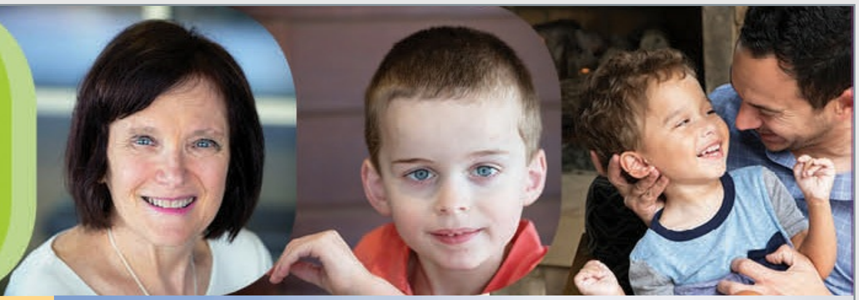
NTSAD

NTSAD Lifeline



In this issue...

It's official. It's fall and with that comes cooler weather, all things pumpkin spice, Halloween, and the inevitable onset of the sniffles, colds and the ominous flu viruses. How do we stay healthy and most importantly how do we keep our kids germ-free? Here is some advice collected over time from the **NTSAD Family Support Group** page. If you have other tips and tricks, please feel free to share them on the page.

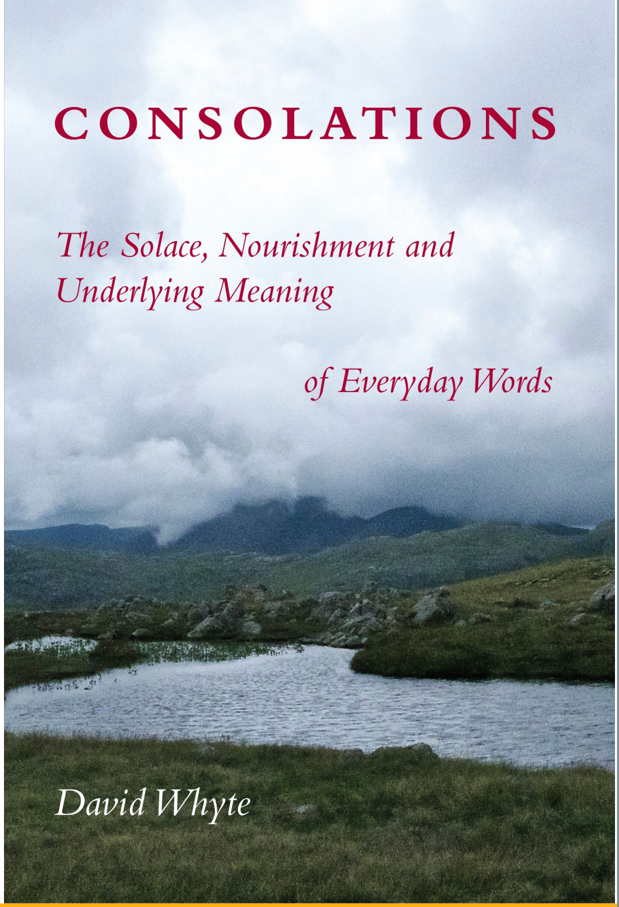
- For prevention, we got into a routine of having the kids change out of school clothes immediately from returning from school, and got them in the habit of washing their hands. When a cold did hit, we did lots and lots of hand washing.
- Masks for those in your home who are potentially sick or who has a virus, and keep them away.
- Hand sanitizers. Vitamin C.
- I am putting up a sign telling people if they are sick or been around people that are sick to stay away from us!
- I put hand sanitizers, gloves and masks in my entry way.

Consolations: The Solace, Nourishment and Underlying Meaning of Everyday Words by David Whyte

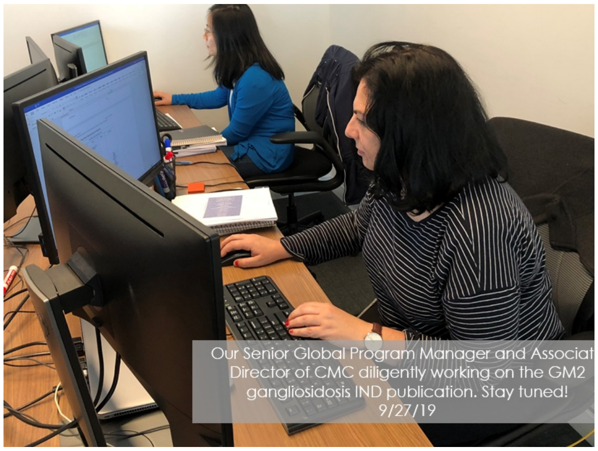
I was introduced to this beautiful book a couple of years ago and realized I should share it with all of you. Perhaps you will connect to some of the definitions at different points in time, but this definition, in particular this segment on "despair" spoke to me after a recent difficult conversation.

