NORD®

LEADING THE WAY TO A

BRIGHTER TOMORROW

A Year of Innovation, Advancement and Progress for Rare Disease.

2021

ANNUAL REPORT

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The impact of the National Organization for Rare Disorders (NORD) has never been greater.

This year, we provided over ***$40 million in financial support*** through NORD Patient Assistance Programs, which ***reached more than 9,400 individuals and families***. Along with our ***336 member organizations*** – a group of incredible nonprofit and patient advocacy organizations around the country – we have made big strides in innovation, advocacy, programs, and strategic leadership in the rare disease space. In 2021, we welcomed ***25 new Corporate Council members***, bringing our network of companies engaging with experts in the rare community to ***128***. Throughout another year of virtual events and engagement, NORD welcomed thousands of attendees to our webinars and conferences and ***allocated more than $312,000 in research funding to seed grant awardees***.

Our work over the last 38 years has been made possible by partners and donors like you. NORD's motto – "Alone we are rare. Together we are strong" – is about the collective voice. From top to bottom, we emphasize collaborative relationships with the patient community, donors, companies researching and developing treatments and therapies for rare disorders, and all those who participate in our mission to improve the lives of people living with rare diseases.

NORD began in 1983 as a collective of patients and caregivers advocating for the Orphan Drug Act. From that first day, through to today, ***NORD has led the way to eliminate the struggle of rare diseases once and for all***. We can achieve this future together, through leadership, advocacy, research, and patient support.

NORD celebrates our past as we strive for a better future. We continue to add chapters to our rich history to make an impact for the 300 million people worldwide affected by rare disease. There are over 25 million Americans – 1 in 10 people – who are living with a rare disease today, but despite incredible advancements in research and technology over the last 40 years, more than 90% of the estimated 7,000 known rare diseases do not yet have an FDA-approved treatment.

With your support, NORD will continue to grow and impact lasting change, so that we can meet the needs of the national and global rare community for many years to come.

PETER L. SALTONSTALL
President and CEO

LEADING THE WAY IN

PATIENT SERVICES

As an integral resource to the rare disease community, we exist to help people navigate the challenges of living with a rare disease. Our goal is to provide access to the services and support the community needs to obtain the best care and treatment, and to help patients live their best lives.

Since 1983, we have been working alongside our community to find and provide resources that can assist with the financial burden that so often accompanies a rare diagnosis. We offer information to help the rare disease community navigate healthcare decisions. We also provide travel and lodging support services to those who are participating in various clinical trials and studies.

Thanks to your participation and support, we've expanded our services to offer the most comprehensive portfolio of person-centric assistance programs available today. One great example is NORD's respite caregiver program – the first-of-its-kind in the country for the rare community. Since the program's introduction in 2019 – and with support requests greatly exacerbated by the pandemic – we have provided 682 respite grants to rare disorder caregivers.

CHANGING LIVES

$40,698,802
in support provided to families

$693,784
given for non-medical relief

65,592
financial support claims processed

9,419
individuals received financial assistance

741
people received non-medical financial relief

145
people received clinical trial travel and lodging services

12
new patient assistance programs launched

50
states supported (plus Washington DC, Puerto Rico and Guam)

OUR COMMITMENT TO CONTINUED PROGRESS

With your support, we will advance our work and continue to:

- Deliver exemplary customer service for patients.
- Stay dedicated to sustainability so that we are here for the rare community as long as they need us.
- Partner with patients, their families and patient support organizations to understand and meet community needs.
- Provide information and services that meet the needs, preferences, and style of those we serve.
- Foster a culture of respect – one that values the life and experience of each individual – so that the individual feels fulfilled in every interaction with us.

> ***"NORD's Rare Caregiver Respite program was an absolute lifeline for our family this past year. With the respite program, I was able to select a family friend who I trusted, who was already adequately trained to handle my daughter's needs, and I could have her provide respite whenever I needed it. The reimbursement process was easy, and payment was received promptly."***
>
> **ANNA W.**
> *Parent*

LEADING THE WAY IN

EMPOWERING RARE NONPROFITS

Anyone affected by a rare disease can count on our 40-year history, vast industry connections, and the true strength that comes from having an organizational powerhouse in their corner. Through our growing network of professional patient organizations and partners, we engage and connect individuals, families and caregivers with the appropriate rare disease communities. We also help garner interest, attention and support for rare diseases from researchers, clinicians, industry stakeholders, regulators and legislators.

NORD's roots are to advocate for equity and inclusion of rare diseases in healthcare, research and treatment development fields. Our important work leveling the playing field continues to today, including through initiatives such as our Diversity, Equity and Inclusion (DEI) three-part webinar and toolkit series.

The program is open to all nonprofits and helped rare disease leaders better understand DEI as a concept and ultimately reach and represent a more diverse swath of patients and caregivers. NORD continues to help rare disease nonprofit leaders improve their capacity, strengthen their governance, connect with researchers and government regulators, and navigate research and drug development.

