NORD®
National Organization
for Rare Disorders

2022
IMPACT REPORT

Alone we are rare.
Together we are strong.®

The Struggle is Real.

Imagine living each day with uncertainty, battling physical limitations, financial strains, and feeling isolated and misunderstood.

Picture facing a rare life-threatening and/or life-altering medical condition without access to proper care or treatments. Consider the challenge of having a loved one with an untreatable condition, with no specialists available or physician familiar with their disease. Now, imagine your health declining rapidly for years without a diagnosis, despite countless medical appointments and tests. Add to this, battles with your health insurance company for medications.

Finally, ponder the exhaustion and heartache of advocating for yourself, your child or someone you love with a rare disease, while also trying to maintain a job and care for your family. For more than 25 million Americans with one or more rare diseases, this is an all-too-familiar way of life.

Individuals and families affected by rare disease(s) deserve the same equitable access to care, resources, support, and investment in research for treatments as do all other diseases.

The mission of the National Organization for Rare Disorders (NORD®) is to improve the health and well-being of people with rare diseases by driving advances in care, research and policy.

More than 93% of contributions to NORD® go to support patient programs and services.

Paving A Path Forward

For nearly 40 years, NORD has been championing advocacy and a path forward for individuals living with rare diseases. NORD's founders led the charge in establishing the Orphan Drug Act (ODA) in 1983, the world's first law incentivizing drug development for all rare diseases.

Every day since, NORD has tirelessly safeguarded the ODA, ensuring continued momentum in drug development while also championing programs and a path forward to improve the lives of those with rare diseases.

Defining the Problem

Rare diseases are not as prevalent as other more common diseases. A rare disease is defined as any disease, disorder, illness or condition affecting **fewer than 200,000 people in the U.S.**[1] Because fewer people have a given rare disease, less is known about them and it makes it harder to diagnosis, research and provide care to individuals with rare diseases.

More than 25 million Americans are living with a rare disease.[2]

Rare disease **devastates families emotionally and financially.**

Half of rare disease patients are children and 30% will not live to see their fifth birthday.[3]

With **over 7,000 rare diseases**, physicians face significant challenges in diagnosing, and treating individuals with a rare disease.[4]

The average time to receive an accurate **diagnosis is 5-7 years with medical costs 3-5 times higher** than non-rare diseases.[5]

Small patient populations make it **hard to find participants for research** which is needed for therapeutic development.

39% of rare disease patients **travel 60 miles or more to access medical care.**[6]

Most rare diseases **lack disease-specific patient organizations** which provide support and drive research for that specific rare disease.

Fewer than 5% of rare diseases have an FDA approved treatment.[7]

Sources:

[1] https://www.fda.gov/patients/rare-diseases-fda

[2] Genetic and Rare Diseases Information Center; National Center for Advancing Translational Sciences; FAQs About Rare Diseases; 11/30/2017.

[3] https://www.thelancet.com/pdfs/journals/landia/PIIS2213-8587(19)30006-3.pdf

[4] https://www.fda.gov/forindustry/developingproductsforraredieasesconditions/howtoapplyfororphanproduct-designation/ucm364750.htm

[5] https://ojrd.biomedcentral.com/articles/10.1186/s13023-021-02061-3

[6] https://rarediseases.org/wp-content/uploads/2020/10/NRD-2098-RareInsights-Telehealth-Report.pdf

[7] https://ojrd.biomedcentral.com/articles/10.1186/s13023-023-02790-7

2022 HIGHLIGHTS

ADVANCING PATIENT CARE

NORD RareCare® Patient Assistance

NORD's Patient Assistance program celebrated 35 years this year and was the first rare disease patient assistance program established in the U.S. The NORD RareCare patient assistance program is uniquely suited to serve the complex needs of rare disease patients. In 2022, NORD provided **$32.6 million** in financial support to individuals and families. See sidebar for **Patient Assistance Impact**.

Continuing Medical Education

To help improve rare disease patient care, NORD worked in partnership with Platform Q Health to educate medical professionals on rare diseases with high quality Continuing Medical Education (CME) courses. Together, NORD and Platform Q Health:

- Provided 83 educational sessions covering 35 rare disorders
- 28,768 Health Care Providers participated in CME sessions, and 14,876 CME credits were awarded.
- 93% of learners reported that the CME programs had a positive impact on their clinical practice and 91% of learners reported that CME programs had a positive impact on patient experience and outcomes.

