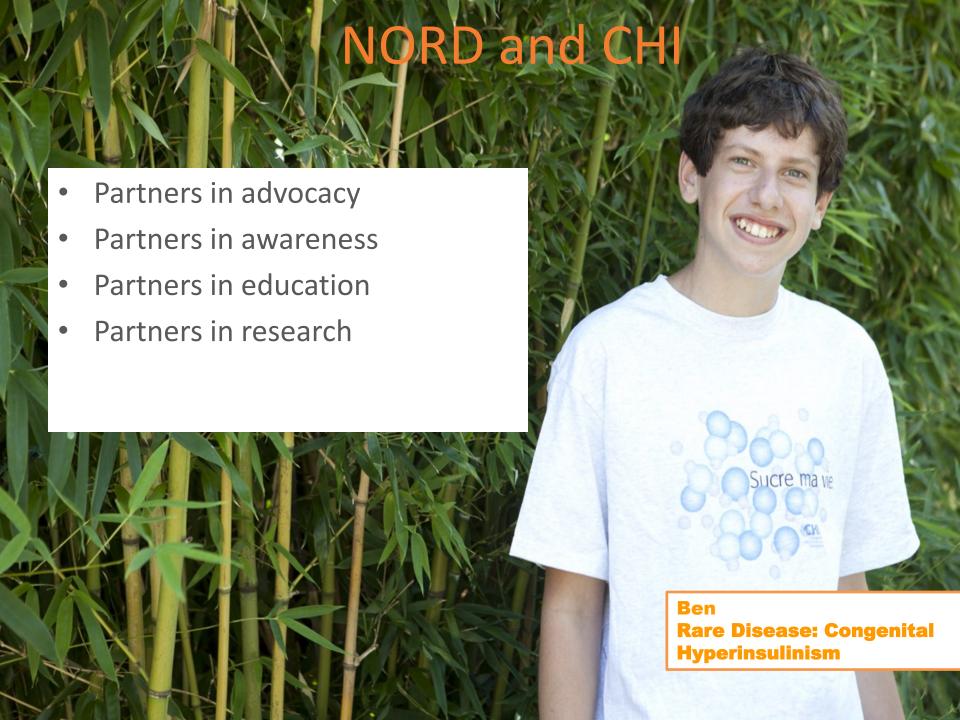
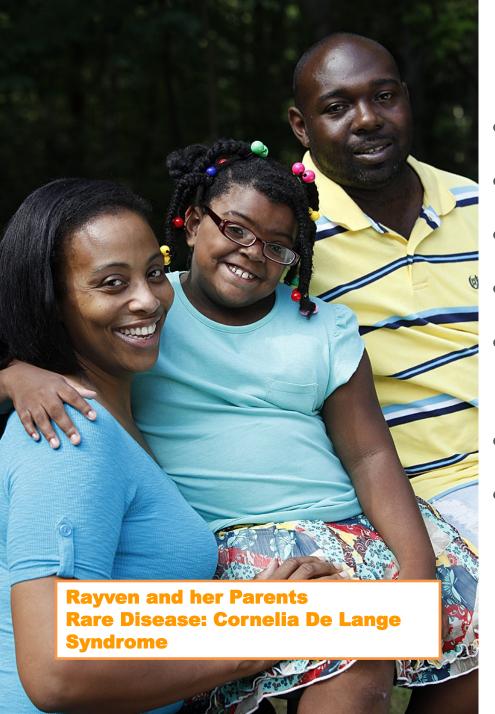


The Role of Patient Organizations in Advocacy

National Organization for Rare Disorders (NORD) www.rarediseases.org





Experience of Patients

- Isolation
- Diagnosis delay
- Too little research
- Too few treatments
- Limited access to treatments & services
- Too few medical experts
- Global public health challenge



What is NORD?











- Established in 1983 by patient advocates
- Non-profit organization representing all Americans with rare diseases
- More than 165 Member Organizations
- Also provides support to people who have rare diseases with no organization
- A national organization with an increasingly global perspective & network



NORD's founders were patient advocates supporting the *Orphan Drug Act*.