EMPOWERING LEADERS

Our work in 2021 resulted in growth across the rare disease community—or in some cases, established a baseline for growth.

336
member organizations

700
nonprofit leaders

25
members of our Rare Cancer Coalition®

3
RareLaunch startup organizations achieved 501(c)(3) milestones

1,171
views of our three-part webinar series "DEI for Rare Disease Nonprofits"

114
free passes awarded for the virtual 2021 NORD Summit®

1,000+
unique visitors worldwide viewed the RareLaunch® Forming a Foundation and Research Ready Workshops

14
webinars conducted that reached more than **3,110** leaders and other stakeholders

In 2021, we created the Hispanohablantes Advisory Committee, which works to engage diverse populations within the greater rare disease community.

RARELAUNCH®
RESEARCH READY WORKSHOP

Within our RareLaunch® program, two of our three ongoing startup organizations received 501(c)(3) designation and the third filed for designation. We developed a Learning Management System (LMS) to make the process of starting a nonprofit turnkey for leaders, while also supporting them in becoming ready to advance research. We held half-day virtual workshops with multiple sessions in April 2021, allowing members to hear from rare leaders and experts on how to move forward in these spaces.

OUR COMMITMENT TO CONTINUED PROGRESS

With your support, we will continue to:

- Expand our Diversity, Equity and Inclusion work with nonprofit leaders.
- Further our mission to reach those in the rare community representing the 6,000+ diseases without a nonprofit home and help them advocate for themselves by developing a nonprofit.
- Develop content and learning modules within the LMS to continue to make starting and growing a rare disease nonprofit turnkey.
- Grow our membership of nonprofit organizations.

> ***"NORD's RareLaunch® Program enabled me to fulfill my previously unattainable goal of launching a foundation. As someone with a rare and orphaned disorder, I enrolled in NORD's RareLaunch® webinar. Through the in-depth knowledge gained and the resources made available, I had the confidence to launch my own 501c(3) foundation. The ongoing support from the knowledgeable RareLaunch® team was invaluable in guiding me through every step of the process. Thank you RareLaunch®!"***
>
> **JO-ANN D'ANGELO**
> *Founder & Chair, Parry Romberg Foundation*

LEADING THE WAY IN

EDUCATION

At NORD, we use the power of education to attack the issues affecting real people's lives from a variety of angles. This work includes innovative partnerships and programs to:

- **EMPOWER AND EDUCATE:** We provide education on rare diseases, how to advocate for one's health, how to cope and find others with shared experiences, and how to navigate insurance and research opportunities.
- **INFORM AND ENGAGE:** We work with students who are entering healthcare studies or who are young advocates. These newcomers will likely encounter rare diseases in their careers and are a key audience to ensure patients are screened for rare diseases.
- **GUIDE AND SUPPORT:** We support healthcare professionals through continuing education and by providing resources to share with patients.

"It is truly hard to have a rare cancer and have financial hardship in time of the pandemic but assistance like what your foundation is giving to us patients [is] really life-changing and for me a miracle on its own. This is truly a gift/blessing!"

ANA
Patient

IMPACT AT A GLANCE

Hosted our third annual *Living Rare, Living Stronger®* NORD Patient and Family Forum – welcoming **389** attendees over the two-day program and made the content available post-event, reaching another **96** attendees.

Held the 2021 NORD *Rare Disease and Orphan Products Breakthrough Summit®* virtually for the second year. More than **940** people registered, representing **33** different countries.

Launched our first e-newsletter for healthcare professionals, called *Caring for Rare Quarterly*, which quickly grew into our **second** most viewed e-newsletter.

Significant growth in our Continuing Medical Education (CME) program. Completed **21** CME programs and partnered with **13** NORD member organizations to host **13,153** healthcare professional participants and **1,227** patients and caregivers.

