



EXPANDING NORD'S NETWORKS

Meeting Continuing Challenges in
Rare Disease Communities

Marybeth McAfee, MA

Director, Medical Affairs, NORD



NORD[®]
National Organization
for Rare Disorders

Alone we are **rare**. Together we are strong.[®]

Disclosure

No Financial Relationships with Ineligible Companies: Nothing to disclose.

Our Mission

Improving the health and well-being of people with rare diseases by driving advances in care, research, and policy.



Learning Objectives

- Be aware of NORD's activities, services, and resources available for the rare disease community.
- Describe the NORD Rare Disease Centers of Excellence Program and how it expanded NORD's network to include clinicians, allied health professionals, support staff, and researchers in a new way.
- Give examples of NORD's efforts to reach historically medically underserved communities.

Agenda

- NORD History
- Patient Advocacy Groups Network
- Patient & Caregiver Network
- Policy and Advocacy Network
- NORD Rare Disease Centers of Excellence Network
- Latino Listening Sessions

HISTORY OF NORD

Parents on the White House Lawn



NORD[®]
National Organization
for Rare Disorders

Alone we are **rare**. Together we are **strong**.[®]

HISTORY

- **We are rooted in advocacy that spurred the rare disease movement.**
 - Passage of the Orphan Drug Act on Jan. 4, 1983
 - Incentives for rare disease drug development
- **Founded by patients, caregivers and patient organization leaders**
 - First national nonprofit to provide a home for rare disease patients and organizations
 - Fostered belonging, collective impact and unification



40 YEARS OF FIRSTS

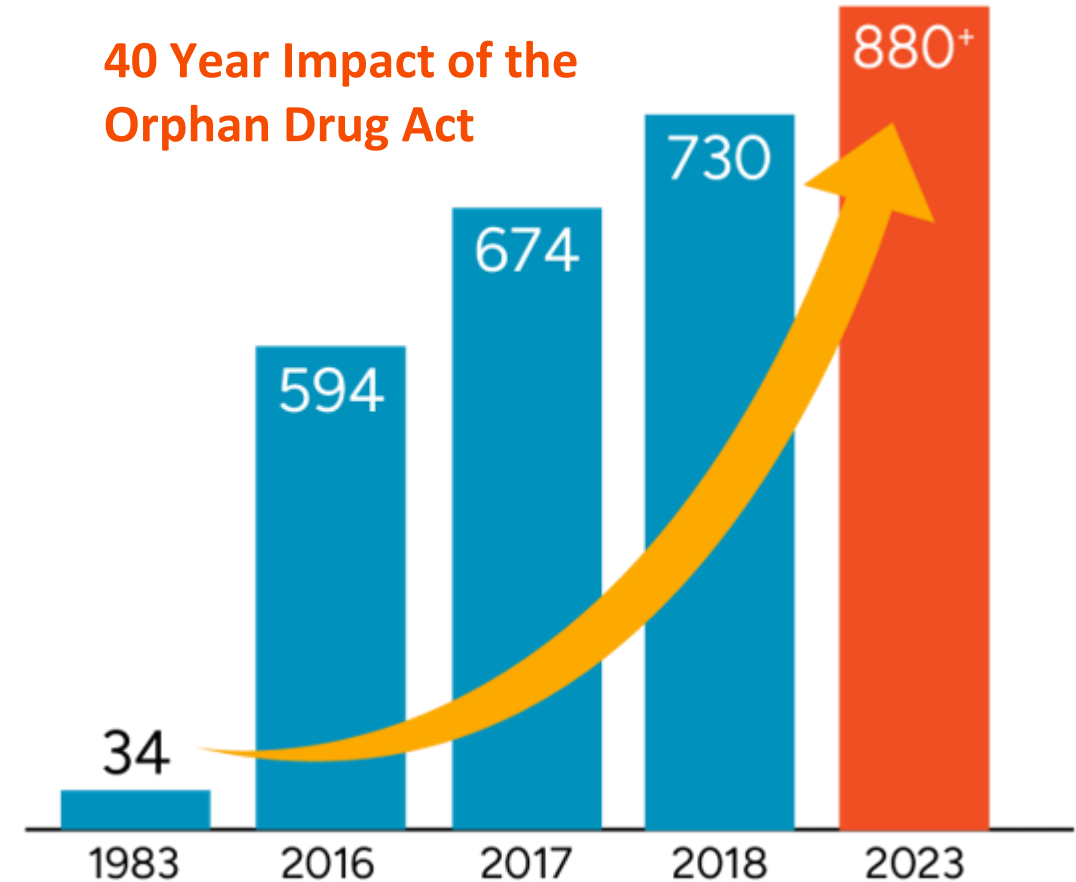
Orphan Drug Act (1983)

