



**NORD**<sup>®</sup>

National Organization  
for Rare Disorders

**NORD 2019 Annual Report**

*At the Heart of Progress  
in Rare Disease*

2019

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## A MESSAGE FROM OUR CEO

Since our founding by a group of parents advocating for their children in 1983, NORD has been dedicated to providing innovative programs and services to unite the rare disease community, eliminating isolation by increasing awareness and supporting patients, families and caregivers. We are committed to driving progress because our hearts are with the rare disease community we serve.

This year, NORD helped over 8,000 patients and families in accessing care and treatment for their rare disorders. We launched the first-of-its-kind Rare Caregiver Respite program, which provides financial assistance for caregivers of a child or adult diagnosed with a rare condition. Our IAMRARE™ registry program marked its fifth anniversary and grew to 41 natural history studies, collecting impactful data from patients that will contribute toward the development of new treatments. NORD's membership grew to just under 300 organizational members and the Rare Action Network reached over 14,000 individuals, who unite with NORD to advance our shared goals for the community. Through new partnerships and alliances, we continue to pioneer important work in the field of regulatory science that will lead to additional FDA-approved treatments for rare diseases.

As we celebrated the 30th anniversary of our Rare Disease Research Grants program, we paused to recognize its impact: the development of life-saving FDA-approved therapies, new discoveries and findings that have led researchers to groundbreaking findings. Many researchers have told us these opportunities have altered the course of their careers—much to the benefit of rare patients who need treatments and cures, as well as the greater scientific community.

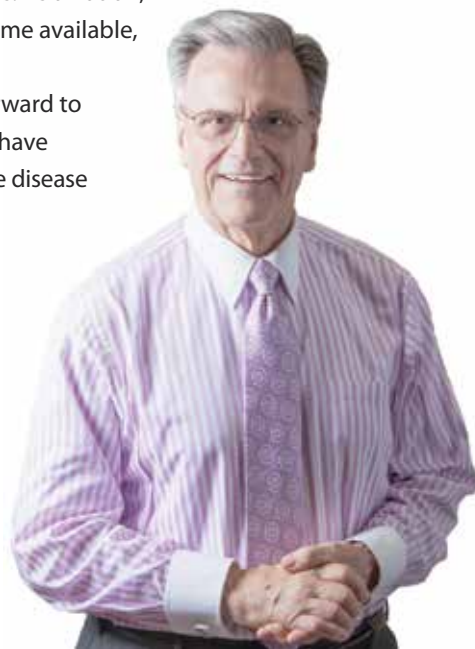
We are honored to know that we are continuing to make a positive impact. This year NORD received the Regulatory Affairs Professional Society's inaugural Patient-Centered Health Award and was recognized by others as a top-rated charity. Adding to these accolades are the letters of thanks from patients and their families. A man who had been enrolled in one of NORD's RareCare® assistance programs sent a letter of thanks and shared that he made NORD his designated charity on Amazon Smile, to do what he could to give back and pay it forward. To know that we have helped and made a positive difference in myriad ways and for so many people makes all of us at NORD want to do even more in the coming year.

There are so many more accomplishments from the past year I hope you will take the time to read about in the following pages. As we recap our work, we also must acknowledge the challenges the rare disease community continues to encounter. While we are experiencing a thrilling period of medical innovation, patients are still facing barriers that require further progress in advocacy, research, education and other services. As a nation, we need continued innovation but as new treatments become available, patients must be able to access them.

We are proud of the work NORD has done, and look forward to another year of continuing to build upon the successes we have achieved alongside over 25 million Americans living with rare disease and those who support them.

Sincerely,

Peter L. Saltonstall | President and CEO  
National Organization for Rare Disorders





**The National Organization for Rare Disorders (NORD)** is at the heart of driving progress for the 25 million+ Americans with rare diseases. Most rare diseases (90%) still do not have an FDA-approved treatment option, and people face an average of 5-7 years wait for an accurate diagnosis. We are committed to changing that. We provide support every step of the way to patients and families and unite our community so that together our voice is stronger.



# BY YOUR SIDE AT EVERY STEP OF THE JOURNEY

We are here before, during and after a diagnosis. Whether connecting people with our resources or the wider community, you can see NORD's impact at every stage of living with a rare disease. That is what it means to be at the heart of progress.

DID YOU KNOW?

