

NEWS FROM NORD

AN UPDATE FOR OUR MEMBERS AND FRIENDS

WINTER 2012 / 2013



JOIN US IN CELEBRATING THE 30TH ANNIVERSARY OF NORD AND THE ORPHAN DRUG ACT!

SPECIAL EVENTS TO TAKE PLACE
THROUGHOUT 2013 FOR THE ENTIRE
RARE DISEASE COMMUNITY.

As we approach the 30th anniversary of NORD and the Orphan Drug Act, we invite you to join us in a year-long series of special events in 2013. With the National Institutes of Health (NIH) and the Food and Drug Administration (FDA), NORD has planned a 30th anniversary observance on the theme, *A Historic Past, A Promising Future*.

WHAT IS PLANNED FOR 2013?

There will be special events – some virtual and some at specific locations – throughout the year. Three highlights of the observance will be:

- **Rare Disease Day 2013** – Feb. 28, 2013
- **NORD Partners in Progress Gala** – May 14, 2013, at the Mellon Auditorium in Washington DC
- **The 3rd Annual U.S. Conference on Rare Diseases and Orphan Products** – Hosted by NORD and DIA, this conference will take place in October in Washington DC.

A Historic Past

The *Orphan Drug Act* is considered an extremely successful piece of legislation, with more than 2,000 orphan products in the pipeline since 1983 and more than 450 approved for marketing. NORD, too, has a strong history of success in advocacy, education, research and patient/family services.

A Promising Future

The science of rare diseases is advancing faster than ever. In advocacy, the *FDA Safety and Innovation Act*, signed into law a few months ago, represents a major success. Also, interest in developing products for rare diseases is at an all-time high, according to recent reports such as one published in October 2012 by Thomson-Reuters.

Throughout 2013, NORD will keep you informed of ways to get involved in 30th Anniversary events, many of which will be virtual events that you can join from home. We encourage you to visit the NORD website (rarediseases.org) and the Rare Disease Day US website (rarediseaseday.us) often to get the latest news about opportunities to get involved.

Share your memories, suggestions for future action, and more with NORD at any time during the year by writing to 30Anniversary@rarediseases.org.

HOW IT ALL BEGAN

In the decade before 1983, only 10 new drugs were developed by industry to treat patients with rare diseases. Congress was considering legislation to address this problem but the debate had become stalled.

The patient community played a key role in focusing national attention on the need for this legislation. The *Orphan Drug Act* was passed by Congress and signed by President Ronald Reagan on January 4, 1983. It created financial incentives to encourage development of treatments for rare diseases.

Patient advocates realized the value of working together and formed NORD with Abbey Meyers, who had been one of the leading advocates for the *Orphan Drug Act*, as its president. Today, NORD continues to serve as the hub of the rare disease community in the U.S.



Mark McClellan, MD, PhD, the Brookings Institution; Richard H. Bagger, Celgene Corporation; Miriam O'Day, Alpha-1 Foundation; and Peter L. Saltonstall, NORD

CONFERENCE HIGHLIGHTS COLLABORATION, INNOVATION

Event Draws Together 500 Stakeholders to “Shape the Future Now”

The 2nd Annual U.S. Conference on Rare Diseases and Orphan Products, hosted by NORD and DIA (the nonprofit Drug Information Association), was judged a major success, with nearly 500 participants representing the entire rare disease community – patients, researchers, government, industry and investors.

The event featured both general sessions and three tracks (Research/Regulatory, Advocacy and Special Challenges). Major recurring themes included:

- *The importance of collaboration*
- *The need for regulatory advances to match the current pace of scientific advancement*
- *The need for increased investment in rare diseases and orphan products*



Flaminia Macchia, Director, European Public Affairs, EURORDIS



Charles Mohan, United Mitochondrial Disease Foundation; Cole Werble, Prevision Policy LLC; David Meeker, Genzyme



FDA Deputy Commissioner Stephen Spielberg, MD, PhD



Patient organization leaders tell their stories in a special session for the media.

JOIN NORD, EURORDIS AND PARTNERS AROUND THE WORLD: SUPPORT RARE DISEASE DAY 2013!



Rare Disease Day 2013 marks the 5th year this international advocacy day is being celebrated in the United States and also coincides with the year-long celebration of the 30th anniversary of the Orphan Drug Act and the founding of NORD. As a result, we intend to make this the best Rare Disease Day yet!

