



EMPOWERING THE RARE DISEASE COMMUNITY



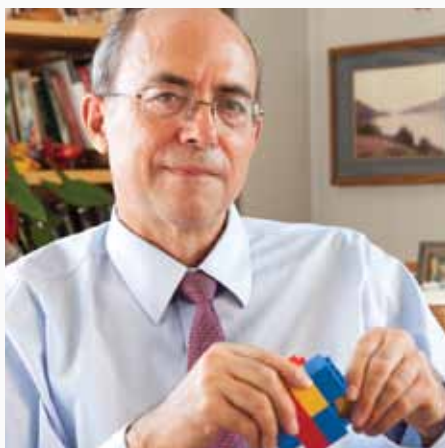
CONNECT



VOICE



RESEARCH



INNOVATE

A report to our constituents

OCTOBER 2012

NORD is the recognized national leader driving progress on behalf of the entire rare disease community.

It provides a unified voice for patients and other stakeholders, promotes research and state-of-the-art regulatory practices, educates medical professionals to encourage earlier diagnosis and appropriate treatment, and raises public awareness of the challenges experienced by patients and their families. Established by patient advocates who led the campaign for the Orphan Drug Act in 1983, NORD is a nonprofit organization representing:

- Everyone who lives with a rare disease
- Everyone who cares about someone living with a rare disease
- Everyone seeking to improve the lives of people with rare diseases

NORD's advocacy initiatives are based upon the following vision and guiding principles:

A national awareness and recognition of the challenges faced by people living with rare diseases and the associated costs to society • A national commitment to assuring that people with rare diseases can secure access to diagnostics and therapies that extend and improve their lives • A social, political, and financial culture of innovation that supports both the basic and translational research necessary to create diagnostic tests and therapies for all rare disorders • A regulatory environment that encourages development and timely approval of safe and effective diagnostics and treatments for patients with rare diseases.

NORD provides strong and consistent leadership in advocacy, awareness, education, patient services, and research. Specific recent accomplishments include:

Leadership and Patient Representation in Public Policy

NORD was instrumental in developing and shaping the FDA Safety and Innovation Act of 2012. Provisions in this law of critical importance to the rare disease community will accelerate research on orphan drugs and medical devices, encourage implementation of innovative regulatory practices, and improve communication among all involved.

A Voice for the Rare Disease Community in Federal Agencies

NORD works closely with the National Institutes of Health (NIH), Food and Drug Administration (FDA), Social Security Administration (SSA) and other federal agencies to increase awareness and understanding of the concerns of the rare disease community. For instance, NORD provided key input to FDA about patient needs in preparation for the 2012 reauthorization of the Prescription Drug User Fee Act (PDUFA). NORD has also assisted SSA with its Compassionate Allowances Program to assure timely review of applications from patients with extremely disabling diseases.

Assuring Patient Access to Treatments

NORD pioneered the concept of Patient Assistance Programs and, since 1987, has helped more than 1.3 million patients obtain needed medications. Today, its high-touch, patient-centric programs provide a complete suite of services to companies seeking to streamline and strengthen their patient assistance programs.





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Unifying the Rare Disease Community

With nearly 200 member organizations and a vast number of additional partnerships and collaborative relationships, NORD serves as the hub of the rare disease community, uniting patients, government partners, researchers, industry, and investors in a focused community-wide effort to achieve the end goal we all share – improving the lives of people affected by rare diseases.

Global Connection

Through a strategic partnership with the European Organisation for Rare Diseases (EURORDIS), NORD serves as the U.S. sponsor for the annual global Rare Disease Day in February and hosts online disease-specific global patient communities (RareConnect.org). NORD and EURORDIS also collaborate on globalization of policies, and encourage FDA and the European Medicines Agency to seek ways to eliminate any unnecessary duplication of effort.

Promoting and Facilitating Research

NORD partners with other patient organizations to provide grants for the study of rare diseases. In at least one case, this has resulted in an FDA-approved product. Numerous articles resulting from NORD-funded studies have been published in medical journals. Through its Corporate Council, NORD provides a forum for the discussion of timely topics and interaction with federal officials and thought leaders. In addition, NORD increases awareness among patients of opportunities to participate in clinical trials.

