2016 ANNUAL REPORT

NORD®
National Organization for Rare Disorders

This past year has been one of significant advances for the rare disease community. I am pleased and proud that the National Organization for Rare Disorders (NORD) continued to be the leading voice and advocate for the rare disease patient community.

This report summarizes what NORD accomplished in 2016 in support of our patients. I think it is accurate to say that it was a year of significant accomplishments. Highlights include:

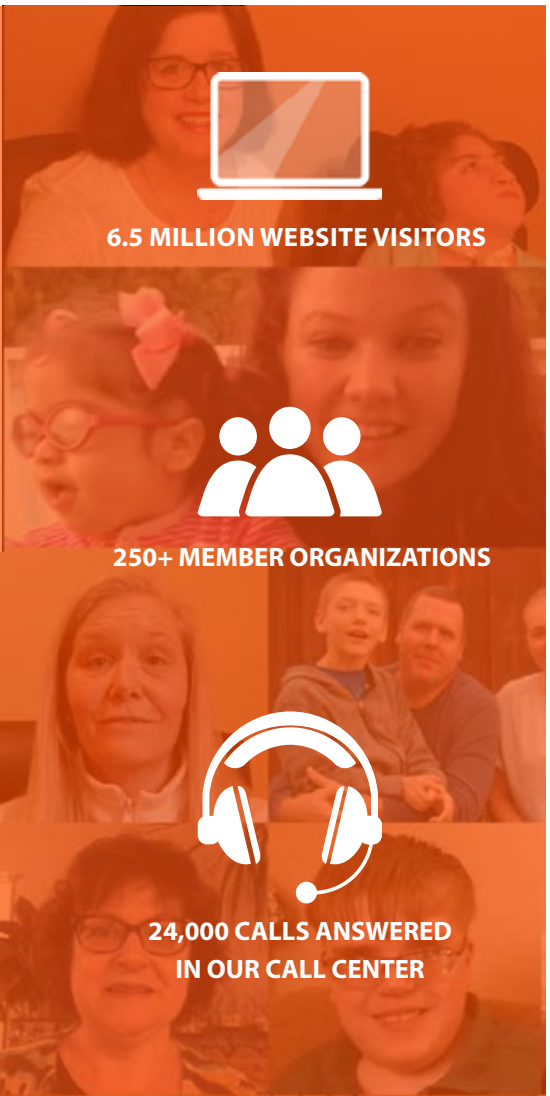
- *NORD actively supported the enactment of the 21st Century Cures Act, which modernized the law to encourage the development of innovative therapies. The Food and Drug Administration once again set records in the approval of orphan drugs – about a third of the new drugs approved each year in the U.S. are for rare diseases.*
- *NORD played an active role in facilitating drug development. There are now 15 registries of patients with rare diseases. Working with the FDA, NORD has advanced the development of natural histories of certain rare diseases to help the medical community gain a better understanding of how to treat them.*
- *NORD programs continued in 2016 to drive research and education about rare diseases. In 2016 NORD awarded $340,000 in grants to study six diseases. More than 6.5 million people visited our website to see up to date information about rare diseases. Clinicians from 169 countries read our online Physician's Guides to help them diagnose and treat rare diseases.*
- *Our patient assistance programs helped thousands of patients obtain needed services and therapies.*
- *NORD membership grew to 250 patient organizations in 2016, our largest number ever.*

