Welcome to the Annual NORD Member Meeting

October 19, 2025

Sign in with your name and organization at menti.com | use code 8104 7688

NORD®
National Organization
for Rare Disorders

Alone we are rare. Together we are strong.®

Our Mission

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

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Let’s Get to Know Each Other

Raise your hand if you are:

- Local or Out-of-towner
- West Coast, Midwest, South, East Coast, International
- All Volunteer Organization or have FTEs
- Your Rare Disease has <100,000, <50,000, <10,000 patients in the USA
- Have a Research Program
- Work with Industry
- Have the best patient community

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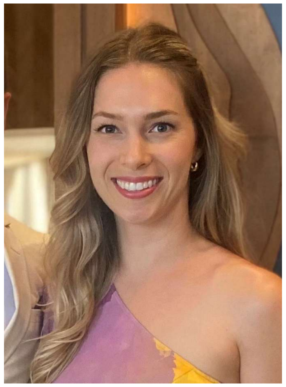
Your NORD Membership Team


Alli Ward
Director of
Membership


Allie Crafton
Membership
Manager


Alex Caron
Membership
Manager


Martha Alyea
Community &
Corporate Affairs
Coordinator


Jake Saltonstall
Community
Engagement
Associate

Membership@RareDiseases.org

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Agenda

- 2:00 – 2:30 pm: Welcome & Member Address
- 2:30 – 3:30 pm: Executive Insights
  Followed by a 10-minute networking break
- 3:40 – 4:10 pm: Aligning on Impact: Member Feedback Forum
- 4:10 – 4:40 pm: Networking Roundtables: Fundraising, Research, and Capacity Building
  Followed by a 10-minute networking break
- 4:50 – 5:30 pm: Registries and Member Opportunity
- 5:30 – 5:50 pm: PAG Engagement in Patient Support Services
- 5:50 – 6:00 pm: Closing Remarks & Group Photo

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Member Meeting Resources

Scan the QR Code at your table to access the Member Meeting microsite with the materials used in today's presentations

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Welcome!

Alli Ward

Director of NORD Membership

NORD® National Organization for Rare Disorders

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ALONE WE ARE RARE.
GETHER WE ARE STRONG.®
Alli Ward

Welcome!

Pamela Gavin

NORD Chief Executive Officer

NORD® National Organization for Rare Disorders

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NORD Rare Impact Awards®

The Abbey S. Meyer Leadership Award
2025 Honoree

Hermansky-Pudlak Syndrome Network

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Executive Insights Panel

Speakers

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Executive Insights

Kelly Esperias
Chief Strategy &
Operations Officer
NORD

Rhonda Rowland,
President
Wilson Disease Association

Ryan Maple,
Executive Director
Global Foundation for
Peroxisomal Disorders

Cynthia Ryan,
Executive Director
Vestibular Disorders
Association

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Questions & Answers

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10 Minute Break

We'll be right back at 3:40 PM

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Aligning on Impact Member Feedback Forum

Moderator: Pamela Gavin, CEO

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Your Feedback Fuels Our Mission

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

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Aligning on Impact Member Feedback Forum

2025 Survey Results

91% Advocate for the development of effective treatments for diseases that have no approved treatments

90% Increase patient access to appropriate care and treatment.

86% Accelerate rare disease research and increase patient participation in research

86% Empower the rare disease community to engage in advocacy and meaningful collaboration to improve the quality of life for people living with a rare disease.

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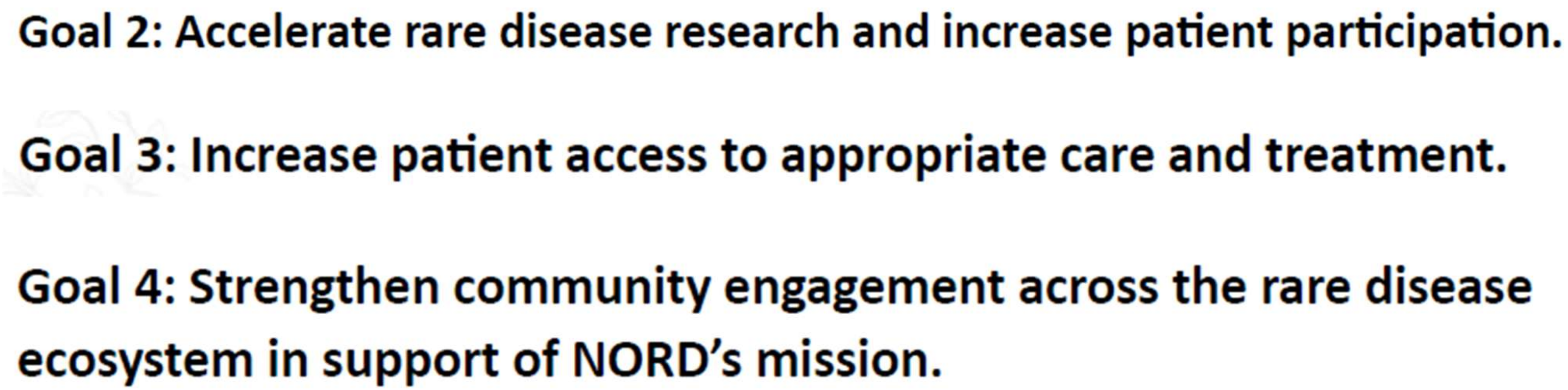
Aligning on Impact Member Feedback Forum

2023 - 2025 Strategic Plan



Goal 2: Accelerate rare disease research and increase patient participation.

Goal 3: Increase patient access to appropriate care and treatment.

Goal 4: Strengthen community engagement across the rare disease ecosystem in support of NORD's mission.

Time for a Poll!

