NORD

COLLABORATION

EDUCATION

LEADERSHIP

RESEARCH

ADVOCACY

MAJOR MILESTONES

Driving Progress on Behalf of Rare Disease Patients

For many years, people with rare diseases walked alone. Patients and their families coped with daunting medical and financial issues with few resources and no one to guide the way.

Then, a small group of patient advocates formed a coalition to unify this community and mobilize support to pass the Orphan Drug Act. In 1983, the coalition became NORD, the National Organization for Rare Disorders. For more than 30 years, NORD has led the way in voicing the needs of the rare disease community, driving supportive policies, advancing medical research, and providing patient and family services for those who need them most.

Read the full story of NORD and the rare disease movement here.

1979

FDA/NIH Task Force Issues Report

Task force chaired by Marion Finkel, MD, of FDA issues report calling for measures to address the need for more resources to be directed toward drugs "of limited commercial value" (drugs for small patient populations).

1979 — 1980

Patient Advocates Form Ad Hoc Coalition

Leaders of rare disease patient organizations form a coalition to provide advocacy together on behalf of legislation to encourage the development of treatments for people with rare diseases.

1979 – 1980

House Subcommittee Gathers Evidence

The Subcommittee on Health and the Environment of the House Energy and Commerce Committee, chaired by Representative Henry Waxman, holds hearings on the orphan drug problem. NORD founders testify.

1980

Popular TV Show Features the Issue

Actor Jack Klugman and his brother, Maurice, assist the patient advocates in focusing national attention on the problem on the TV show, Quincy, M.E.

1980

Pre-Orphan Drug Act

Only 10 new drugs were developed by the pharmaceutical industry for rare diseases in the decade before 1983.

1983

First Orphan Drug Approved

The Food and Drug Administration (FDA) grants first marketing approval to an orphan drug, Panhematin®, for acute intermittent porphyria and other acute porphyrias. Desiree Lyon Howe, co-founder and executive director of the American Porphyria Foundation and for many years a NORD board member, participated in research on this orphan drug.

1983

Orphan Drug Act Enacted

After rare disease patient advocates mobilize support for the Orphan Drug Act, which had been sidelined in Congress, it is approved by the House and Senate in December 1982 and signed by President Ronald Reagan on January 4, 1983. Those same patient leaders then establish NORD to continue their collaboration, realizing that "Alone we are rare. Together we are strong."

1984

Orphan Drug Act Amendment

The Orphan Drug Act is amended to define a rare disease as any disease affecting fewer than 200,000 Americans or one for which there is no reasonable expectation of recovering the cost of therapy development. NORD provides patient input on this and later amendments.

1983

NORD Founded

NORD is incorporated to represent the shared interests and goals of all Americans affected by rare diseases. Abbey Meyers, considered the primary consumer advocate responsible for the Orphan Drug Act, is named president.

1987

NORD Establishes First-ever Patient Assistance Program

NORD establishes the first patient assistance program dedicated to helping patients obtain medications they cannot afford or that their insurance does not cover.

1989

National Commission Issues Report

The National Commission on Orphan Diseases, chaired by Jess Thoene, MD, board chair and medical advisor to NORD, conducts a major study and issues a report on the experiences of patients and families affected by rare diseases.

1989

NORD Establishes Research Program

NORD establishes a Research Program to be overseen by its medical advisors so that patients and patient organizations may provide grants for the study of diseases with limited or no other source of funding.

1999

EU Adopts Orphan Law

NORD shares its experiences with patient leaders in Europe as they promote EU orphan legislation.

2000

NIH Establishes ClinicalTrials.gov

Partly in response to advocacy from NORD and others in the patient community, NIH launches a website (www.clinicaltrials.gov) providing an overview of current clinical trials.

2000

NORD Leads Advocacy for Rare Diseases Act

Senators Ted Kennedy and Orrin Hatch introduce the Rare Diseases Act, advocated by NORD to enhance federal funding for rare disease research and accelerate the development of treatments. The House later splits this legislation into two separate bills – the Rare Diseases Act and the Orphan Products Development Act.

2002

Rare Diseases Act Signed into Law

The Rare Diseases Act, strongly promoted by NORD, is signed into law, codifying the NIH Office of Rare Diseases Research and providing for the establishment of the NIH Rare Diseases Clinical Research Network.

2002

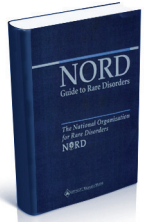
NORD Named One of 100 Best Charities

Worth Magazine names NORD "One of the 100 Best Charities in America."

2003

NORD Publishes Guide to Rare Disorders

NORD, with Lippincott, Williams & Wilkins, publishes a 600-page textbook written by the world's leading rare disease experts, *The NORD Guide to Rare Disorders*, for pediatricians and family physicians to encourage earlier diagnosis and treatment.



2005

First Meeting of ICORD

The first meeting of ICORD (the International Council on Rare Diseases and Orphan Products) takes place in Stockholm, with representatives of NIH, FDA, and NORD participating from the US. At this meeting, Marlene Haffner, MD, of FDA's Office of Orphan Products Development, commits to developing a joint orphan designation application with her office's counterpart in Europe.

2006

Rare Diseases Clinical Research Network Founded

The National Institutes of Health (NIH) announces the establishment of the Rare Diseases Clinical Research Network with $55 million in funding for rare disease research. This was made possible by the Rare Diseases Act of 2002, advocated by NORD, and through the leadership of the NIH Office of Rare Diseases Research.

