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
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 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
“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

• 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
• Provide an avenue for biospecimen collection
Patients Benefit from Natural History Studies

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

• 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
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 software 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 for IPPF and Study Participants

- Safe and easy to use with its modern, cloud-based design that allows for scalability and reliability
- Registry product developed and owned by NORD, no third parties
- IPPF owns data donated by participants
- 1-to-1 guidance and support 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
- Smart surveys reduce participant survey fatigue
- Automated reminders to re-engage participation
- No cost to participate in the IPPF study
- Advanced analysis tools provide real-time aggregate data and information to maintain registry engagement over time
- Future opportunities to compare data across rare diseases
International Pemphigus & Pemphigoid Foundation Launches Largest-Ever Study of Pemphigus and Pemphigoid

Research study is open to participants worldwide to advance understanding and treatments for rare disease causing blistering that may be accompanied by severe pain, itching, burning, and stinging.

Sacramento, California, March 31, 2017—The International Pemphigus & Pemphigoid Foundation (IPPF) has launched the largest-ever study to research pemphigus and pemphigoid that causes blistering of the skin and mucosa. Pemphigus and pemphigoid currently has no cure.

“The IPPF Natural History Registry will provide a complete picture of each patient’s experience with pemphigus and pemphigoid,” said Marc Yale, Interim Executive Director, of the IPPF. “We are launching this initiative to help fill the missing link researchers and medical experts need to advance research and get to a cure.”

To help drive awareness and participation, the IPPF will provide information about the existence of the registry via email, website advertisements, flyers, the Quarterly newsletter, and at its’ Annual Patient Meeting to interested members of the pemphigus and pemphigoid community.

“Our goal is to enroll as many patients, or their parents or legal guardians, as possible,” said Yale. “The success of the registry is dependent upon community participation.”
IPPF Natural History Registry

We are pleased to announce the launch of the IPPF Natural History Registry, a collaborative effort between the International Pemphigus & Pemphigoid Foundation (IPPF) and the National Organization for Rare Disorders (NORD) to study pemphigus and pemphigoid. The IPPF was selected by NORD to create the IPPF Natural History Registry as part of a cooperative project between NORD and the U.S. Food and Drug Administration (FDA) that supports research on rare diseases and how they progress over time - natural history studies.

OVERVIEW OF RESEARCH STUDY

The IPPF Natural History Registry is more than a versatile online system that securely collects and stores data for medical research; it is a dynamic participant-driven resource that can empower and unite the pemphigus and pemphigoid community through shared knowledge. Registry participants not only can complete surveys about their own disease experiences, but also can learn about other participants’ experiences by viewing aggregated survey data. As the registry grows, the IPPF will ensure that data privacy and confidentiality are strictly maintained. Participation in the IPPF Natural History Registry is free and voluntary, and participants may withdraw at any time.

COMMUNITY INVOLVEMENT

The IPPF Natural History Registry is a powerful opportunity for individuals with pemphigus and pemphigoid and their family members to contribute directly to research that will enhance our understanding of pemphigus and pemphigoid, thus facilitating the development of new diagnostic and treatment options. Participation is especially vital given the rarity of pemphigus and pemphigoid — every patient experience is a unique and invaluable part of the natural history of pemphigus and pemphigoid.

About NORD

NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 230 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and support.
Frequently Asked Questions

FAQ For Pemphigus and Pemphigoid Patient Registry Natural History Study

1. What is a Patient Registry?

A patient registry is a collection of standardized information about a group of patients who share a condition and is used for a variety of purposes such as conducting natural history studies and supporting disease specific clinical trial recruitment.

2. What is a Natural History Study?

A natural history study is a study designed to track the course of a disease over time and includes people who have a specific medical condition or disease and those who are at risk of developing such. This method of research explores the disease in a comprehensive way and identifies demographic, genetic, environmental, and other variables that correlate with the disease and its outcomes. Natural history studies have many potential uses such as patient care best practice developments and clinical trial recruitment.

3. What is a Research Study Sponsor?

The National Health Service defines a study sponsor as, "... the individual, company, institution or organization, which takes on ultimate responsibility for the initiation, management [...] of and/or financing [...] for that research." The Study Sponsor ensures that the study is conducted in a reputable manner and upholds regulations as they apply to the study.

4. What is a Principal Investigator?

The Principal Investigator is the research group leader or, the person with the primary responsibility for the design and conduct of the research project or study.

5. What is an Institutional Review Board (IRB)?

According to the Mayo Clinic an IRB is, "a specifically constituted review body established to protect the rights and welfare of human subjects recruited to participate in biomedical or behavioral/social science research." An institutional
The IPPF Registry - pemphigus.iamrare.org
User Friendly Surveys

The questions shown above are a sample and not representative of actual questions that may appear in the IPPF Registry.
Access to Real-Time Aggregate Patient Data

An example of charts displaying de-identified data to participants after survey completion. Charts were not taken from the IPPF Registry.
Future of Rare Disease NHS

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

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 information about the IPPF study, submit an inquiry at registry@pemphigus.org

Visit NORD’s website rarediseases.org
Or email us research@rarediseases.org