NORD®
National Organization for Rare Disorders

2017 ANNUAL REPORT

Alone we are rare. Together we are strong.®

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.

MESSAGE FROM THE CEO

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:

ABC NEWS
TODAY
CNBC
CNN
msn
People
TIME
THE HILL
npr
POLITICO
The New York Times
The Washington Post

PROVIDING PATIENT SERVICES

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

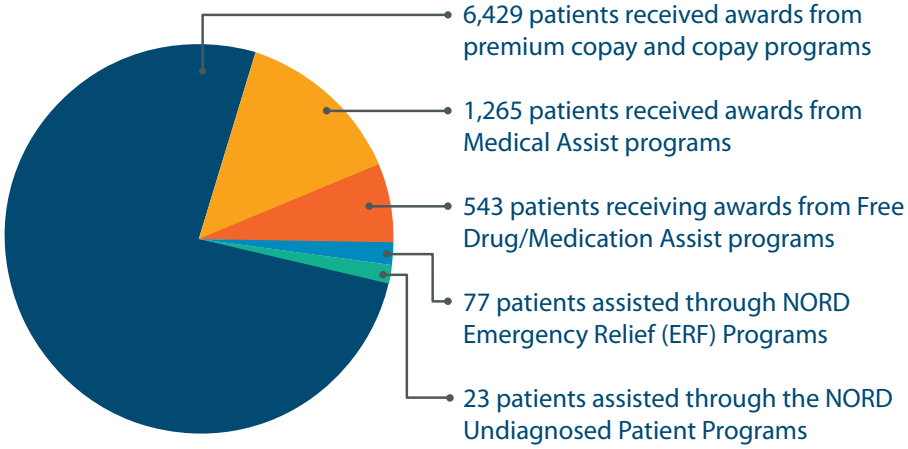
8,337 PATIENTS HELPED

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



Thank You

"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 Distrophy *(Nonsense Mutation Dystrophinopathy)*

Gaucher's Disease

Hodgkins Lymphoma

Hunter's Syndrome *(MPS II)*

Hypophosphatasia IGF-1 Deficiency Ileal Pouch

Lambert-Eaton Myasthinec 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

RARE ACTION NETWORK®

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.

