ABOUT RARE DISEASE

1 in 10 Americans are battling a rare disease, defined as one that affects fewer than 200,000 people in the United States. There are more than 7,000 rare diseases that have been identified. While each one affects a small number of patients, the number of those dealing with rare disease add up to close to 30 million Americans – at least half of whom are children. Holding hands, they would circle the globe 1.5 times.

ABOUT NORD®

OUR PEOPLE:

271 Member Organizations
84 Corporate Council Members
3,666 Rare Action Network® Members and 28 State Ambassadors
459 Student Members and Campus Leaders
6,000+ Participants Entering Data in 19 Natural History Studies
125,000 Social Media Followers

OUR VISION:

A national awareness and recognition of the challenges endured by people living with rare diseases
A culture of innovation that supports basic and translational research to create diagnostic tests and therapies for all rare diseases
Access to adequate medical care and support services that improve patients’ lives
A regulatory environment that encourages development and timely approval of safe, effective diagnostics and treatments

We Care!
Our small but mighty team of 61 staffers helped to make these remarkable accomplishments possible.
Dear Friends,

Because of your support, 2017 was a remarkable year for rare diseases. Together we overcame challenges. We passed new laws and defeated harmful legislation. We helped patients and raised awareness. While supporting research and educating medical professionals, we saw medical advancements—including the first gene therapies—become available.

This year was particularly special at NORD, filled with many important firsts and program launches. A high point, for me personally, was watching one of our Rare Action Network® State Ambassadors, a mother of a rare disease patient who had never done any advocacy with legislators, transform from being a quiet novice to a powerhouse in meetings with her elected officials.

On the research side, several patient groups launched natural history studies with NORD’s IAMRARE™ platform and received an overwhelming response, with many from their patient communities eager to join. Fulfilling two long-time goals, we hosted NORD’s first-ever Continuing Medical Education program and brought to life an inspiring, year-long public awareness campaign. This type of work is what NORD is all about – building programs and events that have a ripple effect throughout the rare disease community and beyond.

As we move forward, we know that we will face new challenges and yet we remain dedicated to the cause that keeps us going: the urgent needs of patients and the knowledge that we are having a direct impact on their health and well-being every day.

We look forward to working with our advocates and members and to supporting each other in the years ahead. We offer our gratitude to all of the friends and donors who are part of our fight.

Thank you,

Peter L. Saltonstall,
President and CEO
THE NORD® IMPACT:

Patients and families battle rare diseases on multiple fronts: medically, financially, physically and emotionally. With programs of advocacy, research, education and financial assistance, NORD is there for our rare community providing support and leading transformative change.

2017 BY THE NUMBERS:

8.6M people and organizations reached through NORD’s programs.

14.2M page views on our website.

150k phone calls answered at our call center.

19 states passed new laws benefiting the rare community because of NORD’s efforts.

570 events that we hosted or attended that brought people together for rare diseases.

3.6k people became effective advocates with Rare Action Network® training.

9.1B earned media impressions generated publicity for our cause.

NORD raised awareness of rare disease across numerous media outlets:
Since 1987, NORD—a pioneer in Patient Assistance Programs—has been helping children and families gain access to specialized medical care they could not otherwise afford.

Our RareCare™ programs provide patients with 360° support.

**OUR IMPACT IN 2017**

8,337 patients helped in all 50 states, D.C., Guam and Puerto Rico

1,732 were children

$31.7M in aid given directly to patients to help with insurance costs, premium and co-pay expenses, medical expenses not covered by insurance, and other assistance for people who were without insurance

6,429 patients received awards from premium copay and copay programs

1,265 patients received awards from Medical Assist programs

543 patients receiving awards from Free Drug/Medication Assist programs

77 patients assisted through NORD Emergency Relief (ERF) Programs

23 patients assisted through the NORD Undiagnosed Patient Programs

“"I want NORD to know how much I appreciate all you do for me in this difficult time in my life.”

-Charles, a patient with Familial Amyloid Polyneuropathy

“On behalf of Zachary and our family, we are extremely grateful to NORD for being so responsive to our needs and helping us through what seemed like irresolvable issues.”