NORD Provides:











- Advocacy
- Education (Patients, Professionals, Public)
- Mentoring (Patient Organizations)
- Patient Assistance Programs
- Representation (FDA, NIH)
- Research Program





Current Advocacy Initiatives:











- TREAT Act -- Senator Kay Hagan (NC)
- FAST Act -- Representatives Cliff Stearnes (FL) and Ed Towns (NY)
- PDUFA (new rare disease provisions)
- Conflict of Interest, Risk/Benefit, Flexibility
- Preserving Insurance Reforms





Current Educational Initiatives:











- Rare Disease Database
- Physicians & Other Clinicians: www.nordphysicianguides.org
- Exhibiting at Major Medical Conferences
- Expanded Media Outreach



NORD encourages our Member Organizations to find their common ground.









NORD/EURORDIS Alliance











- Rare Disease Day
- RareConnect Global Communities:
 RareConnect.org
- Rare Disease Advocacy Blog: RareDiseaseBlogs.net
- FDA/EMA collaboration
- A global approach to clinical trials





RARE DISEASE DAY USA

ALONE WE ARE RARE. TOGETHER WE ARE STRONG.

FEBRUARY 29, 2012

ABOUT RARE DISEASE DAY

RARE DISEASE DAY EVENTS

TAKE ACTION NOW

THANK OUR SUPPORTERS

MEET THE COMMUNITY





View photos in the Handprints Across America Gallery ... and submit one of your own. It's one of the easiest ways to get involved!

Countdown to Rare Disease Day!

358 Days, 12 Hrs, 45 Min, 14 Sec



JOIN THE CONVERSATION

Tell your story or follow ours in our active online community.

facebook

twitter

Tell Your Story



Tell your story to your local media!

Write a letter to the editor or send a press release to a local newspaper. Sharing experiences helps people understand what it is like to live with a rare disease.

This press kit will make it easy for you to share the general theme and messages of the day, as well as your own story.

View the official Rare Disease Day Video!

Join Events



Share your events with NORD and find out what is happening in your area!

RAISE YOUR HAND

Click here and Lundbeck will donate \$1 to NORD's general research fund.

MAKE A DIFFERENCE



Grassroots Activities







This year we are looking at new ways people from across the US to get involved. We encourage you to:

- · Submit a photo of yourself for the Handprints Across America Gallery
- . Write your reps to teach them about rare diseases
- · Bring rare disease day into your school
- . Buy Rare Disease Day Merchandise
- · Become an ambassador or partner or view more suggestions!

RareConnect.org:

- Global online communities
- Connecting patients & patient organizations
- Translation into 5 languages
- 18 disease-specific communities to date
- Private as well as public messages
- "Facebook" features, as well as educational information

Global Blog: RareDiseaseBlogs.net

OCTOBER 30TH, 2009

When Should Patients Be Given Access to Investigational Drugs?



Seriously ill patients who have exhausted all treatment options often seek access to investigational drugs by way of government-sanctioned "expanded access programs". Recently, the U.S. Food and Drug Administration (FDA) implemented new regulations to clarify and increase patient access to investigational drugs.

However, even with the new rules, this topic is more complex than it may seem on the surface. And it has particular relevance to people with rare diseases since most rare diseases have no approved treatment.

To explore the various sides of this issue, NORD recently co-hosted (with Idis Pharmaceuticals) a media briefing on this topic. Both NORD and Idis are providers of expanded access programs.

Representatives of FDA, patient organizations, industry, and a university bioethics program spoke from their various perspectives. They raised some fascinating points in this ongoing discussion. Some of the things the speakers said were.

- People with rare diseases are highly motivated to try investigational drugs because they have few treatment options. In fact, there is no approved drug for most rare diseases today.
- FDA considers access to investigational drugs for treatment purposes important and supports it as long as it doesn't compromise the clinical trial process and gives appropriate consideration to patient safety.

