



NTSAD Lifeline



Be strong, be fearless, be beautiful. And believe that anything is possible when you have the right people there to support you. - Misty Copeland

News about Clinical Trials and Natural History Studies

Sanofi Genzyme's Venglustat Study

This trial is not yet recruiting but the details have been posted [here](#) on clinicaltrials.gov. We worked with the team at Sanofi Genzyme on a set of FAQs that can be found [here](#). If you have other questions that aren't answered, please contact Diana Pangonis, Director of Family Services, [here](#).

Axovant Gene Therapies Update

The good news is that Axovant submitted their Investigational New Drug (IND) application for their GM2 gene therapy program at the end of 2019. The FDA had questions and Axovant has responded. We will share updates with you as soon as we can.

GM1 Natural History Study (UPENN) is open for enrollment

Two sites are now open for families to enroll their children in the natural history study for infantile and juvenile GM1 gangliosidosis. The sites are Great Ormond Street Hospital in the UK and Children's Hospital of Philadelphia (CHOP). The goal is to have 20 infantile and 20 juvenile patients enrolled in this study to ultimately gather data that can benefit future clinical trials. See the full study from UPenn [here](#).

For CHOP - contact Bianca (FERREIRAB@EMAIL.CHOP.EDU) to discuss scheduling an appointment for the study.

For GOSH - contact Julien Baruteau (julien.baruteau@gosh.nhs.uk). Families are welcome to email him directly

Canavan Natural History Study still enrolling!

We encourage our Canavan families to also enroll in the study sponsored by Aspa Therapeutics.

The more data the better!

Please visit treatcanavan.com to more about enrolling in this important study that is so critical to future clinical trials.



NTSAD Be Informed Webinar Series

Did you know that we've already hosted three webinars?

You can watch them all on the NTSAD website [here](#) to learn about IntraBio's trial, How Clinical Trials Work, and How the FDA Works. If there are topics you think might be helpful, let us know! Email Diana [here](#).



Stay tuned for our next webinar in February 2020. Topic will be announced soon.

February 29, 2020 is World Rare Disease Day.

It's a perfect opportunity to share your voice and pictures that week to bring awareness to our unique rare community. If you happen to be in the Washington, D.C. area and want to join Diana and other advocates on Capitol Hill, email Diana [here](#) if you're interested! You can find out about **Rare Disease Week** [here](#).



In the meantime, a new profile Facebook frame will be available on February 1st so we encourage you to change your profile picture for the month. And send a favorite picture of who is rare in your life to Diana [here](#) and we'll feature them on our Facebook page during the month of February.

NTSAD 42nd Annual Family Conference

May 28 - 31, 2020 | Renaissance Stapleton | Denver, CO

Hotel reservations, registration and Helping Hand Grant Applications are now open.

Visit www.ntsadannualfamilyconference.com to reserve your room, register, access the applications and learn how you can sell virtual raffle tickets!

A few notes:



There will be RARE Bears again this year for families who are attending the conference and have not yet received a bear from this program. If your child has received a RARE Bear either through the conference, another meeting or organization, they will not be eligible to receive another one. This program is separate from NTSAD with their own requirements.

To our newly diagnosed or newly bereaved families - please reach out to Becky [here](#) if you have questions, concerns and/or fears about coming to your first conference. We know it's scary, overwhelming and daunting to think about - we're here to make it as easy and as unintimidating as possible.

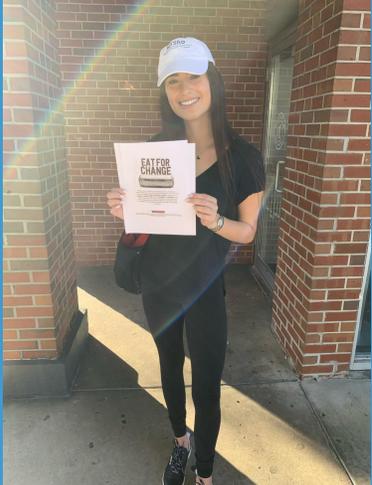
Helping Hand Grant applications are open now [here](#) and we encourage you to apply as soon as you can - even if you're unsure about coming. If you have questions, let Becky know [here](#).

Helping Raffles Update - We know it's a bit of change moving to all [online](#) and to make that easier, we'll be sending updates to those of you selling raffles. Feel free to share [this link here](#) with your family and friends if you want to raise funds for the conference experience. The drawing will be on Saturday, May 30th and there are three cash prizes - \$1,000 - \$500 - \$250.

There will be a family slideshow running throughout the conference at our meals. Please send in special photos of you and/or your family at home to Bonnie [here](#) no later than May 6th to give her time to create it. This includes our Late Onset family too!

Sibling Highlight

Carly Gettleman planned and hosted a fundraiser at The University Florida's local Chipotle benefitting NTSAD. With over 150 fellow students in attendance, Carly was able to raise funds for NTSAD and honor her sister Brooke.



We welcome you to share your own sibling highlights as well – please send them to Diana [here](#).

NTSAD Family Service Hours Online

with Becky Benson and Diana Pangonis

Every Friday from 10:00-11:00 am (EST). There is no pressure to stay for the whole hour so you can still stop by even if you have just 15 minutes. We'd love to see you.



Follow this link [here](#) to access the meeting every Friday.



Say their names...

(November 2019 - January 2020)

Drew Anderson

August 10, 2015-December 21, 2019
Son of Brianne and Adam Anderson
Brother to Kayleigh and Julie

Carolina Pereira

May 19, 2013-January 6, 2020
Daughter of Ricardo and Mafalda Pereira
Sister to Santiago

Grayson Kemp

March 18, 2014-December 7, 2019
Son of Felicia and Chris Kemp
Brother to Logan and Ellie

Rebekah Schiffman

April 25, 1986-December 29, 2019
Daughter of Karin and Howard Schiffman
Sister to Matthew

Grayson Lunchick

January 4, 2017-November 10, 2019
Son of Paisley and Jon Lunchick

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



Welcome to the World Little Ones!

Reagan Jade Redfield

Born on January 15, 2020

Little sister to Ava Rose
Daughter of Kristen Frederic
and Dan Redfield



Rylan Stringer

Born on January 9, 2020

*Little brother to Carson and Brennan
Son of Holly and Royce Stringer*



Staff

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