We're answering

173,800+

phone calls and emails each year



We're training

14,000+

advocates in 50 states



We're helping

8,000+

patients through assistance programs



We're strengthening

299

patient organizations as members



We're educating

1,000s+

of health care providers  
through CME and other  
programs



We're informing

1.3 Million

people on average each  
month through our website



We're empowering

25+ Million

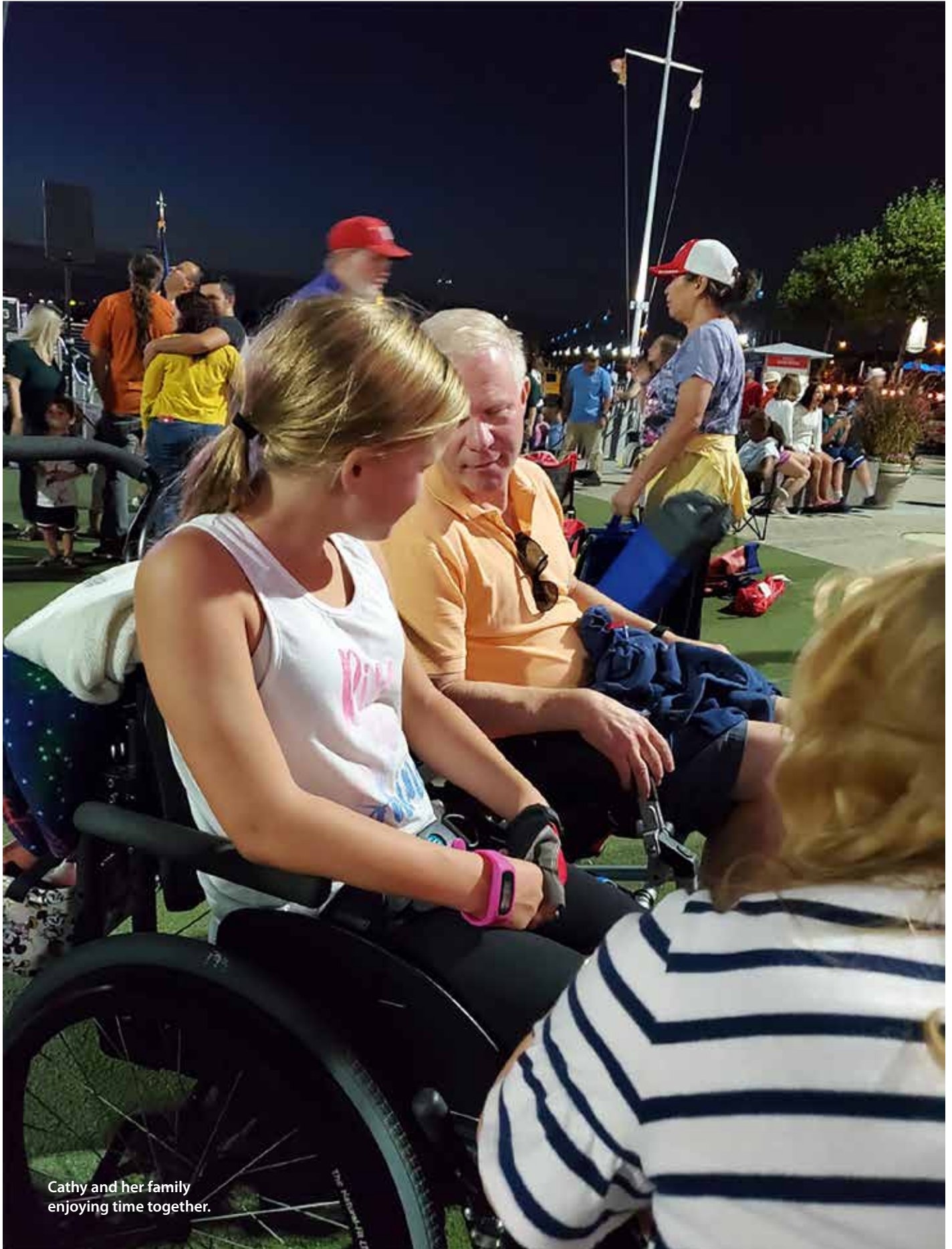
Americans with rare diseases.



We're trusted

Recognized as an outstanding charity by:





Cathy and her family enjoying time together.



# SUPPORT WHEN YOU NEED IT

Progress continues to improve patient outcomes. Because of generous donations to NORD's RareCare® assistance programs, we have been able to help more people on their rare disease journey than ever before.

## PROGRESS FOR RARE: CATHY'S STORY

Cathy, whose daughter has a rare disease, knows firsthand that being a rare caregiver demands significant time, dedication and patience. "We spend most of our extra resources finding ways to help our special needs daughter have a normal life," she says. Cathy is also one of the first participants in NORD's Rare Caregiver Respite Program, which launched in 2019. We created this first-ever program to help give eligible caregivers a much-needed break by granting financial assistance so that a respite provider may be secured to care for a loved one.

Cathy adds, "To know that we had a qualified CNA taking care of our little one was such a blessing. Family offers to help but it was SO important for me to have someone who knew how to handle any medical situation that might arise while we were gone. The funds from NORD gave us 'permission' to have a 'normal' weekend for us as a couple. I have recommended NORD to several other special needs parents who have kids with rare disorders. It is such a great organization for us and has a ton of great resources!"

## TESTIMONIALS

**"I want to thank you from the bottom of my heart for the help you've given me and my family. In what was easily the toughest year of our lives there have been several people that have helped make it just a little easier and you are one of them."**

► Aaron, NORD RareCare enrollee

**"Thank you so much for all your work with me for my brother!! No words can describe our feelings of joy and thankfulness for you and the whole NORD organization!"**

► Craig, caregiver for NORD RareCare enrollee

**"My family cannot express enough how thankful we are for you and everyone who works for us so hard at NORD and all the donors for this program. Please let your fellow co-workers know today that they are making a difference and are appreciated by the families they serve--especially mine!"**

► Belinda, parent of a daughter with a rare disease and NORD RareCare enrollee

# 8,621

patients in financial distress received assistance to pay for expenses related to their rare disease.

We booked and paid for

# 783

flights so patients could travel to meet with specialists for their rare disease.

Patients were able to enroll in

# 14

clinical trials with NORD's assistance.



Read more caregiver stories at [rarediseases.org](https://rarediseases.org/rarediseases.org/?s=rare+caregivers+corner&submit=)  
(or scan QR code)



# RESEARCH THAT FUELS PROGRESS

One of NORD's most impactful contributions to research has been building infrastructure that helps serve the needs of 7,000 unique rare diseases. As an umbrella organization, our mission has always been to help drive progress for all.

The Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) has the potential to unify rare disease data, move critical projects past bottlenecks and help speed the development of treatments and cures. We are proud to work with the Critical Path Institute as a partner on this project, with funding support from the US Food & Drug Administration (FDA).

We received

# 4

federal grants this  
year - a first for NORD  
as an organization.

## TESTIMONIALS

**“People with rare diseases need treatments; we need to do what we can to make development of those treatments as efficient, effective and fast as possible. The way to do that is to have all the data we’ve been talking about brought to bear on how we test the interventions—the Rare Disease Cures Accelerator-Data and Analytics Platform is the vehicle that can deliver that data to the developers and the community,”**

► Janet Woodcock, MD, FDA Center for Drug Evaluation and Research Director







# PROGRESS THROUGH PATIENT REGISTRIES

NORD's landmark natural history program, IAMRARE™, is at the heart of progress for research and is not only increasing knowledge of individual rare conditions, but advancing what we know about rare disease as a whole. We are proud to have built a platform where none existed - one that ensures patients maintain ownership of their data and how it is used - and are eager to continue this work for years to come.