Training the Next Generation with Students for Rare®

NORD's Students for Rare program is actively paving the way to educate and cultivate the next generation of rare disease healthcare professionals, medical experts and others interested in a career tied to rare disease. Highlights include:

- The addition of 23 new student chapters (up to 54 from 31) which is an increase of 74%.
- A total of 18 students traveled to the 2022 NORD Breakthrough Summit in Washington DC. With students from the University of Pennsylvania and Georgetown leveraging their learnings to publish a white paper, "Zebras Among Us: Advocating for the 30 million Americans Living with Rare Disease," published in Medical Science Educator *(AMA citation: Med Sci Educ. 2023 Aug 15; 33(5):1239-1242. doi: 10.1007/s40670-023-01856-2. eCollection 2023 Oct.).*

Patient Assistance Impact

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

$32.6 million
in support provided to families

$85,266
in respite care support provided to 121 patient families and caregivers

$347,229
provided in non-medical emergency relief support to 347 patients

55,586
patient claims fulfilled

221,685
travel miles reimbursed and nearly 800 hotel night stays

106,414
calls and emails responded

THE FUTURE OF RARE DISEASE PATIENT CARE & RESEARCH

NORD® Rare Disease Centers of Excellence - Year One

Launched in November 2021, the NORD® Rare Disease Centers of Excellence (NORD RD CoE) program is the first national network of U.S. hospitals and medical institutions dedicated to diagnosing, treating and researching all rare diseases. In its inaugural year, these Centers teamed up to solve the greatest medical challenges and unmet needs of the rare disease community.

Year-one accomplishments include:

- **Established Working Groups:** Identified the top 10 unmet needs and barriers to care for the rare disease community and established 10 working groups focused on building solutions.
  - > Established Co-Chairs for each group and successfully hosted monthly meetings with 75% attendance. Also, identified initial projects and delivered year-end progress reports.
- **Knowledge Sharing Platform:** Developed and successfully conducted beta launch of a knowledge sharing platform to help facilitate clinical care and research across the network.
- **Volunteer Network:** Established an active volunteer network made up of 250 medical and other healthcare professionals including: clinicians, researchers, advanced trainees, allied health professionals and support staff from across 20 areas of specialty, including pediatrics.
- **Launched Second Application Cycle:** Successfully launched a second application cycle open to any qualified medical institution. Held information webinar on November 16 and a virtual Q&A on November 30 with (54 attendees) combined. Applications due on February 23, 2023.
- **Meetings:** Held three virtual meetings with 97% attendance and 100% representation at the first in-person meeting held in October in Washington, D.C.
- **New Hires:** Hired a Program Assistant focused on Working Group and application cycle management and an Ontology Specialist to curate and validate rare diseases for the knowledge and resource sharing platform.

THE FUTURE OF RARE DISEASE PATIENT CARE & RESEARCH

CONTINUED....

2022 HIGHLIGHTS

IAMRARE® Program

Helped launch 2 patient registries with **All Thinks Kabuki,** Kabuki Syndrome Registry and the **Snow Foundation**, Wolfram Syndrome Global Patient Registry

At the end of 2022, there were **17 organizations' natural history studies** that were in progress.

We reached a total of **19,921 individuals across 31 different disease specific registries**, who have collectively reported data for 175,713 surveys.

In 2022, NORD, with funding from the patient member organization the APS Type 1 Foundation, **issued 1 grant in the amount of $50,000**.

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

In 2022, there were **3 RDCA-DAP sessions held** at NORD Breakthrough Summit:

- Patient Voice at FDA: Tips for Impactful PFDDs and Listening Sessions (130 attendees, standing room only)
- Why Data Matters and What You Can Do with It (100 attendees)
- Creating a Culture of Data Sharing (105 attendees)

DRIVING POLICY & ADVOCACY

In 2022, NORD actively engaged and empowered volunteers, its Rare Action Network® of more than 18,000 people, member organizations and other community members to improve the lives of people with rare diseases and their families through public policy advocacy. Together, with the rare community NORD helped amplify the voices of those with rare diseases to advance policy change at all levels of government to:

- Protect the Orphan Drug Act
- Improve the regulatory process and development of safe and effective therapies
- Quicken access to affordable and quality health care and treatments
- Reduce the time to diagnosis from an average of 5 years or longer

TOP HIGHLIGHTS

Published 2022 State Report Card

Released the 8th edition of the NORD State Report Card which which grades each U.S. state on critical issues impacting access to care for the 1 in 10 Americans living with a rare disease. This year's edition highlights areas of state policies that can benefit or jeopardize health care coverage, access, and affordability for rare disease patients.

