



ANNUAL REPORT 2014



*"Because of NORD and the assistance you provided, I can forget that [financial] stress and concentrate on the continuing battle with the cancer. Thank you NORD for providing some peace."*

*- Patient in NORD's Assistance Program*

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### **Dear NORD Members and Friends:**

Every day in America, 30 million people wake up to fight to the battle with rare diseases. Sadly, the vast majority are children, and most of the conditions are life-altering and have no treatment.

We know from our decades of experience helping patients, caregivers and families how overwhelming a rare disease diagnosis can be, and how distressing it is to go without a diagnosis. For many individuals, living with a rare disease means living in a race against time. Since the beginning, when NORD was founded by parents of children with rare diseases and created the collective voice of rare, our team has worked each day with the determination to make a difference and to keep patients at the core of everything that we do.

In 2014, we launched several ground breaking programs in advocacy, research, organizational capacity building and education. All of our programs grow out of a desire to help patients in the medical setting and beyond, to raise the voice for the courageous people with unmet medical needs, and to pave the way for scientific developments that can help make the world a better place for those in the rare community.

Our team is inspired by the patients and families we serve, and by each person who joins the fight against rare diseases through their professional or volunteer efforts. We want to thank you for making the 2014 programs possible, and for helping us to achieve our goals. There is more work to be done in 2015 and beyond, and we look forward to working with you.

As Sincerely,

Peter L. Saltonstall, President and CEO

**NORD Puts Patients in Control of Research: Natural History Studies**

With significant input from patients, organizations, and other experts in the field, NORD launched a ground breaking patient-powered registry platform to help transform how patients and caregivers shape and inform research into their disease. Because each disease affects people differently, scientists must study many cases in order to develop a thorough understanding of its natural history. Our platform supports the safe, secure collection of data from people around the world, thereby tackling the inherent problem in rare disease: the lack of understanding on how diseases progress over time due to relatively low cases to study. *(Read more on page 4)*



*“FDA Senior Officials praised NORD’s platform as an exciting new tool researchers are using to study rare diseases.”*

**RareAction<sup>SM</sup> Leads Rare Disease Advocacy at the Local Level**

With the adoption of the Affordable Care Act (ACA), progress for people with rare diseases is increasingly dependent upon state policies concerning medical care. This requires activity at the local level to increase awareness and understanding of the challenges and needs of the patients. NORD launched the Rare Action Network<sup>SM</sup> (RareAction) to serve as a broad spectrum of individuals interested in improving the lives of people affected by rare diseases.

Dedicated advocates from across the country have come together with the support of passionate State Ambassadors and the expertise of local patient organizations to ensure the needs of the rare disease community are being adequately met.

Learn More at [www.rarediseases.org/rareaction](http://www.rarediseases.org/rareaction)



**Helping Organizations to Build, Grow and Evolve**

NORD supports the collective and individual needs of more than 230 rare disease organizations and the patient communities that they serve. As the largest rare disease organization in the U.S., our members turn to us for guidance, mentorship and support in advocacy, disease education, research, communications strategies, and professional development. Our services allow leaders to stay connected and up-to-date on critical information, and to meet immediate needs, while strategically planning for future success.

In 2014, we hosted two regional meetings in California and Washington, D.C. to educate and connect our members, in addition to helping over 75 organizations attend NORD’s Annual Summit.

**Membership Grew with the Addition of 12 New Organizations:**

- Answering TTP Foundation
- Cholangiocarcinoma Foundation
- Cluster Headache Support Group
- Gut Check Foundation
- Hermansky-Pudlak Syndrome Network
- Indian Organization for Rare Disorders
- Joshua Frase Foundation.
- NTM Info and Research, Inc.
- OXL Network
- PKD Foundation
- RASopathies Network USA
- Tarlov Cyst Foundation

**Rare Diseases and Orphan Products Breakthrough Summit a Huge Success!**

This year we hosted the Rare Diseases and Orphan Products Breakthrough Summit in Washington, D.C. This was the largest event in rare diseases, bringing together nearly 500 stakeholders! We welcomed over 60 speakers to address and facilitate conversations on collaboration the rare patient’s experience, targeting new treatments, technological tools to support data for research, and more. The conference was highly acclaimed by NORD members and attendees.