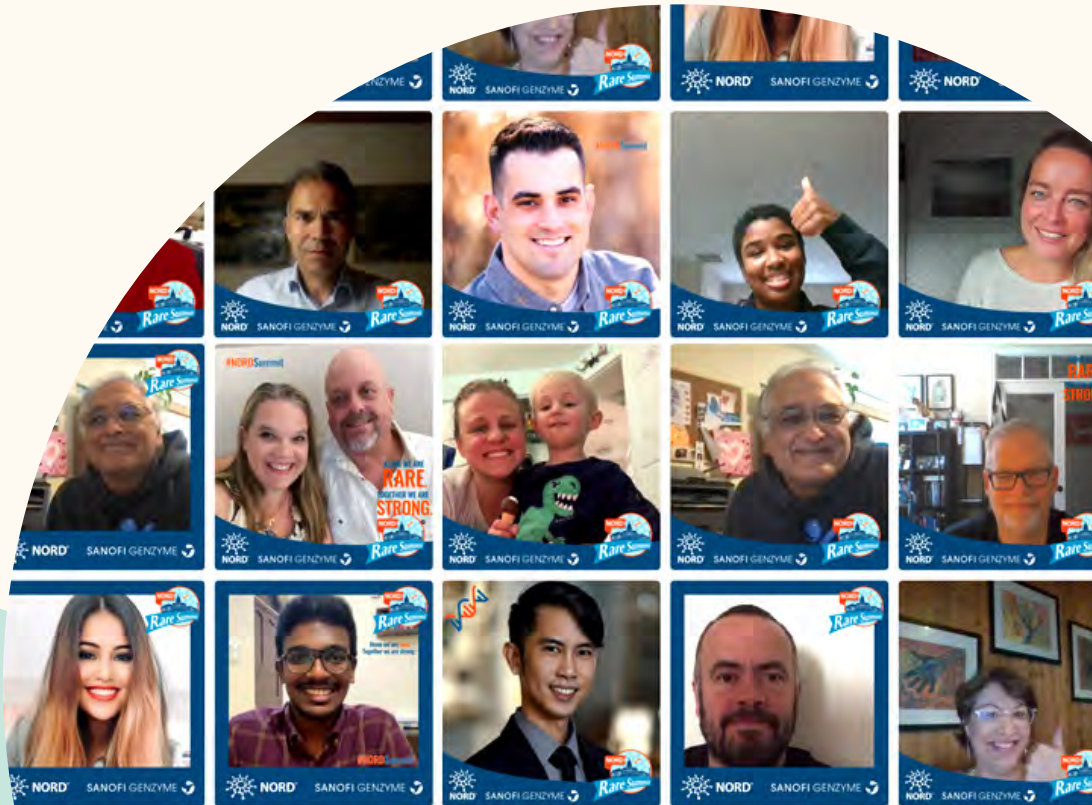
Published **20** new reports in the NORD Rare Disease Database, which is often the first place patients, caregivers, leaders, researchers and media contacts go to learn about a rare disease. Published **19** new rare disease videos in the NORD Video Library to complement rare disease reports and explain rare diseases to visual and auditory learners.

Formed **seven** new Students for Rare chapters. Ended the year with **25** student chapters and **10** student clubs.

OUR COMMITMENT TO CONTINUED PROGRESS

As we advance our work, we will:

- Continue our work on Diversity, Equity and Inclusion issues that affect the rare disease community.
- Produce educational videos on genetic testing capabilities, limitations, experiences and access.



LEADING THE WAY IN

RESEARCH

Patient groups are an important force in driving rare disease research forward. Our research and scientific activities provide the rare disease community with innovative tools and evidence to promote the engagement in impactful research and the fostering of medical advances. Over the past few years, we helped launch more than **40** rare disease patient registries and built systems that serve **tens of thousands of patients,** enabling them to advance groundbreaking scientific research. As part of our work, we:

- **SUPPORT** and empower the research of our member organizations through IAMRARE® – our registry platform – enabling research through the awarding of grants and actively engaging in research projects to improve data utility and research opportunities.
- **ADMINISTER** seed grant funding for basic and translational research that enables scientists to explore investigational treatments and cures.
- **ADDRESS** the gap in available rare disease relevant clinical outcome assessments, which are necessary for clinical trials.
- **PARTNER** with the Clinical Data Interchange Standards Consortium (CDISC) to develop rare disease-specific data standards.
- **CREATE** a culture of data-sharing and collaboration through our partnership with Critical Path Institute on the FDA-funded Rare Disease Cures Accelerator – Data and Analytic Platform (RDCA-DAP®).

RDCA-DAP®
Rare Disease Cures Accelerator
Data and Analytics Platform

> *"An important goal of NORD is to address the diagnostic odyssey, to address the lack of standardization of care-management and lack of coordinated multi-disciplinary care… So the overarching goal is to increase knowledge sharing across all rare diseases and across the country to really accelerate the progress in rare diseases with regards to diagnosis treatment and research."*

OLAF BODAMER
MD, PhD, Associate Chief of Genetics and Genomics at Boston Children's Hospital

IMPACT AT A GLANCE

*We helped **6** organizations launch their natural history studies on NORD's IAMRARE® research collaboration platform:*

The Aplastic Anemia and MDS International Foundation (AAMDSIF)
Paroxysmal Nocturnal Hemoglobinuria (PNH)

The Association for Creatine Deficiencies (ACD)
Cerebral Creatine Deficiency Syndromes (CCDS)

The Cure MLD and The Calliope Joy Foundation
Metachromatic Leukodystrophy (MLD)

The Cute Syndrome Foundation
SCN8A (also known as SCN8A Mutation, Cute Syndrome, SCN8A DEE and SCN8A Epilepsy)

The Gorlin Syndrome Alliance
Gorlin Syndrome

Tatton Brown Rahman Syndrome Community
Tatton-Brown Rahman Syndrome (TBRS, also known as DNMT3A Overgrowth Syndrome)

Reached a total of **14,875 individuals** across **29** different disease-specific registries through the IAMRARE® platform, who have collectively reported data for **143,253** surveys.

