



Celebrating 30 Years  
of Advocacy for the  
Rare Disease Community





## Annual Report 2013

The Yang family helped organize a Rare Disease Day event at the University of Alabama at Birmingham and joined NORD's media campaign to promote awareness.

## Dear NORD Members and Friends:

As we celebrated the 30th anniversary of NORD and the Orphan Drug Act, we honored NORD's illustrious history and the pioneering individuals who established NORD and focused a national spotlight on the need for orphan drug legislation. Their dedicated and passionate advocacy has improved the lives of millions of Americans.

Our anniversary year was also a time to reassess the community's needs and challenges, and to renew our commitment to work toward a world in which every life is lived to the fullest and every patient has a treatment or cure.

The year began – poignantly – with accolades to actor Jack Klugman, who played such a pivotal role in the Orphan Drug Act and the history of NORD. Jack died at his home in California on December 24, 2012, and NORD issued a statement honoring his life that was widely quoted in the press.

Friends of NORD from its earliest days remember Jack as NORD's "Honorary Chairman" who had highlighted the need for the Orphan Drug Act in two episodes of his popular television show, *Quincy, M.E.*

Jack's death came just one week before a major event hosted by the Food and Drug Administration (FDA) commemorating the 30th anniversary of the Orphan Drug Act. A central theme of that event was the crucial role played by the ad hoc coalition that became NORD in enactment of the Orphan Drug Act.

Today, NORD continues to provide leadership to the rare disease community. As you read the following brief summary of NORD's 30th anniversary year, please be aware that these accomplishments were only possible because of the dedicated members and advocates who generously donated their time, expertise, and guidance to NORD. We thank, in particular, our board of directors, medical advisers, member organizations and Corporate Council for their ongoing and important support.

At this defining moment, when the science of rare diseases is advancing at an unparalleled pace, NORD remains dedicated – as it has been since 1983 – to improving the lives of the patients and families who so desperately need our help.

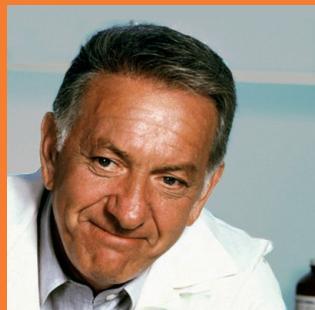
Sincerely,



Peter L. Saltonstall, President and CEO



E. Michael D. Scott, 2013 Board Chair



# Highlights of our 30th Anniversary Year

## Celebrating 30 Years of Progress



NORD's 30th anniversary began on a bittersweet note, with the death of actor Jack Klugman, an early champion of the Orphan Drug Act and NORD. Through his popular TV show, *Quincy M.E.*, Klugman focused national attention on the need for treatments for people with rare diseases. He took a personal interest in the establishment of NORD and continued to follow NORD's progress through its early years.

On January 4th, the 30th anniversary of the signing of the Orphan Drug Act by President Ronald Reagan, the FDA hosted an event featuring a moving video interview with NORD founder Abbey S. Meyers and remarks by current NORD President and CEO Peter L. Saltonstall. We hosted our own celebration in May with children and young adults presenting medals to heroes of the rare disease movement.

## Extending Our Voice Globally



NORD and the Japan Patients Association (JPA) formed a partnership in 2013 aimed at connecting rare disease patients in the two countries. The signing ceremony took place in Washington, DC, with CEO Peter L. Saltonstall representing NORD and Tateo Ito, CEO, representing JPA. Both emphasized the need for global partnership.



In signing the document, President Ito said the two organizations "would like to make use of our valuable knowledge and experiences for the mutual benefit of patients in both countries." Saltonstall added, "Global collaboration will drive progress toward new treatments, improved public policies and better lives for people with rare diseases."

## Mentoring our Members for Growth and Impact



A Sound Bite Can Save a Child's Life  
Victims of rare diseases are seeking help... 200 words at a time.

Helping our members grow and

accomplish their goals is at the heart of our

mission. In 2013, we significantly expanded member services to include a broad range of webinars, teleconferences and in-person regional meetings, all aimed at giving members an inside track on current issues and opportunities.