-Ed, father of a rare patient thanking NORD for helping him and his son
ELIMINATING BARRIERS

DISEASES SERVED BY RARECARE IN 2017

Acute Lymphocytic Leukemia
Alpha-1 Antitrypsin Deficiency
Carnitine Deficiency
Cerebrotendinous Xanthomatosus (CTX)
Cervical Dystonia
Chorea
Chronic Granulamous Disorder
Congenital Sucrase-Isomaltase Deficiency (CSID)
Cryopyrin-Associated Periodic Syndrome (CAPS)
Cushings Syndrome
Cutaneous T Cell Lymphoma
Cystinuria
Dermatitis Hepaformis
Downbeat Nystagmus
Duchenne Muscular Dystrophy (Nonsense Mutation Dystrophinopathy)
Gaucher’s Disease
Hodgkins Lymphoma
Hunter’s Syndrome (MPS II)
Hypophosphatasia IGF-1 Deficiency
Ileal Pouch
Lambert-Eaton Myasthenic Syndrome (LEMS)
Morquio A Syndrome (MPS-IV-A)
Muckel-Wells Syndrome
Narcolepsy
Neonatal Onset Multisystem Inflammatory Disease
Nephropathic Cystinosis
Neurogenic Orthostatic Hypotension
Paroxysmal Nocturnal Hemoglobinuria (PNH)
Periodic Paralysis
Phenylketonuria (PKU)
Seizures Associated with LGS
Short Bowel Syndrome
Spasmodic Torticollis
Tardive Dyskinesia
Urea Cycle Disorders
Ulcerative Colitis
Undiagnosed

Thank You

“There are many additional costs raising a child with a rare disease. Financial support to pay for life sustaining medication is truly a gift… Thank you to everyone at NORD and its donors for being there.”

-Peggy, mom of a child with PKU
The Rare Action Network® works hand in hand with patients to drive policy change through grassroots campaigns. We provide expertise in legislative and regulatory affairs, as well as gather and publish data-driven insights.

This year we mobilized the rare disease patient community in new ways. Through our efforts, we successfully advocated against two harmful federal proposals and took action in 45 states.

**OUR IMPACT IN 2017**

Together, with our Rare Action Network Ambassadors and Advocates we had

*400+ MEETINGS THROUGHOUT THE YEAR*

- **250** on Capitol Hill
- **35** with the FDA (28) and the NIH (7)
- **177** legislative events in **38** states

- Took action on **123** bills in **45** states
- Recruited and trained more than **3.6K** members and **28** state ambassadors to become effective advocates
- Hosted **7** advocacy training workshops
- Sent out **124** email blasts regarding policy alerts and activities

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**Thank You**

“I want to thank you again for a fantastic training program. I am deeply grateful for your time and expertise. The exercise was on point to get us ready for advocating.”

-Stephanie Bozarth
Chairman, Board of Directors,
National MPS Society
YOUR VOICE MATTERS

We work with our members and partners to achieve shared advocacy goals. National alliances include:

- Alliance for Healthcare Reform
- Alliance for a Stronger FDA
- American Plasma Users Coalition (A-PLUS)
- Coalition for Accessible Treatments (CAT)
- Medication Access for Patients Rx (MAPRx)
- National Health Council (NHC)
- Partners for Better Care
- Patients for Biologics Safety and Access (PBSA)
- Research!America
- Regulatory Education and Action for Patients (REAP)
- State Access to Innovative Medicines Coalition (SAIM)
- United States Pharmacopoeia (USP)

2017 RARE DISEASE ADVOCACY & POLICY

States with Rare Action Network Ambassadors (28)
States where Action was taken (123 bills)
MEMBERSHIP

We strengthen the work of our Member Organizations, helping their leaders with resources, capacity-building training and making connections to maximize their impact.

We train members on good governance practices, fundraising, board management, medical education, managing medical advisory boards, registries, research, prioritizing policy, ICD-10 classifications and more.

OUR IMPACT IN 2017

- 271 members from non-profit patient organizations
- 8 webinars presented expert speakers including the Director of the National Center for Advancing Translational Sciences at NIH
- 6 bi-monthly calls shared the latest news from across the rare disease community
- 86 scholarships awarded for Members to attend NORD’s Summit and the World Orphan Drug Conference

- **Launched** the NORD Member Organization Facebook Group, where nearly 210 founders and executives participate daily in robust conversations about emerging issues
- **Launched** and launched the first weekly email newsletter for Members
- **Conducted** a Member Survey to better understand and meet the evolving needs of patient organizations

“Your bi-monthly call was phenomenal. It always energizes me to get involved and do more. For lack of time, we often can’t be present in all the wonderful projects NORD creates; however, knowing that they are there, and that we have a wide context to work from is always empowering...