Thanks to donor generosity, we issued **$312,060** in research funding to seed grant awardees.

Our awareness activities for the RDCA-DAP reached **40,000** individuals with **4,000** unique views of webinars and our podcast.

OUR COMMITMENT TO CONTINUED PROGRESS

With your engagement, we will advance our work with plans to:

- **EXPAND** features within the registry platform to further power robust, high-quality rare disease research, including the ability to launch registry surveys in French and Spanish, and expand our efforts help registry sponsors develop new registries.
- **CONTINUE EDUCATING** for individual patients, caregivers and patient groups to understand the research landscape, drug development process, and data literacy programs.

LEADING THE WAY IN

POLICY AND ADVOCACY

For decades, we have pursued federal and state policies that have improved and continue to improve the lives of Americans impacted by rare diseases. NORD itself was founded by advocates who worked to pass the Orphan Drug Act (ODA), which has resulted in a dramatic increase in research and development of new treatments and cures for rare diseases. Supported by your participation, engagement and funding, our work includes:

- **BUILDING A MOVEMENT:** Through grassroots organizing across the country, we build community, encourage active participation and rally others to join in support of our mission.
- **FOREFRONT OF PUBLIC POLICY:** NORD works on the front lines of policy, government, and regulatory affairs to stand up for individuals with rare diseases and fight for life-changing policies.
- **STRONGER TOGETHER:** We build partnerships and join coalitions to raise awareness and advance our advocacy goals, demanding change from lawmakers that will ensure access to quality, affordable health care and new and better treatments.
- **DEVELOPING AND SHARING RESOURCES:** We connect individuals with local resources and empower them to make their voices heard.

"NORD's Project RDAC (Rare Disease Advisory Council) resources and materials were critical in helping the Massachusetts RDAC bring our first meeting to order in September 2021. Since that first meeting, NORD has connected us with a network of leaders from other established RDACs, who have provided us with guidance and support as we have developed our council's plan to make tangible improvements in the lives of people with rare diseases in Massachusetts."

DYLAN TIERNEY
MD MPH, MA RDAC Chair

OUR COMMITMENT TO CONTINUED PROGRESS

As we advance our work, we will:

- **ADVOCATE** in new states to increase the number of Rare Disease Advisory Councils (RDACs) and continue engagement with RDACs signed into law to ensure their effectiveness.
- **GROW** our reach with our advocacy network through new engagement initiatives and data-driven policy work.

IMPACT AT A GLANCE

STATE REPORT CARD®: Published the 6th edition of our annual State Report Card®, grading all 50 states based on how their policies impacted the lives of patients with rare disorders. We upgraded our website to include issue specific patient stories, as well as interactive maps for each state and policy area.

PROJECT RDAC: Developed several resources, including toolkits and model legislation, to assist with the creation of Rare Disease Advisory Councils (RDACs) at the state level.

21 states had signed legislation into law creating Rare Disease Advisory Councils by the end of 2021.

ADVOCATING FOR AFFORDABLE AND ACCESSIBLE HEALTH COVERAGE: In 2021, we advocated to improve access and affordability of health insurance at both the state and federal level.

A NORD-led sign on letter with **86** patient advocacy organizations was sent to Congress to oppose changes to the Orphan Drug Tax Credit under consideration as part of the Build Back Better Act.

TELEHEALTH ADVANCEMENT: Continued to engage in telehealth legislation at the federal and state levels to permanently protect access that was afforded during the pandemic.

238 organizations added their signatures to a NORD letter to governors to protect access to telehealth services from out-of-state providers.

LEADING THE WAY IN

COMMUNITY DEVELOPMENT

An important aspect of NORD's work is helping others understand what we do, the value of our programs and services, and, ultimately, the impact that we have. Through the tremendous generosity of our donors, we build partnerships, raise funds, and help others understand the depth of our commitment to serve the rare disease community. Likewise, we help donors understand what their support enables us to achieve and that we are good stewards of their financial support.

Thanks to our many financial partners and donors, we can make big strides to:

- **ELEVATE** the impact and value of our programmatic work.
- **DRIVE INNOVATION** and leadership in the rare disease sector.
- **EXPAND** and solidify our reach and collaboration with the community.
- **JOIN FORCES** with others through partnerships, fundraising, end-of-year campaigns and exhibiting at conferences.
- **NURTURE** collaborative relationships with donors (both individuals and family foundations), companies researching and developing treatments for rare disorders (including our Corporate Council Members), Foundation supporters, runners, community partners and all those who participate in and support our events.