Join us at menti.com

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How You Can Get Involved

NORD Board Membership Committee

- Drive development of new member benefits and resources
- Drive development of organizational programming

NORD Board of Executive Directors

- Shape the future of the rare disease community
- Collaborate with national leaders and innovators

Jim Palma
CEO, Target Cancer Foundation;
NORD Board Member

Thank you for three terms of outstanding leadership!
(2016 – 2025)

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Mike Porath
Executive Director, Dup15Q;
CEO, The Mighty
NORD Board Member

January 1, 2025
Welcome to the team!

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Questions & Answers

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Networking Roundtables: Fundraising, Research, and Capacity Building

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Networking Roundtables

- Use the question sheets at your table to choose discussion topics
- Try to discuss at least **3 questions** as a table – don't forget to introduce yourself and your organization!
- Use the index cards at your table to write down key takeaways

Challenge: Learn something new from someone you haven't met before!

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10 Minute Break

We'll be right back at 4:50 PM

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Registries and Member Opportunity

Janet Mauro (NORD), Rebecca Aune (NORD), Jennifer Levy (C3)

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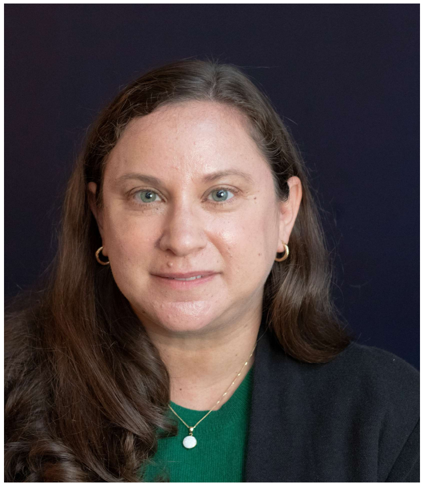
Registries and Member Opportunity

Alex Caron
Membership Manager

Janet Mauro
IAMRARE Implementation Lead & Regulatory Specialist
NORD

Rebecca Aune, MPA
Director of Education Programs
NORD



Jennifer Levy
Scientific Director
Coalition to Cure Calpain

Using Patient Registries to Advance Research and Treatment

Janet Mauro, CCRC

IAMRARE® Implementation Lead, Regulatory Specialist

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Overview

What we will discuss today:

- What are Patient Registries and Natural History Studies
- How collected data can be used
- How the data is protected
- Should my Advocacy Group Sponsor a Registry? Are we ready?
- NORD’s IAMRARE Onboarding Program

Patient Registries and Natural History Studies

Patient Registry – a collection of standardized information about a group of patients with a shared condition. The individual Participants are all responding to the same questions, asked in the same way. The information gathered can be used in many ways.

Natural History Study – tracks the course of a disease over time through data collected in a Patient Registry. Participants answer questions on a set schedule such as every 6 months, annually, etc. This kind of study follows the participant throughout their lifetime.

Patient Registries and Natural History Studies

Natural History Studies – are used in many ways. The data may:

- Identify common traits within the participant population and outcomes that were previously unknown;
- Support the design of clinical trials exploring interventions and treatments;
- Be used as a control within a trial;
- Support clinical trial recruitment;
- Help develop patient care best practices and standards of care, and diagnostic and treatment protocols;
- Provide information for a PFDD (Patient focused drug development) meeting;
- Assists researchers studying the pathophysiology of the disease; and
- Include varied sources such as Participant entered surveys, medical reports, chart extraction and other kinds of records.

Data Protection

Patient registries collect a great deal of private, protected health information. A registry platform should be built with data protection in mind, such as:

- Applying the principles of HIPAA , PHI protections, and other regulations related to data privacy and safety to the platform itself, as well as in the training of study staff – novices and experienced researchers alike;
- Applying a randomly generated code to each Participant's data;
- Setting standards to the types of data to be shared with researchers
- Using safety mechanisms such as data encryption for transmitted data;
- Ensuring secure servers are compliant with US and International regulations; and
- Engaging the services of an IRB to provide ethical oversight to the registry.

Is our Patient Advocacy group ready to sponsor a registry?

Some things that should be in place for success before starting this journey

- You've established a presence in your Rare Disease community as a trusted resource;
- You are already a 501c3 entity;
- You are a member of NORD or well into the process of joining;
- You have a cohort of medical and research professionals to provide support through a registry advisory board;
- You have a cohort of patients and family members to provide input to what parts of their diagnostic and treatment journey should be captured in surveys; and
- You have employees or dedicated volunteers who collectively can devote at minimum 20+ hours a week to the registry in development and once launched.

What is the IAMRARE Onboarding Program?

The IAMRARE Onboarding Program is delivered in cohorts of PAGs three times a year.

Key components:

- Education on topics for novice and experienced researchers.
- Guidance in preparing regulatory documents.
- Guidance in developing custom, disease-specific surveys to your specific needs.
- Templates to use as starting points for every aspect of developing a registry.
- Registry staff receive training in Good Clinical Practice.
- All registries hosted on the NORD IAMRARE platform must be under the guidance of an Institutional Review Board (IRB) to assure that the rights and welfare of research participants are protected

Why should my members participate in our Registry?

In your registry, each participant or their loved one

- Gets to tell the story of their journey
- Their journeys are added to the journeys of others
- Many voices together can make a compelling story, and gain attention from researchers

Patient Registries are:

Participant (Caregiver) Entered, Participant Centered, Participant Driven

Thank You!