*“Incredibly valuable meeting to connect stakeholders, share information and provide inspiration and motivation.”*  
– Genzyme

*“Excellent networking opportunity... The number of patient advocates in attendance speaks volumes for your organization’s commitment to meaningful patient engagement.”*  
– Canadian Organization for Rare Disorders

**Addressing Medical Education in Diagnosing and Treating Rare Diseases**

NORD entered into a strategic partnership agreement with Frontline Medical Communications to collaborate and develop innovative and custom educational programs for healthcare providers (HCPs). Frontline Medical Communications is the leader in medical communications, reaching 1.2 million physicians and HCPs through 30 print publications, 35 active web sites and 15 live events annually.



By harnessing the expertise of each of NORD’s member organizations, we will seek to meet the urgent, unmet medical needs of patients. On average an individual will see seven physicians over 5-10 years before they receive an accurate diagnosis. The key stumbling block is the lack of awareness of understanding among healthcare providers. Members of NORD and their medical advisors are working with us to provide disease-specific information for our multi-channel approach.



**Making an Impact on Rare Disease Day<sup>®</sup>**

NORD was thrilled to once again serve as the U.S. host and sponsor of this international campaign, in partnership with EURORDIS and the global community. We joined efforts with 83 countries and generated new levels of awareness for rare diseases using the official 2014 slogan, “Join together for better care.” NORD reached 268 million people through its media campaign, engaged in 12 State House Events and with advocates in 37 states, co-hosted the first ABC News tweetchat with Dr. Richard Besser on ‘Conquering Rare Diseases’, launched a designated Rare Disease Day U.S. Twitter profile, and received 228 handprints across America<sup>®</sup> submissions.

**Supporting Patients: Access to Education, Treatment and Care**

Each year, our patient services team prepares to help nearly 4,000 patients through our assistance programs. Through donations and support, NORD assists patients obtain life-saving or sustaining medication that they could not otherwise afford. These programs provide medication, financial assistance with insurance premiums and co-pays, diagnostic testing assistance, and travel and lodging assistance for clinical trials or consultation with disease specialists.

**In 2014, we helped 3,988 access life-saving treatment and care**

Our team traveled across the country to host 36 meetings for patients across five different diseases to connect, educate and share new information and resources with them. Combined, our meetings brought together over 800 expert physicians, organizations, licensed facilitators, patients and families.

## PATIENT-CENTERED NATURAL HISTORY PLATFORM

“Since VHL patients battle a series of tumors throughout their lives, they are an ideal population for studying tumor growth and identifying ways in which a patient’s lifestyle or environment may affect the progression of the disease,” says Ilene Sussman, executive director of the VHL Alliance, on developing the first of many registries on NORD’s Natural History stories platform.

Von Hippel-Lindau (VHL), is a genetic form of cancer in which patients battle a series of tumors throughout their lives. And, while von Hippel-Lindau disease is rare, the VHL gene is involved in many other forms of cancer. This project, the Cancer in our Genes International Patient Databank (CGIP), is a promising tool to collect data from patients with several rare forms of genetic cancer, and could ultimately influence the treatment of many other types of cancer including more common forms.

“The CGIP will provide a complete ‘picture’ of each patient’s experience with these diseases, making it possible to assess the effects of diet, exercise, environment and other variables,” said Sussman. “It will also provide a safe platform for patients and researchers to connect and confidentially exchange data in order to accelerate treatments and a cure.”

As the registry program at NORD grows, our hope is for patients to collect high-quality data for disease-specific research that can lead to cures, and collectively enable us to study across diseases and make informative connections.

*“Having such a resource can yield vital information about biomarkers, demographic, genetic, and environmental factors that correlate with the disease, as well as helping to identify patient subpopulations with different characteristics and effects.”*

– Margaret Hamburg, M.D., former FDA Commissioner



**NATURAL HISTORY IS THE SCIENTIFIC TERM FOR HOW A DISEASE WOULD PROGRESS IF NO TREATMENT WAS GIVEN**

## FUNDING BASIC AND TRANSLATIONAL RESEARCH IN RARE DISEASES

NORD awards research grants to advance safe, effective new treatment options for patients and their families. These new grants uphold our time-honored tradition of funding basic and translational research in rare diseases.