About the author



Peter Saltonstall
CEO National Organization for Rare
Disorders (NORD)

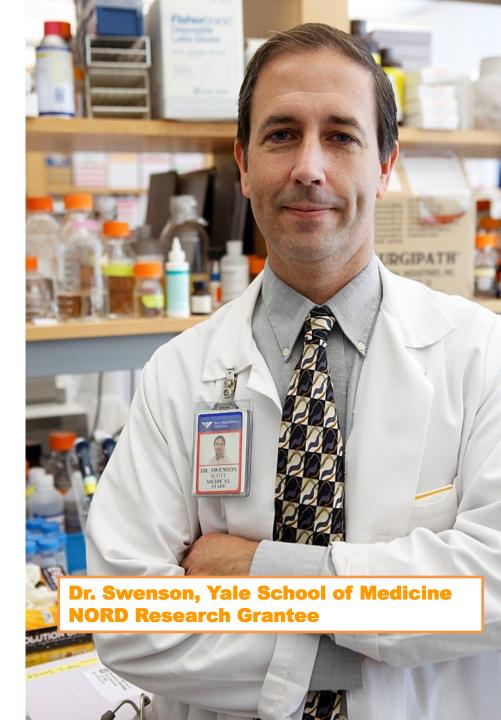
Peter is NORD's President and Chief Executive Officer. He has over 30 years of healthcare experience in both for-profit and not-for-profit environments, as well as extensive federal and commercial market knowledge. Peter has held senior positions within a number of major academic medical centers and organizations, including Harvard's Brigham and Women's Hospital, Tuth-lewe England Medical Center and St. Elizabeth's Medical Center of Boston.

Read his other articles

 When Should Patients Be Given Access to Investigational Drugs?

In the same category...

 When Should Patients Be Given Access to Investigational Drugs? NORD research grants encourage the study of rare diseases that might not otherwise be funded.





NORD Represents Patient Community:



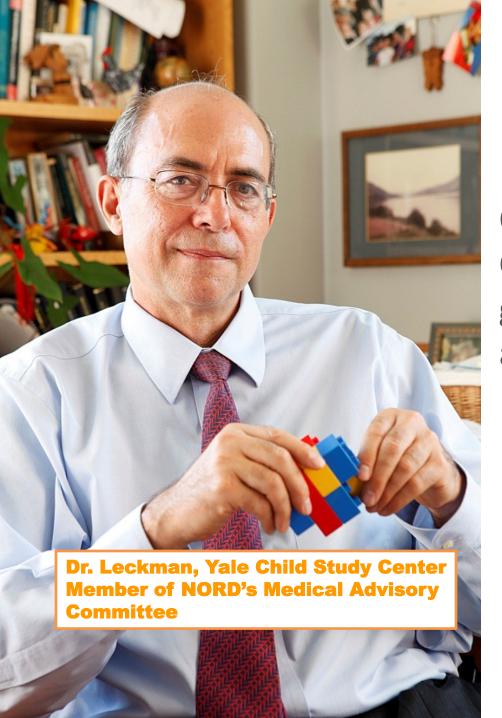








- Program Administered by NORD
- All Medical Decisions Made by our Medical Advisory Committee
- RFPs posted on NORD website: www.rarediseases.org
- More than 100 restricted funds
- Donations from individuals, mostly patients, their friends and families
- Partnerships with patient organizations



Our Medical Advisory
Committee provides essential guidance on medical and advocacy issues.



rarediseases.org



Q Search Site



CONTACT US

DONATE NOW

Alone we are rare. Together we are strong.





For Patients & Families

For Patient Organizations For Medical Professionals For Industry

Rare Disease Information Advocacy

About NORD

News & Events



I AM 1 IN 10

NORD serves the 1 in 10

Americans who have a rare disease through education, advocacy, research and patient assistance.

1 2 3 4 5

News and Events

News for Patients

News for Patient Organizations News for Medical Professionals News for Industry



NORD Releases Landmark Study of FDA Orphan Drug Approvals

NORD has released a major new study examining FDA approvals of



13 New Compassionate Allowances!

Social Security Commissioner Michael Astrue announces 13 new Compassionate Allowances at

WHAT IS NORD?

The National Organization for Rare Disorders is dedicated to helping the nearly 30 million Americans with rare diseases, and the organizations that serve them, through programs of education, advocacy, research, and patient services. More >

I WANT TO:

Find a patient organization >

Get help with medication costs >

Build a patient organization >

Give in honor of someone >

Write my Congressman >

The RARE DISEASE DATABASE

Search NORD's Database for reports on more than 1,200 diseases. Search Now>



GET OUR LATEST E-NEWS



Contact: Mary Dunkle Vice President, Communications mdunkle@rarediseases.org