IAMRARE
Natural History Study (NHS)
Patient Registry
1 in 10 Americans has a rare disease.
About NORD

The National Organization for Rare Disorders (NORD), established in 1983, is the leading advocacy organization addressing the challenges faced by patients and families impacted by rare diseases and the organizations that serve them. NORD, along with its more than 250 patient organization members, is committed to the **identification**, **treatment**, and **cure** of rare disorders through programs of education, advocacy, research, and patient services.
Current Landscape for Patients

• Delays in diagnosis
• Few medical experts
• Little known about the natural progression of a disease and burden of illness
• Social isolation

• Limited FDA approved treatments (95% without)
• Extensive life-long medical needs
• High cost of care and treatment
• Complex health care system
Natural History of Disease

The natural course of a disease from the time immediately prior to its inception, progressing through its pre-symptomatic phase and different clinical stages to the point where it has ended and the patient is either cured, chronically disabled, or dead without external intervention.

A Natural History Study is a specific kind of patient registry which uses information collected to

- Describe the disease over time
- Identify demographic, genetic, environmental and other variables that are associated with the disease
- Define the disease population, including a description of the full range of disease manifestations and subtypes
Purpose of NHS Registry Project

“To empower patient organizations, patients, and families, NORD is collaborating with NIH and FDA to advance the development of more and better natural history data. The cornerstone of this effort is a practical and affordable platform NORD developed for the design, launch, and maintenance of rare disease natural history studies.”

Pamela Gavin – NORD Chief Operating Officer
The Importance of Natural History Study Data

Natural History Study data may

• Inform patient care and best practices

• Assess patient and caregiver experiences and preferences

• Contribute to disease understanding

• Identify research priorities such as genetic, molecular, and physiological basis of rare diseases

• Estimate the number of affected patients and patients potentially available to participate in research

• Evaluate the individual and global economic burden of disease

• Inform drug development
Patients Benefit from Natural History Studies

Natural History Studies

• Educate patients, caregivers, researchers and other stakeholders

• Provide opportunities for researchers to collaborate on projects locally, internationally and across rare disease states

• Provide the incentive of leveraging patient centered outcomes research (PCOR) to optimize the use of existing drugs and/or create novel treatments

• Empower the patient community to participate in research and provide the flexibility for participation regardless of geographic location
IAMRARE™ Registry

**Alone, your data is rare. Together we have strength in numbers.**

Patient-powered natural history studies and networks are transforming how patients and their caregivers inform and shape medical research and translational science for rare diseases.

NORD’s registry platform is an easy to use tool that allows organizations to rapidly launch a high-quality, customized registry to collect the natural history data they need.

**Not just a platform...**

Designed with input from patients, patient organizations, U.S. Food and Drug Administration, National Institutes of Health and other experts in the field, IAMRARE is a compilation of services that help in developing, launching and managing natural history studies.
IAMRARE™ Registry

• Safe and easy to use with its modern, cloud-based design that allows for scalability and reliability

• Developed and owned by NORD, no third parties and no hidden costs

• Advanced analysis tools provide real-time insight and information to maintain registry engagement over time

• 1-to-1 guidance provided by NORD’s dedicated IAMRARE team to create and launch a successful registry

• Access to standardized data dictionaries, as approved by an Institutional Review Board

• Opportunities to compare your data to other rare diseases

• Unlimited surveys, questions and participants

• Smart surveys allow you to target questions based on participant responses

• Automated reminders to re-engage participation
Registry Look and Feel

The Power of Patients Registry

Welcome to the Power of Patients Registry

Learn more »

Rare Disease Research
This is a unique rare disease patient registry. Are you interested in using our data to further your rare disease research?

Researchers »

Participating in this Study
Information collected during this study may be used to help provide opportunities for patients and researchers to collaborate in the rare disease community.

Patients »

Join the Registry
Please create an account and provide consent to participate in the study.

Register »
User Friendly Surveys

**Treatment**

Please enter all of the Participant's past and present medications (prescription, over the counter, homeopathic, other) by clicking the "add another record" button for each medication.

*Select a medication:*

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Did the Participant take any dietary supplements during the past year, at least once a week?

- Yes
- No

Does the Participant take any medical foods or follow a special diet for treatment of his/her rare disease?

- Yes
- No
- Don't know
Access to Real-Time Aggregate Patient Data
Future of Rare Disease NHS

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Experts agree that these registries are transforming patient/caregiver support and advocacy groups into research organizations. They also provide patients and family members another way to become engaged in research beyond the role of adviser or informant to researcher-generated studies.

AHRQ (Agency for Healthcare Research and Quality: Community Forum White Paper)
Learn More

For more information, submit an inquiry at

research@rarediseases.org