

National Organization Members

Alagille Syndrome Alliance
Alpha 1 Association
Alpha-1 Foundation
American Brain Tumor Association
American Laryngeal Papilloma Foundation
American Porphyria Foundation
American Sphingomyelinase Project
Aplastic Anemia & MDS International Foundation
Assn. for Glycogen Storage Disease
Assn. of Gastrointestinal Motility Disorders
Batten Disease Support & Research Association
Benign Essential Blepharospasm Research Fdn.
Charcot-Marie-Tooth Association
Chromosome 18 Registry & Research Society
Cleft Palate Foundation
Cornelia De Lange Syndrome Foundation
Cystinosis Foundation
DEBRA
Dysautonomia Foundation
Dystonia Medical Research Foundation
Ehlers Danlos National Foundation
Epilepsy Foundation
Families of Spinal Muscular Atrophy
Fdn. for Ichthyosis and Related Skin Types
Genetic Alliance
Guillain Barre Syndrome Foundation
Hemochromatosis Foundation
Hereditary Colon Cancer Association
Hereditary Disease Foundation
HHT Fdn. International
Histiocytosis Association of America
Huntington's Disease Society of America
Immune Deficiency Foundation
International FOP Association
International Joseph Diseases Foundation
International Rett Syndrome Association
Interstitial Cystitis Association
Lowe Syndrome Association
Mastocytosis Society
Moebius Syndrome National Support Group
Mucopolidosis Type IV Foundation
Myasthenia Gravis Foundation of America
Myositis Association of America
Narcolepsy Network
National Adrenal Disease Foundation
National Alopecia Areata Foundation
National Ataxia Foundation
National Foundation for Ectodermal Dysplasias
National Hemophilia Foundation
National Marfan Foundation
National MPS Society
National Multiple Sclerosis Society
National Neurofibromatosis Foundation
National PKU News
National Spasmodic Torticollis Association
National Tay Sachs & Allied Diseases Association
National Urea Cycle Disorders Foundation
Neurofibromatosis, Inc.
Osteogenesis Imperfecta Foundation
Parkinson's Disease Foundation
Platelet Disorder Support Association
Prader Willi Syndrome Association USA
Pulmonary Hypertension Association
Rett Syndrome Association

Stevens Johnson Syndrome Foundation
and Support Group
Sturge-Weber Foundation
The Erythromelalgia Association
The Oxalosis & Hyperoxaluria Foundation
The Paget Foundation
Tourette Syndrome Association
Trigeminal Neuralgia Association
United Leukodystrophy Foundation
United Mitochondrial Disease Foundation
VHL Family Alliance
Wegener's Granulomatosis Association
Williams Syndrome Association
Wilson's Disease Association

Associate Organization Members

A-T Children's Project
Acid Maltase Deficiency Association
American Autoimmune Related Disease Association
American Behcet's Disease Association
American Self-Help Group Clearinghouse
ALS of Gr. Philadelphia
Association for People with the
Van Lohuizen Syndrome (CMTC)
Canadian Organization for Rare Disorders (CORD)
Children's PKU Network
Chromosome Deletion Outreach
Chronic Granulomatous Disease Association
CLIMB
Consortium of Multiple Sclerosis Centers
Contact A Family
Cushing Support & Research Foundation
EURORDIS
Family Caregiver Alliance
Family Support Network of North Carolina
Freeman-Sheldon Parent Support Group
GOLD
Hydrocephalus Association
Incontinence Pigment International Foundation
Klippel-Trenaunay Support Group
Late Onset Tay-Sachs Foundation
Les Turner ALS Foundation
Mercy Medical Airlift
National Lymphedema Network
National Niemann-Pick Disease Foundation
National Spasmodic Dysphonia Association
Organic Acidemia Association
Osteoporosis and Related Bone Diseases
National Resource Center
Parent to Parent New Zealand
Recurrent Respiratory Papillomatosis Foundation
Sarcoid Networking Association
Shwachman-Diamond Syndrome Int.
Society for Progressive Supranuclear Palsy
Sotos Syndrome Support Association
Taiwan Foundation for Rare Disorders
Takayasu's Arteritis Association



NORD®



*...out of the
darkness,
into the light...®*

NORD®
**National Organization
for Rare Disorders, Inc.**

<http://www.rarediseases.org>

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What is NORD?

The non-profit National Organization for Rare Disorders (NORD) is a unique federation of voluntary health organizations and individuals dedicated to helping people with rare diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and service.

Is NORD a government agency?

No. It is a national charity that was formed in 1983 by individuals and organizations who supported the *Orphan Drug Act*, an important piece of federal legislation that provides incentives to encourage the development of new treatments for rare disorders.

What is a rare disorder?

A rare disorder is any disease or health condition that is known to affect fewer than 200,000 people in the United States. This is the definition used by the U.S. Food and Drug Administration (FDA) to determine whether a treatment may be eligible for an "orphan drug" designation.

How many rare diseases are there?