An excerpt from DESPAIR...

The antidote to despair is not to be found in the brave attempt to cheer ourselves up with happy abstracts, but in paying a profound and courageous attention to the body and the breath, independent of our imprisoning thoughts and stories, even strangely, in paying attention to despair itself, and the way we hold it, and which we realize, was never ours to own and to hold in the first place...Refusing to despair about despair itself, we can let despair have its own natural life and take a first step onto the foundational ground of human compassion, the ability to see and understand and touch and even speak, the heartfelt grief of another.



Research: The topic of sharing updates


Our Senior Global Program Manager and Associat Director of CMC diligently working on the GM2 gangliosidosis IND publication. Stay tuned! 9/27/19

We are in a time of great hope and promise for potential treatments, but we also recognize it is a stressful time as we wait to hear more information about these trials.

No news means they are working 24/7 on all that it takes to get these treatments moving into clinical trials. Our job, as a patient advocacy organization, is to be a conduit to convey the latest news from the companies. They're job is to make sure every T is crossed and every I is dotted. They are also bound by their own rules to keep data and information confidential which means we are oftentimes bound by a confidentiality agreement. These protocols are in place to protect the program to ensure we get to clinical trials. You can be assured that we will share the latest news when we receive updates. In the meantime, our friends at Axovant shared this picture of their "war room" where the work on the GM2 IND publication is happening - a critical step toward launching clinical trials.

Stay tuned for future NTSAD "Be Informed" webinars about the process of drug development, clinical trials and the importance of the patient experience.

Canavan Natural History Study Launches

CANinform, the new Canavan Disease Natural History Study is now open! We encourage all families to participate in this important study.

To enroll, call 1-833-764-227 or email CanavanMedRec@veristat.com.

Aspa is providing a record retrieval service at no charge, since your child's medical record will be an important component of Can*inform*.

TO ENROLL IN *CAN-inform*:

CALL	1 Families can **call** 1-833-764-2267 or email CanavanMedRec@veristat.com to use a free service to collect medical records
CONSENT	2 Fill out a short online form to provide **consent** to share medical provider information and begin the process of obtaining the records
CONTRIBUTE	3 Once records are retrieved, **contribute** to the Canavan community by enrolling in *CAN-inform*, the Canavan natural history study. Support other Canavan families by joining this important effort

GM1 Type I Natural History Study

NIH U.S. National Library of Medicine

ClinicalTrials.gov

A natural history study of GM1 Gangliosidosis Type I and Type II will be launched shortly to better understand the progression of the disease that will ultimately inform outcome measures for clinical trials.

More information can be found here.

GM1 Family Meeting & Global Genes Summit

NTSAD was represented at the Global Genes Summit and the **GM1 Family Meeting** organized by our friends at the Cure GM1 Foundation. The updates at the GM1 meeting were enlightening and hopeful from Axovant, Gain Therapeutics, NIH, and PassageBio. It was terrific to see families from Australia, the Philippines, Sweden, Germany and the U.S. come together!

The **Global Genes Summit** was filled with many interesting sessions, meetings of the minds and insights gained from other patient advocates and their organizations. Our goal is to continue seeking new and improved ways in which we support families, advance research and encourage awareness. Creating relationships is critical in pursuing those goals as collaboration is important in a community as rare as ours. We look forward to more progress and even a new Five-Year Strategic Plan for NTSAD!

Support the Reauthorization of the Newborn Screening Act in the Senate.

As an advocate for patients with rare diseases you are a very important part of the legislative process. You can make the difference as you are the voices your legislators want, or in some cases do not want, but need to hear. Please complete the form on the right to take action and contact your Members of Congress.

After you take action your job is not done! The final step is to share the action alert with your family, friends, co-workers and any other people that might be interested in taking action on behalf of the rare disease community.

Contact Your Representative

From EveryLife Foundation - the NBS Act needs the support of your representatives.

Day of Hope: Getting Closer to $500,000!

Thank you to the families, their friends and communities for rallying hope and raising funds to support families as we march toward treatments! As of September 29th, we are just $21,500 away from the HALF MILLION DOLLAR mark since 2011! Wow! You all have been a part of that success!

Imagine & Believe Boston Benefit

JOIN US FOR
AN EVENING TO
Imagine & Believe
WITH NTSAD

OCTOBER 30, 2019 6-9 PM
ROYAL SONESTA, CAMBRIDGE, MA **DINNER | SILENT AUCTION | PROGRAM**

If you have a message or photo you'd like to share in our Imagine & Believe digital program book, please email Patrick Woods (**here**) or Oxana Surugiu (**here**) for more information on a 50% discount for families.

We are excited to honor NTSAD's good friend, Dr. Cynthia Tifft, in recognition for her decades long career in finding treatments for these diseases. If you have a picture with Dr. Tifft you'd like to share, please send them to Oxana.