> ***"Our belief is that the Center of Excellence program is the next big stride forward for rare disease treatment and patients—to improve health equity and create critical new connections to resources and specialists across our nation. NORD is committed to breaking down silos and building bridges so that people living with a rare disease can achieve their best health and well-being."***

ED NEILAN
Chief Scientific and Medical Officer, NORD

NEW INITIATIVE SPOTLIGHT: THE NORD RARE DISEASE CENTERS OF EXCELLENCE

Launched in November 2021, the NORD Rare Disease Centers of Excellence (CoE) Program brings together teams of clinical experts in a nationwide network of cutting-edge facilities, with the goal to provide standards of specialized care and disease management for people living with rare disease.

Patients will be able to access **31 designated centers** nationwide, with each center offering access to the best possible coordinated multi-specialty clinical care and diagnostic opportunities for rare diseases.

The centers that form the NORD CoE network will work in partnership to share expertise, advance education, define standards and protocols, and shape the field of rare disease care, ultimately enabling rare disease patients to better navigate their diagnostic journey and find qualified medical homes for their complex medical conditions.

OUR COMMITMENT TO CONTINUED PROGRESS

Together with partners like you, we will:

- Continue looking for opportunities to diversify fundraising streams through multi-year funding support and philanthropic partnerships.
- Expand the impact of our rare disease work to positively touch more lives.

OUR PARTNERS

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A Cure in Sight
A Twist of Fate-ATS
Achalasia Awareness Organization
Acid Maltase Deficiency Association
Acromegaly Community, Inc
ADNP-Kids Research Foundation
Adrenal Insufficiency United
Adult Polyglucosan Body Disease Research Foundation
Advocacy & Awareness for Immune Disorders Association
Alagille Syndrome Alliance
All Things Kabuki
Alpha-1 Foundation
Alport Syndrome Foundation
Alternating Hemiplegia of Childhood Foundation
American Behcet's Disease Association
American Multiple Endocrine Neoplasia Support
American Partnership for Eosinophilic Disorders
American Porphyria Foundation
Amniotic Fluid Embolism Foundation
Amyloidosis Research Consortium, Inc
Amyloidosis Support Groups, Inc
Angioma Alliance
Aplastic Anemia & MDS
Appendix Cancer / Pseudomyxoma Peritonei Research Foundation (ACPMP)
Association For Creatine Deficiencies
Association for Frontotemporal Degeneration
Association for Glycogen Storage Disease
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ASXL Rare Research Endowment Foundation
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AutoImmune Hepatitis Association
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AXYS
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Children's PKU Network
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Chromosome 18 Registry & Research Society
Chromosome Disorder Outreach, Inc
Cicatricial Alopecia Research Foundation
Cloves Syndrome Community
Clusterbusters, Inc
Cmt Research Foundation
CMTC-OVM Netherlands
Coalition to Cure Calpain 3
Congenital Central Hypoventilation Syndrome Family Network (CCHS Family Network)
Congenital Hyperinsulinism International
Consortium of Multiple Sclerosis Centers
COPA Syndrome Foundation
CRMO Foundation
CSNK2A1 Foundation
Cure Cmd
CURE HHT Foundation
Cure SMA
Cure VCP Disease, Inc
CureCADASIL/CADASIL Association, Inc
CureDuchenne
CureGRIN Foundation
CurePSP
Curing Retinal Blindness Foundation
Cushing's Support & Research Foundation, Inc
Cutaneous Lymphoma Foundation
Cystic Fibrosis Foundation
Cystic Fibrosis Research Inc. (cfri)
Cystinosis Research Network, Inc
Danny's Dose Alliance
Defeat Msa Alliance
Desmoid Tumor Research Foundation
DESSH DeSanto-Shinawi Syndrome Corp
DHPS Foundation
Dreamsickle Kids Foundation
Dup15q Alliance
Dyrk1a Syndrome Us
Erdheim-Chester Disease Global Alliance
Erythromelalgia Association
EURORDIS
Familial Dysautonomia Foundation
FamilieSCN2A Foundation
Fanconi Anemia Research Fund
Fat Disorders Resource Society, Inc
Fibrolamellar Cancer Foundation
Fibromuscular Dysplasia Society Of America
Foundation Fighting Blindness
Foundation For Angelman Syndrome Therapeutics
Foundation for Ichthyosis and Related Skin Types
Foundation For Prader-willi Research
Foundation for Sarcoidosis Research
Foundation for USP-7 Related Diseases
Foundation to Fight H-abc
FPIES Foundation
Friedreich's Ataxia Research Alliance (FARA)
Galactosemia Foundation
Gaucher Community Alliance
GBS|CIDP Foundation International
Genetic Alliance
Genetic Alliance Australia
Global Dare Foundation
Glut1 Deficiency Foundation
Gorlin Syndrome Alliance
Gould Syndrome Foundation
Grin2b Foundation
Gut Check Foundation
Guthy Jackson Charitable Foundation
Hcu Network America
HCU Network Australia
Helping Hands for GAND, Inc.
Hemophilia Federation Of America
Hepatitis Delta Connect - Hepatitis B Foundation
Hereditary Neuropathy Foundation
Hermansky-Pudlak Syndrome Network, Inc