Patient Assistance Program (1987)

Launch Office of Rare Disease Research (1993) +
ClinicalTrials.org (2000)

Natural History Study Patient Registry – IAMRARE
(2014)

NORD Rare Disease Centers of Excellence (2021)



WHO IS NORD TODAY?

- Community Engagement
- Educational Initiatives
- Medical Affairs
- Membership
- Patient Services
- Public Policy
- Research
- Development
- Finance
- Human Resources
- Information Technology
- Marketing and Communications
- Strategic Programming & Planning
- Executive Team

PATIENT ADVOCACY ORGANIZATIONS

Our Membership Network



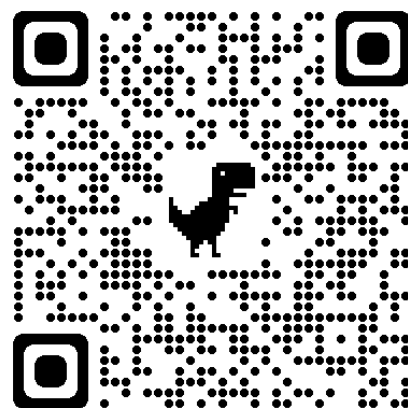
NORD[®]
National Organization
for Rare Disorders

Alone we are **rare**. Together we are strong.[®]

NORD'S PATIENT ADVOCACY GROUP NETWORK

- 330 patient organization members
- 1000+ in our Organizational Database
- 570 leaders engaged in Members Facebook Groups
- 301 Rare Launch Students
- 28 Leaders involved in Rare Cancer Coalition
- 60% led by founders
- 60% of founders are parents
- 50% all volunteer
- 36% 1-4 FTEs
- 47% < \$100,000
- 52% have a registry/ 33% plan to
- 65% work with industry
- 54% need help working with industry
- 71% help promote clinical trials

**Find a Rare Disease
Patient Organization**



THE ROLE OF PATIENT ADVOCACY GROUP NETWORK



- Develop Rare Disease Reports
- Inform Educational Webinar Topics
- Inform NORD's Policy Positions
- Advocate with NORD on Policy Issues
- Build Patient Registries & Offer Research Grants
- Request Patient Listening Sessions or Externally Led PFDDs
- Connect with NORD Corporate Council Members to Advance Their Research

HIGH-LEVEL OVERVIEW OF MEMBERSHIP PROGRAM

- **Membership Goals**

- To help members to mature and expand their ability to advance research & improve care
- To work with members to address gaps in information and resources they provide, including uplifting marginalized populations within their community
- Example – \$200K in COVID capacity grants, 60 + webinars, 100 scholarship awards annually to attend NORD's Rare Diseases & Orphan Products Breakthrough Summit

- **RareLaunch® is a program focusing on two audiences:**

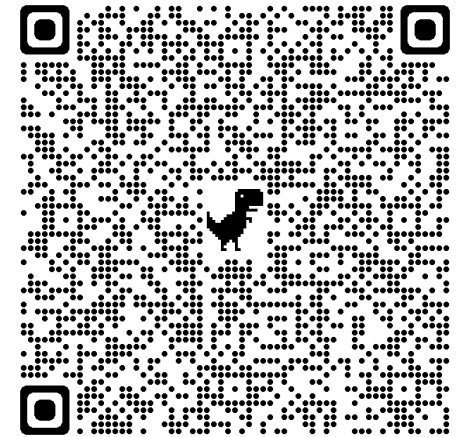
- “Forming a Foundation” helps individuals launch and grow a rare disease nonprofit organization
- “Research Ready” assists leaders of existing organizations to become better positioned to work with medical advisors and engage in research