FOR 30
YEARS
IMPROVING
THE
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OF THE
1 IN 10
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A RARE
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NORD's Programs and Services

Empowering the entire rare disease community



Patients and Families

- Information about diseases in patient-friendly language
- Patient Assistance Programs to help patients access their medications
- Referrals to patient organizations and other helpful resources
- Regional disease-specific meetings for networking and learning
- Representation on Capitol Hill and in federal agencies



Patient Organizations

- Global connection through RareConnect.org hosted by NORD and EURORDIS
- Mentoring and sharing of best practices
- Opportunities to partner in advocacy and education
- Regional meetings to share information and learn from each other



Medical Professionals

- Education and awareness through a new online platform: NORDPhysicianGuides.org
- NORD educational partnerships with professional medical societies
- Research grants
- Research support such as patient awareness of clinical trials



Industry

- Collaboration toward the shared goal of safe, effective treatments for patients
- Participation in NORD's Corporate Council
- Partnering with NORD in Patient Assistance Programs
- Supporting Rare Disease Day and other community-wide initiatives



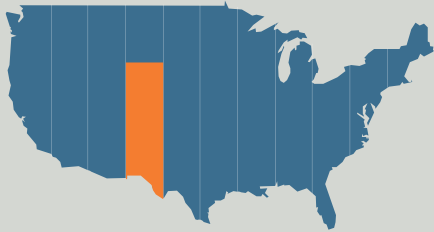
Government Partners

- Assistance in understanding the challenges of living with a rare disease
- Opportunities to interact with, and learn from, the rare disease patient community
- Participation in events that draw together all rare disease and orphan product stakeholders

Why the Rare Disease Community Needs NORD

30 MILLION Americans live with a rare disease

That is **1/10** of the U.S. population



NORD is the hub connecting patients and patient organizations with government, industry, academic researchers and medical experts.

Nearly **2/3** of people with a rare disease are children.



NORD works closely with the American Academy of Pediatrics and other partners to meet the special needs of children with rare diseases. This includes advocacy on behalf of the development of new and better treatments, such as pediatric drugs, medical devices, and medical foods.

Many people with rare diseases can't access needed medical services.

SINCE
1987

NORD \$60 million
has provided

worth of free drug and co-pay assistance through its pioneering patient-centric Patient Assistance Programs.

For **1/3** of the people with rare diseases, it takes **5 YEARS** or longer to get an accurate diagnosis.

NORD provides educational materials for patients and their families and for medical professionals. Through social media, medical conferences, regional patient workshops, and online learning, NORD promotes a better quality of life for everyone with a rare disease.

NORD drives progress for rare disease patients

- Promoting progressive research and regulatory policies
- Voice of patients on Capitol Hill
- Access to medications
- \$6 million in research grants
- Educating medical professionals
- Information in patient-friendly language
- Raising public awareness

THERE ARE
7,000
RARE
DISEASES
and

FEWER
THAN
500
APPROVED
TREATMENTS



1 IN 10
AMERICANS LIVE
WITH A RARE DISEASE

1.5 MILLION

PHYSICIAN GUIDES
DISTRIBUTED TO
**MEDICAL
PROFESSIONALS
BY NORD**



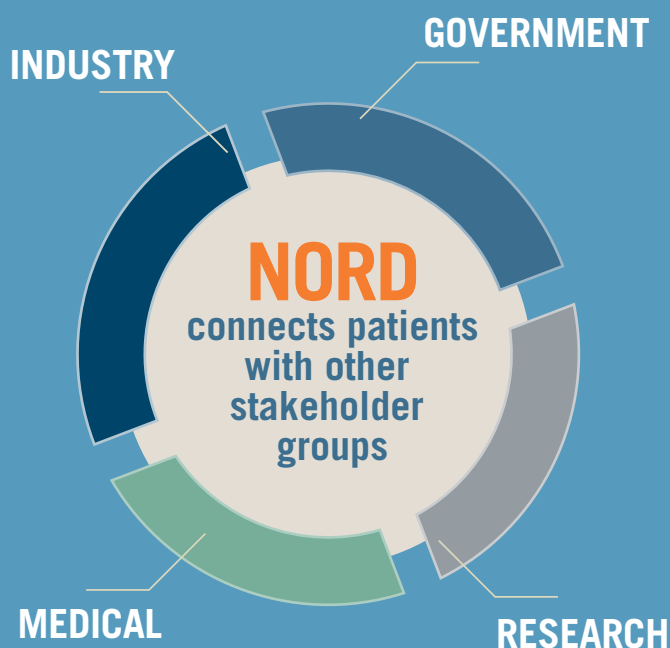
NORD NOW

DISTRIBUTES ITS
RARE DISEASE
PHYSICIAN
GUIDES
ONLINE
REACHING AN
UNLIMITED
AUDIENCE

NORD in partnership with EURORDIS for GLOBAL REACH



In 2009, EURORDIS and NORD formed a transatlantic, patient-driven strategic alliance for rare diseases, to bring patient advocates from Europe and the United States together to promote rare diseases as an international public health priority.