Histiocytosis Association, Inc
Hope For Hypothalamic Hamartomas
HSAN1E Society
Hydrocephalus Association
Illness Challenge Foundation
Immune Deficiency Foundation
Indian Organization For Rare Diseases
International Autoimmune Encephalitis Society
International FOP Association, Inc
International Foundation for CDKL5 Research
International FPIES Association
International Neuroendocrine Cancer Alliance
International Pemphigus & Pemphigoid Foundation
International Rett Syndrome Foundation (dba. Rettsyndrome.org)
International Sacral Agenesis/Caudal Regression Association
International Wagr Syndrome Association
International Waldenstrom's Macroglobulinemia Foundation
ISMRD
Jack McGovern Coats Disease Foundation
Joshua Frase Foundation for Congenital Myopathy Research
Julia's Wings Foundation, Inc
KAT6A Foundation
Kennedy's Disease Association, Inc
Kif1a.org
Kindness for Kimberlee
Klippel Trenaunay Support Group
Krabbeconnect
LAL Solace, Inc
Li-Fraumeni Syndrome Association
Liv4TheCure
Lowe Syndrome Association, Inc
Lung Transplant Foundation
Lymphangiomatosis & Gorham's Disease Alliance, Inc
Malan Syndrome Foundation
Marfan Foundation
Martin Mueller IV Achalasia Awareness Foundation, Inc
M-CM Network
Mebo Research, Inc
Melanoma Research Foundation
Melorheostosis Association
Mila's Miracle Foundation
Mitoaction
MLD Foundation
Moebius Syndrome Foundation
Mowat-Wilson Syndrome Foundation
MPN Research Foundation
MSUD Family Support Group
Mucolipidosis Type IV Foundation, Inc
Multiple System Atrophy Coalition, Inc
Muscular Dystrophy Association
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Myhre Syndrome Foundation
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National MPS Society
National Niemann-Pick Disease Foundation
National Organization for Albinism & Hypopigmentation
National Pku Alliance
National PKU News
National Tay-Sachs & Allied Diseases Association
National Urea Cycle Disorders Foundation
NBIA Disorders Association
Necrotizing Enterocolitis Society
NephCure Kidney International
Neuroendocrine Tumor Research Foundation
Neurofibromatosis Network
Neurofibromatosis Northeast
Neuromuscular Disease Foundation
NTM Info & Research, Inc
Ocular Melanoma Foundation
OMSLife Foundation
Organic Acidemia Association
Organization for Rare Diseases India
Osteogenesis Imperfecta Foundation
Our Odyssey
PAP Foundation, Inc.
Parent Project Muscular Dystrophy
Parent to Parent New Zealand, Inc
Patient AirLift Services
Perthes Kids Foundation
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)
Project 8p
PSC Partners Seeking a Cure
PTEN Hamartoma Tumor Syndrome Foundation
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PURA Syndrome Foundation
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Reflex Sympathetic Dystrophy Syndrome Association
Remember The Girls
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Scleroderma Foundation
SETBP1 Society
Shwachman-Diamond Syndrome Foundation
Sick Cells
Sickle Cell Association of Houston, Inc.
Sickle Cell Association of Texas Marc Thomas Foundation
Sickle Cell Disease Association of America
Siegel Rare Neuroimmune Association
SLC6A1 Connect
Smith-kingsmore Syndrome Foundation
Snyder-Robinson Foundation, Inc
Sofia Sees Hope
Soft Bones, Inc

Spastic Paraplegia Foundation
Spina Bifida Association
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SSADH Association
Stevens Johnson Syndrome Foundation
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Team Telomere
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The ALS Association
The Avalon Foundation
The Bonnell Foundation: living with cystic fibrosis
The Cute Syndrome Foundation
The E.WE Foundation
The EHE Foundation
The Ehlers-Danlos Society
The FD/MAS Alliance
The Global Foundation For Peroxisomal Disorders
The Hyper IgM Foundation
The Hypersomnia Foundation
The Jansen's Foundation
The Lam Foundation
The Life Raft Group
The Mast Cell Disease Society
The Myositis Association
The Oley Foundation
The Progeria Research Foundation, Inc.
The Snow Foundation
The Yellow Brick Road Project
Transplant Unwrapped
TSC Alliance
Turner Syndrome Society of the United States
Tyrosinemia Society, Inc.
United Leukodystrophy Foundation
United Mitochondrial Disease Foundation
United Msd Foundation
Usher Syndrome Coalition
Vasculitis Foundation
Vestibular Disorders Association
VHL Alliance
Wilhelm Foundation
Williams Syndrome Association
Worldwide Syringomyelia & Chiari Task Force, Inc
Xia-Gibbs Society, Inc
XLH Network, Inc