- **Rare Disease Drug Development Series: What Patients and Advocates Need to Know**

- Part 1: Foundations and Pre-clinical Research - recently launched in English and Spanish

- **NORD and FDA Patient Engagement**

- 16 Patient Listening Sessions under NORD/FDA MOU
- 4 NORD-Coordinated Externally-Led Patient Focused Drug Development Meetings



**Learning Management
System Courses**

PATIENTS AND CAREGIVERS

The Network We Serve



NORD[®]
National Organization
for Rare Disorders

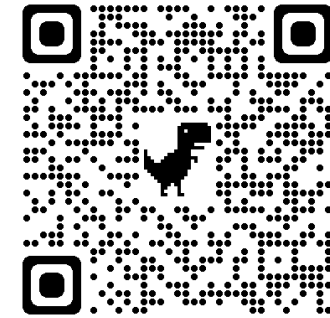
Alone we are rare. Together we are strong.[®]

SEEN AND UNSEEN – NORD PATIENT & CAREGIVER NETWORK

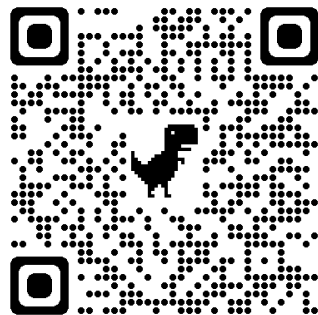
- NORD Rare Disease Reports



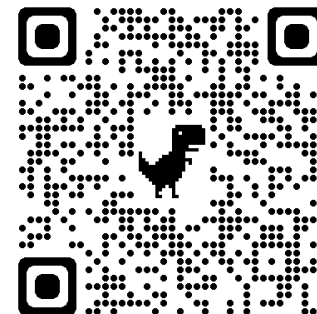
- Living Rare Living Stronger



- State Resource Center



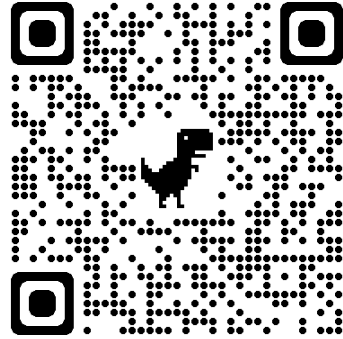
- Rare Diseases & Orphan Products Breakthrough Summit



DIRECT FINANCIAL ASSISTANCE

- Patient Services

- Financial Assistance
- Respite Program
- Educational Support



- NORD Support Helpline

- 1-800-999-6673
- Monday-Thursday 8:30 AM – 7:00 PM ET
- Friday 8:30 AM – 6:00 PM ET
- Si deseas hablar con alguien en español por favor llame al (844) 259-7178 para asistencia.

Making an Impact For Rare

In 2022, we provided assistance through **106** patient assistance and clinical trial support programs.

These programs provided access to care, treatment, diagnostics and clinical trial participation for more than **6,000** individuals.

NORD assisted with:


\$3.5 million
in premium payments


\$26.5 million
in prescription expenses


\$1.1 million
in expenses for travel to a rare disease related medical appointment or treatment


\$348,000
in emergency relief for critical non-medical needs

The impact of this assistance extends far beyond reducing the financial burden.