Mobilized & Educated Volunteers

Coached 25 Community Ambassadors to raise awareness and build state-based coalitions nationwide.

Led nearly 100 volunteers to populate NORD's State Resource Database with local organizations and services to help patients find support close to where they live.

Activated nearly 70 volunteers to execute national Rare Disease Day® events on February 28.

Advocates

More than 18,500 Rare Action Network® (RAN) advocates, representing all 50 states, signed up to receive updates and to take action on federal and state policy issues.

Successfully **rallied RAN network members which led to the passage of key legislation**, including the establishment of Rare Disease Advisory Councils in four states (Colorado, Georgia, Connecticut, and Maine) as part of NORD's Project RDAC.

Together, the RAN network **raised their voices to help improve access to care**, joining action alerts focused on telehealth, accessing care across state lines, and protecting the Orphan Drug Tax Credit and market exclusivity provisions of the Orphan Drug Act.

1,300 new advocates joined the RAN network.

Community Sign-On Letters

RAN advocates sent **nearly 7,000 messages to more than 700 lawmakers**.

NORD HELP Committee Rare Act in FDASLA - **84 sign ons**

NORD Accelerating Kids Access to Care November 2022 - **109 organizations**

NORD Title V Reauthorization November 2022 - **125 sign ons**

BUILDING & EDUCATING THE COMMUNITY

Brritany Clayborne, a Patient Keynote speaker, at the 2022 NORD® Breakthrough Summit®

NORD Rare Diseases and Orphan Products Breakthrough Summit®

The 2022 NORD Rare Diseases and Orphan Products Breakthrough Summit welcomed 755 in-person attendees in Washington, DC and 38 virtual participants. Programming aimed to bring all key stakeholders back together again to address the most critical and timely topics in rare disease, such as: "Strategies for Enhancing Diversity, Equity and Inclusion in Rare Disease Research," "Creating a Culture of Data Sharing" and "Accelerated Approval; Doing it Right." Representation from government partners promoted awareness of rare disease programs at NIH and FDA and included the FDA Commissioner, a Fireside Chat with three FDA Center Directors (CBER, CDER and CDRH) and the NIH Town Hall.

Living Rare, Living Stronger® NORD Patient and Family Forum

Returning to in-person programming after two virtual years, the 2022 Living Rare Forum was held in Cleveland, Ohio. Educational sessions were centered on living your best rare life, and the forum provided educational and networking opportunities with safety as a top priority throughout the event. 299 patients and families living with rare diseases attended in-person, and 78 attended virtually. The most well-attended sessions were on the topics of "Building and Quarterbacking Your Care Team," "Beyond Coping: Resiliency While Rare," and "Rare Breakthroughs: Hope Now and on the Horizon."

Raising Dollars For Rare Disease One Step at A Time

In 2022, in its 14th year, the **NORD Running for Rare® program** raised over $200,000 with participation in the Boston and TCS New York City Marathons as well as three virtual Running for Rare Anywhere races.

ACKNOWLEDGEMENTS

NORD's Rare Giving Society recognizes the extraordinary generosity of individuals and family foundations who donate $1,000 or more to NORD annually. The generosity of these members enables NORD to improve the lives of those with rare disorders.

RARE GIVING SOCIETY MEMBERS

Worthington Olson
Linda Manasee Charitable Fund
Beth Ann Beson
Cinnia Edwards
Erin Fitzgerald
Fred Krimm
John & Nellie Bastien Memorial Foundation
Larry & Marina Wray
Max and Selma Kupferberg Foundation
Spirit For Life Foundation
Susan Burns
Thomas & Sandra Sibley
Tim Trow
Toby Tschirhart
Mr. & Mrs. Randy Stilman
Florence Ferguson Shine
Susan Matthews
The Raynie Foundation
Andrew LeGrand
Edward Patterson
Colin Blanch
Richard Leebove
David Piazza
Phil-Debbie Hoelscher
Donald Rissmiller
Vahe Zeroonian
Steven Grossman
The Roger Drake Charitable Fund
Anthony Hargrove
Rachael Larson
Salve Kutlow
Jamari Foundation
The de Rothschild Philanthropic Trust
Ara D. Cohen Charitable Lead Trust

