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Topic of  
the Week



RESEARCH

PREVENT

SUPPORT

Together we face any challenges  
as deep as the ocean and  
as high as the sky.

- Sonia Gandhi

Friday, November 21, 2014

## *Mark Your Mark and Make a Difference!*

It is easy to forget that advocacy is an important component in caring for families and finding a cure.

In order to receive the best healthcare, the right to benefits to help your affected loved one, and access to drugs that could help,

there must be legislation in place to ensure you can get all of the above.

That is why it is critical that your local congress and state representatives hear your stories. We know it's tough to find time to write a letter, make time to visit your local legislators or make a phone call, but at the end of the day it can really help.



NTSAD is lucky to be a part of an incredible network of rare disease groups and grateful to organizations like the **EveryLife Foundation of Rare Diseases** , **Global Genes**, **National Organization for Rare Disorders**, and

**Rare Disease Legislative Advocates.** Each of them keep us informed as to what is happening on Capitol Hill that impacts the rare disease community. All they ask is for us to share that information with you.

**Consider taking a moment and making your mark today.**

## Your Help is Needed!

The National Organization for Rare Disorders sent out the following advocacy alert for patient groups like NTSAD. If you have questions about what this means, please contact Sue Kahn at [skahn@ntsad.org](mailto:skahn@ntsad.org).

### **FDA Seeks Comments by December 4th on Patient Role**

The Food and Drug Administration (FDA) is seeking input on how best to obtain the views of patients during the medical product development process and how to consider patients' perspectives during regulatory discussions.



This includes both input on current activities and suggestions for new approaches to assure that the patient voice is heard.

**The deadline for submitting comments is December 4, 2014.** Comments may be submitted electronically using the link below. Written comments may be mailed to:

Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Room 1061, Rockville, MD 20852. All comments should be identified with the following docket number: FDA-2014-N-1698.

This request for input is being made in conjunction with the FDA Safety and Innovation Act (FDASIA).

**[READ FDA NOTICE](#)**

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An Opportunity to Make Your Mark!

This is a terrific opportunity for NTSAD to be a voice in the future of how families and individuals can have access to investigational drugs that are developed for our group of rare diseases. **Please contact NTSAD's Executive Director, Sue Kahn, ASAP at [skahn@ntsad.org](mailto:skahn@ntsad.org) if you're interested in attending this workshop in Washington, D.C. on December 10th.**

*Note: There may be travel scholarships available for those coming from a distance.*

## The Role of Investigational New Drugs in Patient Care

### Invitation-only Workshop

**J.W. Marriott, 1331 Pennsylvania Avenue, N.W.,  
Washington, D.C.**

**Wednesday, December 10, 2014, 8:30 a.m.-3:30 p.m.**

The ALS Association, American Autoimmune Related Diseases Association (AARDA), Cancer Support Community (CSC), Center for Information and Study on Clinical Research (CISCRP), Leukemia and Lymphoma Society (LLS), and Pharmaceutical Research and Manufacturers of America (PhRMA), are pleased to invite you to attend an invitation-only workshop to explore issues relating to expanded access programs and patient care on **Wednesday, December 10, 2014** at the J.W. Marriott, 1331 Pennsylvania Avenue, N.W., Washington, D.C.



**Sherri Epstein attended an advocacy-related workshop recently in D.C. We encourage you to make your mark this way too!**

Expanded access, including treatment use in individual patients, is the use of an unapproved investigational drug outside of a clinical trial to treat a patient with a serious or immediately life-threatening disease or condition, when there are no other comparable or satisfactory alternative treatment options. For patients with a serious or life-threatening disease who are ineligible or unable to participate in a clinical trial, use of an unapproved investigational drug via an expanded access program may be an option.

The workshop will convene patient and provider groups, industry, academia, think tanks, and government regulators to explore approaches to optimize the existing Food and Drug Administration (FDA) Expanded Access process, while helping to ensure timely patient access to safe and effective medicines. We hope that you will be able to attend the workshop which will include a

substantive discussion on issues related to expanded access and ways in which stakeholders may be able to work together on this important issue.