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Making Sense of Health Data

Building Data Literacy in the Rare Disease Community

Rebecca Aune, MPA
Director of Education Programs

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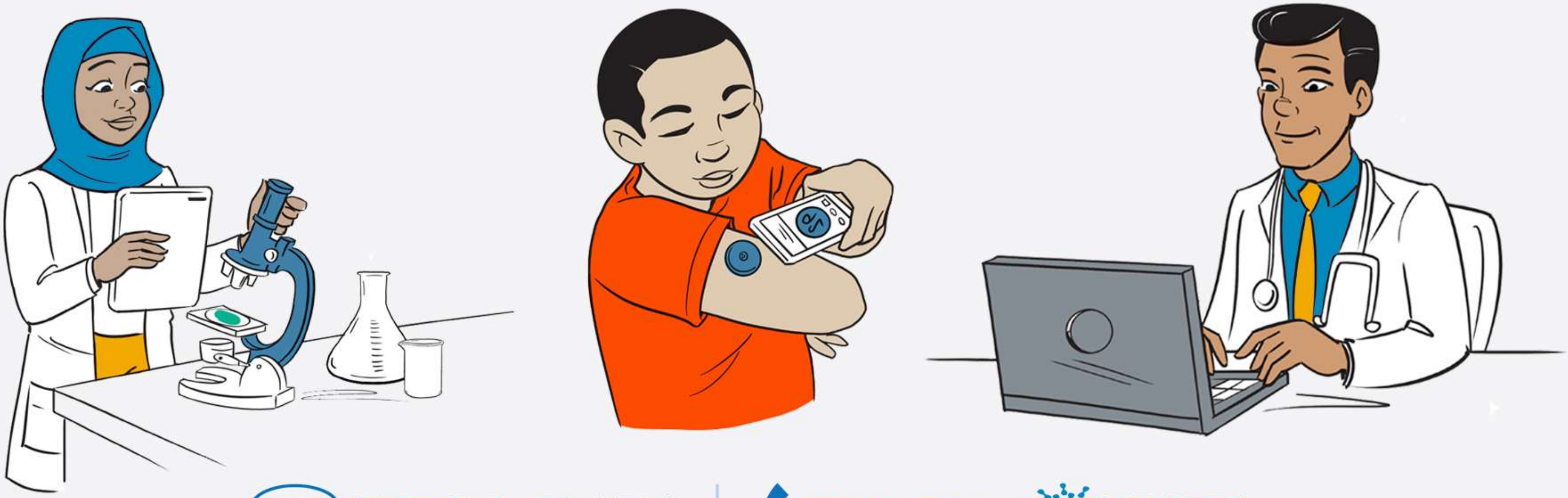
Why Data Literacy Matters

❤ + 📊 = **Impact**

From Records to Research:
Making Sense of Health Data for Rare Diseases



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

CRITICAL PATH
INSTITUTE

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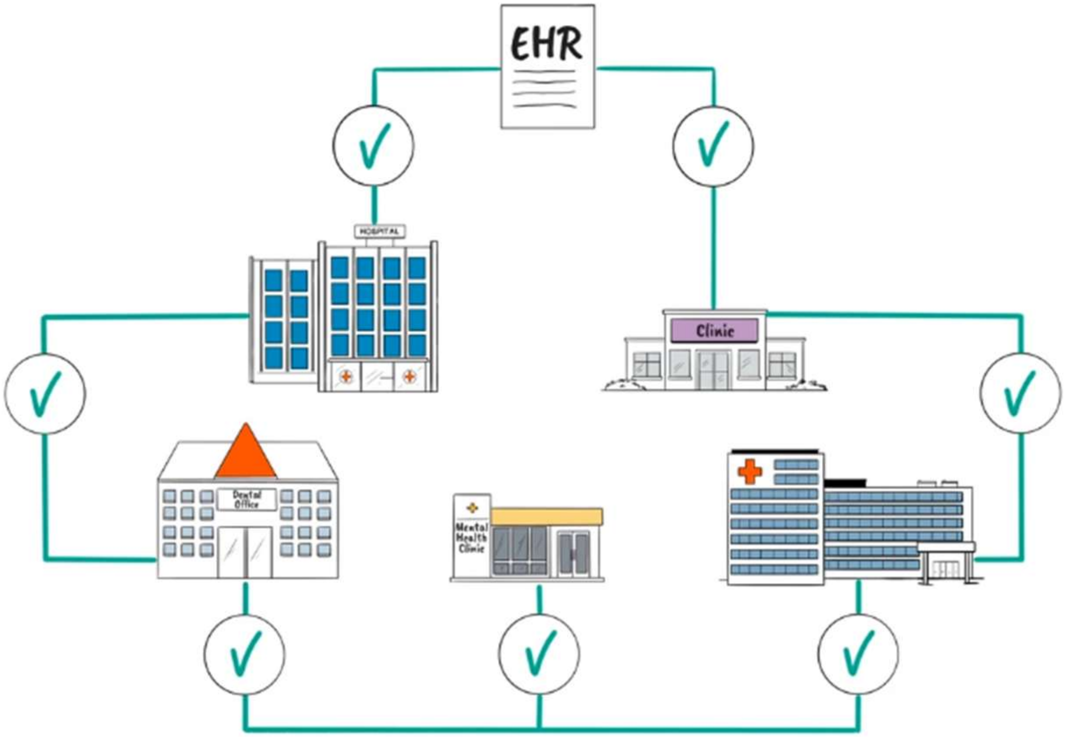
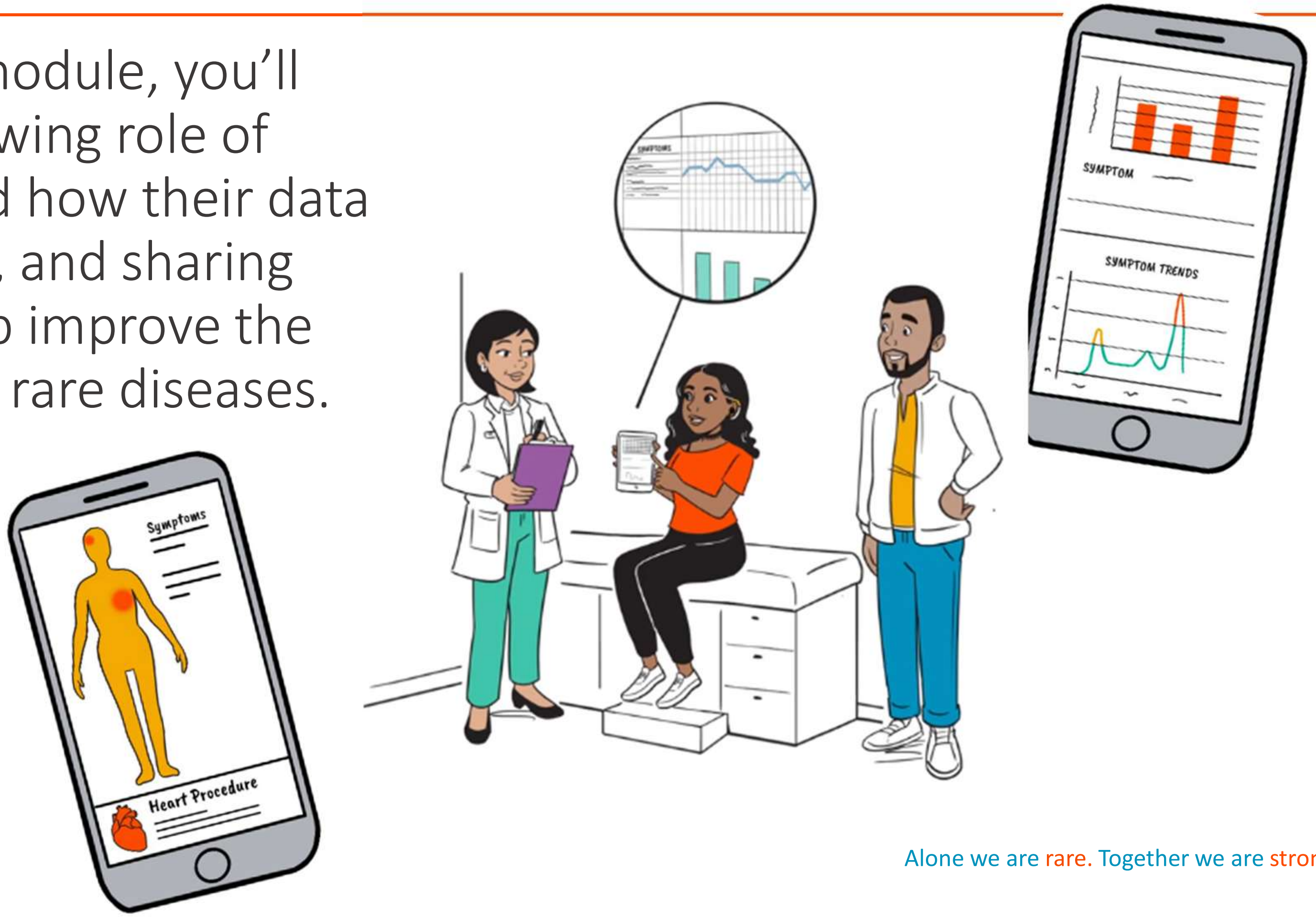
Module 1: Understanding Clinical Data