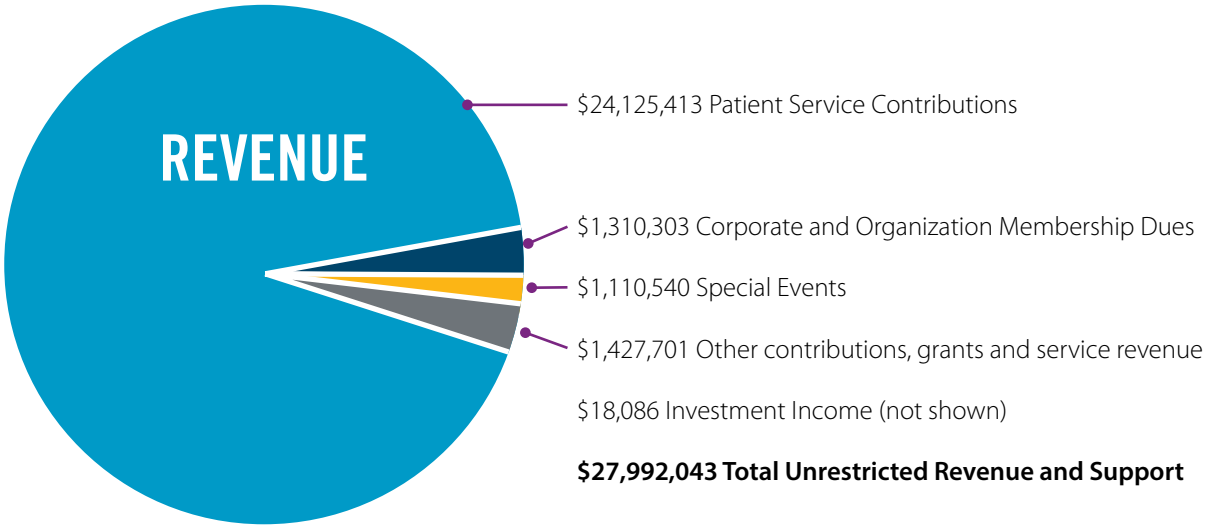
We look to 2017 with optimism, knowing that advances in medical knowledge will further enhance our ability to diagnose and treat these diseases. NORD will continue to provide the leadership and guidance that our community needs to assure that the voice of the rare disease patient continues to be heard.

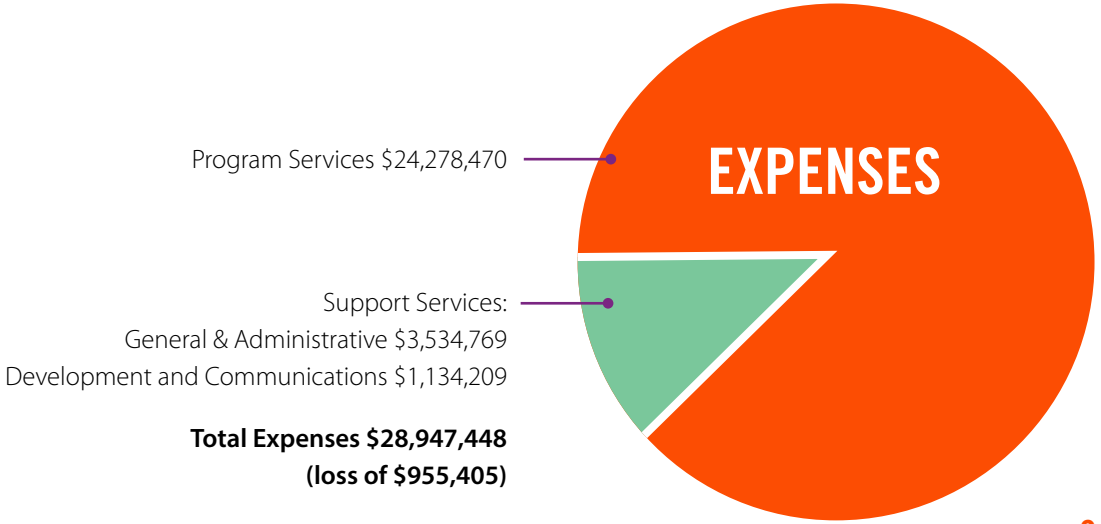
Sincerely,

Peter L. Saltonstall
President and CEO

NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 250 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.







Policy / Advocacy

Driving Health Policies that Help Patients

RALLYING PROGRESS IN OUR NATION'S CAPITAL

Since 1983's passing of the Orphan Drug Act, NORD has ensured that the rare disease perspective is at the table when important policy and regulatory decisions are made. This year, we celebrated the 21st Century Cures Act being signed into law, which marked the culmination of several years of hard work and advocacy by NORD and our members. This game-changing medical innovation bill includes many provisions that will improve the discovery, development, and delivery of orphan therapies for rare disease patients.

Additional 2016 Federal Accomplishments

Led the charge for Congress to reauthorize the Rare Pediatric Disease Priority Review Voucher program, which helps spur new therapies for children with rare diseases. Lead the patient community in passing the Advancing Hope Act, which refined the definition of a rare pediatric disease.	Mobilized the patient community and met with the Armed Services Committees, and both the Senate & House Committees to advocate for the expansion of Medical Foods coverage in TRICARE, creating one of the broadest medical foods coverage policies in the U.S.
Provided a key patient voice in the Zika funding debate on Capitol Hill	We were instrumental in bringing the patient voice to the PDUFA VI and MDUFA IV negotiations.