The workshop will begin at 8:30 a.m. and will conclude around 3:30 p.m. Breakfast and lunch will be served.

Please RSVP by clicking on this link **Stakeholder Workshop on Expanded Access** by November 21, 2014.

We look forward to seeing you on Wednesday, December 10, 2014.

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## Do You Know Someone Courageous?

### Nominate a Patient or Family Member for NORD's 2015 Portraits of Courage Gallery

NORD is seeking nominations from their Member Organizations, Corporate Council, Board Directors, Scientific & Medical Advisory Committee and the broader rare disease community for their 2015 Portraits of Courage Gallery.



The Gallery honors individuals who have:

- Demonstrated courage in their own journey with a rare disease or helped others find courage in their journey;
- Shown remarkable resilience in the face of hardship; and
- Provided advocacy on behalf of all patients living with rare diseases.

Stories and photos of the individuals selected for the 2015 Gallery will be displayed at the NORD Portraits of Courage Celebration in May and at other events throughout the year. Individuals to be honored may be patients (children or adults) or caregivers (parents, spouses or other family members). **December 3rd is the deadline for nominations.**

**Nominate someone courageous today [here!](#)**

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# Mark Your Calendars!

## Rare Disease Week on Capitol Hill February 23 - February 27, 2015

World Rare Disease Day and the week leading up to the day is our chance to be loud and noisy with other patient groups to bring attention to rare diseases like Tay-Sachs, Canavan, GM1, Sandhoff, Niemann-Pick and many others. If you live in or close enough to the Washington, D.C. area, we encourage you to try to make some of the events happening during this week. The Rare Disease Legislative Advocates (RDLA) work hard on our behalf so let's join them in their efforts!



### Scholarship Info:

The EveryLife Foundation for Rare Diseases is providing \$600 travel stipends for two advocates in each of the 48 continental United States (outside of the DC metro area). \$1,000 travel stipends for advocates from Alaska and Hawaii. You must attend both the Legislative Conference & Lobby Day to be eligible.

View Travel Scholarship Application [here](#). **Deadline: December 31, 2014**

Scholarship recipients will be announced: January 4th, 2015

Visit [this page](#) on the RDLA website to see more information and what is in store for Rare Disease Week!

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## New NTSAD Conference Session Debuting in 2015!

The "Honoring Your Loved One through Action" session has a new name that reflects it's new format. "Making Your Mark" will have two purposes now. The first half of the session will stay focused on ways families honor their loved ones whether it's raising awareness, hosting an event or participating in the annual "Day of Hope." Lots of ideas are shared in this session and the energy is terrific! We had nearly 70 people attending this program in 2014!

The second half, however, will focus on advocacy - why it's important and how it makes a difference to the future of rare diseases like ours. It's always been a mysterious topic and hard to define in the context of the conference. That will change and we will all be experts by the time the session is over!



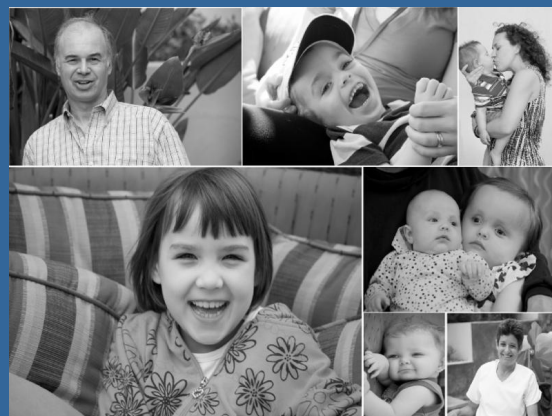
Stay tuned for more information in the coming months!

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## Supporting NTSAD Makes a Difference!

We welcome gifts to support our many efforts to fund research, care for families and their loved ones. Gifts can be made through our website [here](#). *Thank you* for supporting NTSAD and giving hope to all our families!



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## Contact Us

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