We also provided scholarships for 70 representatives from member organizations to attend NORD's Annual Breakthrough Summit on Rare Diseases and Orphan Products, a great opportunity to interact and network with researchers, government officials, and industry leaders. To help prepare these patient leaders for the Summit, we invited them to submit brief essays about living with a rare disease. NORD consolidated the riveting pieces into an op-ed on behalf of the community, which was published in *The Wall Street Journal*.

Membership continued to grow in 2013, with 13 new organizations joining the NORD community.

- Association for Creatine Deficiencies
- CADASIL Association
- Cutaneous Lymphoma Foundation
- Dup15 Alliance
- Genetic and Rare Disorders Organisation
- Global Foundation for Peroxisomal Disorders
- Mesothelioma Applied Research Foundation
- PCD/KS Foundation
- PRISMS - Parents and Researchers Interested in Smith-Magenis Syndrome
- Pulmonary Fibrosis Foundation
- Red Sanfillippo AC
- Rothmund-Thomson Syndrome Foundation
- SSADH Association

## Promoting Research, Innovation and Discovery



Since its infancy, NORD has promoted innovation in medical research, the development of diagnostics and treatments, and regulatory practices. In its 30th anniversary year, NORD was honored to have its president named to the board of directors for the first public-private partnership to advance medical device development. The Medical Device Innovation Consortium (MDIC), launched by the FDA, NIH, industry partners and NORD, will serve as a model for public-private partnerships in other areas.



We also awarded nine research grants in 2013 with a total value of \$255,000. These seed grants provide hope to patients by making possible research on rare diseases for which there are few other sources of funding. Over the years, our grants have contributed to research resulting in two life-saving products approved by the FDA.

## Bringing Rare Disease Day to Communities Across America



NORD organized a Handprints Across America campaign for Rare Disease Day to encourage grassroots participation and support. Hundreds of people from communities across the U.S. submitted brief messages and photos of themselves holding the Rare Disease Day logo.



While Rare Disease Day continues to reach an ever-broader international audience, we are increasing our focus on policy and advocacy activities within states and local communities. In 2013, NORD helped its partners and members host Rare Disease Day "State House Events" to educate legislators and other elected officials about how they can make a difference in the lives of rare disease patients.

## Advancing Diagnosis



NORD published three physician guides in 2013, on Gaucher disease, Pompe disease, and infantile spasms. Written by medical experts, these guides are free and available online at [www.nordphysicianguides.org](http://www.nordphysicianguides.org). The purpose is to promote early, accurate diagnosis and appropriate treatment for patients.

## Training Tomorrow's Advocates

"Having a child with a rare disease makes you feel helpless... powerless. At least, that's how I felt until I found NORD," says Kelli Foster. Kelli and her daughter, Brooke, both live with a rare disease known as mastocytosis.

Kelli attended a NORD conference a few years ago and found that she was greatly empowered by that experience. Connecting with senior officials from NIH and FDA, along with rare disease medical experts and pharmaceutical industry leaders, showed Kelli that she and her family were not alone but part of a community.

That experience gave Kelli the courage to become a more active and vocal advocate. And ultimately, she was able to help her own daughter and others within her patient organization access treatment that had previously not been available to them.

Brooke also benefited from her mother's awakening to advocacy. At a 2013 Rare Disease Day event in New Jersey to educate members of that state's legislature about rare disease challenges, Brooke gave a presentation that was one of the highlights of the event.

"The NORD conference taught me that every voice is important," says Kelli. And Brooke echoed that sentiment in a NORD video to encourage young advocates. "If you want to get involved," she said, "just DO it!"

“ If you want to get involved, just DO it! ”



A close-up photograph of a woman with short brown hair and glasses, speaking into a microphone. She is wearing a dark top. The background is blue.

**SINCE 1983  
NORD HAS  
PROVIDED  
LEADERSHIP  
AND PATIENT  
REPRESENTATION  
IN PUBLIC  
POLICY**

# Providing Lifesaving Patient Services

When Jon and Amanda Miller heard that NORD was hosting a Rare Disease Day event near their home in New Jersey, they decided to come and say thank you. That's because a NORD patient assistance program helped the Millers at a time of crisis in their lives.

