



# NTSAD Community News

Research, Collaboration, and Community



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



## November

November 2023

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Thank You Sponsors and Auction Donors

On November 9th, the NTSAD Community gathered to raise funds for family services and critical research for Tay-Sachs, Canavan, GM1, and Sandhoff diseases. **The event, *Imagine & Believe*, was a huge success and raised more than \$150,000!** Thank you to all who supported this year's event to envision a world with effective treatments for these ultra-rare diseases.

At the event, we honored patient advocate expert and former NTSAD Executive Director Jayne Gershkowitz for her incredible life's work and always championing rare patients and families with compassion and her BIG heart! Congratulations and thank you, Jayne, for putting the focus on patients and their families.

Brian and Sherri Manning, whose son Dylan had Tay-Sachs, presented the award to Jayne at the event. Brian, who is an NTSAD past Board president, shared, "*Jayne wanted medical professionals attending the family conference to meet affected individuals and families. Jayne wanted them to hear our personal stories. She wanted them to see us, to hear us. She wanted them to make a personal connection with us so they would be inspired and remain motivated every day as they work to find treatments.*"



At the event, NTSAD CEO Kathy Flynn lauded Jayne who served as NTSAD's Executive Director from 1998 to 2006.

*"Jayne advocated for families and executed a vision that elevated our organization. She laid the foundation on which the Tay-Sachs Gene Therapy Consortium was built, piloted scientific meetings, and re-engaged the late onset community. Most notably, during her tenure, Jayne launched NTSAD's Research Initiative program, ushering our diseases from pre-clinical research to clinical trial readiness. Since its founding under Jayne in 2002, the Research Initiative has awarded more than \$4.7 million and 72 grants."*

Hear NTSAD families and others thank Jayne for her indelible

impact on the rare community in this [congratulatory video](#).



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## Annual LOTSS Think Tank Meeting



Last month, the [Katie and Allie Buryk Research Fund](#) and NTSAD hosted the 6th Annual LOTSS Think Tank meeting, bringing together a cohort of widely respected researchers and clinicians dedicated to Late Onset Tay-Sachs and Sandhoff (LOTSS). They gathered to strategize ways to design and accelerate research and discuss interventions for disease management.

Researchers gave updates, discussed results, and initiated collaborations on ongoing LOTSS research. Topics included small molecules that may be drug candidates for lysosomal

storage disorders; progress with the efforts to develop biomarkers; the distribution of the gene therapy vector in the brain of the sheep model; and gene-editing correcting the Gly269Ser mutation present in more than 90% LOTSS patients. Other presentations included an overview of lessons learned from other neurodegenerative diseases such as Amyotrophic Lateral Sclerosis (ALS) and Huntington's disease were also discussed and how they may apply to LOTSS.

In addition, three caregivers of LOTSS patients experiencing mental health/psychiatric symptoms shared their experiences during a panel moderated by Director of Family Services, Diana Jussila. **Their testimonies were very powerful, deeply moving, and demonstrated an urgent need for action. As a result, members of the Think Tank and NTSAD's Scientific Advisory Council will address mental health symptoms sometimes associated with LOTSS.**



Special thanks to Cynthia Tift MD, PhD, National Human Genome Research Institute, NHGRI, and Steve Walkley DVM, PhD, Albert Einstein College of Medicine, for organizing and co-chairing the LOTSS Think Tank Initiative, and the Buryk family for their generous hospitality and support.

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## Researchers Can Help NTSAD Grow a Virtual Biorepository of Patient Samples

During the summer of 2023, NTSAD launched its first ever inventory of patient samples from children and adults affected with Tay-Sachs, Canavan, GM1, and Sandhoff diseases to advance research and accelerate treatment. The [virtual biorepository](#) facilitates the sharing of cell lines and other patient samples among researchers and clinicians. NTSAD is currently reaching out to academic institutions and labs to share an inventory of patient samples available for sharing with the research community.

**If you are a researcher interested in contributing samples or requesting samples, please visit [our website](#) for more information or contact Research Director Valerie Greger, PhD, at [vgreger@ntsad.org](mailto:vgreger@ntsad.org).** Please note, NTSAD serves as a connection point between researchers but

does not physically collect or house biological samples.

