

**Families in Florida: We will be keeping you all in our thoughts as Hurricane Darian approaches the Southeast. Be safe, stay as dry as you can, and let us know when you're out of harm's way.**



**NTSAD Lifeline**



**Summer** should get a speeding ticket. - Anonymous

We know this time of year can be bittersweet for many families as the school year begins - a reminder of the milestones that are missed and those that will be missed. We want to take this time to say, "We see you and we're with you." As the dog days of summer begin to wind down, here are some photos of summers, past and present, to hopefully look back on with a smile.



NTSAD's new vice president, Oralea Marquardt, wrote about this time of year for **Courageous Parents Network** when her son, William, would have been entering 4th grade. This year, William would have been in the 9th grade. You can read the full article [here](#).



## Research

**BIG NEWS:** The GM1 Gangliosidosis gene therapy trial has begun recruiting at NIH. Read the latest from NIH [here](#).

### IntraBio Webinar

NTSAD hosted its first webinar with a presentation by IntraBio's Senior Vice President, Taylor Fields, and Dr. Tatiana Bremova from University of Bern in Switzerland. They spoke about their work and the current clinical trial for GM2 Gangliosidosis that is recruiting right now adults over the age of 18.

NTSAD and CTSF have been working closely with IntraBio as they've developed

their trials in the U.S. and Europe.

A recording of the webinar can be seen to the right. Links to publications about their work can be downloaded on the NTSAD website.



LEARN MORE

## Open Natural History Studies for GM1 and Canavan: How you can participate today

### GM1 Gangliosidosis Type 1 and 2

**University of Pennsylvania** and **Passage Bio** are sponsoring a new natural history study focused on infantile and juvenile GM1 Gangliosidosis.

The data gleaned from the study will inform future trials and help to determine clinical endpoints that will show if a therapy is working.

The study is not yet recruiting but to learn more about the two sites and the contact information in the U.S. and the U.K. visit [here](#).

### Canavan Natural History Study

**Aspa Therapeutics** will soon be launching their comprehensive natural history study, CAN-Inform. The more data they collect from families, the better for the trial, and other future treatment trials. They ask for Canavan families who have or have had a child with the disease to participate. It is enormously helpful to have several years of data for a child so we hope all families - younger and older - will participate.

To learn more about record retrieval and participation, please email Veristat [here](#).

## Meet NTSAD's Board President



**Staci Kallish, DO**, assumed the role of NTSAD Board president as of July 1st.

She is a pediatric geneticist who has been a part of the NTSAD family for many years and looks forward to her role as we finalize and implement a new strategic plan based on heading into the world of clinical trials for GM1, GM2, and Canavan diseases.

*Stay tuned to future issues as we introduce other board members to the NTSAD family.*

## Family Support

### Caring for Yourself as a Caregiver: Strategies to Help Yourself

*Borrowed from **Family Caregiver Alliance***

Caregiving is certainly not a 9-5 job where you get to leave at the end of the day, get a good night's sleep and get up refreshed. It is 24/7 multiplied by 1,000+. We've all heard it many times but it still holds a lot of truth - you have to "put the oxygen mask on yourself first, and then you can help others." The group, Family Caregiver Alliance, published an article and we pulled their tips from the NIMH on how to deal with depression, which happens to many of us when it all gets overwhelming.



Remember, there is no shame in asking for help from friends, your doctors, and your NTSAD family.

[READ MORE](#)

## Respite Program Opportunity

The National Organization for Rare Disorders (NORD) has a new program that could be a wonderful resource to help the caregivers out there to get some respite to recharge.

"The Respite Program provides financial assistance to enable the caregiver a break to attend a conference, event or simply have an afternoon or evening away from caregiving."



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## Events | Fall 2019

### Day of Hope | September 21, 2019

This year's Day of Hope is as important as ever ... to help raise the funds to do the work to support families as we prepare for clinical trials. And while families have told us they find real meaning in hosting their events, we also know a little competition is good for the spirit.

Click through to read more, see where the events will be, and join others to push our total goal (since 2011) to over \$500,000 - that's 1/2 a million dollars!



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### Imagine & Believe | October 30, 2019

NTSAD will honor **Dr. Cynthia Tiff** and the many families of NTSAD at this year's **Imagine & Believe** benefit in Boston. We will gather with others who share our mission to support families, fund research, and promote awareness. As always, the family voice is important so if you'd like to be there in person or through a digital ad (50% discount for families) that will be running throughout the evening, let Patrick know [here](#).



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## Remembrance: Say Their Names



“Those we love and lose are  
always connected by heartstrings  
into infinity.” - *Terri Guillemets*

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**Maximus Gutierrez**  
01.19.2017 ~ 8.24.2019

*Keeping Briana, Gabriel and their families  
in our thoughts this weekend. #MaximusStrong*

### NTSAD Family Support

**Diana Pangonis** | Director of Family Services (email [here](#))

**Becky Benson** | Conference Coordinator (email [here](#))

**Monica Gettleman** | Co-chair, Family Services Committee (email [here](#))

**Oralea Marquardt, MSW** | Vice President, NTSAD Board  
Co-chair, Family Services Committee (email [here](#))



2001 Beacon Street, #204  
Boston, MA 02135  
(617) 277-4463  
[www.NTSAD.org](http://www.NTSAD.org)