By the end of this module, you'll understand how clinical data helps improve patient care, supports medical research, and even enables the use of artificial intelligence (AI) in healthcare.



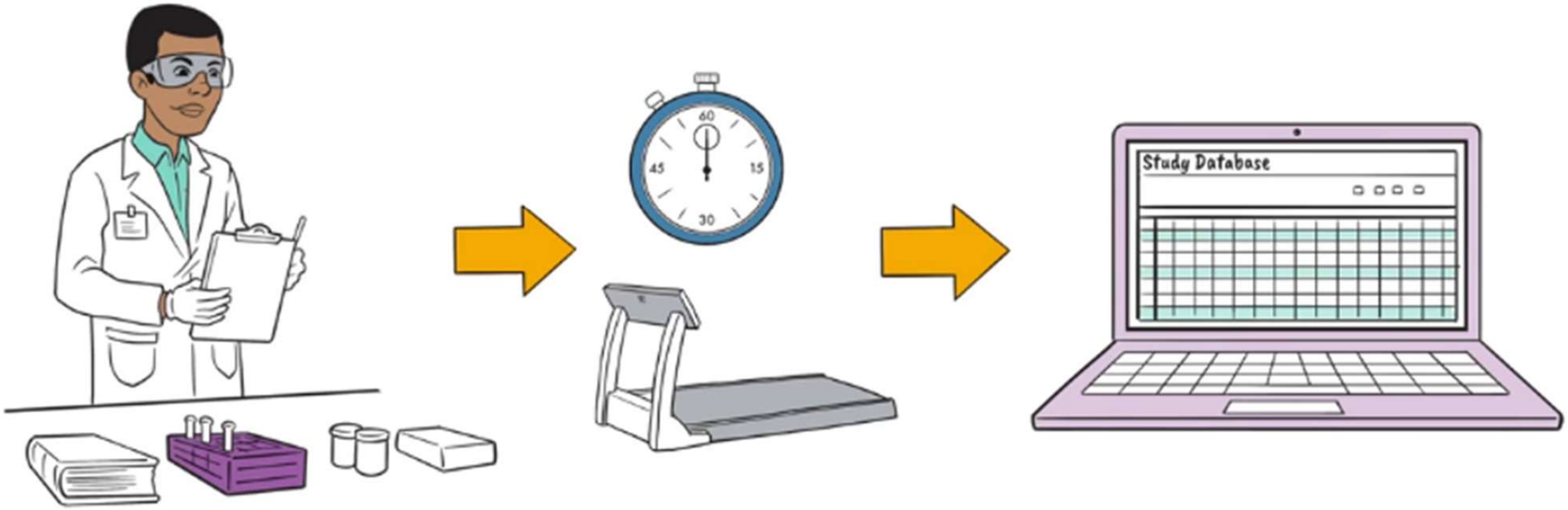
Module 2: Patient-Generated Data for Better Health

By the end of this module, you'll understand the growing role of medical devices and how their data storing, monitoring, and sharing capabilities can help improve the lives of people with rare diseases.