Rare Disease Day® U.S. Community Calls to Action

Show Your Stripes®

- *Wear stripes* on Rare Disease Day (black and white, zebra, or other)
- *Stripe out* your office, building, home, town
- *Show your stripes* social media with your story (I show my stripes fo

Light Up for Rare

- *Light up* your home in the RDD logo colors (Pink, Blue, Green)
- *Light up* local monuments, bridges, buildings and more



<https://rarediseases.org/rare-disease-day/>

POLICY AND ADVOCACY

Network of Change



NORD[®]
National Organization
for Rare Disorders

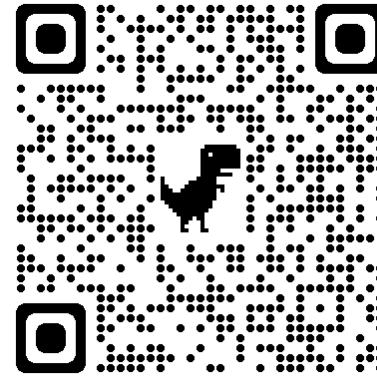
Alone we are rare. Together we are strong.[®]

WHY PATIENT ENGAGEMENT IN POLICY MATTERS

The Policy team primarily supports efforts to improve the health and well-being of people with rare diseases by driving advances in policy.

Problem: Elected officials often do not understand the challenges facing people with rare disorders and how they can help (or hurt!).

Solution: NORD works with our Member Organizations, grassroots advocates through RAN and our Taskforces, patients, and caregivers to **elevate the voice** of the rare disease community in government by identifying the policy problems and advocating for solutions that can benefit our community.



Get Involved in Advocacy



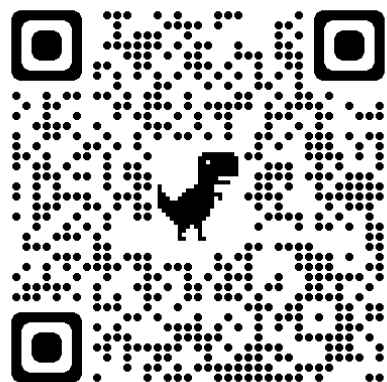
Alone we are rare. Together we are strong.®

RARE DISEASE ADVISORY COUNCILS (RDACs)

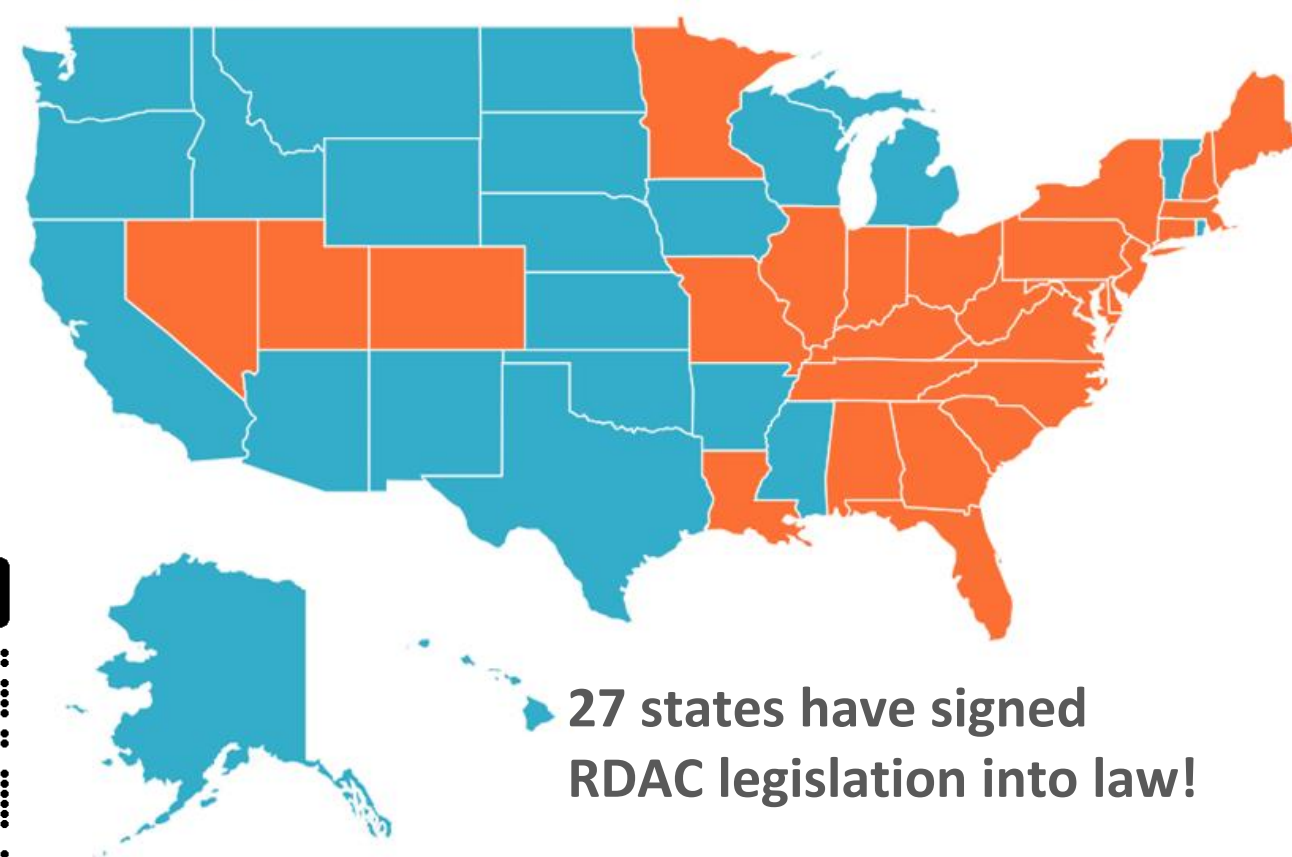
A Rare Disease Advisory Council (RDAC) acts as an advisory body that gives the rare disease community a stronger voice in state government.