STATE-BASED INITIATIVES

To help everyday people become effective advocates for rare diseases, NORD launched a designated website for the Rare Action Network (RAN), offering tools, training and resources. Features include a national summary, state-by-state breakdowns, maps, and key contacts. Visit at www.rareaction.org.

The second annual edition of our State Report Card: A Roadmap to Improve the Lives of People with Rare Diseases was released. The report graded states on the following policy areas and also offered a vision statement for each: coverage of medical foods and newborn screening, prescription drug cost-sharing limits, policies supporting biosimilar prescriber communications, protections against step therapy protocols, the establishment of rare disease advisory councils, and Medicaid expansion program, including the Children's Health Insurance Program (CHIP).

Additional 2016 Legislative Accomplishments

4 states passed step therapy laws (CT, IL, IN, and MO)	7 states passed biosimilars laws (AZ, CA, HI, ID, MO, OH, and RI)
Illinois passed a Rare Disease Commission Bill	Hosted an Advocacy 101 Webinar to offer best practices for meeting with elected officials

RARE ACTION NETWORK®
powered by NORD®

Research

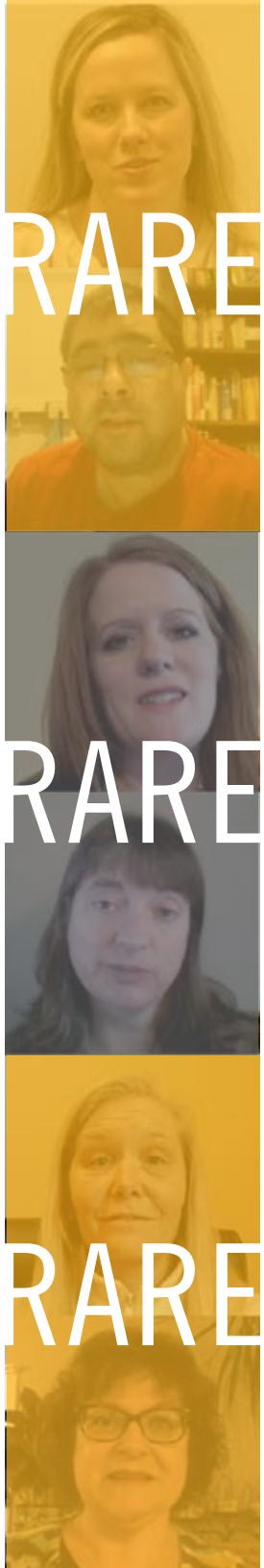
Driving Research that Brings New Treatments to Patients

The statistics can be overwhelming: nearly 95% of the 7,000 known rare diseases have no treatment. As the umbrella organization serving all rare diseases, NORD supports patients and patient organizations by advancing systems and providing resources to support research that can lead to life-saving diagnostics and new therapies.

Through our IAMRARE™ Registry Program, individuals from around the world are empowered to educate others about their day-to-day quality of life issues and the characteristics of their disease. This type of knowledge is currently lacking for most rare conditions and is essential for improving clinical trial designs and accelerating new drug approvals. With support and input from the Food and Drug Administration (FDA), ***we now have 15 active registries.*** *While there are five additional registries in development, 40 disease states are waiting as we grow the program and continue our efforts to generate resources for this work to increase understanding of rare diseases.*

Partnering with the following patient organizations:

- Adult Polyglucosan Body Disease (APBD) Research
- Bridge the Gap's SYNGAP1 (MRD5)
- CCHS Family Network
- Congenital Hyperinsulinism International
- The Desmoid Tumor Research Foundation
- Fibrous Dysplasia Foundation's FD/MAS Patient Registry
- Foundation for Prader-Willi Research
- Galactosemia Foundation
- Hereditary Neuropathy Foundation
- International Pemphigus and Pemphigoid Foundation
- The Morgan Leary Vaughan Fund, Inc.
- National PKU Foundation
- The OMSLife Foundation
- Platelet Disorder Support Association
- XLH Network, Inc.



NORD's natural history studies project empowers patients and families to help eliminate some of the "I don't know" in rare disease research, making way for progress.

NORD's Rare Disease Research Grant Program provides seed funding to academic scientists for translational or clinical studies related to the development of potential new diagnostics or treatments for rare disease. Over the years, NORD grants have led to the development of two FDA-approved treatments and numerous journal articles. More than 150 grants have been awarded nearing $7 million in approved funding since the program's launch in 1989.