Module 3: Research Data

By the end of this module, you'll understand the role of data in research and how it helps drive medical advancements that improve lives.



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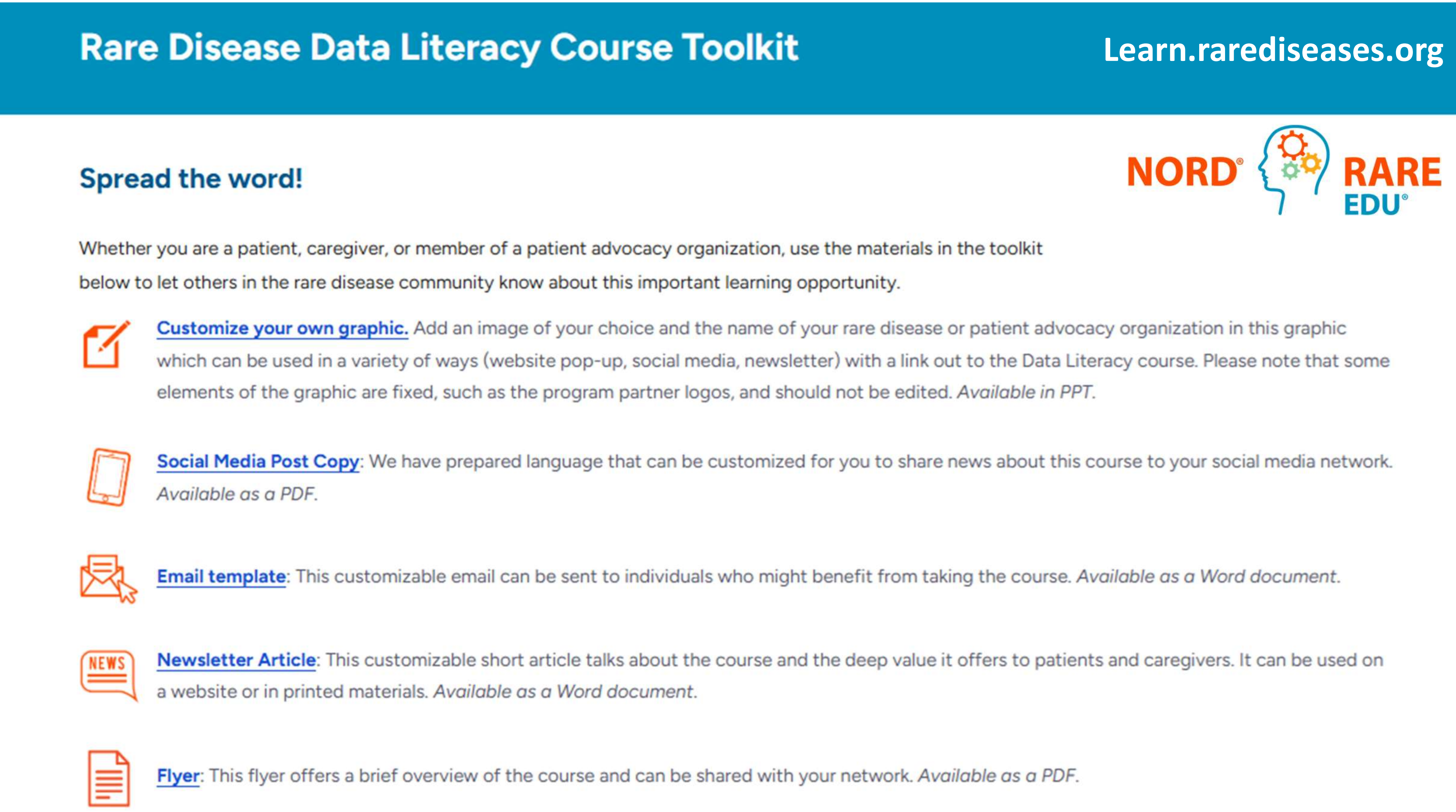
Module 4: How Data Becomes Knowledge



Rare Disease Data Literacy Course Toolkit

Learn.rarediseases.org

Spread the word!

Whether you are a patient, caregiver, or member of a patient advocacy organization, use the materials in the toolkit below to let others in the rare disease community know about this important learning opportunity.

Customize your own graphic. Add an image of your choice and the name of your rare disease or patient advocacy organization in this graphic which can be used in a variety of ways (website pop-up, social media, newsletter) with a link out to the Data Literacy course. Please note that some elements of the graphic are fixed, such as the program partner logos, and should not be edited. *Available in PPT.*

Social Media Post Copy: We have prepared language that can be customized for you to share news about this course to your social media network. *Available as a PDF.*

Email template: This customizable email can be sent to individuals who might benefit from taking the course. *Available as a Word document.*

Newsletter Article: This customizable short article talks about the course and the deep value it offers to patients and caregivers. It can be used on a website or in printed materials. *Available as a Word document.*

Flyer: This flyer offers a brief overview of the course and can be shared with your network. *Available as a PDF.*

From Records to Research:

Making Sense of Health Data for Rare Diseases

This free online course guides learners through the basics of rare disease health data, preparing and empowering patients and advocates to make informed decisions, advocate effectively, and contribute to research.

Now available on RareEDU® at **Learn.RareDiseases.org**

Understanding Clinical Data

This module dives into the world of electronic health records (EHRs) and why they matter.

