NORD®
National Organization for Rare Disorders

2018

NORD 2018 Annual Report

Celebrating Our 35th Anniversary

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Working to improve the lives of people living with rare diseases and their families as they travel on the patient odyssey has long been at the heart of NORD's mission. We strive to provide vital services, support and information every step of the way to the millions of Americans affected by the more than 7,000 rare diseases currently identified by the National Institutes of Health. But we don't operate in a vacuum; it truly does "take a village" to foster change.

2018 was a year of milestones, as we marked the 35th anniversary of the groundbreaking Orphan Drug Act as well as NORD's 35 years of service. We have seen major advances on the treatment front, with 47 FDA orphan approvals and 199 designations granted in 2018. While there is much more work to be done, with statistics like these, it's clear that the pace of progress is accelerating, and it's with those directly affected often leading the way. Patients and caregivers are helping to build serious momentum in new and different ways, such as with patient-reported outcomes and real-world evidence. In fact, early this year, NORD and the FDA announced a memorandum of understanding for listening sessions that have since begun connecting patients and reviewers in real time, allowing for valuable firsthand input. It's for good reason that the 2018 Rare Diseases and Orphan Products Breakthrough Summit, NORD's largest and most well attended to date, was themed "A New Era of Patient-Focused Innovation."

It is only by working with people living with rare diseases, patient organizations, advocates, legislators and other supporters that we can truly make a difference. And so we continue to do just that. NORD's motto of "Alone we are rare. Together we are strong." has never been more true. We thank you for your support and look forward to continuing our work, together, in hopes of building an enduring era of hope.

Sincerely,

Peter L. Saltonstall | *President and CEO*

2018 HIGHLIGHTS

At NORD, we are incredibly grateful to our generous donors and supporters. Because of you, we are making a difference in the rare disease community. Our work touches many, including the more than 25 million Americans impacted by rare diseases, with programs spanning from advocacy to research and beyond.

NORD 35TH ANNIVERSARY

35 years of service to the rare disease community

NORD and the FDA established listening sessions connection rare disease patients and reviewers in real time, allowing for valuable firsthand input.

RARE CARE®

7,459 people received assistance to help with rare disease-related medical expenses that they could not otherwise afford.

150,000+ phone calls from patients and caregivers answered in our call center.

12.7 MILLION website visits to read NORD's online rare disease reports, which offer information about rare diseases written in easy-to-understand language.

280 patient organizations joined or renewed as NORD Member Organizations.

550 patient stories shared to raise awareness.

$7+ MILLION funded in research through grants since 1989.

800+ attendees at the NORD Rare Summit

Recognized as an outstanding charity by:

Charity Navigator · GuideStar 2017 Top-Rated Nonprofit · GuideStar Transparency 2018 Gold

WORKING TOGETHER TO ACCELERATE RESEARCH

Whether it's building the infrastructure to conduct research around the globe or using new techniques to chart the progression of a disease, each of our studies begins with a simple idea: to better understand rare diseases to help patients. This year we introduced a new model of engagement to advance NORD's registry program, IAMRARE™, to the next level. Now patient groups, working with researchers spanning industry, academia and medicine, can establish sub-studies to propel research forward.

DID YOU KNOW? Our 32 patient registries representing different rare diseases have collected 40,000+ lifetime survey responses, with more added each day.

Making Breakthroughs

Collaborating with the Global Prader-Willi Syndrome Registry and biopharma companies, our sub-study program is helping to inform potential new treatments and life-saving clinical trials. Together we are studying behavioral changes, examining de-identified data to understand medication usage, and following serious medical events over a four-year period.

GRANTING HOPE FOR RARE DISEASES

We are committed to helping every person with a rare disease. Our research grant portfolio—more than $7 million since 1989—led to two FDA-approved therapies, numerous publications in medical journals and, in many cases, has served as the only source of funding for the study of particular rare diseases.

What's more is much of this funding comes from donations made by families and friends of people affected by rare diseases, or by individuals living with rare diseases themselves. This past year's investment demonstrated our long-running commitment to empowering patients to advance research, with $375,000 distributed to qualified researchers working to develop new diagnostics or treatments.

TESTIMONIALS

"It allowed us to generate the first publication on this condition in a major medical journal, which helped make doctors better able to diagnose and treat it, and made patients more aware of it."

▶ **Anne Louise Oaklander, Associate Professor Neurology, Harvard Medical School**

"This grant allowed us to do a study that we could not have done and provided then unknown information about the condition now called vascular EDS."

▶ **Peter Byers, Professor, Departments of Pathology and Medicine (Medical Genetics), University of Washington**

"NORD funding kept our laboratory funded and the investment in our work has been met more than 10-fold with subsequent research finding from the NIH, industry and other foundations!"