## PROGRESS FOR RARE: PDSA'S NATURAL HISTORY STUDY

The Platelet Disorder Support Association (PDSA) is a long-standing NORD member organization and a powerful force in serving and unifying the global community of patients, practitioners, caregivers, advocates and key disease stakeholders affected by immune thrombocytopenia (ITP) and other platelet disorders. PDSA's ITP Natural History Studies Registry, hosted under the IAMRARE platform, serves as part of PDSA's research portfolio, collecting data on the natural progression of ITP, helping to examine its pathogenesis and management, and present the most promising outlook to significantly improve diagnosis, therapies and patient quality of life. "Thank you to NORD for our collaborative partnership and the opportunities that you have given to us which has elevated our organization in ways we could have never imagined," says PDSA President and CEO Caroline Kruse.



Our new book, ***The Power of Patients: Informing Our Understanding of Rare Diseases (2019)***, published in partnership with Trio Health, features reflections from rare disease patients and information from NORD member organizations who are participating in our registry program.



Read an excerpt at [rarediseases.org](https://rarediseases.org)  
[rarediseases.org/iamrare-registry-program/](https://rarediseases.org/iamrare-registry-program/)  
(or scan QR code)

# 10,000+

people are enrolled in IAMRARE™ studies, contributing data to fuel lasting progress.

# 41

IAMRARE studies are live and gathering data from patients around the world.

To spur further advancements, NORD traveled to

# 19

conferences in 2019 to present on emerging topics and trends in rare disease research.



# COMMEMORATING 30 YEARS OF GRANTING HOPE FOR RARE DISEASE

Starting with our first research grant 30 years ago, NORD has awarded more than 150 grants and over \$8 million in funding. These grants have led to the development of FDA-approved treatments and powerful discoveries that have helped researchers reach the next level in their work. With NORD-awarded researchers working across 13 countries and 28 states, our reach has never been more broad, yet our mission remains the same: to drive progress for the benefit of patients and families affected by rare disease.

## YOUR JOURNEY MATTERS

To mark the 30th anniversary of NORD's research program we are conducting a follow-up survey to one first conducted in 1989 (and again in 2003), to better understand the barriers and facilitators of rare disease diagnosis, care and treatment in the United States over time. The survey will be open and capturing responses and valuable feedback through early 2020.

## TESTIMONIALS

**“It is a very uphill battle to have a career in rare disease research. There are very few mechanisms for start-up funding, and most institutions do not want to invest in them as they are seen as unlikely to obtain national funding. NORD is one of the few programs that offers opportunities for such startup funding.”**

► Johan Van Hove, MD, PhD, NORD awardee and Professor, University of Colorado

**“I am extremely grateful to NORD’s grant program. It was instrumental in jump-starting my research and helping to secure additional funding\* and further my career so that I dedicate my clinical and research efforts to improve the way we treat individuals with lysosomal storage diseases. My goal is to help the development of definitive therapies for these diseases.”**

► Natalia Gomez-Ospina, MD, PhD, NORD awardee and Assistant Professor, Stanford University

*\*Dr. Gomez-Ospina reports she was able to leverage the data collected through her \$30,000 NORD grant into \$1.2 million in additional research funding.*

Working with patients and families for

# 30

years to fund research on rare diseases that affect them or loved ones.

# 160+

publications have cited NORD research grant funding since our program’s inception.

We have awarded

# 150

grants totaling over \$8 million since 1989 - many of which have been the only source of funding for the study of those conditions.



# BREAKTHROUGHS OF GLOBAL PROPORTIONS

This year marked a significant milestone for all people with rare diseases. At a high-level meeting at the United Nations in September, rare diseases were included for the first time within a UN declaration adopted by all 193 Member States. This accomplishment for our community will help achieve critical awareness for rare diseases whenever healthcare policies are discussed. We are honored to work hand-in-hand with our global partners to make this progress possible.



Learn more at:  
[rarediseases.org/partnerships](https://rarediseases.org/partnerships)  
(or scan QR code)



## HELPING UNDIAGNOSED PATIENTS

In 2019, NORD charged ahead to create progress for people at every stage of their rare disease journey - including the often long, confusing road to diagnosis. We welcomed new and returning runners, walkers and volunteers to our Running for Rare® charity marathon team. This passionate community raced toward making a difference, raising funds to help patients pay for diagnostic testing through NORD's Undiagnosed Patient Assistance Fund.

### PROGRESS FOR RARE

For patients and families affected by undiagnosed conditions, costs associated with a diagnostic workup can be substantial. "The NORD Undiagnosed Patient Assistance Fund has been crucial in providing financial assistance to undiagnosed patients and families who are facing out of pocket costs related to co-pays and diagnostic testing needed before acceptance into the program," says Katrina Dipple, MD, PhD, principal investigator at the UDN Pacific Northwest Clinical Site and UDN liaison to NORD. **To date, 130 families have received support, totaling more than \$63,000 since the program's inception in 2015.**



Staff profile: Read Marissa's Running for Rare story here.  
[rarediseases.org/a-first-time-for-everything-marissas-running-for-rare-story](https://rarediseases.org/a-first-time-for-everything-marissas-running-for-rare-story)  
(or scan QR code)



# A NATION OF ADVOCATES

When we come together, it is not just the advocacy piece that serves a crucial role - it is the feeling of not being alone. At every Rare Action Network event we host, there is at least one person in attendance who has never met another person with a rare disease, sometimes even meeting someone with the same diagnosis. The connections we build give us strength and bring us closer toward continued progress.