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

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

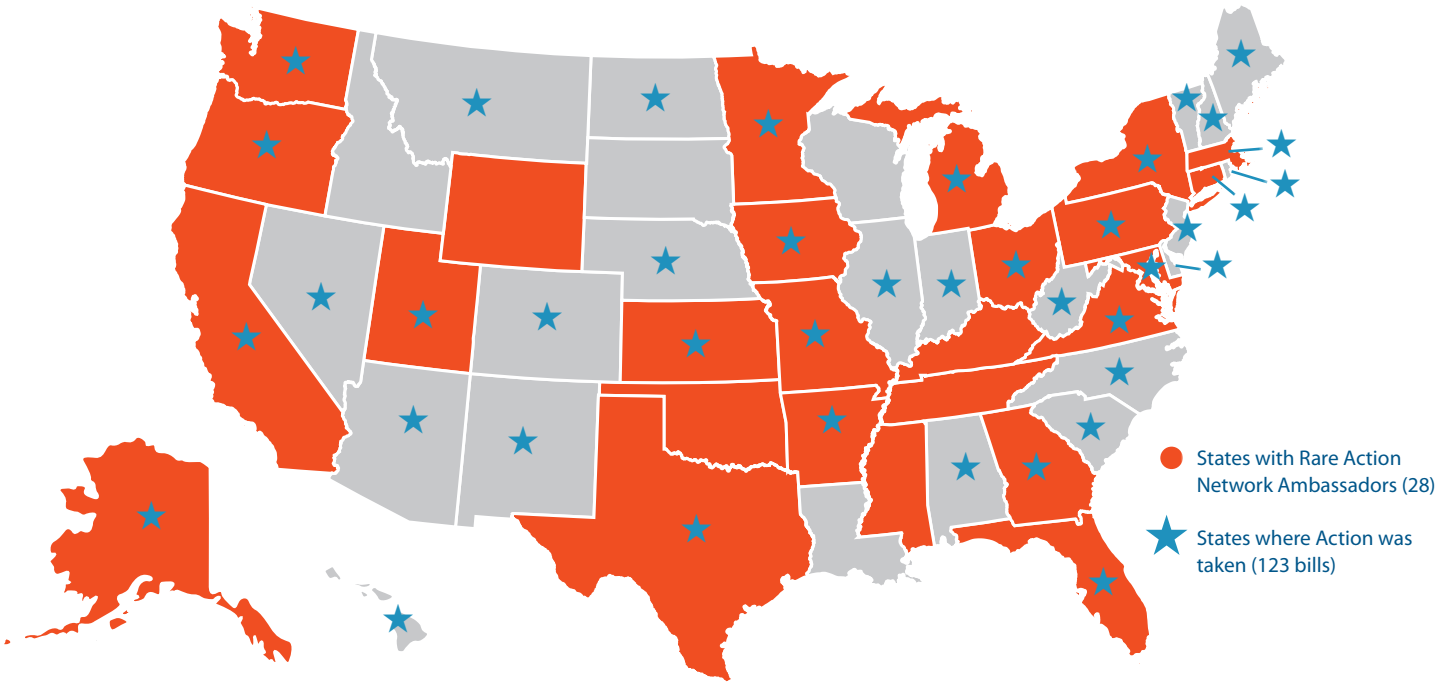
ADVOCACY IN ACTION

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



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 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

Thank You

"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

STRONGER TOGETHER

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

DRIVING RESEARCH

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 caregiviers 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)*

Thank You

"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

ACCELERATING PROGRESS

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.

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)*

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

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
- **Cited by** Time, Newsweek, People, ABC News, CNN, MSN, CNBC, Forbes, San Francisco Chronicle, Houston Chronicle, Nature, Health.com and other news outlets

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.

PROGRAMS FOR MEDICAL PROFESSIONALS & STUDENTS

- **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

ADVANCING EDUCATION

THE NORD RARE DISEASES AND ORPHAN PRODUCTS BREAKTHROUGH SUMMIT®

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.

EVENTS

Our events provide educational content for a broad audience representing all stakeholder groups within the rare disease community.

- Spoke on behalf of patients at a major conference hosted by National Institute of Nursing Research at NIH
- Organized a speaker panel, at the request of the Social Security Administration, for SSA's National Disability Forum on Compassionate Allowances and Rare Diseases.

100%
of CME attendees said the content enhanced their knowledge

91%
were likely or very likely to make changes in their practice based on conference learnings

COMMUNITY EVENTS & AWARENESS

With new programs and record-breaking participation, we took education and awareness to new levels.

Thank You

"I have only 34% lung capacity. People ask me why I am doing this. The simple answer is to raise awareness of PCD and other rare diseases. I'm part of the charity team for the National Organization for Rare Disorders (NORD)'s Running for Rare team.

I want to bring attention to the limitations imposed on people who require oxygen. My mother-in-law literally had her oxygen supply rationed with lethal results. Those of us with rare diseases must fight for treatments which do not have FDA approval. I am taking a stand — a walk — for those of us with rare diseases who will not stay quietly at home."

-Mary Kitlowski,
Running for Rare Participant,
TCS New York City Marathon 2017

OUR IMPACT IN 2017

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

DOING OUR SHARE

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.

34 patient videos submitted

82,334k views

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

TAKING A GLOBAL APPROACH

In addition to representing patients in the United States, NORD also represents the U.S. patient community abroad.