Their son, Evan, was just three months old when he went into a medical crisis and was diagnosed with tyrosinemia type 1, a rare metabolic disease. The good news for the Millers was that there is an FDA-approved treatment for that disease.

The bad news was that the family did not have insurance that would cover the cost of the drug, and the hospital wouldn't release Evan without the medication that was his lifeline.

"It was very stressful," Amanda says. "It was bad enough to have to worry about Evan's medical condition. But to also have to worry about how we were going to get his medication was almost overwhelming."

Then a friend told Jon about NORD and its patient assistance programs. Jon called NORD and – even though it was the day before Thanksgiving – within one day, NORD had arranged for the medication to be delivered to the hospital, and little Evan and his parents were soon on their way home.

Today, Evan is doing well and Jon and Amanda are grateful that a safety net was in place to help them access the lifesaving therapy their baby needed.

“ NORD'S Patient Assistance Program provided Evan with the lifesaving medication he needed. ”





NORD  
PIONEERED  
PATIENT  
ASSISTANCE  
PROGRAMS  
FOR MEDICATIONS  
& CONTINUES  
TO PROVIDE  
LEADERSHIP  
IN THAT FIELD

# Stretching Dollars to Save Lives

When 14-year-old Jacob Maren wanted to do something to help others as a bar mitzvah and school leadership project, he and his parents visited the NORD offices near his home in Connecticut. Jacob was particularly interested in educating physicians about a rare, potentially very serious condition known as infantile spasms that had touched his life briefly when he was very young.

Ultimately, Jacob raised \$10,000 to make it possible for NORD to create and publish a Physician Guide to Infantile Spasms. The guide is available free to physicians around the world at [www.nordphysicianguides.org](http://www.nordphysicianguides.org).

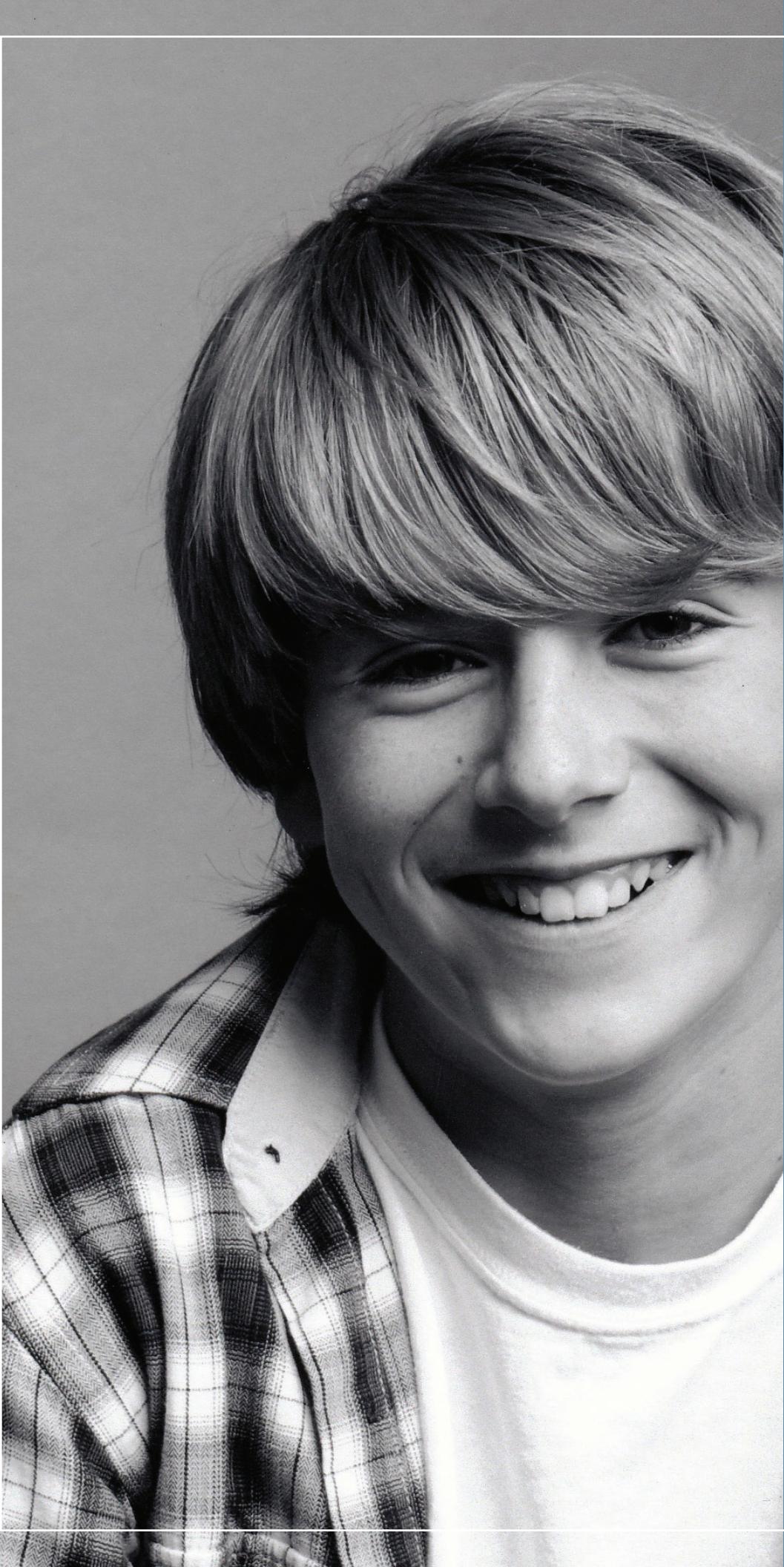
NORD created the physician guide website a few years ago as part of its campaign to reduce the time to diagnosis and treatment for patients. The guides are written by medical experts, and each time a new guide is published, NORD conducts outreach to physicians in targeted practice fields to promote awareness of the new resource.