1.3 MILLION

Number of rare disease patients
NORD has helped through patient
assistance programs



Leadership and Patient Representation in Public Policy

- Orphan Drug Act of 1983
- Rare Diseases Act of 2002
- NIH Rare Diseases Clinical Research Network
- Insurance Reforms in Affordable Care Act
- SSA Compassionate Allowances Program
- Faster Access to Specialized Treatments Act (FAST)
- Transforming the Regulatory Environment to Accelerate Access to Treatments Act (TREAT)
- Humanitarian Use Device Reforms
- Expansion of Rare Disease Program at FDA
- FDA Safety and Innovation Act
- Documenting FDA Flexibility
- Implementation of ACA and FDASIA
- National Pediatric Research Consortium
- Risk/Benefit Policy

 enacted  pending

RECENT HIGHLIGHTS

MAY More than 500 members of the rare disease community attended the Annual NORD Partners in Progress Event on May 15, 2012, where pioneers from research, industry, and public policy were honored. Senator Kay Hagan (NC) and Reps. Cliff Stearns (FL) and Ed Towns (NY) were among the honorees.

NORD has hosted regional meetings for rare disease patient organizations in Chicago, New York, San Francisco and Philadelphia in 2011 and 2012 to encourage sharing of best practices.

MAR With its European partner, EURORDIS (Rare Diseases Europe), NORD hosts global online communities (www.RareConnect.org) where posts may be translated into any of five languages.

FEB In February 2012, NORD launched a new website to educate both physicians and clinicians about rare diseases at www.nordphysicianguides.org.

NORD worked closely with Members of Congress and FDA during the two-year preparation for reauthorization of the Prescription Drug User Fee Act. The resulting FDA Safety and Innovation Act bore NORD's imprint in numerous groundbreaking provisions.

For Rare Disease Day 2012 (February 29), more than 700 patient organizations, companies, academic institutions and government agencies signed on with NORD as Rare Disease Day Partners. NORD assisted FDA with the agency's first-ever FDA Patient Advocacy Day on March 1, 2012.

JAN NORD convened a Risk Task Force in January 2012 that is working with FDA to enhance patient input into decisions related to risk tolerance.

OCT NIH Director Francis Collins, MD, PhD, and Social Security Commissioner Michael Astrue were among the speakers at the U.S. Conference on Rare Diseases and Orphan Products hosted by NORD and DIA in October 2011.

A study commissioned by NORD showed that FDA applied flexibility in the review of two-thirds of the non-oncologic orphan drugs approved from 1983 to 2010. NORD is encouraging FDA to document this flexibility to provide greater transparency in orphan drug reviews.

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RARE DISEASE COMMUNITY
**NORD FOCUSES
ON THOSE ISSUES**

RECENT EVENTS

Mentoring Patient Advocates

Approximately 40 NORD member organizations were represented at a recent meeting in Philadelphia. This included both start-up and seasoned organizations. These meetings encourage mentoring and sharing of best practices.



Bringing New Treatments to Patients

NORD commissioned a major study of FDA flexibility in the review of orphan products to document current practices and how the process may be improved.



Connecting Patients with Government Partners

NORD helped FDA organize its first-ever Rare Disease Patient Advocacy Day in March 2012, educating patient leaders beforehand and bringing them to the White Oak Campus on a bus.



ANNUAL EVENTS

NORD is the national sponsor in the U.S. for **Rare Disease Day**, which takes place on the last day of February each year. Visit the special website NORD hosts (RareDiseaseDay.us) to learn how to get involved.



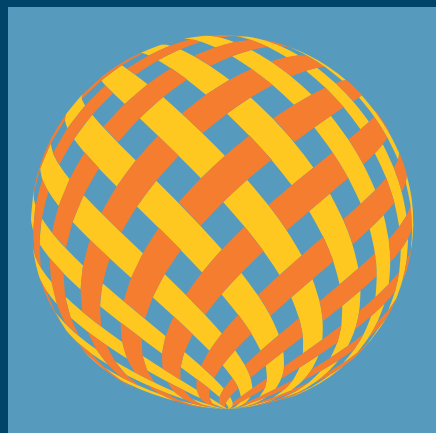
At its **Partners in Progress Celebration** each year in May, NORD honors individuals, public officials, researchers, patient organizations and companies for outstanding achievements in policy, research and service



NORD co-hosts, with the Drug Information Association (DIA), the annual **U.S. Conference on Rare Diseases and Orphan Products**. Collaborators include NIH, FDA, EURORDIS and Duke Department of Pediatrics.



NORD MOVING FORWARD



GLOBAL

RareConnect.org

Evolving and growing the global conversation among patients

RareDiseaseDay.us

An annual international advocacy day to bring widespread recognition of rare diseases as a global health challenge.



EDUCATE

NordPhysicianGuides.org

Creating the tools for doctors and clinicians to diagnose rare diseases



SUPPORT

NORD Patient Assistance Portal

Increasing access and solutions for our cutting-edge programs to advance treatments for patients.



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rarediseases.org