OUR WORLDWIDE PARTNERS

IRDiRC
INTERNATIONAL RARE DISEASES RESEARCH CONSORTIUM

RARE DISEASES INTERNATIONAL
A EURORDIS INITIATIVE

WORLD OrphanDrug

UDN Undiagnosed Diseases Network

CORD
Canadian Organization for Rare Disorders

罕见病发展中心
Chinese Organization for Rare Disorders

EURORDIS
RARE DISEASES EUROPE

ORDI Organization for Rare Diseases India

Japan Patients Association
JPA 一般社団法人 日本難病・疾病団体協議会

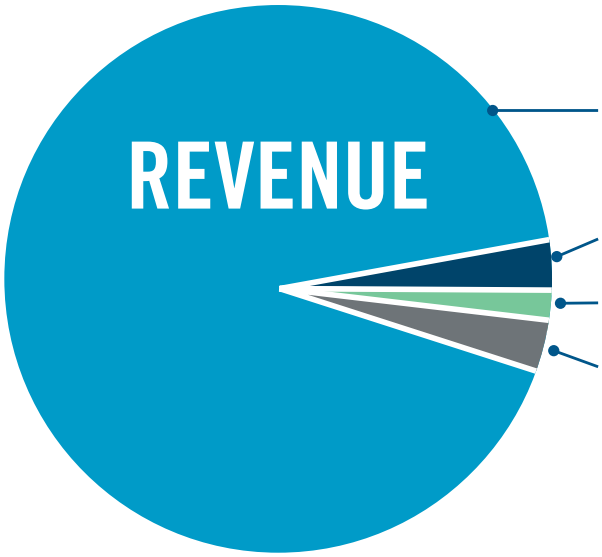
rare voices
AUSTRALIA

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

2017 FINANCIALS

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.



$28,871,006 Patient Service Contributions and Fees

$1,321,624 Organization and Corporate Membership Dues

$1,856,417 Special Events

$1,509,416 Other Contributions, Grants and Service Revenue

$21,741 Investment Income (not shown)

$33,580,204 Total Unrestricted & Released Donor-Restricted Revenue

Program Services $28,301,762

General and Administrative $3,522,216

Development and Communications $1,937,868

Total Expenses $33,761,846

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! ★

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Clinical Professor, Pediatrics, UCSF Medical Director, Genetic Medicine and Metabolism, Valley Children's Hospital

Doris T. Zallen, Ph.D.
Professor Emerita of Science and Technology Studies and Humanities, Virginia Polytechnic Institute and State University

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.
Allergen
Alnylam Pharmaceuticals
Amgen
Amicus Therapeutics, Inc.
Asklepion Pharmaceuticals, LLC
Astellas
AUDENTES THERAPEUTICS INC
AveXis, Inc.
Biogen
BioMarin Pharmaceutical Inc.
Biotechnology Industry Organization
BLUEBIRD BIO
BLUEPRINT MEDICINES
Boehringer-Ingelheim Pharmaceuticals, Inc
BRISTOL-MYERS SQUIBB COMPANY
Catalyst Pharmaceuticals, Inc.
Celgene Corporation
CHEMISTRY RX
CHIASMA PHARMA
CHILDRENS NATIONAL HEALTH SYSTEM
Clementia
CSL BEHRING
CTD Holdings, Inc.
Cytokinetics, Inc.
DOHMEN LIFE SCIENCE SERVICE
Editas Medicine
EIGER BIOPHARMACEUTICALS
Genentech, Inc
Genzyme Corporation
GlaxoSmithKline
Global Blood Therapeutics, Inc.
GlycoMimetics, Inc.
GRUNENTHAL USA INC
HANSA MEDICAL
HORIZON PHARMA
Incyte Corporation
INSMED INC.
INTELLIA THERAPEUTICS
INVITAE
IPSEN BIOPHARMACEUTICALS, INC.
Jazz Pharmaceuticals
Johnson & Johnson
Lundbeck, Inc.
LYSOGENE
MALLINCKRODT PHARMACEUTICALS
Marathon Pharmaceuticals, LLC
MEIRAGTX
MITSUBISHI TANNABE
Moderna Therapeutics
MULTICARE PHARMACEUTICALS
Orchard Therapeutics
Otsuka America Pharmaceuticals, Inc
OVID THERAPEUTICS
Pfizer, Inc.
PHARMACEUTICAL RESEARCH AND MANUFACTURING OF AMERICA
Prevention Genetics
PROMETIC LIFE SCIENCES
Recordati Rare Diseases
Regeneron Pharmaceuticals
REGENX BIO
RETROPHIN, INC
Rhythm Pharmaceutical
RIGEL PHARMACEUTICALS
Rocket Pharmacueticals
SAREPTA THERAPEUTICS
Shire Human Genetic Therapies
SOBI, INC
Soligenix, Inc.
SPARK THERAPEUTICS
STEALTH BioTherapeutics Inc
STRONGBRIDGE BIOPHARMA
SUCAMPO
SYROS PHARMACEUTICALS
Teva Pharmaceuticals
THETIS PHARMACEUTICALS LLC
Vertex Pharmaceuticals Inc.
VITAL THERAPIES, INC
WALGREENS CO
ZAFGEN, INC
Zealand Pharma
ZOGENIX, INC.

NORD ORGANIZATION MEMBERS

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

NORD ORGANIZATION MEMBERS (CONTINUED)

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
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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National Organization for Rare Disorders

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