



NORD[®]
National Organization for Rare Disorders



FAQ for 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?

An individual, company, institution, or organization that takes responsibility for choosing appropriately trained and experienced researchers as well as the initiation, management, and/or financing of a clinical trial. 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)?

Any board, committee, or other group formally designated by an institution or investigator to review, approve the initiation of, and to conduct periodic review of research involving human subjects. The primary purpose of such review is to assure the protection of the rights and welfare of the human subjects. Also known as Ethics Committee (EC).

6. What is the purpose of a Patient Registry?

One of the most important purposes of a patient registry is to bring the rare disease community together and collect data which could be used to create therapeutics and improve the quality of life for patients. Some other goals of the registry are to:

- Conduct a prospectively-planned natural history study that will result in the most comprehensive understanding of a rare disease and its progression over time.
- Characterize and describe a rare disease population as a whole.
- Assist the rare disease community with the development of recommendations for standards of care.
- Assist researchers studying the pathophysiology of a rare disease.
- Assist researchers studying interventional outcomes.
- Support the design of clinical trials for new treatments.

7. What types of data will be collected in the Patient Registry?

The data collected is uniform and includes but is not limited to

- Socio-demographics
- Medical and diagnostics
- Treatment and disease progression
- Management of care
- Quality of life

8. How is the data collected?

Data is collected through a secure web-based system developed by the National Organization for Rare Disorders ([NORD](#)), an independent non-profit committed to the identification, treatment, and cure of all 7,000 rare diseases. Study participants respond to questions grouped within a series of surveys developed per study standards and in collaboration with disease specific experts.

9. Who is a study participant?

A study participant is the individual who takes part in a research study and whose information is collected for that research. Study participants may consent to enter and share their own personal data.

10. Who is a reporter/respondent?

A reporter/respondent is an individual who completes the surveys on behalf of the patient/study participant, when they are unable to do so on their own behalf.

11. What is a legally authorized representative (LAR)?

Individual who is authorized under applicable law to consent, on behalf of a prospective subject, to the subject's participation in the clinical trial. The LAR may be a parent, grandparent, caregiver, or guardian who has the legal authority to grant consent on behalf of another who is eligible to participate in research. When a LAR acts on behalf of a study participant, he/she is considered to be the reporter/respondent in the research.

12. Can data be collected worldwide?

The patient registry uses an online platform which allows participants to contribute data from anywhere in the world. Individuals from other countries who enter data into the registry should be aware that data and privacy laws are different in the U.S. from other countries. For persons living outside the U.S. who choose to share information about themselves, the same protections for privacy and confidentiality are offered as in the U.S.

13. Where is the data stored?

The data is stored on NORD's registry platform system which adheres to industry standards regarding security protections.

14. Is the data safe?

Yes, the data is safe. The registry follows strict government guidelines to assure patient information is protected. The platform is served over HTTPS, which provides traffic encryptions so as to prevent eavesdropping and man-in-the-middle attacks. Communications between the registry platform application server and the database are also encrypted.

15. Who owns the data?

The identifiable and de-identifiable data are owned by the study sponsor, the National Organization for Rare Disorders (NORD). NORD decides how and with whom to share the data. A subset of the de-identified data collected across the NORD Registry Platform is available to NORD to support cross disease analysis and advocacy activities to members of the rare disease community as a whole.

16. How is the Patient Registry maintained?

The registry is maintained by NORD who hosts the registry on its cloud-based Platform and provides oversight and ongoing support of the system. NORD provides the day-to-day management of their patient registry, including the development and adherence to the study procedures.

17. Who is NORD – the National Organization for Rare Disorders?

NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD was founded by patients and families who marshaled grass-roots efforts to secure the passage of the Orphan Drug Act in 1983. Today, NORD represents the united voice of more than 250 rare disease-specific groups and thousands of patient advocates. Together, we are committed to the identification, treatment and cure of rare disorders through programs of advocacy, education, research and patient support services. Learn more about NORD at <https://rarediseases.org/>.