Since the American Academy of Neurology (AAN) had published new treatment guidelines for infantile spasms around the time Jacob first approached NORD, the educational team at NORD worked with AAN and arranged for two physicians who had been involved in the new guidelines to write the NORD guide.

Through the years, NORD has demonstrated its ability to gain maximum benefit for patients and their families from donations of all sizes.

**“Jacob raised funds for NORD to create a physician guide on a rare disease affecting infants where early diagnosis is critical.”**

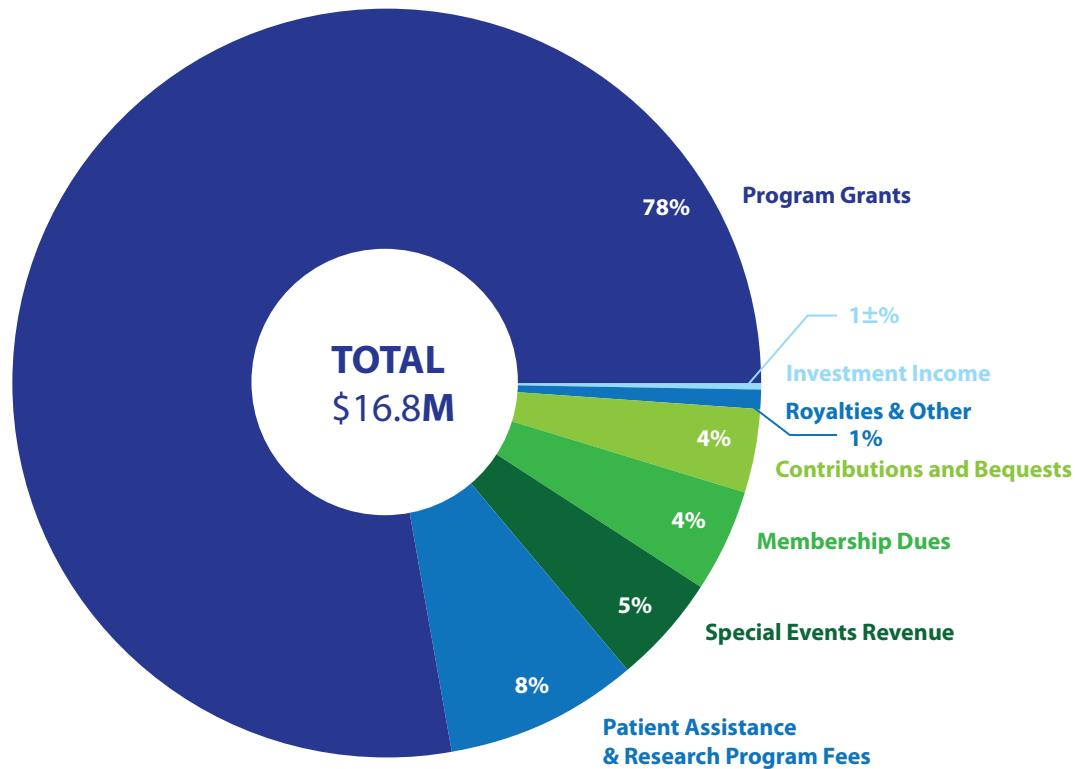




CLINICIANS  