Thank you for all you do for us. We are so proud of being NORD members.”

– Rachel Gomel, PSC Partners
OUR MEMBERS - WHO WE ARE

- **82%** were founded by parents or caregivers of someone with rare disease
- **74%** are led by patients, parents or caregivers
- **82%** have fewer than 5 full-time employees
- **50%** nearly half employ zero full-time staff

VALUE OF MEMBERSHIP

- Advocacy Opportunities and Representation
- Access to Research Tools and Programs
- Event Scholarships and Discounts
- Capacity Building and Leadership Resources
- Access to Breaking News and In-Depth Analyses
- Peer Networking and Guidance
- Visibility and Promotional Opportunities
- Credibility
Our goal is to support the advancement of research so that new treatments can be developed more quickly to help patients.

Through NORD’s IAMRARE™ platform, we provide support, guidance and training to patient organizations launching registries and natural history studies. Patient-powered natural history studies are transforming how patients and caregivers inform and shape medical research and translational science for rare diseases.

### OUR IMPACT IN 2017

- **More than 6,000** participants around the world contributed data to IAMRARE studies
- **16 active** IAMRARE studies, with more under development
- **1 IAMRARE** study discovered a new mechanism for the disease’s gene!
- **9 new** IAMRARE studies launched

### IAMRARE STUDIES LAUNCHED

- CCHS Network One World Registry  
  (for Congenital Central Hypoventilation Syndrome)
- Charcot-Marie Tooth Research Network
- Desmoid Tumor Patient Registry
- International Pemphigus and Pemphigoid Foundation Natural History Registry
- ITP Natural History Study Registry  
  (for Immune thrombocytopenia)
- Natural History Registry for Necrotizing Enterocolitis
- OMS Patient Registry  
  (for Opsoclonus Myoclonus Syndrome)
- The OAA Natural History Patient Registry  
  (for organic acidemia disorders)
- The PKU Registry  
  (for Phenylketonuria)

“The IAMRARE team at NORD is absolutely committed to helping rare disease patient organizations run low-cost, high-quality natural history studies. NORD’s understanding of the needs, goals and challenges of a small nonprofit like ours is in a league of its own.”

- Member, Fibrous Dysplasia Foundation
GRANTING HOPE

For many rare diseases, our grants represent the only source of research funding, providing financial support for researchers and also hope for the rare disease community.

$250k invested in new research grants

5 RESEARCH GRANTS AWARDED

7 published peer-reviewed papers in 2017 cited NORD’s funding support from prior grant cycles.

RESEARCH GRANT SNAPSHOT:

APPENDIX CANCER & PSEUDOMYXOMA PERITONEI (PMP)
(affects 1-2 cases per million individuals)

• J. Silvio Gutkind, PhD, University of California, San Diego
• D. Scott Merrell, PhD, Uniformed Services University of the Health Sciences
• Marc Pocard, MD, PhD, Institut national de la santé et de la recherche médicale (Inserm), Paris, France
• Traci L. Testerman, PhD, University of South Carolina School of Medicine

MAJOR RESEARCH DONATIONS RECEIVED

• ACPMP Research Foundation
  ($164,000 for Appendix Cancer and PMP fund)
• The Hope Fund
  ($55,000 for Malonic Aciduria fund)
• Nicolas Vassalli
  ($30,000 for PACS1 fund)
• The David Ashwell Foundation
  ($22,420.57 for ACD/MPV fund)
• Alveolar Capillary Dysplasia Association
  ($20,100 for ACD/MPV fund)
• William Akers, Jr. & Georgia O. Akers Private Foundation, Inc.
  ($10,000 for ACD/MPV fund)
• Lundbeck “Raise Your Hand” Campaign 2017
  ($10,000 for rare disease research)
EDUCATIONAL INITIATIVES

NORD is proud to deliver education programs that are used around the world.

NORD’s RareEDU® program provides educational resources to the rare community, including those living with a rare disease, caregivers, medical professionals, students, researchers and the media.

NORD’s Rare Disease Database, with expert reviewed reports, together with an always evolving resource center featuring helpful videos, webinars, and other vital educational materials provide the foundation to help people better understand rare diseases and the impact of living with a rare disease.

OUR IMPACT IN 2017

Our online Rare Disease Reports are written in patient-friendly language with the help of independent medical professionals.