NORD MEMBER

2021 CORPORATE COUNCIL MEMBERS

PRE-CLINICAL RESEARCH

Acer Therapeutics
Cabaletta Bio
Cellectar
gMendel
Jaguar Gene Therapies, LLC
Neurogene
Orna Therapeutics, Inc
Praxis Precision Medicines
Shape Therapeutics
Soligenix, Inc.
Stride Bio
Taysha Gene Therapies
Vigil Neuroscience, Inc.

CLINICAL DEVELOPMENT

Acer Therapeutics, Inc.
Aeglea BioTherapeutics
Apellis Pharmaceuticals
Applied Therapeutics
argenx
Ascendis Pharma
Asklepion Pharmaceuticals, LLC
Atara Biotherapeutics
Avadel Pharmaceuticals
AVROBIO
bluebirdbio
Blueprint Medicines
BridgeBio
Camurus
ChemoCentryx
CRISPR Therapeutics
Cytokinetics, Inc.
Dicerna
Editas Medicine
Enzyvant
Exicure
Helixmith Co, Ltd.
Insmed Incorporated
Kezar Life Sciences, Inc.
Kiniksa Pharmaceuticals
Larimar Therapeutics
LEO Pharma
Lysogene
MeiraGTx
Mereo BioPharma
Moderna Therapeutics
Neurocrine Biosciences
Orchard Therapeutics Ltd.
Orphazyme
Ovid Therapeutics
Passage Bio
PharmaEssentia
Prevail Therapeutics
Rallybio
REGENXBIO
Rocket Pharmaceuticals, Inc
Sangamo Therapeutics
Saniona
Santen
Savara
Sigilon Therapeutics
Synlogic Therapeutics
UniQure
Wave Life Sciences
X4 Pharmaceuticals
Zealand Pharma

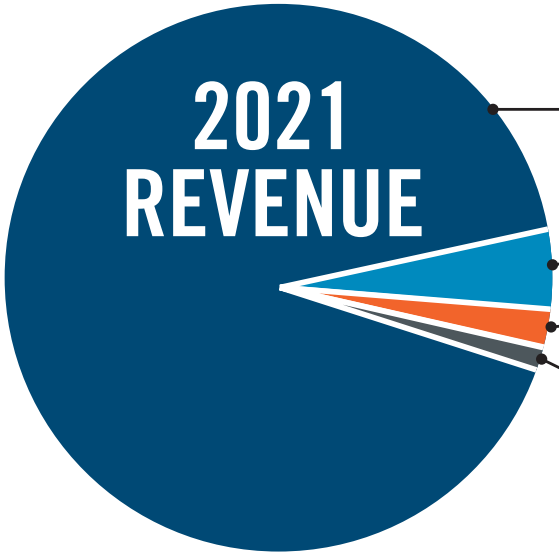
APPROVED PRODUCT

AbbVie Pharmaceuticals
Acadia Pharmaceuticals
Acceleron Pharma
Agios Pharmaceuticals
Alexion, AstraZeneca Rare Diseases
Alnylam Pharmaceuticals
Amgen
Amicus Therapeutics, Inc.
Astellas
AstraZeneca
BioCryst
Biogen
BioMarin Pharmaceuticals, Inc.
Boehringer-Ingelheim Pharmaceuticals, Inc.
Bristol –Myers Squibb
Catalyst Pharmaceuticals
Chiesi Global Rare Diseases
CSL Behring
Daiichi Sankyo
Deciphera
Dompé
Eiger BioPharmaceuticals
Foundation Medicine
Genentech, Inc.
GlaxoSmithKline PLC
Global Blood Therapeutics, Inc.
Greenwich Biosciences, Inc
Harmony Biosciences, LLC
Horizon Therapeutics
Incyte Corporation
Ionis Pharmaceuticals
Ipsen Biopharmaceuticals
Janssen Pharmaceuticals
Mallinckrodt Pharmaceuticals
Neurocrine Continental, Inc.
Novartis
Otsuka America, Inc.
Pfizer, Inc.
PTC Therapeutics
Recordati Rare Diseases, Inc.
Regeneron Pharmaceuticals, Inc.
Rigel Pharmaceuticals
Sanofi Genzyme
Sarepta Therapeutics
Sobi
Spark Therapeutics
Stemline Therapeutics
Strongbridge Biopharma
Takeda
Travere Therapeutics, Inc.
UCB Pharma
Ultragenyx
Vanda Pharmaceuticals Inc.
Vertex Pharmaceuticals Worldwide
Zogenix, Inc.