Patient-Generated Health Data

This module explores how advancements in the capabilities of devices, especially data storing and monitoring, are transforming healthcare.

Research and Health Data

This module explores how scientists and doctors collect and use data to better understand diseases, develop treatments, and improve patient care.

Registry Insights

Jennifer Levy, Scientific Director
Coalition to Cure Calpain 3

The LGMD2A/Calpainopathy Registry:
A Patient Powered Natural History Study and Trial Recruitment Tool

OCTOBER 18, 2025

COALITION TO
CURE
CALPAIN 3
OVERCOMING WEAKNESS
WITH STRENGTH

LGMD2A/Calpainopathy Registry

Replaces the LGMD2A Global Registry, which served primarily as a contact registry

Objectives

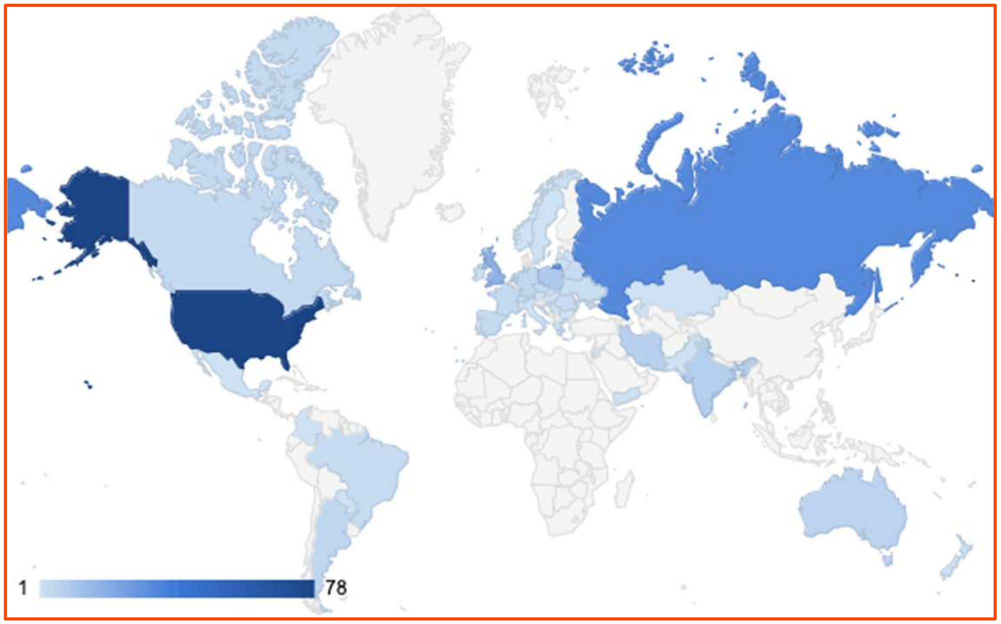
1. Collect longitudinal natural history data
2. Support recruitment for research studies