The following awards were provided in 2014:

### **For the study of Alveolar Capillary Dysplasia with Misalignment of Pulmonary Veins (ACDMPV):**

- Csaba Galambos, Children's Hospital Colorado and University of Colorado Denver
- Przemyslaw Szafranski, Baylor College of Medicine

### **For the study of Autoimmune Polyglandular Syndrome Type 1 (APS Type 1):**

- Mark Anderson, University of California San Francisco Diabetes Center

### **For the study of Primary Orthostatic Tremor:**

- Aparna Wagle Shukla, MD, University of Florida

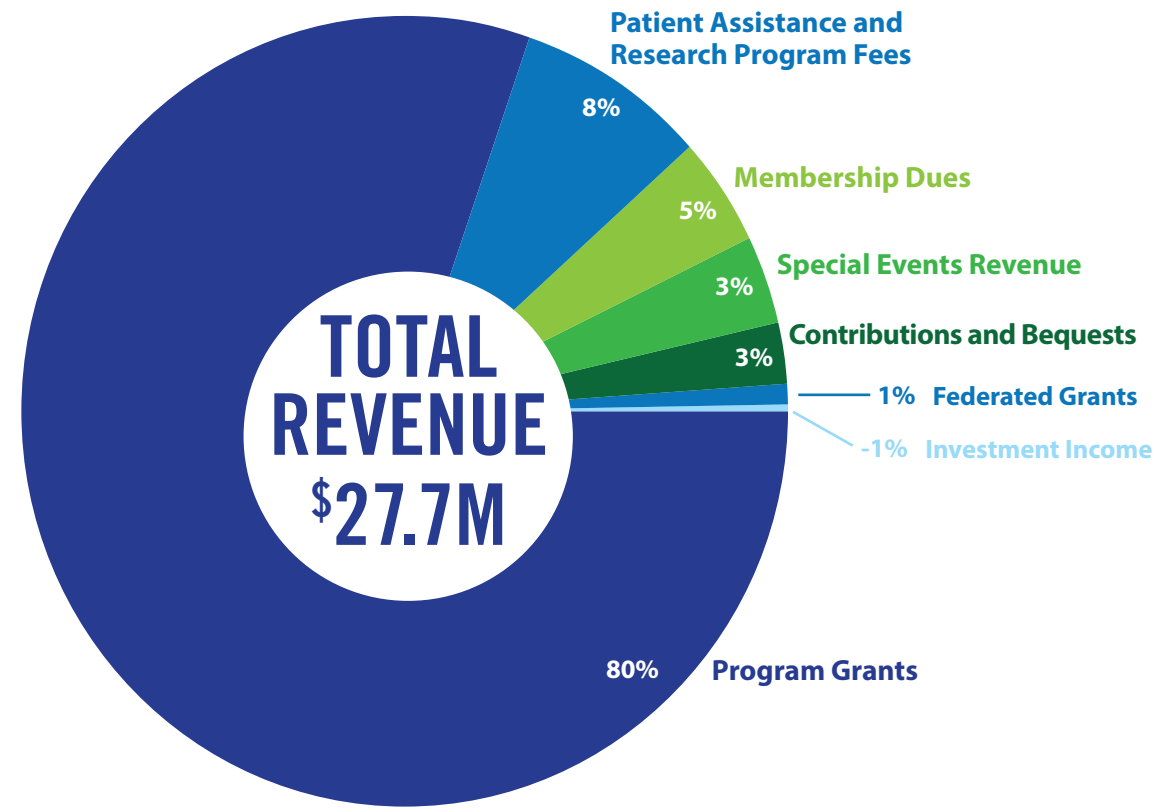
### **For the study of Pseudomyxoma peritonei (PMP):**

- Steven Katz and Cherif Boutros, Roger Williams Medical Center and University of Maryland School of Medicine
- Venkatesh Govindarajan, Creighton University