BUSINESS MEMBER

Biotechnology Industry Organization (BIO)
Eversana
HPS Group LLC
ICON
Illumina
Medidata
Optum Frontier Therapies
Parexel International
PhRMA
Syneos Health

FINANCIALS



$46,999,589 Patient Service Contributions and Fees

$2,145,585 Organization and Corporate Membership Dues

$1,888,596 Special Events

$8,720,385 Other Contributions, Grants and Service Revenue

$59,754,155 Total Unrestricted & Released Donor-Restricted Revenue

Program Services $52,898,299

General and Administrative $3,138,306

Development and Communications $1,694,897

Total Expenses $57,731,502

FINANCIALS

STATEMENTS OF FINANCIAL POSITION DECEMBER 31, 2021 AND 2020

ASSETS	2021	2020
Current Assets:		
Cash and cash equivalents	$13,488,659	$23,512,112
Accounts, grants and contributions receivable, net of allowance for doubtful accounts of $25,000 and $200,000 at December 31, 2021 and 2020, respectively	1,240,510	1,358,053
Prepaid expenses	351,001	368,116
Investments	20,808,366	25,613,110
Total Current Assets	**$35,888,536**	**$50,851,391**
Restricted Cash	--	$200,000
Investments - Deferred Compensation	147,299	108,394
Investments- Endowment	244,364	--
Deferred Hosting Arrangement Costs	74,147	109,919
Property, Equipment and Software, net	1,248,813	1,316,605
Software Under Development	272,087	188,231
Total Assets	**$37,875,246**	**$52,774,540**
LIABILITIES AND NET ASSETS		
Current Liabilities:		
Current portion of long-term debt	$74,640	$70,844
Accounts payable	513,301	587,335
Accrued expenses	922,684	662,999
Current portion of research grants payable	548,527	1,098,220
Deferred revenue	242,900	248,181
Total Current Liabilities	**$2,302,052**	**$2,667,579**
Deferred Compensation	$147,299	$108,394
Research Grants Payable, net of current portion	393,758	265,000
Long-Term Debt, net of current portion	90,440	165,915
Total Liabilities	**$ 2,933,549**	**$3,206,888**
Net Assets:		
Without donor restrictions:		
Operating	$7,789,738	$5,877,691
Property, equipment and software	1,355,820	1,268,077
Board designated endowment	22,863	--
Total Without Donor Restrictions	**$9,168,421**	**$7,145,768**
With donor restrictions:	$25,773,276	$42,421,884
Total Net Assets	**$34,941,697**	**$49,567,652**
Total Liabilities and Net Assets	**$37,875,246**	**$52,774,540**

STATEMENTS OF ACTIVITIES WITHOUT DONOR RESTRICTIONS FOR THE YEARS ENDED DECEMBER 31, 2021 AND 2020

	2021	2020
Changes in Net Assets Without Donor Restrictions		
Patient services:		
Program fees	$182,934	$345,353
Net assets released from purpose restrictions	43,844,645	39,755,627
Patient assistance and reimbursement expense	(44,198,311)	(40,100,980)
Patient services, net	(170,732)	--
Research grants:		
Net assets released from purpose restrictions	--	631,258
Research grant expense	--	(631,258)
Research grants, net	--	--
Other revenue and support:		
Grants, contributions and bequests	$3,363,185	$4,122,303
Special events revenue	1,888,596	1,900,601
Registry, web subscriptions and other related fees	1,000,570	395,674
Investment return, net	64,185	273,125
Drug, travel and lodging program administrative fees	44,877	67,425
Investment return - endowment, net	22,863	--
Net assets released from purpose restrictions - contributions	4,224,705	1,755,460
Net assets released from purpose restrictions - patient service administrative fees	2,972,010	3,756,095
Net assets released from time restrictions - membership dues	2,145,585	1,783,062
Net assets released from purpose restrictions - research grant administrative fees	--	52,000
Total Other Revenue and Support	**$15,726,576**	**$14,105,745**
Other operating expenses:		
Personnel and related	$10,006,667	$8,470,391
Professional fees	1,180,693	1,156,404
Occupancy	497,517	521,385
Data systems and equipment	487,430	456,801
Other	467,025	507,830
Depreciation	457,604	486,500
Conferences, meetings and travel	436,255	401,986
Total Other Operating Expenses	**$13,533,191**	**$12,001,297**
Changes in Net Assets Without Donor Restrictions	**$ 2,022,653**	**$2,104,448**

ALONE WE ARE RARE.
TOGETHER WE ARE STRONG.®

RareDiseases.org

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Connecticut office | 55 Kenosia Avenue, Danbury, CT 06810 | (203) 744-0100
Washington, D.C. office | 1779 Massachusetts Avenue, NW, Washington, D.C. 20036 | (202) 588-5700

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