HI GLOBAL REGISTRY

Q + A

Q: What is a patient registry?
A: A research study that is a collection of surveys organized in a way to better understand how a whole community of patients is affected by a specific condition. A more formal definition of a patient registry is: "An organized system of collecting uniform data for a population defined by a particular disease or condition that serves one or more predetermined scientific, clinical or policy purposes."

Q: Why should I participate in the HI (Congenital Hyperinsulinism) Global Registry?
A: To further understand the disease so that there will be better treatments, access to excellent HI care and one day, a cure.

Q: What are the objectives of the HI Global Registry?
A:
- To provide a convenient online platform for participants (or caregivers) to self-report cases of congenital hyperinsulinism (HI) in order to document the natural history and outcomes of individuals with HI.
- To improve knowledge of how often HI and other health issues associated with it occur
- To better understand the role of timely diagnosis of HI on patient developmental outcomes
- To better understand patient health outcomes of different HI treatment options, settings or provider types
- To identify both positive and negative effects related to different HI treatment options
- To support the evolving global standards of care for HI patients

Q: What is a natural history study?
A: A natural history study is a study that follows a group of people over time who have a specific medical condition or disease.

Q: What type of questions will be asked in the HI Global Registry surveys?
A: Questions relating to contact information, demographics, developmental, diagnosis, pregnancy & birth, medication, surgical, diet, glucose monitoring and quality of life.

Q: Who is the HI Global Registry Sponsor?
A: Congenital Hyperinsulinism International (CHI). As the sponsor, CHI is responsible for ensuring the design, management and overall conduct of the research meets appropriate regulations and standards.

Q: What is a Registry Participant?
A: A registry participant is the HI patient. The participant's data can be entered by the patient himself/herself, a parent, or a guardian who is legally authorized to represent the patient.

Q: What is a Registry Platform?
A: A Registry Platform is where an individually sponsored patient registry is hosted by a service provider on the Internet. The HI Global Registry is hosted by NORD on an IIS server in concert with a Microsoft SQL Server database. The registry platform is metadata-driven and supports data versioning, so the survey contents may be updated, as appropriate, by subject matter experts at any time without loss of data integrity.

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Q + A

Q: What is the National Organization for Rare Disorders?
A: NORD, a 501(c)(3), is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that service 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.

Q: Will the patient's data be safe?
A: The registry platform is served over HTTPS, providing encryption of traffic (to prevent eavesdropping) and helping to prevent man-in-the-middle attacks. Communications between the registry platform application server and the database are also encrypted. Furthermore, sensitive data is encrypted at rest with Advanced Encryption Standard (AES), a form of encryption the US government has deemed appropriate for top-secret information. This encryption is applied to patient information and any files uploaded by the participant while answering surveys.

Q: What is an Institutional Review Board (IRB)?
A: An IRB is a diverse group of qualified individuals that has been formally designated to review research to assure the protection of the rights and welfare of the human subjects. To accomplish this purpose, IRBs use a group process to review research protocols and related materials (e.g., informed consent documents and investigator brochures).

Q: What is the governing structure of the registry?
A: The Registry is a research project governed by the HI Global Registry Steering Committee that was formed by CHI to ensure independent guidance and oversight for the lifetime of the initiative.

Q: Who are the experts on the Registry Steering Committee?
A: The experts on the committee consist of doctors, nurses, researchers, patients, caregivers and executive management from the global HI community.

Q: What types of data reports can be produced by the Registry?
A: Any information that is input into the patient registry can be reflected in data reports generated by the system. Examples of data reports are:

- Demographics and global prevalence (how often it occurs and where)
- Diagnostics and genetics
- Disease progression
- Treatments and outcomes
- Standards of care and quality of life
- Healthcare providers management

Q: What is de-identified aggregated data?
A: Registry data combined in a way which, when shared, contains no personal information that can identify individual participants. This de-identified aggregated data is used to produce reports from the registry for research purposes.

Q: How will the registry be used to support research?
A: The HI Global Registry will generate HI data to support current and future research initiatives. As sponsor of the Registry CHI will work closely with its Registry Steering Committee to oversee sharing of de-identified aggregated data reports and research collaborations. Registry data can validate current understanding of HI and produce new ideas for research to improve treatment, outcomes and quality of life for patients and their families.

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