Launched on September 21, 2023

NORD IAMRARE Platform

Oversight by Steering Committee & North Star Review Board

358 Consented Participants



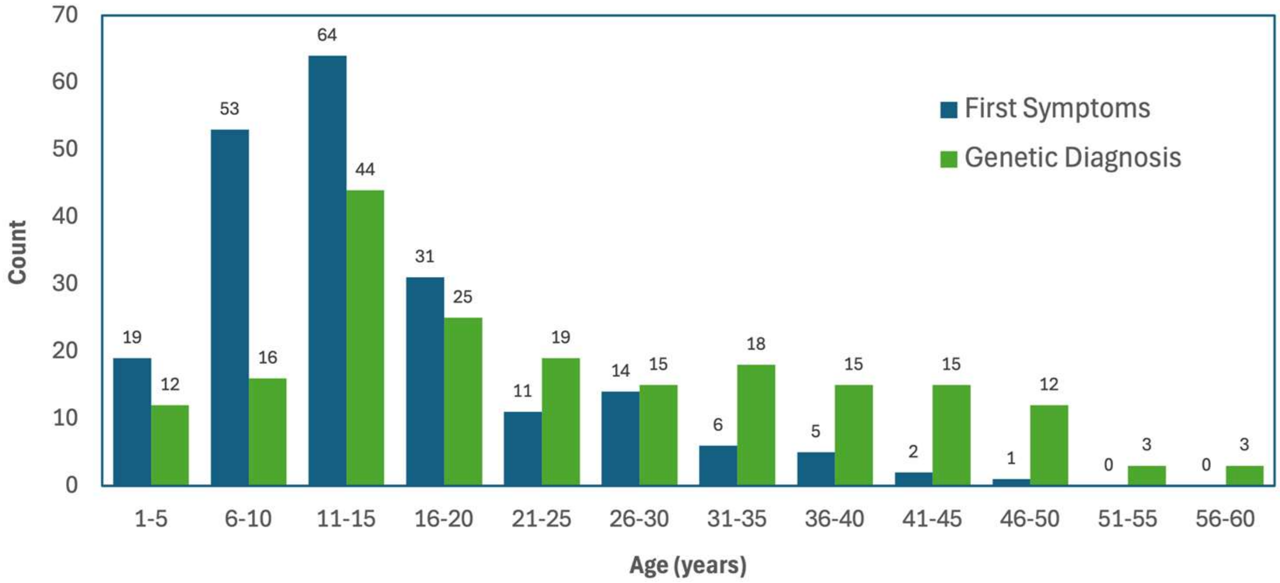
Top 5 Countries in the Registry

United States	78
Russian Federation	33
United Kingdom	18
Poland	10
India	8

41

Countries Represented

Age Symptoms First Noticed and Age at Diagnosis of LGMD2A/R1

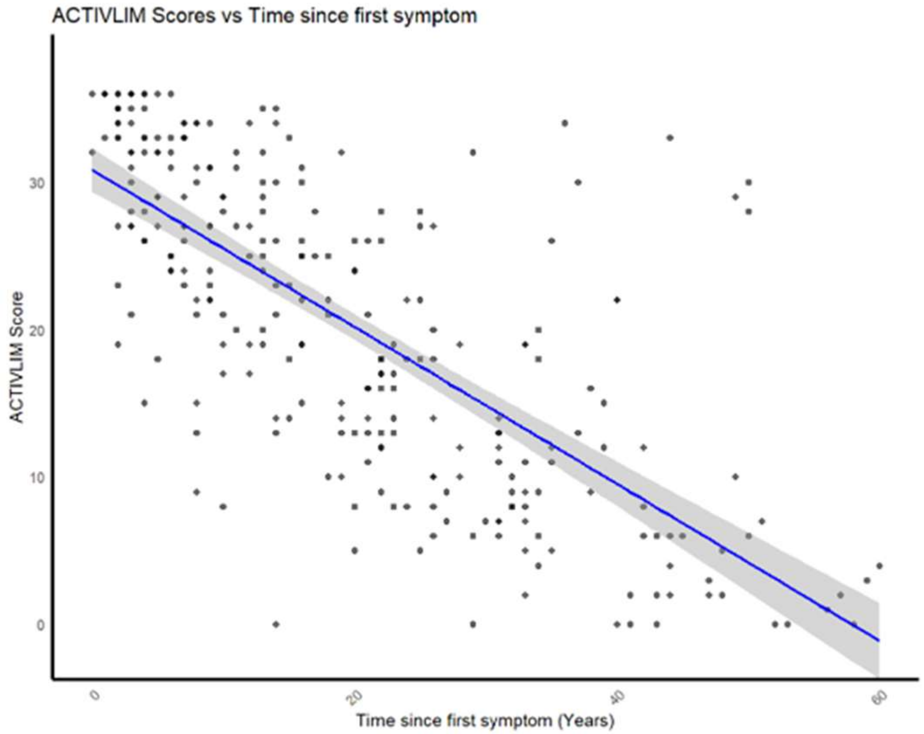


Age at symptom onset (years)

	C3 Registry	**Saenz, 2005**	**Fanin, 2007**	**Angelini, 2010**	**Richard, 2016**
Range	**1-48**	1-67	3-47	3-28	3-45
Mean	**14.9±9.2**	14±11.3	16.5±10.2	14.3±6.9	13.5±7
N	**197**	238	24	22	85

Saenz et al., *Brain*, 2005 Apr; 128(4): 732-742
Fanin et al., *J Med Genetics,* 2007 Jan; 44(1): 38–43
Angelini et al., *Neurol Res,* 2010 Feb;32(1):41-6
Richard et al., *Ann Clin Trans Neurol,* 2016 Mar 4;3:248-265

- A measure of **activity limitations** for **patients with upper and/or lower limb impairments**
- 22 daily activities (4 child items, 4 adult items, 14 items for children and adults).



Adapted from Vandervelde et al., available at Rehab-scales.org

Questions & Answers

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PAG engagement in Patient Support Services

Guest Speaker: Jill Pollander, RN, MSN
Vice President of Patient Services,
NORD

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NORD®

Breakthrough Summit

From Voices to Breakthroughs

Collaboration in Action: Membership and NORD Patient Services

Jill Pollander, RN, MSN
Vice President of Patient Services

OCTOBER 19-21, 2025

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Patient Services

- Patient Services is more than a department—it's a direct expression of our mission to improve the lives of individuals and families affected by rare diseases.
  - Financial assistance
  - Information & resources
  - Clinical trial support

Patient Services

- NORD's Impact: By the Numbers in 2024:

NORD provided nearly $24 million in financial support to 6,450 individuals

NORD Patient Services team engaged with nearly 135,000 callers and emailers

Many of these individuals were referred by our Member Organization partners 🧡

Partnering to Meet Your Community's Needs

- NORD Patient Services can collaborate with your organization to design or expand programs that reflect your mission and the real-world needs of your members.

- Examples of Patient Assistance Program opportunities:
  - Emergency Relief - Support for urgent non-medical needs
  - Copay & Premium Assistance - Help with out-of-pocket costs for medications and insurance coverage
  - Travel & Lodging Support - Assistance for patients traveling to rare disease specialists or clinical trials
  - Educational Access – Travel & lodging expenses for conference attendance, advocacy training, or rare disease education
  - Innovative and custom program design - Tailored solutions based on your community's unique challenges

NORD Breakthrough Summit

How We Partner with Member Organizations

- Step 1: Identify your community's needs **and** funding opportunities to support those needs
- Step 2: Ask us how we can design services to services to support your community
- Step 3: The *details*
- Step 4: Launch and support together
- Step 5: Share outcomes and impact

Stronger Together: Reflections from Our Member Organization Partners

"Dear Jill and Friends at NORD Patient Services,

Your compassion and commitment to supporting those affected by rare diseases are truly inspiring. Thank you for standing with us and our GBS patient community during their most difficult times.

Your program has brought hope and relief to our families as they face the challenges of Guillain Barre syndrome. Your kindness in managing the process is not only helping with the medical costs but also giving families strength to keep fighting.

We are deeply thankful for the incredible generosity of spirit shown by NORD. The financial assistance has provided critical support for the treatment and ongoing care related to GBS. Your compassion brings light and hope to those navigating rare diseases!"