2007

FDA and EMEA Collaboration

NORD advocates for more seamless regulatory processes in the US and Europe. FDA and EMEA adopt a shared orphan designation application.

2008

NIH Establishes Undiagnosed Diseases Program

NIH launches a program for patients with undiagnosed diseases. NORD later works with NIH to establish a patient assistance program, funded by the Genzyme Running for Rare Diseases team, to pay for required testing for applicants.

2009

NORD Opens DC Advocacy Office

NORD expands its presence in the nation's capital, establishing an office in the Carnegie Endowment for International Peace building to serve as headquarters for its advocacy initiatives.

2008

New NORD Leadership

Abbey Meyers retires after serving for 25 years as NORD's president. She is succeeded by Peter L. Saltonstall.

2008

NORD Launches Disease-Specific Regional Meetings

NORD launches a program of regional, disease-specific meetings for patients and caregivers. These meetings provide opportunities for networking and to interact with medical experts.

2008

Compassionate Allowances Program Established

Social Security Commissioner Michael Astrue announces at a NORD patient/family conference that he will establish a "Compassionate Allowances Program" to fast-track the processing of assistance applications from patients with certain severely disabling diseases. NORD facilitates input from rare disease organizations and experts.

2009

NORD and EURORDIS Formalize Partnership

NORD and EURORDIS (Rare Diseases Europe) enter into a strategic partnership to connect patients and patient organizations in the US and EU in shared initiatives.

2009

NORD Launches Rare Disease Day in US

Through its partnership with EURORDIS, NORD establishes Rare Disease Day in the US.


Quantum of Effectiveness Evidence in FDA's Approval of Orphan Drugs

Configuring FDA's Flexibility in Regulating Therapies for Persons with Rare Disorders

2011

NORD Releases Major Report on FDA Orphan Approvals

NORD releases major new report written by board member Frank Sasinowski documenting ways in which flexibility has been applied by FDA in the review of all non-oncologic orphan drugs approved between 1983 and summer 2010. The study revealed that for two of every three orphan drugs, FDA exercised flexibility in its review of effectiveness data.

2011

NORD Conference Draws Together Stakeholders

NORD and DIA (the non-profit Drug Information Association) co-sponsor the first US Conference on Rare Diseases and Orphan Products.

2010

Rare Disease Office Established in FDA CDER

NORD successfully advocates for a new FDA position, Associate Director for Rare Diseases, in the Agency's Center for Drug Evaluation and Research (CDER), Office of New Drugs (OND).

2011

NORD Hosts Major Medical Foods Conference

To promote awareness of access issues for patients whose treatment is a medical food or formula, NORD hosts a major conference on this topic, drawing together key stakeholders.

2011

Healthcare Reform Includes Desperately Needed Insurance Reforms

In two-year debate leading up to the Affordable Care Act, NORD advocates for insurance reforms, such as ending annual and lifetime insurance caps and eliminating discrimination based on pre-existing conditions, which are included in the final law.

MDIC
MEDICAL DEVICE INNOVATION CONSORTIUM

2012:

NORD is Founding Member of First Public-private Medical Device Partnership

NORD helps launch the first public-private partnership to promote innovative medical device development. NORD President Peter L. Saltonstall is later named to the board of the Medical Device Innovation Consortium, whose members include FDA, NIH, and several companies involved in developing medical devices.

2012

FDA Safety and Innovation Act Approved

This new law formally embeds the rare disease patient voice within the drug review process. For two years leading up to its enactment, NORD meets regularly with senior officials at FDA, legislators and Congressional staff to educate them about patient needs and concerns. NORD calls this new law the most important since the Orphan Drug Act for the rare disease community.

2011

NORD Hosts Forum with FDA Commissioner

In its continuing promotion of the patient voice at FDA, NORD hosts the first-ever forum at which patient leaders sit down one-on-one with the FDA Commissioner to express their needs and concerns.

2012

NORD Establishes Physician Guide Website

To promote earlier diagnosis and treatment, NORD launches a website where physicians and other medical professionals can access free disease-specific guides written by medical experts.

FDA

2012

NORD Brings Patient Advocates to FDA

For Rare Disease Day 2012, FDA hosts its first-ever Patient Advocacy Day. Since many patient leaders have never been to the FDA campus, NORD arranges for a hotel room block and bus, and hosts an orientation beforehand.

NORD 30 YEARS EMPOWERING THE RARE DISEASE COMMUNITY

2013

30 Years: NORD and The Orphan Drug Act

The rare disease community celebrates 30 years of progress on the 30th anniversary of NORD and the Orphan Drug Act. The celebration highlights a new generation of advocates being trained by NORD.

2014

NORD Launches Natural History Patient Registry Platform

A new natural history/patient registry platform created by NORD is unveiled with the launch of a disease-specific registry established in partnership with the VHL Alliance.

NORD's leadership on behalf of rare disease patients does not end.

We invite you to follow our progress and check for new successes on our online timeline. As a nonprofit 501c3 organization, NORD relies on the support of donors to remain active and strong. Please consider a contribution to assure we will always be here as a unified voice for the rare disease community.

2013

NORD and JPA Announce Strategic Partnership

JPA

NORD and the Japan Patients Association sign a Memorandum of Understanding creating a strategic partnership to connect patient organizations in the US and Japan.

2014

With Rare Action Network™ NORD Promotes State-Based Advocacy

NORD establishes this network of individuals and organizations to address state issues on behalf of rare disease patients.

NORD

National Organization for Rare Disorders

Alone we are rare. Together we are strong.™

rarediseases.org

DONATE

Be part of NORD's future.