- More than 200 new and updated reports added to the website
- 10.3 million views, up by 23% since 2016
- Residents from 237 different countries accessed rare disease reports

Our partnerships with medical publishers allow us to publish news from NORD and our member organizations in journals for medical professionals. Through our student programs, we are educating future health care professionals.

- Hosted our first-ever Continuing Medical Education (CME) program
- Reached 30k medical professionals through our Neurological Diseases Special Report (3rd edition) published with Neurology Reviews
- Partnered and published content with Medscape, WebMD, Frontline Medical Communications and Rare Disease Report
- 8 new Student Chapters and 5 Student Clubs created
The 2017 Rare Summit was our largest conference to date, with more than 60 research posters and 60 lunch and learn roundtable discussions. We exchanged ideas, made personal connections and planned future collaborations.

✔️ **700 participants** attended the 2017 NORD Summit

We were thrilled to see several NORD Member Organizations getting together with FDA leaders to talk one-on-one about rare diseases and were inspired by keynote speakers: FDA Commissioner Scott Gottlieb, M.D., and Mike Porath, founder of The Mighty.
Running for Rare, brought together 50 dedicated runners and 54 community partners to raise more than $125,000 with the goal to assist patients seeking a diagnosis.

They inspired us as participants in events across the country, including:

- TCS New York City Marathon
- Boston Marathon
- Providence Full- and Half-Marathons
- Eversource Hartford Marathon
- Skechers Performance Los Angeles Marathon

Rare Disease Day, an international day of awareness, is the biggest day of the year in rare diseases. NORD was honored to serve as the host of the campaign in the U.S. for another successful year!

OUR CAMPAIGN FEATURED:

- 43 educational and advocacy events
- Shared 1,700 new patient stories trended on social media
- Generated 4,300 media articles to raise awareness
DO YOUR SHARE FOR RARE Campaign

Launched on Rare Disease Day, “Do Your Share for Rare” featured the voices and stories of many living with a rare disease to inspire and encourage those living outside of the community to get involved in ways big or small. A community-driven public service announcement kicked off the campaign.

GOOD MORNING PEYTON Documentary

Imagine never being allowed to feel the sun on your skin. For 11-year-old Peyton, who has a rare disease that makes him allergic to sunlight, that is a reality.

We partnered with Peyton’s hometown to turn one special night into day for him, and developed an award-winning documentary about the experience for our year-long “Do Your Share for Rare” campaign. “Do Your Share for Rare” was created to shed light on the need for more discussion on rare diseases and inspire individuals and communities to show their support.

OVER 87,000 VIEWS, 3 HEALTHCARE MARKETING AWARDS:

- Global Gold Award, Health Awareness & Advocacy (HWC): Activations/Events/Live Experiences
- Silver Winner, Clio Health, Disease Awareness
- Gold Video MM&M Awards
In addition to representing patients in the United States, NORD also represents the U.S. patient community abroad.

OUR WORLDWIDE PARTNERS

NORD’S INTERNATIONAL MEMBERS

- Canadian PBC Society
- Genetic Alliance Australia
- EURORDIS
- Canadian Organization for Rare Disorders (CORD)
- CMTC-OVM Netherlands
- Parent to Parent New Zealand, Inc.
- Taiwan Foundation for Rare Disorders
- HCU Network Australia
- Wilhelm Foundation
- Canadian CMTC Foundation
- Sanfilippo Children’s Foundation
- iSEEK Pulmonary Hypertension Hope Center
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 271 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research and patient services.

In 2017, there was a $181,642 deficit to the changes in unrestricted net assets. However, there was a positive $15.1 million change in total net assets.
THANK YOU!

BOARD OF DIRECTORS

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Associate Professor for Metabolic Diseases University of Zurich; Head, Division of Metabolics Children’s Hospital Zurich

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Professor Emerita of Science and Technology Studies and Humanities, Virginia Polytechnic Institute and State University
NORD ORGANIZATION MEMBERS

