FAQ for Krabbe Community United Research and Engagement Study (KrabbeCURES)

1. What is KrabbeCURES?

KrabbeCURES is a data-collection study for patients around the world to share information about globoid cell leukodystrophy (Krabbe disease). Its purpose is to build a unified globoid cell leukodystrophy patient community, serve as an international resource that can be used by researchers in industry and academia and, to inform and accelerate regulatory approval of therapies.

2. 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, including but not limited to, providing further understanding around quality of care, aids clinical trial recruitment, and can help contribute to disease specific guidelines across many disciplines.

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).

Informed Consent FAQs: https://www.hhs.gov/ohrp/regulations-and-policy/guidance/faq/informed-consent/index.html. Accessed May 15, 2017.

6. What is the purpose of KrabbeCURES?

One of the most important purposes of KrabbeCURES is to bring the globoid cell leukodystrophy 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 study are to:

- Systematically gather information to fill current gaps in research around newborn screening, transplantation, and gene therapy.
- Characterize and describe the Krabbe disease population as a whole.
- Assist the Krabbe disease community with the development of recommendations for standards of care.
- Assist researchers studying the pathophysiology Krabbe disease.
- Assist researchers studying interventional outcomes.
- Support the design of clinical trials for new treatments.

7. What types of data will be collected in KrabbeCURES?

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

- Socio-demographics
- Diagnostics
- Treatment and disease progression
- Management of care
- Quality of life
- Symptoms
- Diagnostic odyssey

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 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 i.e. the individual with a diagnosis of globoid cell leukodystrophy. 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)?

An 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. What is an Informed Consent?

The Office for Human Research Protections (OHRP) states that, "… the informed consent process is the critical communication link between the prospective human subject and an investigator beginning with the initial approach of an investigator to the potential subject (e.g. through a flyer, brochure, or any advertisement regarding the research study) and continuing until the completion of the research study. The informed consent process involves three key features: (1) disclosing to potential research subjects' information needed to make an informed decision; (2) facilitating the understanding of what has been disclosed; and (3) promoting the voluntariness of the decision about whether or not to participate in the research." (https://www.hhs.gov/ohrp/regulations-and-policy/guidance/faq/informed-consent/index.html)

13. Who can join the study?

This study is open to anyone who has a confirmed diagnosis of globoid cell leukodystrophy (Krabbe disease).

14. Is there a cost to participate?

There is no cost to the patient to join this study. The KrabbeCURES sponsor absorbs the cost of the study for its members.

15. How long will this study last?

This study will be open for at least five years with the option to renew registration. There is no date of termination or closure at this time.

16. Can data be collected worldwide?

This study uses an online platform which allows participants to contribute data from anywhere in the world. Individuals from other countries who enter data into KrabbeCURES should be aware that data and privacy laws are different in the U.S. from other countries. This U.S. based study will protect data and privacy according to U.S. requirements.

17. Where is the data stored?

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

18. Is the data safe?

Yes, the data is safe. The platform 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 platform application server and the database are also encrypted.

19. Who owns the data?

The identifiable and de-identifiable data are owned by the study sponsor, KrabbeConnect. The study sponsors decide how and with whom to share the data. A subset of the de-identified data collected across the NORD's IAMRARE Platform is available to NORD to support cross disease analysis and advocacy activities to members of the rare disease community as a whole.

20. How is KrabbeCURES maintained?

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

21. Who is KrabbeConnect?

KrabbeConnect's mission is to be the source of comprehensive information and access to resources for patients with Krabbe disease. The foundation will drive state-of- the- art research by bridging the gap between science and patient knowledge. The organization seeks to revolutionize the practice of medicine by identifying, optimizing, and implementing advances in the care and cure of globoid cell leukodystrophy (Krabbe disease), utilizing a multicenter network. The organization's goal is to achieve complete disease eradication through cooperation between patients, patient advocacy groups, clinicians, researchers, industry, and government. The organization is dedicated to the cooperative planning, implementation, analysis and reporting of controlled clinical trials, as well as observational studies and educational activities within the Krabbe disease community. KrabbeConnect is a charitable non-profit organization with 501(c)(3) status.

Learn more about KrabbeConnect at https://krabbeconnect.org/

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

The National Organization for Rare Disorders (NORD®), an independent nonprofit, is leading the fight to improve the lives of rare disease patients and families. We do this by supporting the rare community, its people and organizations. We work together to accelerate research, raise awareness, provide valuable information and support, and drive public policy that benefits the estimated 25-30 million Americans impacted by rare diseases. NORD is a charitable non-profit organization with 501(c)(3) status.

Learn more about NORD at https://rarediseases.org/.