▶ **Alison Moliterno, Associate Professor of Medicine, Johns Hopkins University School of Medicine**

Watch to see how the community relies on our research grant program – together we make a difference. **https://youtu.be/xbP8K7PKguY** (or scan QR code)

MAKING STRIDES AS A COMMUNITY

In recognition of the 7,000 identifed rare diseases and to mark NORD's 35th anniversary, we issued a challenge to rare disease advocates to walk, run or bike 7,000 miles collectively throughout the month of February. The 7,000 Mile Rare Movement raised over $36,000 towards NORD's programs and services.

Running for Rare®, NORD's charity marathon running team, added five events to its roster in 2018 for a total of 12 races, representing full and half marathons across the US. With 50 runners and 54 community partners, we have a growing team making moves and raising awareness and funds in support of the rare disease community, with more than $125,000 raised this year.

$125,000+

raised in 2018 through this movement

PROVIDING HELP WHEN IT'S NEEDED MOST

Dealing with the financial and logistical realities of living with a rare disease can be frustrating and overwhelming. NORD provides services to people living with rare diseases in all 50 states, Washington, DC, Puerto Rico and Guam. This year, in addition to its existing programs, NORD launched three new RareCare® Patient Assistance Programs:

CTTL - Boston Children's Hospital (BCH) sponsored Randomized Double-Blind Controlled Trial of Everolimus in Children and Adolescents with PTEN Mutations

Bile Acid Synthesis Disorders/ Zellweger Spectrum Disorders Copay and Medical Assistance Program

Neurotrophic Keratitis Copay Assistance Program

Owen and his service dog, Dandelion

TESTIMONIALS

"Owen gets weekly injections that are helping him preserve muscle and lung function. However, maintaining his access to this drug is a difficult process. Your help means the world to us and is a huge relief on our stress level."

▶ Christine and Scott, Owen's parents

"Since my wife went down to working part-time to be able to stay home with our daughter, our finances have certainly been tighter than they previously were. The assistance I receive from NORD to cover my [medication] has been incredible in our lives."

▶ Ryan, rare disease patient

95%

of the people we helped live below the Federal Poverty Line and could not otherwise access necessary medical treatment.

ANSWERING YOUR MOST URGENT QUESTIONS

More than 80% of rare diseases are genetic. In light of treatments recently approved by the Food and Drug Administration, gene therapy is a topic at the forefront of many rare disease conversations. This year we released a video to address patients' hopes, questions and concerns, across many different diseases. Watched 12,000+ times, "Gene Therapy: Your Questions Answered" ignited conversation with hundreds of comments and shares across social media.

TESTIMONIALS

"The NORD video is an excellent resource and will help a great deal of patients and patient advocates."

▶ David M. Barrett, JD, MS, Executive Director, American Society of Gene & Cell Therapy

NORD makes it easier for rare disease patients to understand the latest medical advancements that might help their condition.

Watch the video here. https://www.youtube.com/watch?v=5ChXI6cSQs0 (or scan QR code)

PROGRESS THROUGH POLICY

Throughout 2018, volunteers and staff of NORD's grassroots advocacy arm, the Rare Action Network® (RAN), advanced our goals to unite, educate and drive progress for people with rare diseases. We helped pass new laws in 12 states (plus near passage in four states), hosted 10 advocacy training workshops and released our annual RAN State Report Card®—the only report of its kind—evaluating each state's progress on reducing the burden of rare diseases, and where to make future improvements. As a community we celebrated a major milestone when we reached 5,000 RAN members, representing all 50 states and the District of Columbia.

TESTIMONIALS

"As an advocate, I like giving a voice to those who cannot give a voice to themselves. I measure success as something as simple as connecting a rare disease patient with a support group so that they can speak to others and don't feel so isolated. Or it could be something as big as getting legislation that benefits the entire rare disease community and my state."

▶ **Deborah Skolaski, RAN State Ambassador for Texas**

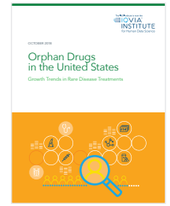
We helped pass new laws in

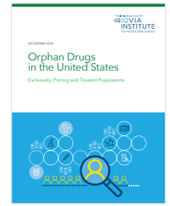
12

states

Turbo-Charged Advocacy

Two studies released in 2018 through NORD's RareInsights™ platform helped boost advocacy efforts with new data showing the true cost of rare diseases to the US health care system.





