The Role of Patient Organizations in Advocacy

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

• Partners in advocacy
• Partners in awareness
• Partners in education
• Partners in research

Ben
Rare Disease: Congenital Hyperinsulinism
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

Rayven and her Parents
Rare Disease: Cornelia De Lange Syndrome
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
NORD provides representation on Capitol Hill and at FDA and NIH for our Member Organizations

NORD CEO Peter Saltonstall and Vice President of Public Policy, Diane Dorman
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
Providing effective advocacy requires a strong foundation of awareness and education. Families play a major role in this.

Gaby and her parents
Rare Disease: APS Type 1
Current Educational Initiatives:

- Rare Disease Database
- Physicians & Other Clinicians: [www.nordphysicianguides.org](http://www.nordphysicianguides.org)
- Exhibiting at Major Medical Conferences
- Expanded Media Outreach
NORD encourages our Member Organizations to find their common ground.

Staff Members of a NORD Member Organization:
Cornelia de Lange Syndrome Foundation
Alone we are rare. Together we are strong.
NORD has a strategic partnership with its European counterpart, EURORDIS.
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
View photos in the Handprints Across America Gallery ... and submit one of your own. It’s one of the easiest ways to get involved!
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
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 treatments.

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

Representatives of FDA, patient organizations, industry, and a university bioethics program spoke from their unique 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, Tufts New 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.

Dr. Leckman, Yale Child Study Center
Member of NORD’s Medical Advisory Committee
I AM 1 IN 10

NORD serves the 1 in 10 Americans who have a rare disease through education, advocacy, research and patient assistance.

I am Lulu
Rare Disease: Paraneoplastic Syndrome

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>
Contact:
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