# NTSAD Lifeline

## Community and Connections for Families and Individuals

**OCTOBER 2020**

### In this Issue

## Help accelerate drug development for GM2

Your experience with GM2 is vital to helping researchers advance treatments for our community.

By partnering with AllStripes, formerly RDMD, patients and patients' families allow their de-identified medical records to be shared with multiple research studies for GM2 easily and from the comfort of your own home. AllStripes does the work in collecting medical information, and patients receive all of their medical records in one secure account, at no cost.

The goal is to have at least 30 patients participate. **Join and register here**.

---

## Passage Bio's Orphan Drug Designation in Europe

Passage Bio recently received an Orphan Drug Designation for GM1 treatment in Europe from the EMA - the European equivalent to the U.S Food and Drug Administration. What does this mean? "With EMA orphan drug designation, Passage Bio will receive certain benefits and incentives including 10-year market exclusivity for the approved therapeutic indication once PBGM01 receives marketing authorization as well as clinical protocol assistance and reduced regulatory fees." Read the full press release **here**.

---

## Join NTSAD's Imagine & Believe Virtual Event!

**Tuesday, November 10, 2020**
4:30 p.m. (Eastern) / 3:30 p.m. (Central) /
1:30 p.m. (Pacific)

Families are welcome! It's free to attend for anyone and everyone! Register to join **HERE**.

**NTSAD's Imagine & Believe** is a live virtual event where we honor patients and families, who will share their stories, experiences, and hopes for the future. After all supporting families is the center of everything we do! NTSAD families and community members Ryan Miller, Lorelei Sandoval, Justin Ungerleider along with a dozen more families will participate in the evening's program.

The inspiring evening also includes NTSAD’s Board President Staci Kallish and Executive Director Sue Kahn who will highlight NTSAD's recent accomplishments, advances in research, and our direction for the new year. In addition, Dr. Guangping Gao, a gene therapy pioneer and researcher, Dr. Florian Eicheler NTSAD Advisory Board member and respected clinician, and Sherri Sigel, NTSAD Mom will talk about the journey to a clinical trial.

**Please be sure to register** for the live, virtual event on **Tuesday, November 10th - 4:30 - 5:30 EST**, so you may receive the link to join us! The event will be hosted via Zoom and Facebook Live. Imagine & Believe raises vital funds for supportive services for individuals and families affected by Tay-Sachs, Canavan, GM1, and Sandhoff diseases. Thank you to our sponsors.

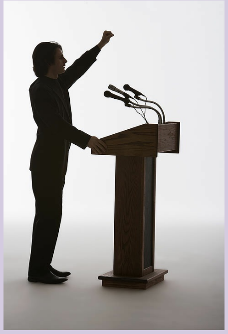
---

## Caregiving in the Age of COVID

FCA
FAMILY
CAREGIVER
ALLIANCE®
National Center on Caregiving

The holidays are coming up and now more than ever it's important to remember to take care of yourself. The team at the **Family Caregiver Alliance** has put together a webinar titled "**Well-Being Tips: Stress Management for Family Caregivers**" that will be held on Sunday, November 18th from 11-12 p.m. (Pacific).

Register **here** to attend.



## Sharing Your Experience

As the landscape of research and clinical trial development advances, companies often ask us about the "patient" experience, and we want to give you the opportunity to share your story via a survey, a Zoom chat, or a letter.

If you are interested in sharing your story and want to join our network of families willing to speaking with industry members, click **here** to email Diana to include you.

## NTSAD Family Connections November Zoom Chat

RARE MAMAS
Empowering Rare-Disease Moms from Distress to Prowess

We're excited to announce November's topic for the NTSAD Family Connections Zoom Chat!

November’s topic, *The Fight of our Lives* will consist of a discussion and Q&A with **Rare Mama’s** Founder, **Nikki McIntosh**.

**Save the Date: Tuesday, November 17, 2020**
**7 p.m. (ET) / 6 p.m. (CT) / 4 p.m. (PT)**

For this special event, we invite all our families caring for a child to please join us for an in-depth conversation with Nikki centered on the challenges of daily life in caring for children fighting a rare disease, and building resiliency in “**The Fight of Our Lives**”.

Please join us in congratulating the
**Stanley & Jeffrey Gottlieb Sibling 2020 Memorial Scholarship Fund Recipients!**

Jeremy Davis
Kyla Marquardt
Jake Rabinowitz
Abby Seacord
Justin Ungerleider

Details about 2021 grant cycle will be announced in the spring of 2021.

The monthly NTSAD Family Connections October Zoom chat about the Parent & Sibling Perspective was a profound conversation between parents and older siblings. We plan to continue the conversations between parents and their healthy older children in 2021.

If you want to learn more about connecting your healthy children with their NTSAD peers, please contact Becky Benson, NTSAD's Family Services Coordinator **here**.

## A Resource in Development: Sneak Peek into Content

There is no rule book,
No time frame,
No judgement.

Grief is as individual
as a fingerprint.
Do what is right for
your soul.

The NTSAD Family Services team is developing a series of newsletters for families in the months following a diagnosis and after connecting with NTSAD, as well as the months following the death of a child. We recognize and understand the challenges navigating life after it has been turned upside down. We always welcome your thoughts on what would have been helpful in the months following diagnosis and loss - please feel free to share them with Becky **here**.

One piece we thought could be helpful for our bereaved families as we approach the holidays is Allan Wolfelt's "Mourner's Bill of Rights" which states the following:

***Though you should reach out to others as you do the work of mourning, you should not feel obligated to accept the unhelpful responses you may receive from some people. You are the one who is grieving, and as such, you have certain "rights" no one should try to take away from you.***

Read the full bill of rights **here** and stay tuned for more!

## Say Their Names

**Finnegan Loughran**
*April 25, 2019 - October 3, 2020*
Son to Jennifer and Mike Loughran
*Little brother to Eilish and Serafina*

**If your loved one's name is missing, our deepest apologies.*
*Please send Diana the right information and dates to make those corrections.*

**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**

**Donate**

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

**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**