NORD's Project RDAC is designed to optimize the existing RDACs and to increase the number of RDACs across the country.

More info about
RDACs



■ State has an RDAC ■ State does not have an RDAC

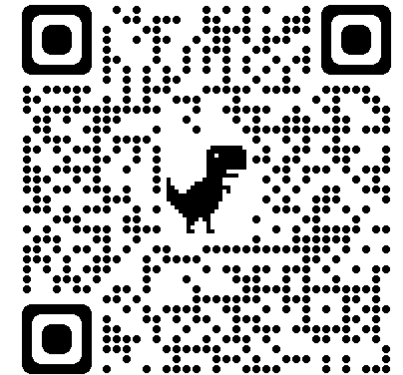


27 states have signed
RDAC legislation into law!

Alone we are rare. Together we are strong.®

9th ANNUAL STATE REPORT CARD JUST RELEASED

- ✓ Medicaid Financial Eligibility
- ✓ Medical Nutrition
- ✓ Newborn Screening
- ✓ Prescription Drug Out of Pocket Costs
- ✓ Protecting Patients in State Medicaid Programs/ State-Regulated Insurance
- ✓ Rare Disease Advisory Councils
- ✓ Step Therapy (Fail First)
- ✓ Telehealth



**2024 State Report
Cards**

RARE DISEASE CENTER OF EXCELLENCE

Network of Clinicians and Researchers



NORD[®]
National Organization
for Rare Disorders

Alone we are rare. Together we are strong.[®]

NORD RARE DISEASE CENTERS OF EXCELLENCE PROGRAM

NORD Rare Disease Centers of Excellence (NORD RD CoE) is the first of-its-kind network of U.S. medical institutions dedicated to diagnosing, treating, and researching ALL rare diseases.

The Program was officially launched on November 4th, 2021, with the announcement of the first 31 designated RD CoE.

On May 4th, 2023, an additional 9 RD CoE were designated bringing the total to 40 on NORD's 40th Anniversary.

Currently the Program only accepts applications from institutions based in the United States.



- ***Vision:*** All persons living with a rare disease, regardless of disease, socioeconomic level, or demographics, have access to timely diagnosis, quality, compassionate clinical care, research opportunities, and supportive resources.

