.

From when we first met in 2007, I have been awed by you and your remarkable recollections of each patient and their families. Whether it’s a child or an adult, you see the person, not just their disease. One Mom told me that you're her HERO! Another Mom shared how much it meant to her that you simply wanted to help her daughter.

Dr. Kolodny, your life has been marked by graciousness, compassion, and chesed; your overflowing loving-kindness for your patients and their families. Thank you from the bottom of our hearts.

And thank you, Sanofi Genzyme, for your generous donation to NTSAD and unwavering support of rare families around the world."

NTSAD Welcomes First Research Director

This month, Valerie Greger, Ph.D. joins NTSAD as the organization's first Research Director. She is an accomplished academic, scientist and leader with extensive experience in human genetics, genomics, molecular biology, and bioinformatics.

In her role, Valerie will lead NTSAD's research grant program and implement initiatives in clinical development, newborn screening, and early diagnosis. She also will be a resource for communicating scientific and medical information to patients.

Valerie is passionate about creating value from scientific discoveries, transforming ideas into reality and making an impact on patients' lives. Valerie will be working part-time at NTSAD, as she also works with the Yaya Foundation for 4H Leukodystrophy. Her combined experience at these organizations will enhance research in the rare leukodystrophy community.

NTSAD's Board of Directors recently welcomed Valerie where she shared, ***"Knowledge alone doesn't mean anything until it leads to the building of a product–that's when knowledge can make an impactful change."***

Since 2002, NTSAD's Research Initiative has made direct investments of more than $4 million in grants that have been leveraged to more than $30 million of investments in additional research, leading to new therapies.

Sue Kahn Receives 2022 Patient Advocate Leader Award

Congratulations to NTSAD's very own Sue Kahn for receiving the WORLDSymposioum 2022 Patient Advocate Leader (PAL) Award. Sue will be presented the award at the 18th Annual WORLDSymposium in San Diego, California on Tuesday, February 8, 2022.

Read the press release.

Making the Rare Diagnosis

No one knows the frustration and months, sometime years of desperation seeking a rare diagnosis better than members of the NTSAD Community.

In an effort to speed the time to diagnosis, so families and individuals may receive support, access to treatment, and better outcomes, NTSAD has launched an awareness campaign. Thanks to a donation from Passage Bio, NTSAD is running a "Make the Rare DX" campaign in *Neuro Review* magazine as well as on the NTSAD website and social media. The comprehensive campaign designed to educate the medical community features NTSAD families and their journey to diagnosis. The campaign highlights signs, symptoms, and risk factors. It also empowers families and promotes the importance of carrier screening. NTSAD gratefully acknowledges the pro bono creative work on the campaign by Acom Healthcare and NTSAD Board member Jonathan Katz.

Make the DX Campaign

Day of Hope 2021

This year marked the 11th Anniversary of Day of Hope and a decade of Hope! Day of Hope events unite people in our shared cause to find effective treatments. Since September 2011, our Community has raised nearly **$550,000 for the NTSAD Research Initiative.**

Thanks to families and companies participating in Day of Hope and raising rare awareness and money for research. Because of you, there are now clinical trials for Tay-Sachs, Canavan, GM1, and Sandhoff diseases.

THANK YOU: Skylar Benson, Kim Bursak, Ashley Cornett, Alan Croft, Amber Franzen, Katie Frys, Heidi Gaffney, Samantha Gamble, Lily Halim, Merlie Jackson, Shelley and Ian Karp, Felicia Kemp, Jakub Kupiszewski, Passage Bio, John Pollman, The Ronaldsons, Kim Rudness, Sio Gene Therapies, Faith and Derrick Stidham, Taysha Gene Therapies, Rose Urban, Andrea Weinbrect, Kirsten White, and Mindi Zollar.

Our partner **Passage Bio** show they **#CareforRare** on **#DayofHope2021**! Thank you for your support of NTSAD and our community!

Thank you to our partner **Taysha Gene Therapies** for the support of **Rare Families** this **#DayofHope2021** and everyday!

Make a Gift of Hope

IntraBio's GM2 Clinical Trial Update

IntraBio reports statistically significant and clinically meaningful improvements in the use of IB1001. The company held a multinational GM2 clinical trial for both pediatric and adult patients.

Read the full press release.

Lysogene Doses First Patient in Gene Therapy for GM1

Lysogene doses first patient in the United States with LYS-GM101 investigational gene therapy for the treatment of GM1 gangliodosis. The company seeks to enroll a total of 16 patients in the UK, US, and France.

Read the press release.

For information on the clinical trial.

Sio Doses First Early Infantile GM1 Patient

Sio Gene Therapies doses of first GM1 Gangliosidosis Early Infantile (Type I) patient in the company's ongoing Phase 1/2 Study of AXO-AAV-GM1 Gene Therapy. This is an exciting step for the company, NIH, and the GM1 community.

Read the press release.

American Society of Gene & Cell Therapy Patient Education Program

American Society of Gene & Cell Therapy has developed its Patient Education Program to help patients and families find comprehensive information about gene and cell therapy. The goal of the patient education program is to provide accessible, accurate, and responsible information and resources about gene and cell therapy to patients and caregivers. **There are now resources for Tay-Sachs and Sandhoff diseases in addition to GM1.**

Read the guide here.

Spread Awareness in September

September marks the National Awareness Month for Tay-Sachs, Leukodystrophy, and Newborn Screening. There are many opportunities to spread awareness, raise funds for research, and advocate for rare.

Read and share NTSAD's social media campaign, #BeRareAware, to build awareness about Tay-Sachs and Canavan, GM1, and Sandhoff diseases with new posts every Wednesday and Friday throughout September on **Facebook, Twitter, Instagram,** and **Linkedin.**

NTSAD 2022 Calendar

The NTSAD 2022 Calendar is dedicated to Sue Kahn. The Calendar will recognize Sue for creating a culture of unprecedented collaboration, advancing research leading toward multiple clinical trials, and above all else, supporting families.

Learn more about the calendar and how to purchase an ad in honor of Sue Kahn.

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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