RARE GIVING SOCIETY LEGACY MEMBERS

Alfred Selgas
Raymond Salza
Kay Family Foundation
Smidt Family Foundation Trust
Gregory & Carla Matthews
The Denise D'Ascenzo Foundation Inc
JF Maddox Foundation

2022 BOARD OF DIRECTORS

KAY HOLCOMBE, CHAIR
Senior Advisor, Milken Institute Center for Public Health

FREDERICK (RICK) E. BARR, MD, MBA
Executive Vice-President and Chief Clinical & Academic Officer, Arkansas Children's Hospital

SUSAN A. BERRY, MD
Professor, Medical Genetics & Metabolism, Dept. of Pediatrics, University of Minnesota

LEON EIDELMAN, CFA, TREASURER
JP Morgan & Chase

STEVEN GROSSMAN, JD, SECRETARY
President, HPS Group, LLC

SUSAN HEDSTROM
Executive Director, Foundation for Prader-Willi Research

DENNIS J. JACKMAN
Independent Board and Organizational Consultant

SHAFALI SPURLING JESTE, MD
Chief, Division of Neurology, Children's Hospital, Los Angeles

JIM PALMA
Executive Director, Target Cancer Foundation

PHILLIP L. PEARL, MD
Director, Epilepsy and Clinical Neurophysiology, Boston Children's Hospital

MIKE PORATH
Founder and CEO, The Mighty

PETER L. SALTONSTALL
President and CEO, NORD®

MARK SKINNER
President and CEO, Institute for Policy Advancement

2022 MEDICAL & SCIENTIFIC ADVISORS

SHAFALI SPURLING JESTE, MD, CHAIR
Children's Hospital Los Angeles / USC Keck School of Medicine

OLAF BODAMER, MD, PHD
Boston Children's Hospital / Harvard Medical School

GERALD COX, MD, PHD
Gerald Cox Rare Care Consulting, LLC

ADA HAMOSH, MD, MPH
Johns Hopkins University

MARTIN HO, MS, PHD
Google

PRIYA S. KISHNANI, MD
Duke University Medical Center

BRENDAN LEE, MD, PHD
Baylor College of Medicine

2022 CORPORATE COUNCIL MEMBERS

PRE-CLINICAL RESEARCH

Astria Therapeutics
Entrada
Jaguar Gene Therapies
Acer Therapeutics
Aeglea BioTherapeutics
Beam Therapeutics
Cabaletta
Coherus BioSciences
GMendel
Neurogene
Reneo Pharmaceuticals
Satellite Bio
Shape Therapeutics
Sigilon Therapeutics
Soligenix, Inc.
Stride Bio
Tenaya Therapeutics
Vigil Neurosciences

CLINICAL DEVELOPMENT

Acadia
Apellis Pharmaceuticals
Applied Therapeutics
Arcturus
Argenx
Ascendis Pharma
Atara Biotherapeutics, Inc.
Avadel Pharmaceuticals
bluebirdbio
BridgeBio
Camurus
CRISPR Therapeutics
Cytokinetics, Inc.
Dicerna
*** Acquired by Novo Nordisk*
Fulcrum Therapeutics
Kezar
Lysogene
MeiraGTx
Mereo Biopharma
Moderna Therapeutics
Ocugen
Orchard Therapeutics Ltd.
Ovid Therapeutics
Passage Bio
Praxis Precision Medicine
Prothena
Rallybio
Recursion Pharma
REGENXBIO
Rocket Pharmaceuticals Ltd.
Sangamo Therapeutics
Saniona
Santen
Savara, Inc
Springworks Therapeutics
uniQure
Wave Life Sciences
X4 Pharma
Zealand Pharma