In 2016, NORD awarded a total of $340,000 in research grants for the study of six rare diseases, including:

Aveolar Capillary Dysplasia with Misalignment of the Pulminary Veins	Appendix Cancer and Pseadomyxoma Peritonei
Autoimmune Polyglandular Syndrome Type I	Homocystinuria
Malonic Aciduria	Stiff Person Syndrome

Educational Initiatives

Driving Awareness and Education

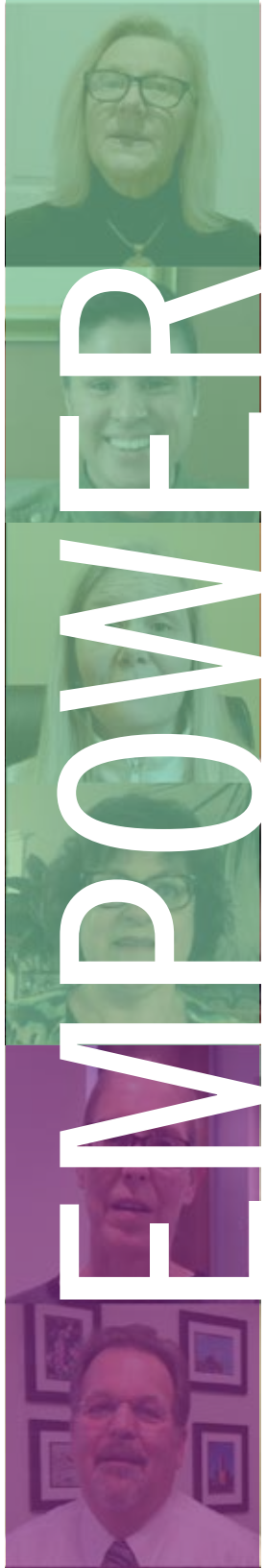
This year, a record 6.5 million people from all 50 states and 236 countries came to our website looking for updated and often hard-to-find information. We remain committed to educating patients, caregivers, clinicians, and students about rare diseases.

Our annual Rare Diseases & Orphan Products Breakthrough Summit™ took place in Arlington, VA, on Oct. 17-18. It was our most-attended Summit to date, bringing together 600 guests from all areas of rare diseases. During the two-day conference, we discussed strategies to address patient challenges, orphan product development, the National Cancer Moonshot Initiative, genetic innovation, collaborations across borders, and much more. Keynote speakers included FDA Commissioner Dr. Robert Califf, who called this an era of remarkable progress; parent advocate Kristen Gray (The Charlotte & Gwenyth Gray Foundation), who shared her family's story of trying to cure Batten disease; and Kate Rawson (Prevision Policy), who discussed national election implications.

Additional 2016 Educational Accomplishments

Clinicians from 169 countries accessed our online Physician's Guides	185 reports updated in NORD's Rare Disease Database, which now features 1,200+ disease-specific reports
Developed 21 new educational videos with our Member Organizations to facilitate timely diagnosis and optimal care	Hosted 10 disease-specific patient and caregiver regional meetings
Partnered with experts from Texas Children's Hospital to co-host two tweetchats focused on Navigating the Emergency Room and Pediatric Cancers	Educated medical students by hosting a booth at the 2016 annual convention of the American Medical Student Association (AMSA), the largest annual meeting of medical students in the U.S.
Hosted an Undiagnosed Diseases Network Webinar to help those struggling to find a diagnosis	Grew our Student Membership to 230 members

Our new 1:1 networking tool created additional opportunities for Breakthrough Summit™ attendees to make valuable connections.



Membership

Driving Programs that Empower Patient Communities

33 YEARS OF COLLABORATION

Helping our patient Member Organizations grow and accomplish their goals is at the heart of our mission. In 2016, we were thrilled to work closely with Members and offer a broad range of teleconferences, in-person regional meetings, and advocacy events, all aimed at giving members an inside track on current issues and opportunities.

We also provided scholarships for 95 representatives from our Membership to attend NORD's annual Breakthrough Summit™ – the largest multi-stakeholder event in rare diseases and the only one co-sponsored by the FDA – offering many opportunities to interact and network with researchers, government officials, and industry leaders.