Rare Disease Day began in Europe to bring recognition to rare diseases as a global health challenge, and NORD works closely with the global planning team to select a theme, poster, and messages to be used around the world each year. This year's theme is Rare Disorders Without Borders.

HERE ARE SOME OF THE WAYS YOU CAN GET INVOLVED:

Go to RareDiseaseDay.US for details

- Sign-up to be a partner or ambassador to access a press-kit and receive regular updates from NORD about what others are doing across the U.S. and around the world.
- Submit a photo of yourself holding the Rare Disease Day logo for the Handprints Across America Gallery.
- Download the High School Rare Disease Day Curriculum or other classroom resources from the Rare Disease Day U.S. website and encourage teachers you know to use these materials.
- Join us on social media to follow the conversations and see how others are celebrating. Go to facebook.com/RareDiseaseDay.US
- Plan an event at your state capitol or statehouse to bring awareness to the major state-level policy issues: NORD will provide tools for this at rarediseaseday.us.
- On Rare Disease Day, send a letter to your political representatives through NORD's website (rarediseases.org) in our Handprints on the Hill campaign.

MAKE YOUR VOICE HEARD: JOIN THE CONVERSATION ON NORD'S NEW BLOG!

NORD has launched a blog for the entire rare disease community. You can access it from NORD's home page (rarediseases.org) or go directly to blog.rarediseases.org.

In a "Welcome to our Blog" message, NORD President and CEO Peter L. Saltonstall outlined the broad-ranging nature of the conversation, noting that "We'll cover 'hard news' related to developments on Capitol Hill as well as the softer side of the news with personal reflections from members of NORD's staff and guest bloggers about their experiences with rare diseases."

Topics to be covered, he said, include the looming "budget cliff" and how it may impact rare disease patients; implementation of the insurance reforms in the *Affordable Care Act* and the extensive new rare disease provisions in the *FDA Safety and Innovation Act*; and innovative efforts by patients and patient organizations to encourage the development of treatments for rare diseases. 🍌



blog.rarediseases.org



NORD HOSTS REGIONAL MEMBER MEETING IN PHILADELPHIA

In September, NORD hosted a one-day workshop for its member organizations in the Philadelphia area. This is the most recent in a series of regional member meetings. Previous ones have taken place in Chicago, San Francisco and New York.

Speakers included NORD President and CEO Peter L. Saltonstall, Senior Vice Presidents Pamela Gavin and Mary Cobb, and other staff members. In addition, representatives of the NORD Board participated, including Board Chair Michael Scott; Pat Furlong, founding president and CEO of Parent Project Muscular Dystrophy; and Estelle Benson, founder of GBS/CIDP International.

Also, several leaders of NORD member organizations spoke since these meetings highlight learning from each other and sharing experiences. Topics included the role of patient organizations in accelerating research, an update on the NORD policy agenda and a preview of Rare Disease Day 2013.

As this publication went to press, a regional meeting was being planned for Dec. 6 in Orlando, Florida, to focus on growing and developing a patient organization. The regional meetings are made possible by a grant from the Medtronic Foundation. 🍊

EVERY DONATION HELPS!

The approaching year holds many challenges. Congress and the President will be making difficult decisions that could greatly impact the rare disease community. Your donation will help to ensure that NORD will continue to provide strong representation for rare disease patients and their families. Every gift helps. Donations fund vital programs and services such as the following:

- Advocacy on public policy issues
- Awareness for medical professionals to encourage early diagnosis and appropriate treatment
- Education for patients and their families
- Global connection for patients and patient organizations
- Mentoring for patient organizations
- Patient assistance programs to help patients access life-saving medications
- Representation of rare disease patients and patient organizations at NIH, FDA, SSA and other government entities
- Research to advance understanding of rare diseases

NORD is a 501(c)3 organization. All donations are tax-deductible. Make your donation online today at www.rarediseases.org or mail it to: NORD, 55 Kenosia Avenue, Danbury, CT 06810. Donations may be made in honor or memory of a loved one.

PLEASE MAKE YOUR DONATION TODAY!



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SAVE THE DATE

Tuesday, May 14, 2013

SPECIAL 30TH ANNIVERSARY GALA

See you at the Mellon Auditorium!