Get inspired: Watch our advocates in action in this special series produced as part of NORD's 35th Anniversary celebration. https://www.youtube.com/watch?v=LqzWEnwBrP4&list=PLMmYBWQscoiEei4xEtzj74GxA2vvC-WP2 (or scan QR code)

STRONGER WHEN WE COME TOGETHER

RARE CANCER COALITION

Many people may not realize that there are hundreds of types of rare cancers, including all pediatric cancers. This year we formally united 21 patient groups to form the Rare Cancer Coalition—the first of its kind in the United States—and established a presence at ASCO, the largest oncology conference in the world, to help raise awareness and meet the needs of rare cancer patients. The group provides opportunities for information sharing, networking, collaborative educational programs and a presence at major conferences, bringing hope and results to patients.

Building hope and progress for rare cancer patients

TRAINING TOMORROW'S LEADERS

What does the future look like for rare disease patients and clinicians? We think it will be brighter than ever. This year we teamed up with Healthline Media, the second largest health information website in the United States, to create a stronger and healthier world by assisting and empowering college students who have demonstrated involvement in the advancement of rare and/or chronic disease. The 2018 Healthline and NORD Stronger Scholarship program awarded four students each with a $5,000 scholarship. We are excited to see what they will accomplish next!

Matthew Pearl

When Westminster College senior Matthew Pearl was four years-old, he received a diagnosis of Fanconi Anemia, a rare blood disorder that leads to bone marrow failure. At nine, he underwent a bone marrow transplant and the following year, he was invited to speak at a scientific symposium held by the Fanconi Anemia Research Fund. In the years since, Matthew has gone on to speak at various functions and scientific symposiums for the National Marrow Donor Foundation and Fanconi Anemia Research Fund. As he pursues a self-designed major of leadership consulting with an emphasis in nonprofit management, Matthew plans to speak to both patients and researchers in Germany in 2019. Matthew has been extensively involved in fundraising, and he and his family have been instrumental in adding more than 100,000 new donors to the National Marrow Donor Program's Registry.

Simran Handa

Simran Handa is a senior at Lewis & Clark College working towards her degree in biochemistry and molecular biology. She is also a caregiver for her teenage sister, who lives with ulcerative colitis, a chronic inflammatory bowel disease. Simran's research at Lewis & Clark investigates the biology behind two rare genetic diseases, Hermansky-Pudlak syndrome and Chediak-Higashi syndrome.

Sanika Rane

Upon entering the direct medical program at Rice University, junior Sanika Rane began working towards a bachelor of science degree in kinesiology and a minor in global health technologies. As part of her minor, she and her peers were assigned a semester-long project to design a less expensive alternative to help treat gastroschisis, a rare congenital birth defect that occurs when a baby's intestines form outside of the body. While the survival rate of babies born with gastroschisis in the United States is approximately 90%, the survival rate in developing nations is minimal. The team presented their work at Rice University's annual George R. Brown Engineering Design Showcase in April 2018, where they were honored with the top award, Excellence in Engineering: 1st Place Design.

Daniel Stanley

Daniel Stanley is a junior at the University of Wyoming and is pursuing a double major in biology and environment and natural resources. His younger sister, Emily, had her first seizure when she was 6 months old, but didn't receive a diagnosis of Dravet syndrome—a rare, lifelong form of epilepsy—until she was 5. Watching Emily manage her condition has inspired Daniel's desire to pursue a career in research. He won funding through a competitive IDEA Network Biomedical Research Excellence (INBRE) Fellowship program from the National Institutes of Health (NIH), which has contributed to his ongoing epilepsy research. Daniel is also active with the Dravet Syndrome Foundation, and at a recent conference, he and his older sister participated in a panel made up of siblings of Dravet patients.

IN MEMORIAM

ROBERT M. CAMPBELL, JR., MD

This year, the rare disease community lost one of its most dedicated and accomplished supporters. We were deeply saddened to learn of the passing of Dr. Robert M. Campbell. A great friend to NORD, Dr. Campbell served on our Medical Advisory Committee for many years, donating countless hours as a scientific adviser and reviewer of research proposals.

He was also supportive of NORD's educational programs, reviewing information for patients and serving as a chapter editor for The NORD Guide to Rare Disorders, a textbook on rare diseases for pediatricians and family physicians that NORD published with Lippincott, Williams & Wilkins in 2003.

He was not only involved in the quality of life for rare disease patients through his work as a researcher and clinician, but Dr. Campbell also supported NORD's advocacy on pediatric medical device development on many occasions over the years. In 2010, his advocacy on behalf of children with rare medical conditions was recognized with a Congressional Resolution (H.Res.1499).