FROM MORE  
THAN 160  
COUNTRIES  
HAVE ACCESSED  
NORD'S NEW  
**ONLINE**  
PHYSICIAN  
GUIDES TO  
PROMOTE  
DIAGNOSIS  
AND TREATMENT  
FOR PATIENTS

# 2013 Financial Position

## Revenue



Program Grants. . . . . \$13,090,825

Patient Assistance and Research Program Fees. . . . . \$1,418,853

Special Events Revenue . . . . . \$776,744

Membership Dues. . . . . \$751,040

Contributions and Bequests. . . . . \$617,280

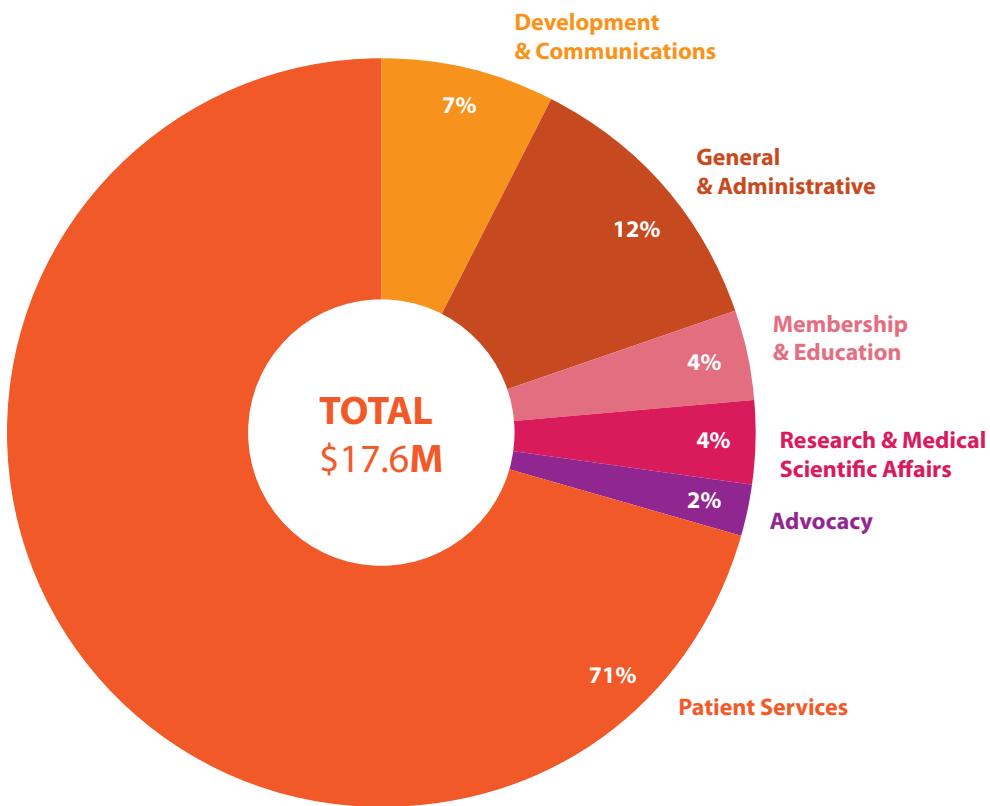
Royalties and Other . . . . . \$146,154

Investment Income . . . . . \$18,998

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**Total Revenue** **\$16,819,894**