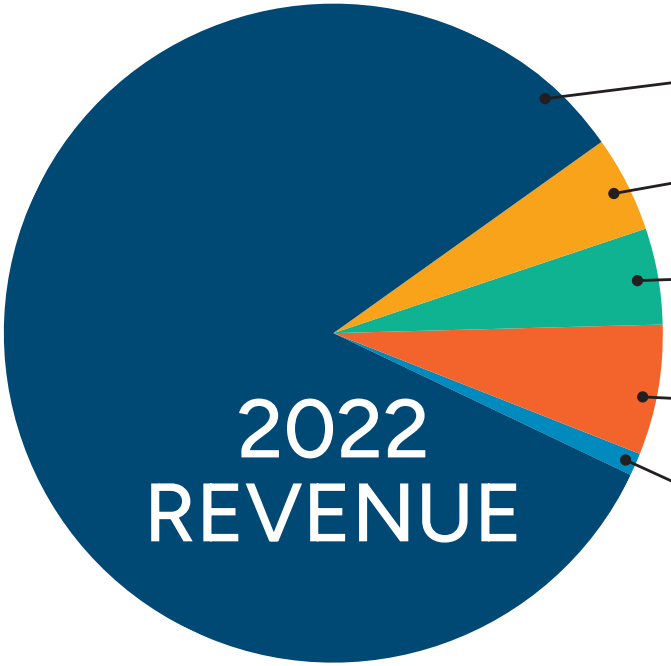
APPROVED PRODUCT

AbbVie Pharmaceuticals
Agios
Albireo
Alexion, AstraZeneca Rare Disease
Alnylam
Amgen
Amicus Therapeutics, Inc.
Amryt
Astellas
BioCryst
Biogen
BioMarin Pharmaceuticals, Inc.
Blueprint Medicines
Boehringer-Ingelheim Pharmaceuticals
Bristol-Myers-Squibb
Catalyst Pharmaceutical
ChemoCentryx
Chiesi Global Rare Diseases
CSL Behring
Daiichi Sankyo
Deciphera
Dompe
Editas Medicine
Eiger BioPharmaceuticals
Eli Lilly
Enzyvant
Foundation Medicine
Galderma
Genentech, Inc.
GlaxoSmithKline PLC
Global Blood Therapeutics, Inc.
Harmony Biosciences, LLC
Horizon Therapeutics
Incyte Corporation
Insmed Incorporated
Ionis Pharma
Ipsen
Janssen
Jazz Pharmaceuticals
Kiniksa
Kyowa Kirin
Mallinckrodt Pharmaceuticals
Merck
Neurocrine Biosciences
Novartis
Otsuka America
Pfizer, Inc.
PharmaEssentia
Pharming
PTC Therapeutics
Recordati Rare Diseases
Regeneron
Rigel Pharmaceuticals
Sanofi Genzyme
Sarepta Therapeutics
Sobi
Spark
Takeda
Travere
UCB Pharma
Vertex Pharmaceuticals Worldwide

BUSINESS MEMBER

Alira Health
AnovoRX Group
Biotechnology Industry Organization (BIO)
Eversana
ICON
Illumina
Medidata
Optum Frontier Therapies
Parexel
PhRMA
Syneos Health Inc

FINANCIALS



$39,907,322 Patient Service Contributions and Fees

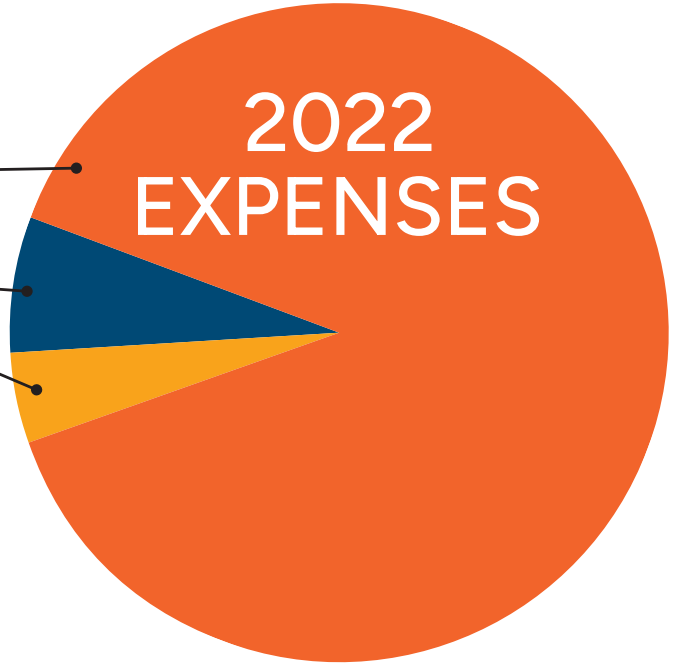
$2,269,050 Organization and Corporate Membership Dues