Dr. Campbell was best known for his groundbreaking invention–the Vertical Expandable Prosthetic Titanium Rib (VEPTR)–a medical device that has been life-saving for many children with Thoracic Insufficiency Syndrome. Due to his incredible accomplishments and dedication to the rare disease community, NORD proudly honored Dr. Campbell with the 2018 Lifetime Achievement Award during our 35th Anniversary Celebration presenting the Rare Impact Awards on May 17. During his remarks, the audience was moved by his words of encouragement and laughed with him as he recalled joyful memories of his years working with the community.

Dr. Campbell will be missed by his family, friends, colleagues, patients and by all of those whose lives he touched.

Dr. Campbell's Legacy: Watch his Lifetime Achievement Remarks. https://youtu.be/_kEz6ldw0gM?t=2h16m21s (or scan QR code)

VOICES OF NORD

Launched originally as a telephone and mail information clearinghouse 35 years ago, we have grown and added many services over the years. We are honored to come to work each day to help make a difference for rare disease patients.

TESTIMONIALS

"In 1995 I lost my mom to cancer. After watching all the VNA staff take care of her and seeing how sad they all were after her passing, I decided I wanted to be able to help people. I applied at NORD and was hired. I have enjoyed my job here because I can go home each day knowing that I've helped someone."

▶ **Nancy Nadig, Administrative Assistant (23 years)**

"What I most appreciate about working at NORD is that my team and I are able to make a real difference in people's lives. We are proud to provide help and support directly to patients and their families when they need it most, and are humbled by their appreciation."

▶ **Jill Pollander, Director of Patient Services (1 year)**

"My favorite part of working at NORD is meeting the amazing patients, caregivers and supporters of the rare community! I'm always amazed by the patients and families who struggle daily but don't ever let their disease define them. Inspiration comes from these people and I'm lucky to work with them every day."

▶ **Kristen Angell, Associate Director of Advocacy (5 years)**

"I'm so fortunate that so many of the core components of what I love about research—building sustained relationships, partnering with communities, designing with creativity, and creating meaningful impact—are embodied by my position at NORD, where I have the opportunity to help shape the scientific vision for the organization."

▶ **Vanessa Boulanger, Director of Research (2 years)**

"I enjoy working at NORD because it gives me the opportunity to help everyone in the organization, giving them more understanding and comfort with today's technology. I find it meaningful to help update their technology and find new ways to improve their processes—and, ultimately, NORD's efficiency."

▶ **David Romero, Systems Administrator (3 years)**

"I believe in the mission, first and foremost. The education, exposure and involvement with the rare disease community and the development of our department is something I've never had in any previous workplace. Of course, the fact that we have a direct positive impact on our patients' lives is pretty motivational, too!"

▶ **Noël Williams, Patient Service Specialist (6 years)**

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Glut1 Deficiency Foundation
Gorlin Syndrome Alliance
Gut Check Foundation
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International Pemphigus & Pemphigoid Foundation
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International Sacral Agenesis/Caudal Regression Association
International WAGR Syndrome Association
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Myelodysplastic Syndromes Foundation, Inc
Myocarditis Foundation
Myositis Association
Myotonic Dystrophy Foundation
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National Foundation for Ectodermal Dysplasias
National Health Council
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National Tay-Sachs & Allied Diseases Association
National Urea Cycle Disorders Foundation
NBIA Disorders Association
NephCure Kidney International
Neuroendocrine Tumor Research Foundation
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Neurofibromatosis Northeast
NGLY1.org
NICER Foundation
NTM Info & Research, Inc
Ocular Melanoma Foundation
Oley Foundation
OMSLife Foundation
Organic Acidemia Association
Organization for Rare Diseases India
Osteogenesis Imperfecta Foundation
Parent Project Muscular Dystrophy
Parent to Parent New Zealand, Inc
Parents of Infants and Children with Kernicterus
PHACE Syndrome Community
Phelan-McDermid Syndrome Foundation
Pheo Para Alliance
Pituitary Network Association
PKD Foundation
Platelet Disorder Support Association
Prader-Willi Syndrome Association (USA)
Primary Ciliary Dyskinesia Foundation
PRISMS (Parents & Researchers Interested in Smith-Magenis Syndrome)
PSC Partners Seeking a Cure
PTEN Hamartoma Tumor Syndrome Foundation
Pulmonary Fibrosis Foundation
Pulmonary Hypertension Association
PURA Syndrome Foundation
Rare & Undiagnosed Network
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Connecticut office: 55 Kenosia Avenue, Danbury, CT 06810 | 203-744-0100
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Massachusetts office: 1900 Crown Colony Drive, Quincy, MA 02169 | 617-249-7300

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