-Lisa Butler, President & CEO

GBS|CIDP Foundation International™

Stronger Together: Reflections from Our Member Organization Partners

"Owen, 10, was diagnosed with TSC at 3 weeks of age following the onset of seizures. Following this diagnosis, it soon became clear the local medical community did not have the resources that Owen would need for such a complex disease. We sought a second opinion with the TSC Clinic located at Cincinnati Children's that would soon change the course of our son's future for the better. However, this was not an easy feat financially. We would soon be put in touch with the NORD Bcureful Travel Fund administered, and not only would our son have the opportunity to receive care from one of the best TS Clinics in the country, but he would also get the chance to partake in research studies that would further help future children with TSC. Without programs like the NORD Bcureful Travel Fund, options would have been limited for children like Owen."

-Shared with permission from Owen's family, by Kari Luther Rosbeck, President & CEO

Stronger Together: Reflections from Our Member Organization Partners

"The International Waldenstrom's Macroglobulinemia Foundation (IWMF) is deeply grateful for our partnership with NORD for our **Travel & Lodging Program**. We consistently emphasize the critical need for second opinions from WM Specialists and access to clinical trials. When community members reach out to us, desperate for expert guidance, it is an honor to share this vital program and refer them to NORD. We wholeheartedly entrust our community members to NORD, with confidence that they will receive the very best of care and support."

-Michelle Postek, IWMF Director, Information & Support

IWMF
International Waldenstrom's
Macroglobulinemia Foundation

Eva’s Butterfly Wishes® for Rare Children

- …to give rare diagnosed children their own butterfly wish, an experience designed especially for them, to enhance their quality of life.

Let’s explore how we can build something meaningful—together.

NORD
Breakthrough Summit

From Voices to Breakthroughs

Thank you.

We're grateful for your partnership!

Jill Pollander

jpollander@rarediseases.org

OCTOBER 19-21, 2025

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Questions & Answers

NORD®
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Closing Remarks

Thank you for joining us!

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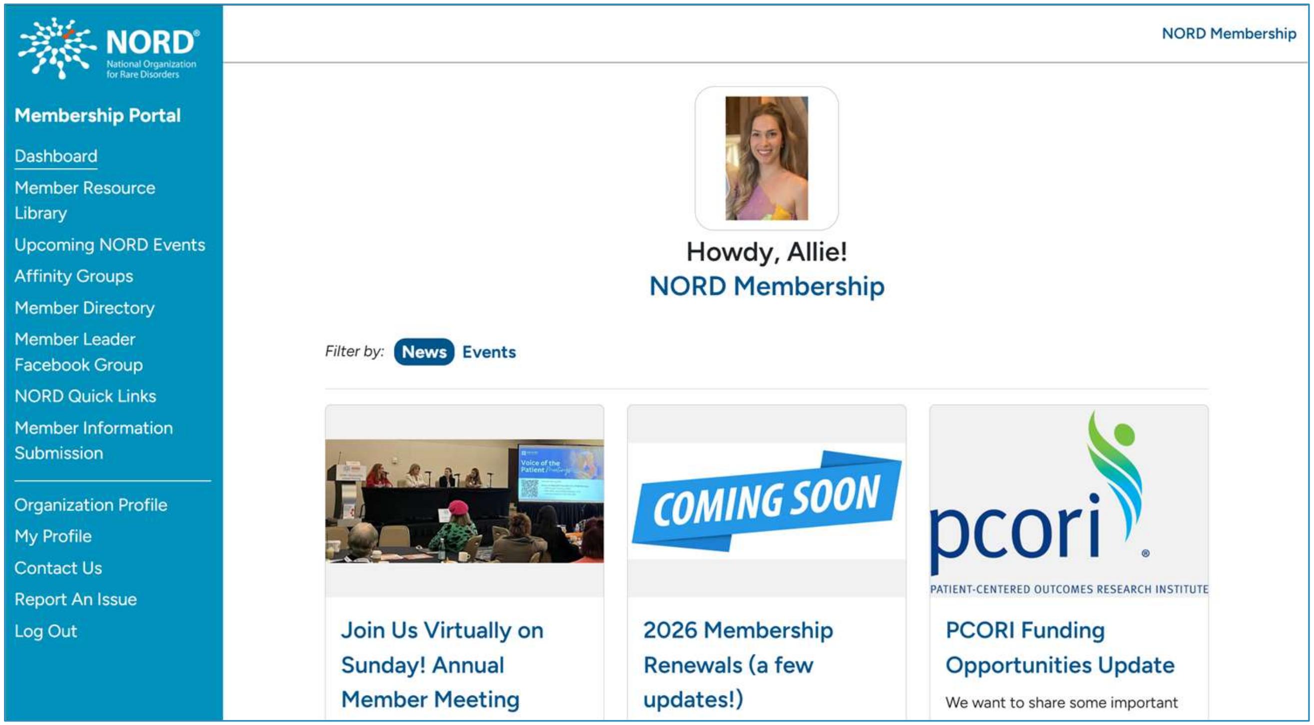
Access the Membership Portal

- https://members.rarediseases.org/



Howdy, Allie!
NORD Membership

Join Us Virtually on Sunday! Annual Member Meeting

2026 Membership Renewals (a few updates!)

PCORI Funding Opportunities Update

We want to share some important

Please Complete Our Survey

Thank you for your feedback!

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Your NORD Membership Team Membership@RareDiseases.org

Alli Ward
Director of
Membership

award@rarediseases.org

Alex Caron
Membership Manager

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Jake Saltonstall
Community
Engagement
Associate

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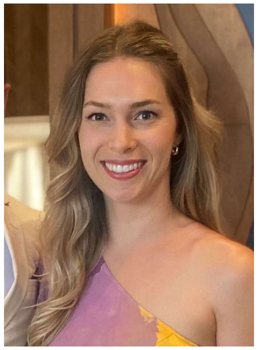

Allie Crafton
Membership Manager

acrafton@rarediseases.org

Martha Alyea
Community & Corporate
Affairs Coordinator

malyea@rarediseases.org

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Join us outside in the hall for a group photo!

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