NORD Member Organization Testimonials

"You've opened so many horizons for us. You made us dare and see possibilities. A couple of weeks ago we were at the FDA for a workshop exclusively on primary sclerosing cholangitis. We couldn't have done it without NORD that brings together all stakeholders we need to be aware of and makes them accessible to us. We are filled with gratitude."

- PSC Partners Seeking A Cure (Primary Sclerosing Cholangitis), a NORD Member Organization

"For both ATOF and myself, attendance to last year's Summit changed our nonprofit and put us years ahead in research and collaborations with our medical partners. It is imperative we are able to participate again this year. We are saving lives because of you!"

- A Twist of Fate (ATOF), a NORD Member Organization

This year NORD welcomed 40 new patient groups, bringing our membership total to more than 250 organizations.

Patient Services

Driving Programs that Save Lives

Because of the generosity of our supporters, NORD is able to help lessen the financial burden placed on individuals and families affected by rare disease. The following are some words of gratitude from the more than 7,000 people we were able to help this year.

If a treatment exists, patients should be able to access it.

NORD entered into a partnership with Trio Health® to capture data about the drug delivery process. Marrying our commitment to patient advocacy with real-world data, the collaboration will produce insights about the overall patient experience and any unforeseen roadblocks that can undermine effectiveness, which can be used to help drive policy change and improve the quality of care.

Patient and Caregiver Testimonials

"Thankfully, your organization… took the time to listen to our situation, to empathize, and to ultimately search for a solution that in the end resulted in a much more positive outcome and a very grateful family... we're eternally grateful for one small "win" in an otherwise terrible journey."

- Nicholas, Chase's Father

"We want to thank you again for your care and kindness toward our family! This time last year she began having break through seizures that were life-threatening. You and your organization have been a big part of relieving some of the financial stress associated with Lily's disease. It makes us feel like we are not in this alone. Thank you so much!"

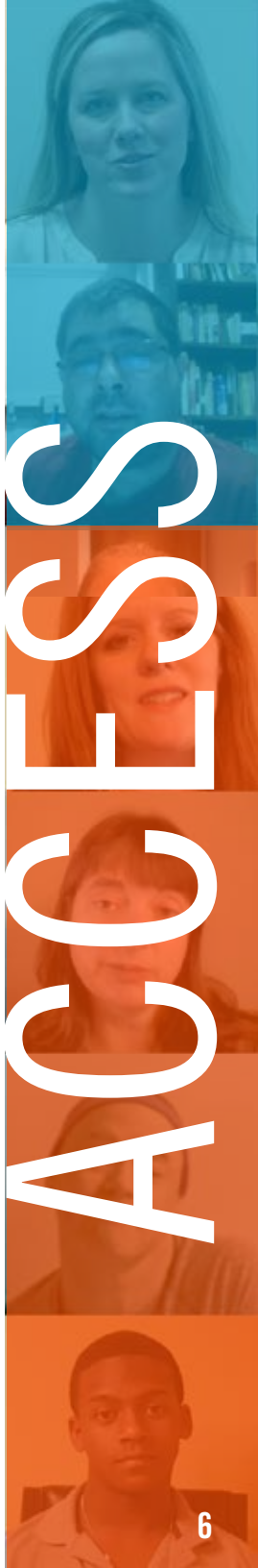
- Lily's family

"I am writing this letter to express my deepest thanks for your organization, NORD. The financial assistance extended to us during the most difficult times in our life is an answered prayer."

- Jenna's mother

"Thank you for all of your support - Merry Christmas. Please know that NORD has given our family the greatest gift."

- Zsaklin's family



Community

Innovative Ways to Get Involved Year-Round

No one with a rare disease should feel alone. New ideas came to fruition in 2016 to help build community, provide support, raise awareness, and inspire action. We engaged new audiences in their communities, at work, in clinical settings, and at home.

Patient/Caregivers Speaker's Bureau

NORD established a Patient/Caregivers Speaker's Bureau to provide a platform for individuals to promote better understanding of the challenges of their rare disease. We successfully placed speakers in more than a dozen events around the country.

Rare Disease Day®

As the U.S. sponsor of Rare Disease Day each year, NORD is proud to help raise awareness and spread the word about rare diseases. For the 2016 campaign, we planned and hosted 37 State House Events that were attended by 2,100+ people and 279 legislators & staff. Advocates in all 50 states obtained governor-issued proclamations for "Rare Disease Day" and the Senate passed a resolution declaring Feb. 29, 2016 as "Rare Disease Day." Hundreds of advocates convened at the National Institutes of Health and together we achieved greater awareness through social media by making the official hashtag, #RareDisease-Day, trend on Facebook and Twitter. In addition, 4,000+ people shared photos and stories in the Handprints Across America® campaign.