$2,239,534 Special Events

$3,076,161 Other Contributions, Grants and Service Revenue

$248,962 Investment Income

$47,741,029 Total Unrestricted & Released Donor-Restricted Revenue



Program Services $43,146,755

General and Administrative $3,101,339

Development and Communications $2,141,304

Total Expenses $48,389,398

FINANCIALS

STATEMENTS OF FINANCIAL POSITION DECEMBER 31, 2022 AND 2021

ASSETS	2022	2021
Current Assets:		
Cash and cash equivalents	$7,189,095	$13,488,659
Accounts, grants and contributions receivable, net of allowance for doubtful accounts of $25,000 at December 31, 2022 and 2021	11,078,737	1,240,510
Prepaid expenses	232,983	351,001
Investments	28,046,028	20,808,366
Total Current Assets	**$46,546,843**	**$35,888,536**
Investments - Deferred Compensation	106,602	147,299
Investments- Endowment	201,076	244,364
Deferred Hosting Arrangement Costs	106,140	74,147
Operating Lease Right-of-Use Asset	421,247	--
Property, Equipment and Software, net	2,160,212	1,520,900
Total Assets	**$49,542,120**	**$37,875,246**
LIABILITIES AND NET ASSETS		
Current Liabilities:		
Current portion of long-term debt	$77,294	$74,640
Accounts payable and accrued expenses	1,421,641	1,435,985
Current portion of research grants payable	752,777	548,527
Operating lease liability - short-term	207,322	--
Deferred revenue	214,816	242,900
Total Current Liabilities	**$2,673,850**	**$2,302,052**
Research Grants Payable, net of current portion	218,875	393,758
Operating Lease Liability - Long-Term	224,323	--
Deferred Compensation	107,385	147,299
Long-Term Debt, net of current portion	13,146	90,440
Total Liabilities	**$3,237,579**	**$2,933,549**
Net Assets:		
Without donor restrictions:		
Operating and board designated endowment	$6,460,678	$7,812,601
Property, equipment and software	2,059,374	1,355,820
Total Without Donor Restrictions	**$8,520,052**	**$9,168,421**
With donor restrictions:	37,784,489	25,773,276
Total Net Assets	**$46,304,541**	**$34,941,697**
Total Liabilities and Net Assets	**$49,542,120**	**$37,875,246**

STATEMENTS OF ACTIVITIES WITHOUT DONOR RESTRICTIONS FOR THE YEARS ENDED DECEMBER 31, 2022 AND 2021

	2022	2021
Changes in Net Assets Without Donor Restrictions		
Patient services:		
Program fees	$301,072	$182,934
Net assets released from purpose restrictions	32,627,130	48,069,350
Patient assistance and reimbursement expense	(33,039,894)	(44,198,311)
Patient services, net	(111,692)	(170,732)
Research grants:		
Net assets released from purpose restrictions	198,624	--
Research grant expense	(198,624)	--
Research grants, net	--	--
Other revenue and support:		
Grants, contributions and bequests	$2,683,947	$3,363,185
Special events revenue	2,239,534	1,888,596
Registry, web subscriptions and other related fees	781,560	1,000,570
Investment return, net	292,249	64,185
Drug, travel and lodging program administrative fees	102,071	44,877
Investment return - endowment, net	(43,287)	22,863
Net assets released from time restrictions - membership dues	2,269,050	1,828,085
Net assets released from purpose restrictions - research grant administrative fees	--	--
Total Other Revenue and Support	**$47,741,029**	**$59,754,155**
Other operating expenses:		
Personnel and related	$10,723,039	$10,006,667
Professional fees	1,155,992	1,180,693
Occupancy	495,394	497,517
Data systems and equipment	514,067	487,430
Other	431,493	467,025
Depreciation	477,010	457,604
Conferences, meetings and travel	1,383,887	436,255
Total Operating Expenses	**$48,389,398**	**$57,731,502**
Changes in Net Assets Without Donor Restrictions	**($648,369)**	**$2,022,653**

Alone we are rare. Together we are strong.®

NORD®
National Organization for Rare Disorders