QUALITIES OF NORD RARE DISEASE CENTERS OF EXCELLENCE

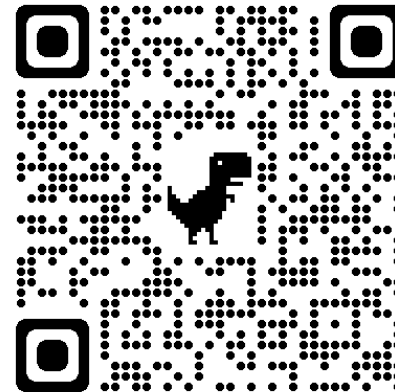
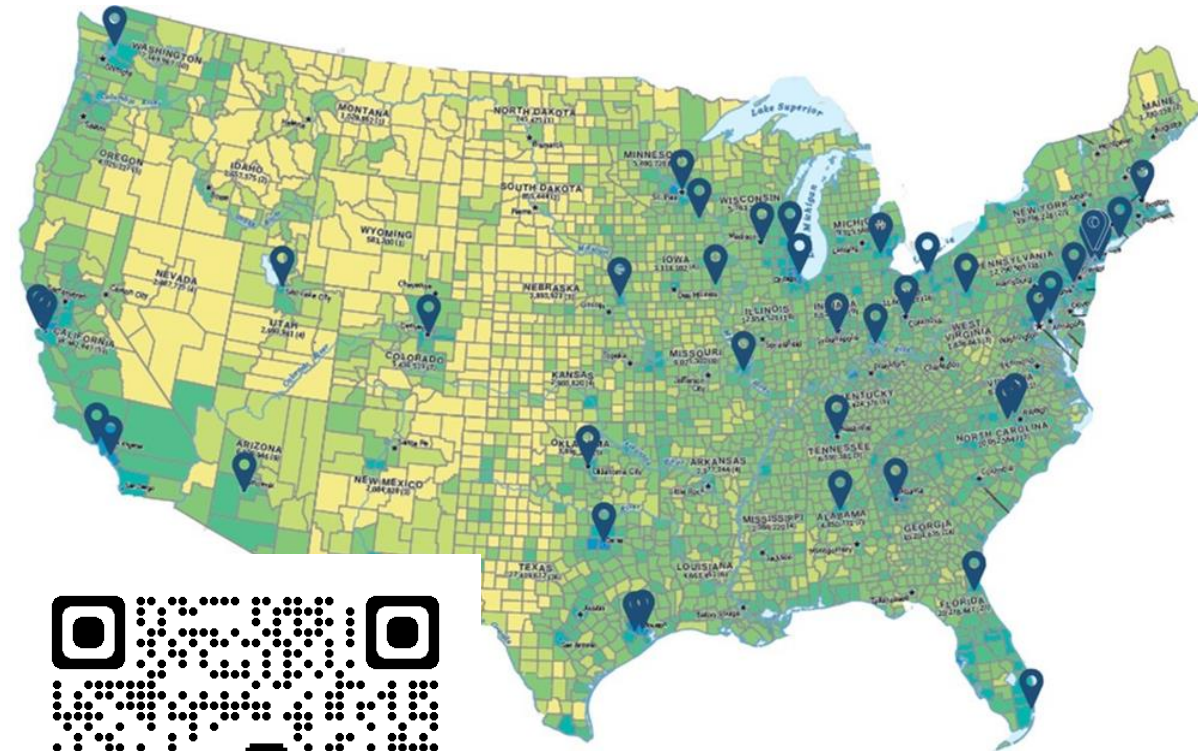
Commitment to rare disease patients & families, including education

Experts in all specialties needed for diagnosis & treatment of rare diseases in children & adults, including coordinated, multi-specialty care

Support for the transition of care

Train the next generation of rare disease specialists and researchers.

