



NTSAD Community News

Research, Collaboration, and Community



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

October

September 2022

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Imagine & Believe is three weeks away! Have you reserved your spot, yet?

Don't miss out on this opportunity to connect with the NTSAD Community as we commemorate our 65th Anniversary.

[RSVP Today](#)

On Thursday, November 10, we will gather in-person for the first time in three years at the Royal Sonesta Boston, and together envision a world with effective treatments for Tay-Sachs, Canavan, GM1, and Sandhoff diseases.

At ***Imagine & Believe***, we will honor research pioneer **Guangping Gao, PhD**, for his life's work in identifying the Canavan gene, revitalizing gene therapy, and his many accomplishments leading to potential treatments for Canavan disease and many rare diseases.

"Dr. Gao, is a pioneer in gene therapy and development of gene vectors for gene therapy and has been for a long time, really since his training. Dr. Gao is smart, brilliant, incredibly energetic, engaging, not only with other researchers and industry folks, but with families, and that's fabulous."

-Cynthia Tifft, MD, PhD, 2019 Imagine & Believe
Honoree

Every year, *Imagine & Believe* is attended by more than 150 industry members, researchers, clinicians, and rare individuals and families and raises more than \$120,000 for NTSAD programs and services as well as research. Thank you to our sponsors and supporters.

[Learn more about *Imagine & Believe* sponsorship opportunities or reserve your spot here.](#)

Imagine & Believe admission begins at \$200 per person, and sponsorships range from \$750 to \$25,000.

[Deadline to RSVP is November 4.](#)

[You can pay tribute to your loved one](#) or highlight your company by purchasing an ad in the event's digital program. Full screen \$1,000, Half screen \$500, Quarter screen \$250. **[Purchase an ad.](#)**

The event includes a reception, silent auction, and speaking program featuring NTSAD's leadership, an NTSAD family who shares their story, and a tribute to Dr. Gao. This year's silent auction includes many exciting, must-have items.

Silent Auction
preview*:



- Experience the beauty of Maine with a two-night stay on ROAM, a stunning houseboat.
- Roll the dice and enjoy a stay at the Encore, including dinner for two at Rare.
- Are you a big Red Sox fan? Add a Rafael Devers autographed baseball to your collection.



If you would like to donate an auction item, email Development and Communications Manager Sydnie Dimond at sdimond@ntsad.org.

**Thanks to Rheanna Sinnett, Encore, The Red Sox, and all of our generous auction item donors.*

Thank you to sponsors of *Imagine & Believe* 2022! It's not too late to become a sponsor. If interested, contact Director of Development and Communications Susan Keliher at skeliher@ntsad.org.

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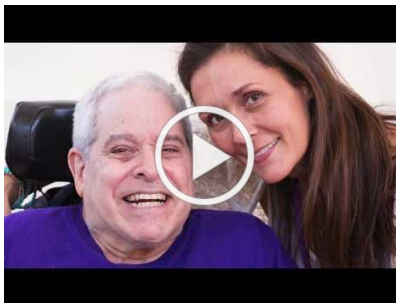
*NTSAD Board Member

(Sponsor listing as of October 20, 2022.)

65 Years of Imagining and Believing

Each year at *Imagine & Believe*, we remember the vision and determination of the families who founded NTSAD in 1957, and honor all the families, researchers, clinicians, and rare allies who continue to support our shared mission.

NTSAD is one of the oldest patient advocacy organizations in the United States. Learn more about our rich history and achievements. [Watch the video from our 60th Anniversary.](#)



In 2020 NTSAD Families shared their stories, experience, and advice at Imagine & Believe.

[Watch NTSAD families support other rare families.](#)



November 2019 is the last time Our Community gathered in-person for Imagine & Believe.

[Check out the 2019 event photos here.](#)



Successful GM1 Externally-led Patient-Focused Drug Development Meeting

On October 14, the GM1 community met virtually with the U.S. Food and Drug Administration (FDA) for the first ever GM1 Externally-led Patient-Focused Drug Development Meeting (EL-PFDD). This meeting, which took place during Cure GM1 Foundation's annual virtual conference, was an opportunity for affected families to share their experiences with GM1 directly with the FDA, medical product developers, researchers, advocacy organizations, and clinicians. Families' perspectives provide critical context when the FDA makes regulatory decisions for new drugs.

[Watch the GM1 EL-PFDD meeting.](#)

An FDA Patient-led Listening Session is being planned by NTSAD for early 2023 for Canavan disease. NTSAD is working toward hosting an EL-PFDD meeting for Tay-Sachs and Sandhoff diseases in the future.

Jaxson's Train of Hope

The Karp Family recently hosted the 3rd Annual Jaxson's Train of Hope Poker Run! Their Day of Hope event consisted of a poker run, silent auction, raffle, and lots of fun.

Shelley Karp, Mom to Jaxson, created a beautiful slideshow featuring many Rare Families.

[Watch the video featuring the special people in the NTSAD Community.](#)

Thanks to Shelley, Ian, Christine, and Jaxson Karp for all they do to raise awareness and funds for research. And thank you to their incredible community who came out to support Rare Families!



NTSAD to Hire Research Associate

NTSAD is seeking a part-time Research Associate to play an important role in coordinating NTSAD's Research Initiative and to aid Research Director, Valerie Greger, Ph.D. The ideal candidate will support scientific work groups and manage an international network of researchers, clinicians, and industry partners.

[Learn more about responsibilities, qualifications, and how to apply.](#)

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

NTSAD

Kathleen M. Flynn, CEO
Becky Benson, Family Services Manager
Sydnie Dimond, Development and Communications Manager
Valerie Greger, Director of Research
Susan Keliher, Director of Development and Communications
Diana Pangonis, Director of Family Services

2001 Beacon Street
Suite 204
Boston, MA 02135

info@ntsad.org
www.NTSAD.org