RARE DISEASE DAY®

Running for Rare™

Our inspiring charity marathon team, Running for Rare, expanded this year. We took on New York City for the first time as an Official Charity Partner of the 2016 TSC New York City Marathon. We also participated in the Boston, Providence, and Hartford marathons and announced plans to expand to Los Angeles next year. Funds raised by the team support NORD's patient assistance fund for undiagnosed patients.

RUNNING FOR RARE®

Rare Impact Awards

Continuing our annual tradition of celebrating those who are making a difference in the fight against rare diseases, NORD hosted the 2016 Rare Impact Awards at the Warner Theatre, in Washington, D.C., on May 17. The honorees included patients, doctors, researchers, advocates, legislators, a bioethicist, and industry innovators. Prior to the ceremony, we hosted a roundtable conversation with the awardees to discuss ways to drive further progress. The show was live-streamed and served as a fundraiser to support NORD's mission.

Summer Camp

We were honored to partner with The Hole in the Wall Gang Camp® on the first-ever Rare Disease Summer Family Camp. Established by the late actor Paul Newman, The Hole in the Wall Gang Camp opens its doors to children and families to ensure that every child, no matter their illness, can experience the transformational spirit and friendships that go hand-in-hand with camp. The camp was offered completely free, at no charge.

THE HOLE IN THE WALL GANG CAMP

a seriousfun camp

... for Being Part of Our Community!

Generosity takes many forms. It can mean volunteering time to work at an event, visiting an elected official, speaking on a webinar, joining a social media campaign, gathering signatures, or answering questions.

Each day we are inspired by people coming together to do extraordinary things in the rare disease community. Courageous patients and families refuse to give up fighting for answers. Researchers and clinicians work tirelessly to find answers and provide care. Industry innovators push science beyond the limits of what we know to be possible. Advocates and legislators come together to enact policies that improve lives. Volunteers offer their hearts, hands, and time.

For 33 years, NORD has been bringing the needs of the rare disease community to the forefront, in a world where it is often easier to walk away or turn a blind eye. The path in rare diseases is not always an easy one. Today, our work is as essential as always – and we take our responsibility seriously to lead the fight for people with rare diseases in a way that no other organization can.

As a nonprofit, we are grateful for the generosity of our donors who allow us to continue this important work to support patients and our members who serve them. We remain an independent charity. Together, as one community, our voices will be strong in the call for action to improve diagnoses, treatments and cures.

Thank you to everyone who took part in our campaigns and events in the past year. Thank you also to all of the individual donors, foundations, corporate supporters, and others whose gifts helped to drive our mission forward.