Make your voice heard! Get involved at [rareaction.org](http://rareaction.org)

## TESTIMONIALS

**“It’s me and my daughter, we have no family here in Connecticut. You, this group of rare disease advocates, are our family and we are thankful for events like these to connect us and make us know we’re not alone.”**

► Carmen, mother of 40-year old daughter with a rare disease, speaking at NORD’s Rare Action Network event in New Britain, CT

**“I was so happy to be able to have the entire family with me to connect with you and the other families and advocates. We are blessed to have circles of support and folks who ‘get it’.”**

► Nick, father of a 4-year old with a rare disease, speaking at NORD’s open house event



New York’s Empire State Building was lit up for Rare Disease Day 2019.

Together, our

# 14,285

advocates were vocal with their elected officials to demand better for rare patients and their families.

Working with our volunteers, we held and attended

# 130+

events around the country.

NORD’s Rare Action Network® grew to include members across all

# 50

states and DC, with Ambassadors now in 31 states.

Peer-to-peer fundraising grew more than

# 10%

over 2018, thanks to Facebook fundraising, Crowdrise and other efforts.



# PROGRESS THROUGH RAISING AWARENESS

## PROGRESS FOR RARE: CAMPAIGNS TO RAISE AWARENESS



Volunteers brought their voices to Capitol Hill for NORD’s Hill Day on October 23 to tell their stories and advocate for policies benefiting the rare community. Ambassadors from 20 states met with 30 Congressional offices to talk about a host of issues confronted by the rare disease community.



View more photos here.  
[rarediseases.org/nords-rare-action-network-mobilized-for-a-successful-hill-day](https://rarediseases.org/nords-rare-action-network-mobilized-for-a-successful-hill-day)  
(or scan QR code)



**October 1 is #RareCancerDay**  
Show Your Support by Spreading the Word

Together with our Rare Cancer Coalition members, we launched Rare Cancer Day on October 1, marking the first-ever day to recognize rare cancers. One in 5 people living with cancer in the US have been diagnosed with a rare cancer, and, for those individuals, the 5-year survival rate is lower than that for more common cancers - clearly there is more to be done. Nicole, one survivor, says, “Being diagnosed with cancer is a traumatic event and the more I talk about it and connect with others, the more I can process, heal, grow, and help others like me who might feel like they are alone or on an island. I am here to say that you’re not alone.”



Read more stories at [rarediseases.org](https://rarediseases.org)  
[rarediseases.org/?s=Voices+of+Rare+Cancer&submit=](https://rarediseases.org/?s=Voices+of+Rare+Cancer&submit=)  
(or scan QR code)



NORD launched the #ShowYourStripes awareness campaign in time for our 10th Rare Disease Day® celebration on February 28, with a goal of increasing attention and engaging our community. Judging by the number of events, attendance at events, extensive media coverage, a resolution passed by the Senate, robust social media engagement and beyond (even the Empire State Building was illuminated in the Rare Disease Day colors), the day was a success and had rippling effects throughout the year.



Watch our Rare Disease Day advocates in action at [youtube.com/raredisorders](https://youtube.com/raredisorders)  
[www.youtube.com/playlist?list=PLMmYBWQscoiEOsTPzqLn3cq6N1oUH5heN](https://www.youtube.com/playlist?list=PLMmYBWQscoiEOsTPzqLn3cq6N1oUH5heN)  
(or scan QR code)



# SPOTLIGHT ON POLICY

Since 1983, NORD has worked to ensure that the voice of the rare disease patient is front and center when important policy and regulatory decisions are made at both the federal and state levels. The challenges of living with a rare disease are many, including the initial diagnostic odyssey, accessing needed therapies, navigating insurance, paying for unending medical expenses and dealing with systems and individuals that fundamentally do not understand the realities of the rare disease community. Even when a treatment exists, people living with rare diseases often face challenges obtaining insurance coverage. They may also face problems in affording the coverage itself, both in the form of premiums and cost sharing. NORD's mission is to ensure that these barriers and significant obstacles are addressed.

## PROGRESS FOR RARE: NEWBORN SCREENING CAN MAKE ALL THE DIFFERENCE

Newborn screening (NBS) programs throughout the United States have had great success at increasing the number of newborns screened at birth and, as a result, saving lives. Each year, approximately four million babies are screened through these programs. Of that four million, screening identifies over 12,000 infants each year with a disorder that, left undiagnosed and untreated, would cause severe developmental disability or death. Newborn screening programs are administered at the state level, but federal agencies do much to improve and expand them. NORD worked tirelessly throughout 2019 to advocate in Congress for passage of the Newborn Screening Saves Lives Act which would reauthorize the law under which the federal newborn screening programs operate. NORD continues to fight for robust, well-funded newborn screening programs in every state.



Visit our State Action Center for a complete breakdown on how your state measures up on newborn screening and other key issues. [rareaction.org/resources-for-advocates/state-profiles/](https://rareaction.org/resources-for-advocates/state-profiles/) (or scan QR code)

In 2019, FDA approved

# 76

new indications to treat rare diseases, each one a step closer to improving patient outcomes.

With our member organizations, we hosted

# 7

FDA Listening Sessions, elevating the patient voice to help speed up drug development.

## RARE DISEASE ADVISORY COUNCILS

Rare Disease Advisory Councils (RDAC) act as an advisory body that helps give the rare disease community a stronger voice in state government. In 2015, the first RDAC was created in North Carolina by patients, caregivers, families and providers. Since then, NORD has sought to build councils in many states to help better represent their community. With the support of NORD and other patient organizations, RDACs are enabling each of the eleven states that have passed them into law to address barriers that

prevent individuals living with a rare disease from obtaining proper treatment and care for their condition. NORD will continue its efforts to ensure that every state enacts legislation to establish a robust, well-functioning RDAC.