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## National Family Caregivers Month



Commemorated every November, National Family Caregivers Month is a time to recognize and honor family caregivers across the country. With the holidays approaching, we understand the mix of emotions this season can bring for people who are newly diagnosed, providing care, and experiencing grief and loss. **The NTSAD Community sees you. We are here to support you. We appreciate everything you do as a caregiver for your loved ones.**

If your loved one for whom you provided care has passed, thank you for easing their days, continuing to care for their memory, and keeping their legacy alive.

We are sending you love and wishing you comfort.

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## Giving Tuesday: November 28, 2023



Created in 2012, [Giving Tuesday](#) is a day that encourages people to do good. Since then, it has grown into a global movement that inspires hundreds of millions of people to give, collaborate, and celebrate generosity on the Tuesday following Thanksgiving. [Join in this year and make a gift to NTSAD and help support more than 750 rare individuals and families worldwide.](#)

Hear from families directly on why your support of NTSAD makes a difference in their lives [in this video.](#)

[DONATE](#)

Hear from families directly on why your support of NTSAD makes a difference in their lives [in this video.](#)

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## Fine Jewelry, College Football, and Family Fun!

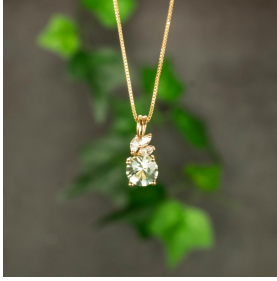
**Do you love fine jewelry, college football, or fun with family and friends?** Here's your chance to support families and buy a present for a loved one, or even yourself via our online auction!

**If you would like to purchase any of these online auction items, please email Development and Communications Manager Sydnie Dimond at [sdimond@ntsad.org](mailto:sdimond@ntsad.org) with your bid by 4 p.m. Eastern Time today, November 17th.**

### Artful Eye's Mint Garnet and Diamond Necklace

*Donated by Artful Eye Jewelers*

Buy yourself or a loved one this stunning custom 14k yellow gold pendant with a mint garnet and diamonds totaling .10 cwt. on a 14k yellow gold box chain, 18" length.



Est. Fair Market Value: \$1,998



### **Holy Cross vs. Georgetown Football Game on Nov. 18**

*Donated by Holy Cross Athletics*

Got plans for Saturday, November 18th? These four tickets to Holy Cross vs. Georgetown football at Holy Cross would make for a fun afternoon! Tailgate with a case of Samuel Adams beer.

Est. Fair Market Value: \$136



### **Boston College vs. Miami Football Game on Nov. 24**

*Donated by Boston College Athletics*

Enjoy the Friday after Thanksgiving (11/24) at the BC vs. Miami game at Alumni Stadium! These four tickets are for section U, row 19, seats 31-34.

Est. Fair Market Value: \$292



### **Family Fun Experiences**

*Donated by Xtremecraze, Ron's Ice Cream*

This package offers two different fun activities for family and friends. Visit Xtremecraze in Woburn, Massachusetts with this Family Fun certificate good for up to five people for either one session of laser tag, or one session at the air park, which includes trampoline and blow-up obstacle course. You also can enjoy \$100 worth of Ron's Ice Cream and Bowling in Hyde Park, or just Ron's Ice Cream in Dedham. Perfect for kids and adults, birthday parties, or the holiday break.

Restrictions: Xtremecraze: Good for one year from date of issue. Laser tag is ages 7 and up.

Est. Fair Market Value: \$158

# Thank You to Our 2023 Sponsors!

## BELIEVE



## HOPE

Tedra Cannella

Christine Chapman  
and William Ohle

Gerald Cox, MD, PhD\*

Jayne and Bruce  
Gershkowitz

Michael and Caitlin  
Gladstone



## INSPIRATION



Robin Lynn and  
Lawrence Blumberg

Sedra and Alan Schiffman



## DETERMINATION

Aaron's Fund



Adelman Associates

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Susan\* and Alan Roden  
and Family

## DREAM



Bradley and Nadia Campbell

Dawn Laney, MS, CGC, CCRC\*



\*NTSAD Board Member

## Thank You to Our 2023 Auction Donors!

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Xtremecraze

**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

**Kathleen M. Flynn**, CEO  
**Becky Benson**, Family Services Manager  
**Sydney Dimond**, Development and Communications Manager  
**Valerie Greger**, Director of Research  
**Diana Jussila**, (formerly Pangonis) Director of Family Services  
**Susan Keliher**, Director of Development and Communications

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