Alone we are rare. Together we are strong.®

rarediseases.org

2016 NORD Member Organizations

A Cure in Sight
A Twist of Fate-ATS
Acid Maltase Deficiency Association (AMDA)
Acoustic Neuroma Association
ACPMP (Appendix Cancer / Pseudomyxoma Peritonei Research Foundation)
Acromegaly Community, Inc.
Adrenal Insufficiency United
Alagille Syndrome Alliance
Alpha-1 Foundation
Alport Syndome Foundation
Alternating Hemiplegia of Childhood Foundation (AHCF)
American Autoimmune & Related Diseases
American Behcet's Disease Association
American Brain Tumor Association
American Cleft Palate-Craniofacial Association/ Cleft Palate Foundation (For Patients/families) ACPCA is for medical professionals.
American Multiple Endocrine Neoplasia Support
American Partnership for Eosinophilic Disorders (APFED)
American Porphyria Foundation
American Syringomyelia & Chiari Alliance Project, Inc
Amyloidosis Research Consortium, Inc.
Amyloidosis Support Groups, Inc.
APBD Research Foundation
Aplastic Anemia & MDS International Foundation, Inc (AAMDS)
APS Type 1 Foundation
Association for Creatine Deficiencies
Association for Frontotemporal Degeneration (AFTD)
Association for Glycogen Storage Disease
Association of Gastrointestinal Motility Disorders, Inc (AGMD)
Ataxia Telangiectasia Children's Project, Inc (A-T)
Autoimmune Hepatitis Association
Autoinflammatory Alliance (formerly NOMID Alliance)
Basal Cell Carcinoma Nevus Syndrome Life Support Network
Batten Disease Support & Research Association
Benign Essential Blepharospasm Research Foundation, Inc
Bohring-Opitz Syndrome Foundation, Inc.
BORN A HERO, Pfeiffer's Health and Social Issues Awareness
Bridge the Gap-SYNGAP Education & Research Foundation
Calliope Joy Foundation
Cardio Facio Cutaneous International (CFC)
Castleman's Awareness & Research Effort (CARE)
CCHS Network
Charcot-Marie Tooth Association
Charlotte & Gwenyth Gray Foundation to Cure Batten Disease at The Giving Back Fund
Children's Cardiomyopathy Foundation
Children's Craniofacial Association
Children's PKU Network
Children's Tumor Foundation, Inc
Chloe's Fight Rare Disease Foundation
Cholangiocarcinoma Foundation
Chordoma Foundation
Chromosome 18 Registry & Research Society
Chromosome Disorder Outreach, Inc
Chronic Granulomatous Disease Association, Inc.
Cicatricial Alopecia Research Foundation (CARF)
Cloves Syndrome Community
Cluster Headache Support Group, Inc.
Clusterbusters, Inc.
CMTC-OVM - US
Congenital Hyperinsulinism International
Consortium of Multiple Sclerosis Centers
Cornelila de Lange Syndrome Foundation, Inc.
Council for Bile Acid Deficiency Diseases
CURE HHT Foundation
Cure SMA
CureCADASIL/CADASIL Association, Inc.
CurePSP
Curing Retinal Blindness Foundation
Cushing Support & Research Foundation, Inc.
Cutaneous Lymphoma Foundation
Cystinosis Foundation, Inc.
Cystinosis Research Network, Inc.
Daybreak Children's Rare Disease Fund
Desmoid Tumor Research Foundation
Dravet Syndrome Foundation
Dup15q Alliance
Dysautonomia Foundation Inc.
Dyskeratosis Congenita Outreach, Inc. (DCO)
ECD Global Alliance
Erythromelalgia Association
Evans Syndrome Foundation
Family Caregiver Alliance
Family Support Network of North Carolina
Fat Disorders Research Society, Inc.
Fibrolamellar Cancer Fdn
Fibromuscular Dysplasia Society of America
Fibrous Dysplasia Foundation
Foundation Fighting Blindness
Foundation for Ichthyosis & Related Skin Types, Inc.
Foundation for Prader-Willi Syndrome
FPIES Foundation
Friedreich's Ataxia Research Alliance (FARA)
Galactosemia Foundation
GBS/CIDP Foundation International
Genetic Alliance
Global Foundation for Peroxisomal Disorders
Glut1 Deficiency Foundation
Gut Check Foundation
Guthy Jackson Charitable Foundation
Hemophilia Federation of America
Hereditary Leiomyomatosis & Renal Cell Cancer Family Alliance (HLRCCFA)
Hereditary Neuropathy Foundation
Hermansky-Pudlak Syndrome Network, Inc.
Histiocytosis Association, Inc.
Hope for Hypothalamic Hamartomas
Huntington's Disease Society of America
Hydrocephalus Association
Immune Deficiency Foundation
Incontinentia Pigmenti International Foundation
Indian Organization for Rare Diseases
International FOP Association, Inc. (Fibrodysplasia Ossification Progressive)
International Foundation for CDKL5 Research
International FPIES Association (International Association for Food Protein Entercolitis)
International Myeloma Foundation
International Pemphigus & Pemphigoid Foundation (IPPF)
International Rett Syndrome Foundation (Cure Rett)
International WAGR Syndrome Association
International Waldenstrom's Macroglobulinemia Foundation
Intractable Childhood Epilepsy Alliance-ICE Epilepsy Alliance
ISMRD
Jack McGovern Coats Disease
Joshua Frase Foundation for Congenital Myopathy Research
Julia's Wings Foundation, Inc.
Kennedy's Disease Association, Inc.
Klippel Trenaunay (KT) Support Group
LAL Solace, Inc.
LAM Foundation
Liam's Land Organization, Inc.
Life Raft Group
Lipoprotein a Foundation
Lowe Syndrome Association, Inc.
Lymphangiomatosis & Gorham's Disease Alliance, Inc. (LGDA)
Marfan Foundation
Martin Mueller IV Achalasia Awareness Foundation, Inc.
Mastocytosis Society, Inc.
M-CM Network
MEBO Research, Inc.
Melorheostosis Association
Mesothelioma Applied Research Foundation
MitoAction
MLD Foundation
Moebius Syndrome Foundation
Morgan Leary Vaughan Fund, Inc.
Mowat-Wilson Syndrome Foundation
MPN Research Foundation
MSUD Family Support
Mucolipidosis Type IV Foundation, Inc.
Multiple System Atrophy Coalition, Inc. (MSA Coaltion)
Myasthenia Gravis Foundation of America, Inc.
Myelin Project
Myelodysplastic Syndromes Foundation, Inc. (MDS Foundation)
Myocarditis Foundation
Myositis Association
Myotonic Dystrophy Foundation
Narcolepsy Network, Inc.
National Adrenal Diseases Foundation
National Alopecia Areata Foundation
National Ataxia Foundation
National Brain Tumor Society
National Eosinophilia Myalgia Syndrome Network
National Foundation for Ectodermal Dysplasias
National Health Council (NHC)
National Lymphedema Network, Inc.
National MPS Society
National Nieman-Pick Disease Foundation, Inc. (NNPDF)
National Organization for Albinism & Hypopigmentation (NOAH)
National PKU Alliance
National PKU News
National Spasmodic Dysphonia Association
National Tay-Sachs & Allied Diseases Association
National Urea Cycle Disorders Foundation
NBIA Disorders Association
NephCure Kidney International
Neuroendocrine Tumor Research Foundation (formerly Caring for Carcinoid Foundation)
Neurofibromatosis Network
NGLY1 Foundation
NICER Foundation
NTM Info & Research, Inc
Ocular Melanoma Foundation
Oley Foundation
OMSLife Foundation
Organic Acidemia Association
Osteogenesis Imperfecta Foundation
Pachyonychia Congentia Project
Pancreatic Cancer Action Network
Parent Project Muscular Dystrophy
Parkinson's Disease Foundation, Inc.
Phelan-McDermid Syndrome Foundation
Pitt Hopkins Research Foundation
Pituitary Network Association (PNA)
PKD Foundation
Platelet Disorder Support Association
Prader-Willi Syndrome Association, USA
Primary Ciliary Dyskinesia Foundation - PCD Foundation
PRISMS (Parents & Researchers Interested in Smith-Magenis Syndrome)
PRP Alliance, Inc.
PSC Partners Seeking A Cure (Primary Sclerosing Cholangitis)
Pulmonary Fibrosis Foundation
Pulmonary Hypertension Association