## DRUG PRICING AND ACCESS TO INNOVATIVE THERAPIES

In 2019, NORD announced its development of key drug pricing principles, created with the needs of the rare disease community in mind. People living with rare diseases need to be able to afford the therapies that come to the market. The high cost of prescription drugs has a direct impact on the ability of patients to access lifesaving care. The small patient populations and medical complexities associated with rare diseases can lead to costly therapies, but it is vital that these therapies remain affordable and, therefore, accessible to rare disease patients. Throughout 2019, NORD advocated at the federal and state levels for policies that would foster access to affordable and innovative rare disease therapies.



*“What has been so encouraging is seeing other children born with the same disease as Ruby and Landon, who, because they were screened prenatally or via newborn screening, began to receive treatment before symptoms began. Those children are very often developing typically, overall. That is a miracle in the world of SMA. Because of newborn screening becoming more common, our and other children’s lives are being saved and quality of life is improving in ways we never would have thought possible even just two years ago.”*

**Danyelle Sun, WI RAN Volunteer State Ambassador**



Read more about NORD's new drug pricing principles here:  
[rarediseases.org/nord-releases-principles-for-assessing-proposals-designed-to-lower-the-cost-of-prescription-drugs-in-the-united-states/](https://rarediseases.org/nord-releases-principles-for-assessing-proposals-designed-to-lower-the-cost-of-prescription-drugs-in-the-united-states/)  
(or scan QR code)



# USHERING PROGRESS FORWARD WITH GENE THERAPY

After decades of research and development, gene therapy is becoming a reality. With the first gene therapies already approved and many more in the pipeline, we may soon gain the ability to treat more rare conditions. To address this exciting topic, NORD hosted a 5-part webinar series to hear from FDA leaders, gene therapy patients and clinicians, among other experts in the field. We were honored to work in collaboration with the American Society for Gene and Cell Therapy to present on this important subject. Through our efforts we aim to also provide hope for the future for people living with rare diseases.

#### Topics covered include:

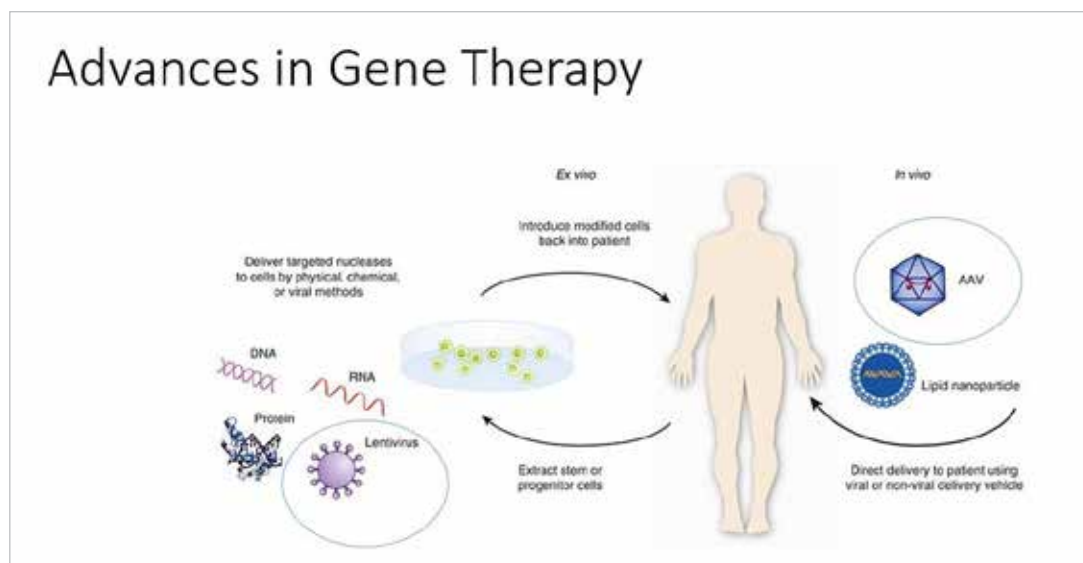
- Gene Therapy: Yesterday, Today and Tomorrow
- The Science Behind Gene Therapy
- The FDA's Role in Gene Therapy
- Understanding the Gene Therapy Process and Aftercare
- Life After Gene Therapy

In 2019, FDA approved the third gene therapy to treat rare diseases, with hundreds more research studies underway.

Our webinar series on gene therapy has been viewed more than

# 3,000

times.



Watch the webinars at [youtube.com/raredisorders](https://www.youtube.com/raredisorders)  
[youtube.com/watch?v=qKxnHDj1cbc&list=PLMmYBWQscoiH3t2SzSP1gYjP2q8sZxCo9](https://www.youtube.com/watch?v=qKxnHDj1cbc&list=PLMmYBWQscoiH3t2SzSP1gYjP2q8sZxCo9)  
(or scan QR code)





# INFORMATION IS POWERFUL

Since our founding, NORD has been a leader in presenting rare disease information in accessible language. Our online Rare Disease Database is responsible for educating millions of readers annually. This year we augmented these reports with videos from our new library and, thanks to our partnership with NIH's Genetic and Rare Diseases Information Center (GARD), now provide educational information on all 7,000 rare diseases on our website.

## PROGRESS FOR RARE: SOPHIA'S STORY

Before she was born, Sophia was diagnosed with Miller-Dieker syndrome, a rare genetic condition primarily associated with a type of abnormal brain development called lissencephaly. Sophia's family had little time to prepare themselves for a diagnosis of this magnitude, so they quickly turned to NORD's Rare Disease Database to find information on this condition and connect with a network of people going through similar challenges. Although doctors were uncertain if Sophia would survive birth, she lived 22 remarkable months of a life her family knew was uncertain. Her family shares their story to help other families facing similar difficulties.