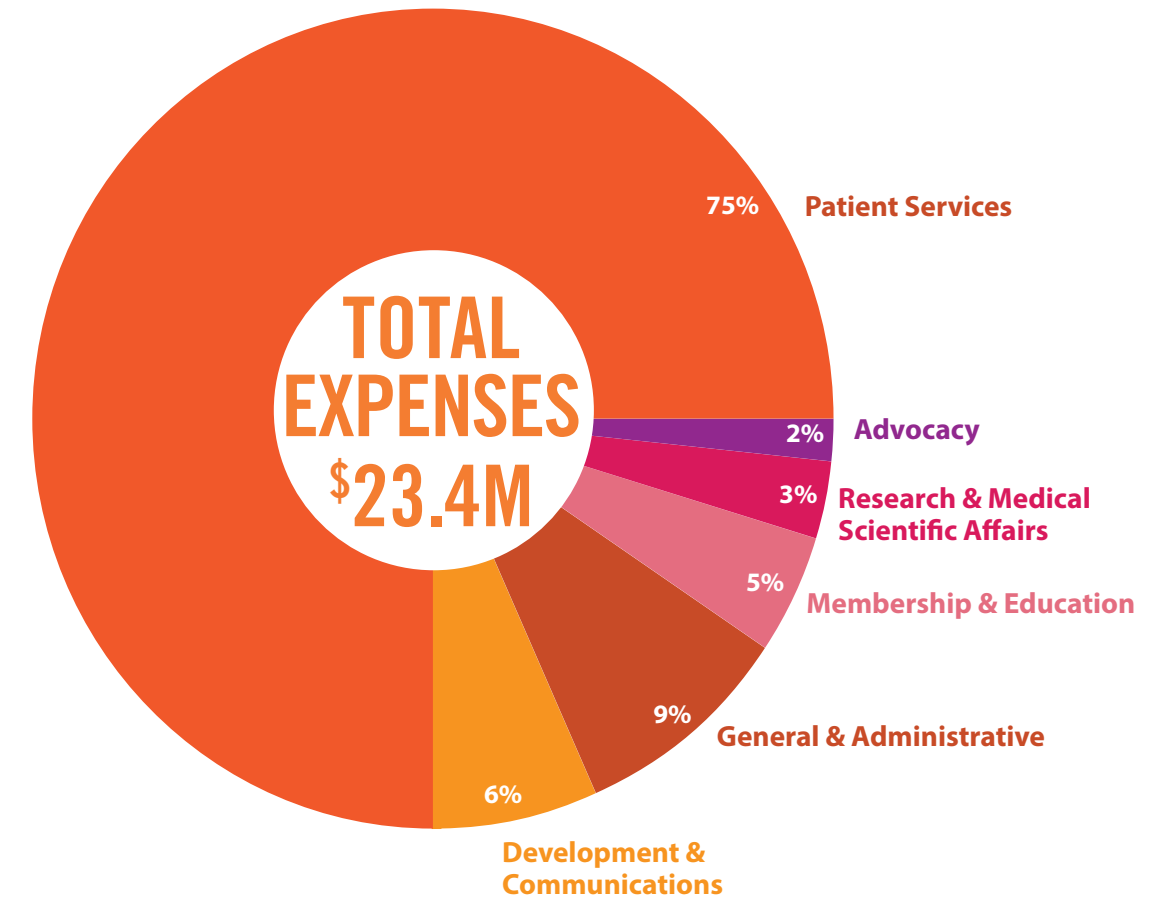
Throughout its 26 year history, **NORD's research grant program has administered more than 150 grants.** Two FDA-approvals for a medical device and treatment were made possible through NORD grants.



## 2014 FINANCIAL POSITION



Program Grants . . . . .	\$22,343,734
Patient Assistance and Research Program Fees . . . . .	\$2,243,559
Membership Dues . . . . .	\$1,257,819
Special Events Revenue . . . . .	\$951,074
Contributions and Bequests . . . . .	\$741,884
Royalties and Other . . . . .	\$191,748
Federated Grants . . . . .	\$41,540
Investment Income . . . . .	\$20,445
<b>Total Revenue</b>	<b>\$27,791,803</b>



Patient Services . . . . .	\$17,625,423
Advocacy . . . . .	\$406,798
Research and Medical Scientific Affairs . . . . .	\$736,109
Membership and Education . . . . .	\$1,058,262
General and Administrative . . . . .	\$2,109,853
Development and Communications . . . . .	\$1,515,212
<b>Total Expenses</b>	<b>\$23,451,657</b>

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Morgan Vaughan (right)  
was diagnosed with Necrotizing  
Enterocolitis (NEC) at four days old.



**Alone we are rare. Together we are strong.**<sup>®</sup>

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