2017 ANNUAL REPORT

Alone we are rare. Together we are strong.
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 30 million Americans – at least half of whom are children. Holding hands, they would circle the globe 1.5 times.

2017 GROWTH:

271 Member Organizations
84 Corporate Council Members
3,666 Rare Action Network® Members and 28 State Ambassadors
459 Students 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 for all patients to the diagnostics and therapies that will extend and improve their 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 made possible 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 advancing researching 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
Patients and families battle rare diseases on multiple fronts: medically, financially, physically and emotionally. With programs of advocacy, research, education and financial assistance, NORD’s help is always just one click or phone call away.

**KEY FACTS FROM 2017:**

- 90¢ of every dollar donated went directly to patients through assistance programs.
- 8.6M people and organizations reached through NORD’s programs.
- 14.2M page views on our website.
- 150k phone calls answered in our call center.
- 19 states passed new laws because of our advocacy efforts.
- 510 events that we hosted or attended brought people together for rare diseases.
- 3.6k people became effective advocates with training offered through Rare Action Network®.
- 91B earned media impressions generated publicity for our cause.

NORD’s expertise and resources were sought out and shared by the numerous media outlets, including but not limited to:

- abc NEWS
- TODAY
- CNBC
- CNN
- msn
- People
- TIME
- THE HILL
- npr
- POLITICO
- The New York Times
- The Washington Post
Since 1987, NORD has been helping children and families gain access to specialized medical care they could not otherwise afford.

Relied on by patients, FDA, NIH, and other government officials, our RareCare™ programs provide patients with 360° support.

8,337 PATIENTS

$31.7M in aid given directly to patients

$6.6M to pay for medical expenses not covered by insurance

$25M to cover insurance premium and co-pay expenses

1,265 PATIENTS

6,429 PATIENTS

1,265 WERE CHILDREN

23 PEOPLE RECEIVED DIAGNOSES

543 RECEIVED FREE MEDICATION

in all 50 states, D.C., Guam and Puerto Rico

“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, a patient’s father thanking NORD for helping him and his son

Thank You

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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
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Paroxysmal Nocturnal Hemoglobinuria (PNH)
Periodic Paralysis
Phenylketonuria (PKU)
Seizures Associated with LGS
Short Bowel Syndrome
Spasmodic Torticollis
Tardive Dyskinesia
Urea Cycle Disorders
Ulcerative Colitis

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
ADVOCACY IN ACTION

Working hand in hand with patients, we drive policy change - through grassroots campaigns in NORD’s advocacy arm, the Rare Action Network®. 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...

- Hosted 250 meetings
- 35 with the FDA and the NIH
- 177 legislative events in 38 states
- Took action on 123 bills in 45 states
- Recruited more than 3.6K new members!

NORD’S Policy Team:

- Recruited and trained 28 ambassadors
- Launched multiple days of action
- Hosted 7 advocacy training workshops
- Sent out 124 email blasts regarding policy alerts and activities

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
STRONGER TOGETHER

We work with our members and many 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)
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.

“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% employ nearly 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 advance 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 natural history registry studies. This helps better equip them to engage with their respective patient communities and to drive participation in research.

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)

More than 6,000 participants around the world contributed data to IAMRARE studies

16 active IAMRARE studies, with more under development

9 new IAMRARE studies launched

1 IAMRARE study discovered a new mechanism for the disease’s gene!

“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

Thank You

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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 cited NORD’s funding support.

10 events and conferences featured NORD presenting on the state of rare disease research.

RESEARCH GRANT HIGHLIGHTS:

ALVEOLAR CAPILLARY DYSPLASIA WITH MISALIGNMENT OF THE PULMONARY VEINS (ACD/MPV)
(approximately 200 cases reported)

• Arun Pradhan, PhD, Cincinnati Children’s Hospital Medical Center

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.

EVERYONE has the power to change someone’s life...

...whether you have 5 YEARS or 5 MINUTES

OUR IMPACT IN 2017:

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

- **10.3 million views**, up by 23% since 2016
- **More than 200** new and updated reports added to the website
- **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. And 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**
Our events provide educational content for a broad audience representing all stakeholder groups within the rare disease community.

**700 participants** attended the 2017 NORD Summit

**Invited** to speak 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

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

**The NORD Rare Disease and Orphan Drug Rare Summit** was our largest conference to date, with more than 60 research posters and 60 Lunch & Learn Roundtable discussions. We exchanged ideas, made personal connections and planned future collaborations.

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.
**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 New York City Marathon participant

**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 the TCS New York City Marathon, Boston Marathon, Providence Full- and Half-Marathons, Eversource Hartford Marathon, and our first 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 and generated 4,300 media articles to raise awareness.

**NORD’s Rare Action Network (RAN)**, serves as a broad spectrum of stakeholders ranging from patients and their families, caregivers and friends to researchers in industry and academia, as well as medical professionals. Working on both state and national levels, RAN filters information to engage rare communities to take action.
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 of 2018, 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.

We further collaborated with the international community by serving as a member of the Council of National Alliances for the global Rare Disease Day® campaign. To learn more about the impact of Rare Disease Day in the United States, see the Community Events and Awareness section.

OUR IMPACT IN 2017:

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 250 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research and patient services.
THANK YOU! ★ 2,315 new donors in 2017

We have been committed to our mission of helping rare diseases patients for over three decades. We could not do it without the generous support of our donors, and we are honored to have your trust and support.

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Cure SMA
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International FPIES Association
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International Pemphigus & Pemphigoid Foundation (IPPF)
International Rett Syndrome Foundation [Cure Rett]
International MPS Society
International WAGR Syndrome Foundation
International WAGR Syndrome Foundation (Cure Rett)
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Indian Organization for Rare Diseases
International FOP Association, Inc.
Fibrodysplasia Ossificatio Progressive
International Foundation for CDKL5 Research
International FPIES Association
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International  FOP Association, Inc.
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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
Outy Foundation
OMSLife Foundation
Organic Acidemia Association
Over the Rainbow Syndrome Network Foundation
Paramus Neurological Group
Parent Project Muscular Dystrophy
Parkinson’s Disease Foundation, Inc.
Phelan-McDermid Syndrome Foundation
Pitt Hopkins Research Foundation
Pituitary Network Association (PNA)
Pitt Hopkins Research Foundation
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
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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.
Sofa Sees Hope
Soft Bites, 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 Foundation
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)
VHIL Alliance
Williams Syndrome Association
Wilson Disease Association
Worldwide Syringomyelia & Chiari Task Force Inc.
XLH Network, Inc.
Xtraordinary Joy