Improving physicians' rare disease knowledge through continuing medical education (CME) programs is vital to enabling families like Sophia's to have a faster and more accurate diagnosis. This year NORD teamed up with PlatformQ to create accredited online education programs for healthcare professionals, helping to ensure clinicians have the information needed to provide the best care possible for rare disease patients and their families.



Search for rare disease information here.  
[rarediseases.org/rare-disease-information/](https://rarediseases.org/rare-disease-information/)  
(or scan QR code)



Visit [rarediseases.org/cme](https://rarediseases.org/cme) to take our courses  
at any time, from anywhere in the world.  
(or scan QR code)

Reports in NORD's  
online Rare Disease  
Database were read

# 18,537,315

times (a 47% increase  
over 2018).

We launched the  
NORD Rare Disease  
Video Library with 27  
videos (and growing)  
on rare diseases and  
related topics for  
patients, caregivers,  
students, professionals  
and the public, watched  
more than

# 100,000

times in the platform's  
first year.

# 4,600+

people registered  
for our educational  
webinars, presented  
with rare disease  
specialists.



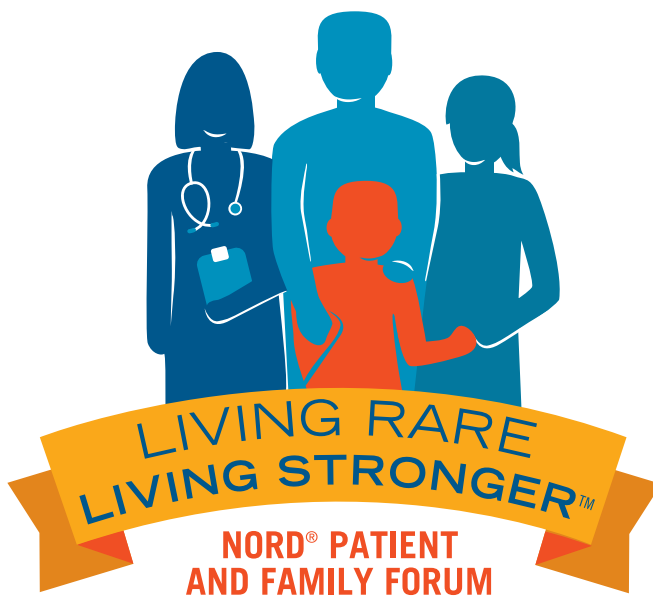
# EVENTS THAT BRING THE RARE COMMUNITY TOGETHER

Serving patients and families with resources and offering connections to information has long been integral to NORD's mission. This year we connected with more people than ever by expanding our signature events and reintroducing a type of live programming for patients and caregivers that had been one of our hallmarks for many years.

## HOUSTON, WE HAVE LIFT OFF

At our inaugural **Living Rare, Living Stronger™ NORD Patient & Family Forum** (June 21-23), held in Houston, TX, more than 500 community members came together for three days of learning, connecting and fun. We shared tools on how to be powerful forces for change; offered practical tips for patients, caregivers, clinicians and medical students; and bonded by sharing stories and decompressing in the dedicated wellness room and at the welcome barbeque. The event will travel to a different part of the US each year. In 2020, the event will move to Cleveland, OH.

The weekend's festivities also included NORD's annual Rare Impact Awards ceremony, celebrating those making extraordinary contributions to the lives of rare disease patients and caregivers. Our fantastic honorees were joined by emcee and Olympic gold medalist, Mary Lou Retten; retired astronaut Col. Kenneth Cameron; and violinist and advocate Allison Lint, who treated attendees to a special musical performance.



Learn more at [rarediseases.org/living-rare-forum](https://rarediseases.org/living-rare-forum)  
Watch highlights from this amazing weekend at [youtube.com/raredisorders](https://youtube.com/raredisorders)  
(or scan QR code)





# EVENTS THAT BRING THE RARE COMMUNITY TOGETHER

## THE TIME IS NOW: NORD SUMMIT OFFERS SUPPORT AND SOLUTIONS

2019 marked an historic **Rare Diseases & Orphan Products Breakthrough Summit**<sup>®</sup> (Oct. 21-22) in terms of numbers and content. More than 900 attendees met in our nation's capital to explore our theme of "The Time is Now" and why this is the moment for action to help rare disease patients and families. The event featured our largest research poster session to date and keynote speeches from passionate advocates and high-ranking officials. Secretary of Health and Human Services Alex Azar, talked about the need to spur innovation and fix a system that has led some diseases to be neglected for too long, and FDA then-Acting Commissioner, Ned Sharpless, MD, discussed important progress and challenges in the field—their presence signaling how much of a priority rare diseases are for the federal government.

There were also many heart-tugging behind-the-scenes moments, such as when a biopharmaceutical scientist who helped develop a new precision therapy met a patient whose life had been saved by it; when a caregiver expressing frustration about a newborn screening issue was introduced to a Rare Action Network advocate educating legislators in that state; and conversations between new and long-time advocates that fuel the momentum of even greater progress in the years to come.



Learn more at [nordsummit.org](http://nordsummit.org)  
Watch our Summit highlights video here:  
[youtube.com/raredisorders](https://youtube.com/raredisorders)  
(or scan QR code)



Read Secretary Azar's speech here.  
[hhs.gov/about/leadership/secretary/speeches/2019-speeches/remarks-to-the-national-organization-for-rare-diseases.html](https://hhs.gov/about/leadership/secretary/speeches/2019-speeches/remarks-to-the-national-organization-for-rare-diseases.html)  
(or scan QR code)





# EVENTS THAT BRING THE RARE COMMUNITY TOGETHER

## FDA MEETINGS

We strive to create opportunities for patients to be heard, especially when a patient community has an unmet need. This year, collaborating with the Foundation for Rare Blood Diseases, we hosted the first-ever meeting at FDA for a rare condition known as pyruvate kinase deficiency, which currently has no targeted treatment and a severe burden of disease, especially for children. For the first time, patients had the opportunity to speak directly with FDA staff, drug developers, doctors and other patients to discuss what it is like living with this condition. The results of this meeting will be shared publicly in a “Voice of the Patient” report that we hope will inform future progress for this disease.