According to the National Institutes of Health (NIH), there are approximately 6,000 rare disorders affecting more than 25 million Americans. This means that one of every ten people has a rare disorder, so even though each disease may affect only a small number of people, rare diseases have a significant impact when considered collectively.

What services does NORD provide?

- **Information.** On NORD's web site, and through its publications, it provides information about rare diseases and support organizations. Each month, NORD responds to thousands of phone calls, letters, and e-mails from patients and their families, physicians, teachers, social workers, librarians, genetic counselors, therapists, and nurses. These inquiries come from all over the world.
- **Research.** NORD supports and promotes biomedical research on rare disorders to encourage the development of new treatments and cures. Donations may be made to NORD for general research, or earmarked for research on particular diseases. NORD's Medical Advisory Committee, a group of physicians with rare-disease research expertise, oversees this process, selecting research projects based on their scientific excellence and monitoring the progress of each grantee.
- **Advocacy.** NORD's Washington, DC, office keeps members informed about issues of importance to the rare-disease community and ensures that people with rare diseases are not forgotten when federal funds are being allocated. NORD has played a pivotal role in increasing government funding for research on rare diseases, protecting access to treatments through government health programs, and affecting health policy to ensure that the interests of rare disease patients are not ignored. The Volunteers in Public Policy initiative provides a way for individuals and families to become involved.
- **Networking.** NORD's Networking Program puts families with the same diagnosis in touch with each other. This helps people find mutual support and encourages the formation of new support groups for specific rare diseases.



- **Clinical Broadcasts.** Although it never endorses particular research projects, NORD increases awareness of opportunities to participate in research through its newsletter, *Orphan Disease Update*, its web site, and mailings to patients inviting them to contact researchers.
- **Database Subscriptions.** Institutions such as libraries, universities, schools, and hospitals may gain 24-hour, 7-day-a-week access to NORD's databases with annual subscriptions. Subscribers select either password or IP access.
- **Other Patient Services.** NORD provides other assistance to families, such as helping patients find free or low-cost travel options to distant treatment centers and answering questions related to insurance, Medicare, disability rights, and other issues. Patients and family members may communicate by phone, letter, or e-mail with NORD's registered nurse and genetic counselor.
- **Medication Assistance.** NORD administers several Medication Assistance Programs that provide more than 20 different medications to uninsured and underinsured people who could not otherwise afford them. NORD also administers programs for specific experimental therapies. We do this through arrangements with humanitarian-minded pharmaceutical firms. Every year, thousands of needy patients receive free or low-cost pharmaceuticals through these vital programs.

Why should you support NORD?

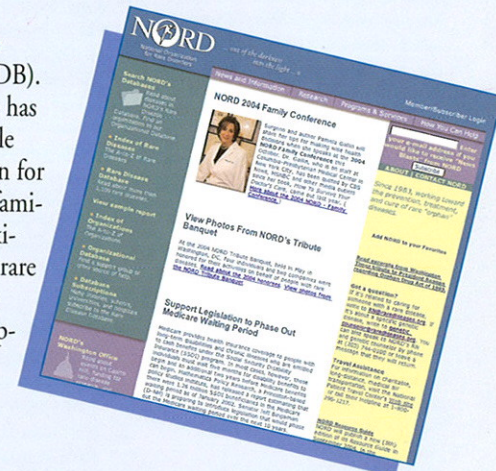
In 2002 and 2003, NORD was honored by *Worth* magazine as one of the 100 best charities in America. It was selected for this citation because it uses donated dollars effectively to provide services that truly make a difference. NORD relies on these donations for its existence, and this is why your contributions are so critically important.

With a small but committed staff, volunteers, and the support of many of the nation's best rare-disease physicians, NORD has served since 1983 as a central source of information and assistance for patients, families, and health-care providers. In many cases, the services NORD provides to patients and families would not be available if NORD did not exist.

NORD's Web Site

On its web site (www.rarediseases.org), NORD provides news of interest to people with rare diseases. It also has searchable databases:

- **Rare Disease Database (RDB).** This database has understandable reports written for patients and families on approximately 1,140 rare diseases, with referrals to support groups and other resources.
- **Organizational Database (ODB).** This database includes information on more than 2,000 organizations and government agencies that help rare-disease patients and families.



NORD's Special Events

NORD hosts two annual events of special interest to the rare-disease community. These are the NORD Tribute Banquet in the spring and the Family Conference in the fall.

- **At the Annual Tribute Banquet,** held in Washington, DC, NORD honors individuals from Congress, public service, and health-related industries who have made a significant contribution to the ongoing battle against rare diseases. NORD also honors companies for development of important new therapies that prevent, alleviate, or cure rare disorders.
- **The NORD Family Conference** provides workshops and programs on two tracks: The first, for patients and families, includes presentations by medical experts and researchers who talk about rare diseases, as well as how to cope with daily life issues. The second track, for leaders of support groups and voluntary health organizations, focuses on teaching them to become more effective, to conform to non-profit regulations, and to use their resources to best advantage.