Actively contribute to rare disease research and to new models of therapy and care for rare diseases



Directory of NORD
Centers of Excellence

Key Unmet Needs ?	Working Groups
Special Challenges in operating a general rare disease center of excellence	Director’s Workgroup* on Rare Disease CoE Mechanics and Best Practices
Long Diagnostic Odyssey	Diagnostic Approaches / Access to Testing (Diagnostic Odyssey)
Scattered Patients & Rare Expertise	Telemedicine / Inter-site Consultation
Very Few Approved Treatments	Facilitating Multi-site Research & Clinical Studies
Often Limited Clinical Data (Especially for new and ultra-rare diseases)	Shared Data Collection & Analysis
Few Treatment Guidelines and Standards of Care	Treatment Guideline Curation & Development
New demands in delivering innovative therapies in the clinical setting	Clinical Application of Newly Approved Treatments/Label Indication Expansion
Gaps in patient education resources	Patient Education
Workforce limitations / Gaps in medical professionals’ rare disease knowledge and education	Medical Professional Education
Gaps in insurance coverage and adolescent to adult transition of care	Policy & Collective Advocacy/ Transition of Care
Inequities in access to rare disease treatment and in the education and training of rare disease specialists	Diversity, Equity, and Inclusion
Efforts to implement WGS as primary NBS often circumvent State NBS Advisory Committees and often don’t reflect the expertise of key stakeholders with a deep understanding of the challenges and processes of the complex U.S. NBS system.	Newborn Screening
Limited resources and education available in other languages/countries for patients and providers.	International Collaboration

PROFESSIONS REPRESENTED ON THE WORKING GROUPS

- Bioethicist
- Bioinformaticians
- Certified Genetic Counselors
- Clinicians
- Clinician/Researcher
- Clinical Trials Coordinator
- Dietician
- Directors of Clinical Trials Program
- Directors of Research & Training Programs
- Genetic Counseling Assistant
- Lab Directors
- Nurse Practitioners
- Pharmacist
- Physician Assistants Physicians
- Project Managers
- Professors (Assistant, Associate, Full, Emeritus)
- Rare Disease Program Coordinator
- Rare Disease Program Directors
- Registered Nurses
- Research Coordinators
- Researchers
- Social Worker
- Senior Practice Manager
- Senior Business Manager
- Senior Trainees
- Telehealth Coordinator

SPECIALTIES /SUBSPECIALTIES REPRESENTED ON WORKING GROUPS

- Cardiology
 - Dermatology
 - Endocrinology
 - Gastroenterology
 - Hematology Oncology
 - Immunology
 - Internal Medicine
 - Interventional Radiology
 - Medical Genetics and Genomics
 - Clinical Biochemical Genetics
 - Clinical Genetics and Genomics
 - Laboratory Genetics and Genomics
 - Neonatology
 - Nephrology
 - Neurodevelopmental Pediatrics
 - Neurology
 - Pediatrics
 - Pharmacology
 - Pulmonology*
 - Reproductive Health
 - Rheumatology*
- **all except where noted include ped & adult*

GOALS OF NORD RD CoE PROGRAM FROM PATIENT PERSPECTIVE

- Sharing expert rare disease knowledge so patients and caregivers will have to travel less to receive optimal care.
- Increasing rare disease research so more rare disease patients have opportunities to enroll in clinical trials (often the only form of treatment available).
- Advocating for equity in access to diagnosis and care. Ensuring every rare disease patient feels welcome at any RD CoE.
- Spearheading efforts to increase the number of rare disease specialists so more doctors are available to diagnose and manage rare diseases.

REACHING NEW COMMUNITIES

Expanding for the Future



NORD[®]
National Organization
for Rare Disorders

Alone we are **rare**. Together we are strong.[®]

RARE DISEASE DIVERSITY SURVEY - UNSUCCESSFUL

- Worked in partnership with the Rare Disease Diversity Coalition
- Meant to capture the experience of people of color living with a rare disease
- Also targeted Latino, Immigrant, and LGBTQIA+ communities
- Majority of the respondents were adult white females in low to middle SES.



LATINO COMMUNITY LISTENING SESSIONS - SUCCESS

- From April to October, we held 6 In-Person in NYC, DC, Houston
- 1 virtual session was held in June
- The listening sessions were open to all Latino rare disease patients regardless of disease state.
- Multiple disease classifications were represented including undiagnosed
- All participants were followed up after sessions.



Thank You for Your Attention

Marybeth McAfee, MA

mmcafee@rarediseases.org



Alone we are rare. Together we are strong.®