Staff perspective: Read Debbie Drell's story about working with FDA as both a rare caregiver and NORD's Director of Membership here: [rarediseases.org/nords-work-with-fda-gives-patients-a-chance-to-be-heard-in-drug-development/](https://rarediseases.org/nords-work-with-fda-gives-patients-a-chance-to-be-heard-in-drug-development/) (or scan QR code)





# DELIVERING ON A SHARED MISSION

## PROGRESS FOR RARE: UNIQUE PARTNERSHIPS AND BENEFITS

All of our membership programs are designed to strengthen rare disease patient organizations and help them increase capacity to serve patients and families, educate medical professionals and bring awareness to the general public. We are committed to helping each organization evolve and thrive, and provide guidance and support at all phases of development.

In 2019, we connected members with multiple regulatory and government leaders who shared knowledge through exclusive webinars on topics to drive progress, such as how to apply for Patient-Centered Outcomes Research Institute (PCORI) funding, engage with FDA, host externally-led Patient-Focused Drug Development meetings and apply for Department of Defense medical grants. We facilitated new opportunities, including pro-bono communication consulting services and connections with the Rochester Institute of Technology IdeaLab to solve complex challenges faced by the rare disease community. Complimentary membership to BoardSource and access to NORD's private member leader social network offered opportunities to seek practical answers and solutions from experts and peers.

In person, we met with our members on topics of importance to them, offering good governance workshops and leadership development sessions that were attended by 235 leaders from our member organizations, nearly 90% of whom said they were able to identify new governance strategies to improve the management of their board and for fundraising and development.



Learn more at [rarediseases.org/membership](https://www.rarediseases.org/membership)  
(or scan QR code)

# 25

new members joined NORD in 2019, bringing our total to 299 member organizations.

We gave over

# \$120,000

in travel scholarships for members to attend key rare disease and biotech conferences.

# 8

members-only webinars and 6 bi-monthly calls provided rare disease updates and capacity-building tips throughout the year.



# TRAINING TOMORROW'S LEADERS

To assure a future where rare diseases are not overlooked, NORD works with student leaders and organizations to facilitate opportunities to learn about these conditions and patients' experiences. We are continually inspired by the dedication and passion of our student volunteers, each of whom shows us there are only brighter tomorrows ahead.

## PROGRESS FOR RARE: STUDENTS FOR RARE LEADER JAIME TAN

Jaime Tan is a Central Michigan University (CMU) medical student and NORD Students for Rare Chapter Leader.



### *Where did your interest in rare diseases come from?*

When I was an undergraduate and during my post-baccalaureate, I became involved in extensive basic science research. Most of the research involved rare diseases (Waardenburgh-Shah syndrome, Hirschsprung disease, etc.). I also did work involving desmoplastic infantile ganglioma and intrahepatic bile duct cancer. I was consistently amazed by the numbers and statistics while doing research on those diseases.

Then, during my first year of medical school, I attended a lot of student meetings and events. The rare diseases student interest group's events always stuck with me. Talking to patients and families and learning about academic presence of rare diseases were enlightening. To this day, I remember the first rare disease speaker I saw, a woman with Marfan syndrome. Hearing about her experiences and perspectives was a transformative and inspiring event.

### *Why did you decide to start a NORD Chapter?*

I thought it was something that should be told and starting a NORD chapter is a great way to get the word out. My classmate, Mary Gipson, and I then decided to promote the NORD chapter at CMU. We have worked hard to bring speakers and hold events that hopefully make an impact to our fellow students the way it has impacted us.

### *What would you say to other students who are interested in rare diseases?*

It's not a matter of learning about all the rare diseases, but it's more of getting into the habit of thinking outside of the box and considering a diagnosis no matter how rare it may be.

This year

# 92

university students around the country raised awareness of rare diseases on campus in their role as Students for Rare leaders.