# Expenses



Patient Services . . . . . \$12,459,521

Advocacy . . . . . \$385,505

Research and Medical Scientific Affairs. . . . . \$620,136

Membership and Education. . . . . \$708,174

General and Administrative . . . . . \$2,117,125

Development and Communications . . . . . \$1,308,380

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**Total Expenses** **\$17,598,841**

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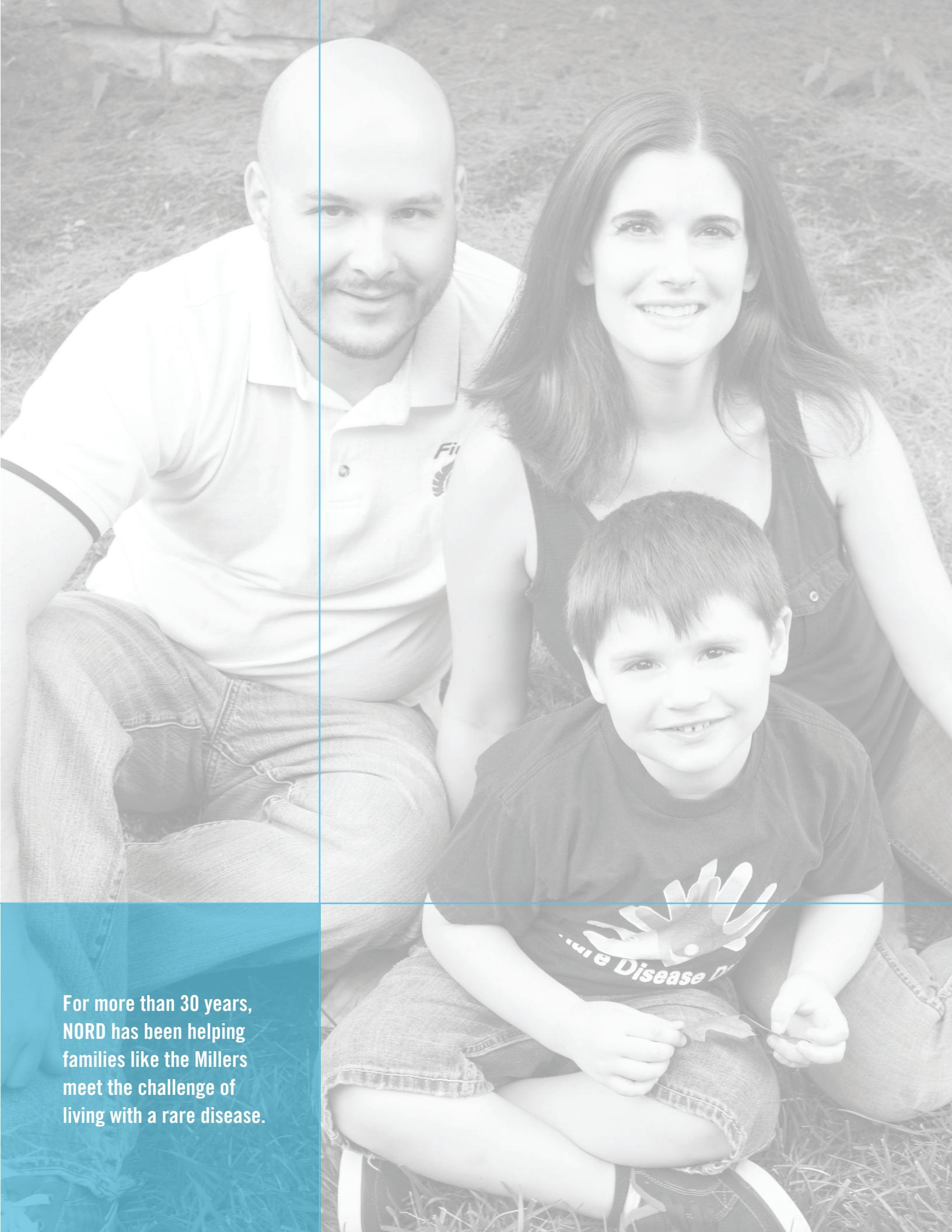
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For more than 30 years,  
NORD has been helping  
families like the Millers  
meet the challenge of  
living with a rare disease.



National Organization for Rare Disorders

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

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