A Cure in Sight
A Twist of Fate-ATS
Acid Maltese Deficiency Association (AMDA)
Acoustic Neuroma Association
ACMP (Appendix Cancer / Pseudomyxoma Peritonei Research Foundation)
Acromegaly Community, Inc.
Adrenal Insufficiency United
Ahalgle Syndrome Alliance
Alpha-1 Foundation
Alop Syndrome 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 Creative 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
Autoflammatory Alliance (formerly NOMID Alliance)
Basal Cell Carcinoma Nevis Syndrome Life Support Network
Batten Disease Support & Research Association
Benign Essential Blepharospasm Research Foundation, Inc
Bohning-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 & Gwennyth Gray Foundation to Cure Batten Disease at The Giving Back Fund
Children’s Cardiomyopathy Foundation
Children's Cranial Facial Association
Children’s PKU Network
Children’s Tumor Foundation, Inc
Chloé’s Fight Rare Disease Foundation
Cholangiocarcinoma Foundation
Chordoma Foundation
Chromosome 18 Registry & Research Society
Chromosome Disorder Outreach, Inc
Chromosome 18 Register & Research Society
Chromosomal Disorders Outreach, Inc
Chronic Granulomatous Disease Association, Inc.
Cicatricial Alopeica Research Foundation (CARF)
Cloves Syndrome Community
Cluster Headache Support Group, Inc.
Clusterbusters, Inc.
CMTC-OVM - US
Congenital Hyperinsulinism International
Consortium of Multiple Sclerosis Centers
Cornelia 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
Dup 1Sq 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 Foundation
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

CORPORATE COUNCIL MEMBERS

The following organizations participated in NORD’s Corporate Council membership program.

ABB VIE
ACHILLION PHARMACEUTICALS
Actelion Pharmaceuticals US, Inc.
Aegerion
Agilis Biotherapeutics, Inc.
Agios
AGTC
Akcea
Alexion Pharmaceuticals, Inc.
Allergan
Alnylam Pharmaceuticals
Amgen
Amicus Therapeutics, Inc.
Amgen
Alnylam Pharmaceuticals
Alexion Pharmaceuticals, Inc.
Akcea
AGTC
Agios
Agilis Biotherapeutics, LLC
ACHILLION PHARMACEUTICALS
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Incyte Corporation
HORIZON PHARMA
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GRUNENTHAL USA INC
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Editas Medicine
DOHMEN LIFE SCIENCE SERVICE
Cytokinetics, Inc.
CTD Holdings, Inc.
Cytokinetics, Inc.
DORHMEN LIFE SCIENCE SERVICE
Edistas Medicine
EGER BIOPHARMACEUTICALS
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STRONGBRIDGE BIOPHARMA
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Teva Pharmaceuticals
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Vertex Pharmaceuticals Inc.
VITAL THERAPIES, INC
WALGREENS CO
ZAFGEN, INC
Zealand Pharma
ZOGENIX, INC.
NORD ORGANIZATION MEMBERS (CONTINUED)

Friedreich's Ataxia Research Alliance (FARA)
Galactosemia Foundation
GBS/CIDP Foundation International
Genetic Alliance
Global Foundation for Peroxisomal Disorders
Glut 1 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 Progressiva)
International Foundation for CDKL5 Research
International FPIES Association
International Association for Food Protein Enterocolitis
International Myeloma Foundation
International Pemphigus & Pemphigoid Association
International Myoclonus Association
International Retinitis Pigmentosa Foundation
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
Measles Hemorrhagic Apoplexy 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 Coalition)
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 Alpopecia Areata Foundation
National Ataxia Foundation
National Brain Tumor Society
National Eosinophilia Myalgia Syndrome Network
National Foundation for Ocular and Periorbital Disease
National Health Council (NHC)
National Lymphedema Network, Inc.
National MPS Society
National Nieman-Pick Disease Foundation, Inc. (NWPDF)
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 Carcinooid Foundation)
Neurofibromatosis Network
NGLY1 Foundation
NICER Foundation
NTM Info & Research, Inc
Ocular Melanoma Foundation
Oley Foundation
OMSLife Foundation
Organic Acidaemia Association
Osteogenesis Imperfecta Foundation
Osteosarcoma Foundation
Ovarian Cancer National Alliance
Pectus Carinatum Foundation
Pelvic Inflammatory Disease Foundation
PKD Foundation
Platelet Disorder Support Association
Prader-Willi Syndrome Association, USA
Primary Ciliary Dyskinesia Foundation
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
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
Sideropenic Dysphagia Foundation
Singer-Robinson Foundation, Inc.
Sofa Sees Hope
Soft Tissue Cancer Foundation
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
(General 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 S sympathy & Chiari Task Force Inc.
XLA Network, Inc.
Xtremeordinary Joy