2016 NORD Member Organizations (continued)

PURA Syndrome Foundation
Rare & Undiagnosed Network
Rare Cancer Research Foundation
RASopathies Network USA
Recurrent Respiratory Papillomatosis Foundation
Reflex Sympathetic Dystrophy Syndrome Association (RSDSA)
Rett Syndrome Research Trust
Rothmund-Thomson Syndrome Foundation
RYR-1 Foundation
Sarcoma Foundation of America
SBS Cure Project
Scleroderma Foundation
Scleroderma Research Foundation
Short Bowel Syndrome Foundation
Shwachman-Diamond Syndrome Foundation
Sitosterolemia Foundation
Snyder-Robinson Foundation, Inc.
Sofia Sees Hope
Soft Bones, Inc.
Sotos Syndrome Support Association
Spastic Paraplegia Foundation
Spinal CSF Leak Foundation
SSADH Association (Succinic Semialdehyde Dehydrogenase Deficiency)
Stevens Johnson Syndrome Foundation
Sturge-Weber Foundation
Target Cancer Foundation
Tarlov Cyst Disease Foundation
Tess Research Foundation
TNA - The Facial Pain Association
Tourette Association of America
Transverse Myelitis Association
Tuberous Sclerosis Alliance (National Tuberous)
Turner Syndrome Society of the United States
United Leukodystrophy
United Mitochondrial Disease Foundation
US Hereditary Angioedema Association
Vasculitis Foundation
Vestibular Disorders Association (VEDA)
VHL Alliance
Williams Syndrome Association
Wilson Disease Association
Worldwide Syringomyelia & Chiari Task Force Inc.
XLH Network, Inc.
Xtraordinary Joy

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Division of Gastroenterology
Director, Wilson Disease Program
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