NORD's Students for Rare program grew to include

# 24

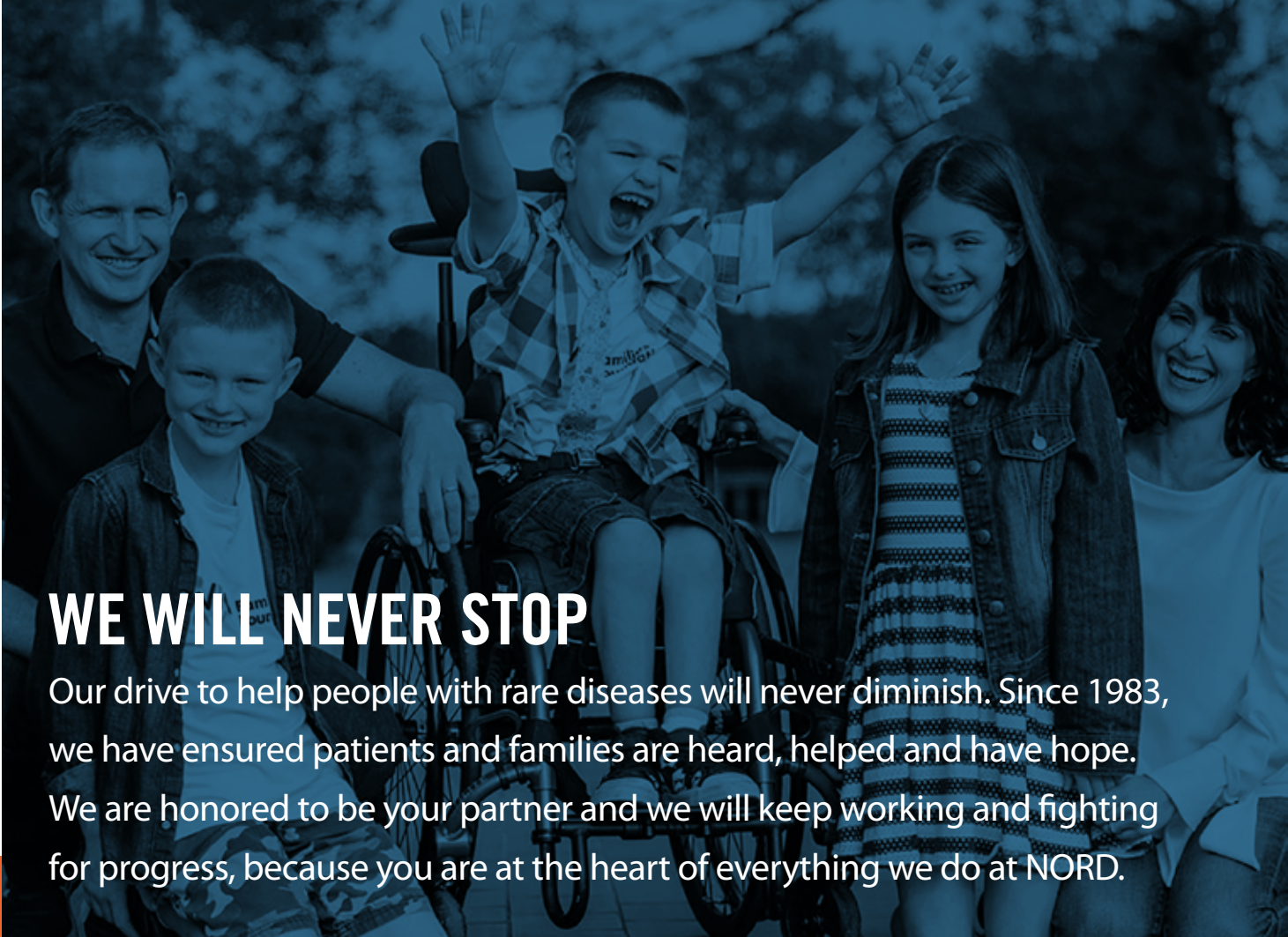
chapters and clubs at high schools and universities around the country.

In 2019, we hosted 10 laboratory rotation students in NORD's offices, contributing to a multidisciplinary understanding of rare diseases.



Meet our Students for Rare Leaders  
[rarediseases.org/tag/students-for-rare](https://rarediseases.org/tag/students-for-rare)  
(or scan QR code)





## WE WILL NEVER STOP

Our drive to help people with rare diseases will never diminish. Since 1983, we have ensured patients and families are heard, helped and have hope. We are honored to be your partner and we will keep working and fighting for progress, because you are at the heart of everything we do at NORD.



## BECAUSE OF YOU

Our heartfelt thanks to our supporters. As a nonprofit organization, we would not exist without you. Together, we will continue our work at the heart of progress in rare disease.

Turn your spare change into progress for rare diseases. Download the Cheerful app and select the National Organization for Rare Disorders (NORD) as your charity of choice.

Visit our website for more ways to help and get involved

[rarediseases.org/ways-give](https://rarediseases.org/ways-give)

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Acromegaly Community, Inc.

ADCYS.org

ADNP-Kids Research Foundation

Adrenal Insufficiency United (AIU)

Advocacy & Awareness for Immune Disorders Association (AAIDA)

Alagille Syndrome Alliance

All Things Kabuki

Alpha-1 Foundation

Alport Syndrome Foundation

ALS Association

Alternating Hemiplegia of Childhood Foundation (AHCF)

American Behçet's Disease Association

American Brain Tumor Association

American Multiple Endocrine Neoplasia Support

American Partnership for Eosinophilic Disorders (APFED)

American Porphyria Foundation

Amniotic Fluid Embolism Foundation (AFE Foundation)

Amyloidosis Foundation

Amyloidosis Research Consortium, Inc.

Amyloidosis Support Groups, Inc.

APBD Research Foundation

Aplastic Anemia & MDS International Foundation, Inc.

Appendix Cancer/Pseudomyxoma

Peritonei Research Foundation (ACPPM)

APS Type 1 Foundation

Association for Creatine Deficiencies

Association for Frontotemporal Degeneration (AFTD)

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Association of Gastrointestinal Motility Disorders, Inc. (AGMD)

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AXYS

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Children's Craniofacial Association

Children's PKU Network

Children's Tumor Foundation

Chloe's Fight Rare Disease Foundation

Cholangiocarcinoma Foundation

Chromosome 18 Registry & Research Society

Chromosome Disorder Outreach, Inc.

Chronic Recurrent Multifocal

Osteomyelitis Foundation (CRMO)

Cicatricial Alopecia Research

Foundation (CARF)

CLOVES Syndrome Community

Cluster Headache Support Group, Inc.

Clusterbusters, Inc.

CMT Research Foundation (CMTRF)

CMTC Alliance

CMTC-OVM Cutis Marmorata

Telangiectasia

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Cystinosis Research Network, Inc.

Danny's Dose Alliance

Defeat NSA

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DHPS Foundation

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Erythromelalgia Association

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Fat Disorders Resource Society

Fibrolamellar Cancer Foundation (FCP)

Fibromuscular Dysplasia Society of America

Fibrous Dysplasia Foundation

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Foundation for Angelman Syndrome

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Foundation for Ichthyosis & Related Skin Types

Foundation for Prader-Willi Research

Foundation for Sarcoidosis Research

Foundation for USP-7 Related Diseases

FOXG1 Research Foundation

FPIES Foundation

Friedreich's Ataxia Research Alliance

Galactosemia Foundation

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**NORD: Fighting for the rare community every day for more than 35 years.**

NORD is committed to the identification, treatment and cure of rare disorders through programs of education, advocacy, research and patient support services. NORD does not recommend or endorse any particular medical treatment but encourages patients to seek the advice of their clinicians.

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