Congenital Hyperinsulinism International

The HI Global Registry

Patient-Powered Research Connecting All HI Stakeholders

CONGENITAL HYPERINSULINISM

HI GLOBAL REGISTRY

TIMELINE

HI GLOBAL REGISTRY

RESEARCH
2010 - 2013

- Rare disease patient registry review
- Rare disease registry assessment
- Adoption of natural history study (NHS) model
- Rare disease NHS registry committment

CONCEPT DESIGN
2013 - 2015

- Project blueprint
- Expert Steering Committee review & recruitment
- Registry provider analysis
- CHI seed funding
- Community engagement
- Registry provider selection & contract

SET UP
2015 - 2017

- Protocol development NHS registry data set
- Data management plan
- Communication plan & deliverables
- Registry design & congifuration
- IRB approval
- Registry testing & training
- Funding analysis
- Partnership development
- Community engagement

LAUNCH
2017 - 2018

- Patient community engagement & recruitment
- Clinician engagement & promotion
- Researcher engagement & promotion
- Other collaborating parties engagement & promotion
- Multi-country recruitment

POST-LAUNCH
2018 -

- Multi-country recruitment
- Data management
- Data reports, analysis & dissemnation
- Clinical trial support services
- Research collaboration requests
- Governance board
- Registry committees
- Protocol amendments
- IRB submissions
- Fundraising & securing funding
- Community engagement & retention
- Resource management
- Secure HIGR funding

LONG TERM
FUTURE

- Further research
- Partner developments
- Regulators & funders relationships
- Boards & committees
- Registry outcomes publications
- Registry developments
- Community engagement & retention
- Resource management
- Strategic planning

The HI Global Registry has many Global partners and is sponsored by

CHI Congenital Hyperinsulinism International

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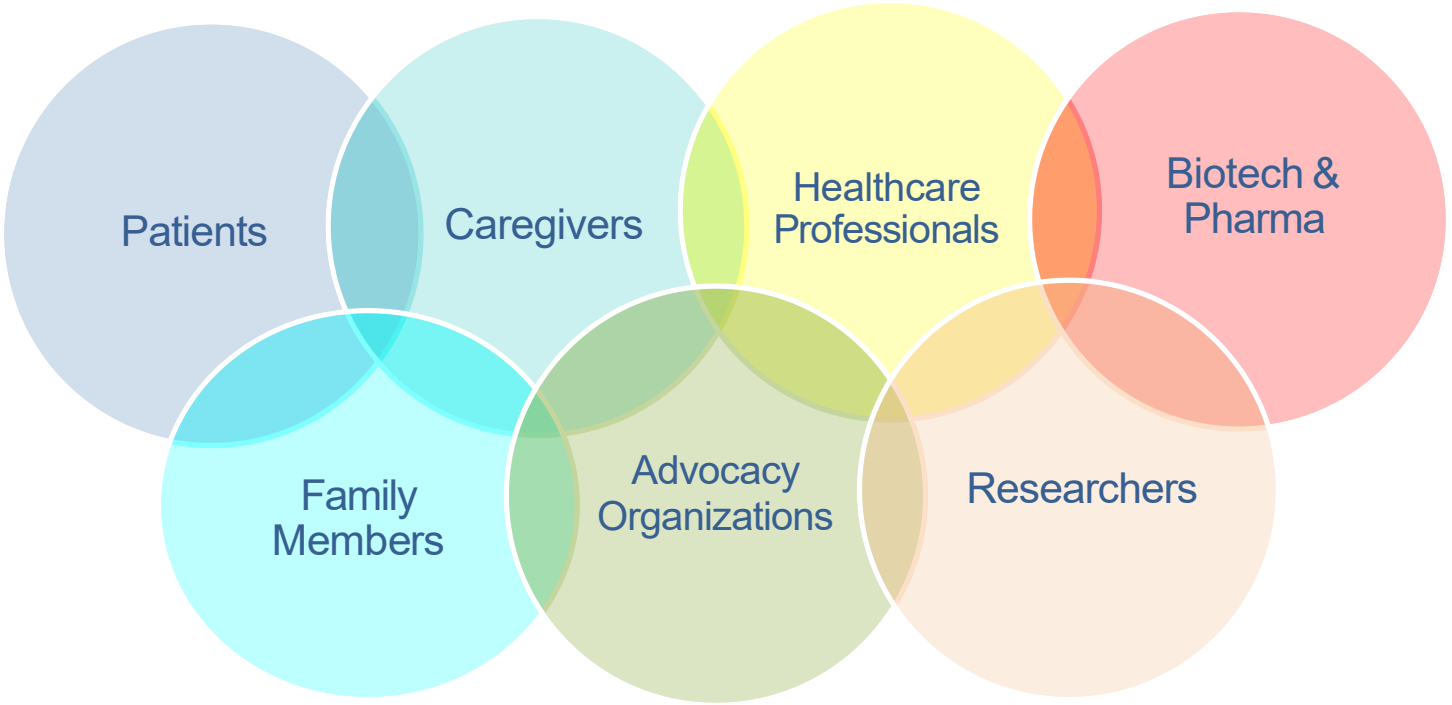
Collaboration of HI Experts



Leads to knowledge, treatments and improved patient care

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KEY FACTS

- CHI is the sponsor of the HI Global Registry (HIGR)
- IAMRARE registry platform, hosted by NORD
- Long-term natural history study including QoL measures
- Primary Objectives:
  - Better treatments
  - Improved quality of life
  - Cures
- Protocol and study documents IRB approved
- Collaborative, diverse HIGR Steering Committee

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HOW DOES THE HI GLOBAL REGISTRY WORK?

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SURVEY	Type	# of Questions	CONTENT
Contact Information	Updatable	13	Basic contact info and preferences (GRDR-compliant)
Demographics	Updatable	20	Key characteristics of the participant
Pregnancy	Data submitted once	13	Pertinent information about mother's pregnancy with participant
Birth	Data submitted once	27	Pertinent information about participant's birth
Diagnosis	Updatable	81	Detailed questions about how the participant was ultimately diagnosed with HI
Medication Management	Updatable	132	Current & past HI medication treatment(s)
Diet & Feeding Management	Updatable	32	Current & past feeding routes and regimens

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SURVEY	Type	# of Questions	CONTENT
Surgical Management	Updatable	44	Whether pancreatectomy was considered, and relevant details if surgery was performed, including glycemic outcomes
Other Diagnosis	Updatable	42	Commonly related diagnoses noted in the HI community, such as epilepsy, diabetes, pancreatic insufficiency, neurologic and other conditions
Glucose Monitoring	Longitudinal – every 6 months	26	Method and frequency of checking blood sugar levels
Developmental	Updatable	22	Growth & development as well as any therapeutic interventions
QOL (Parent/LAR)	Longitudinal – Annual	39	General, physical, social, emotional wellbeing & school/work, healthcare experience
QOL (Participant)	Longitudinal – Annual	42	General, physical, social, emotional wellbeing & school/work, healthcare experience, 14+ year old

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DATA VALIDATION

- Started with existing surveys created/used by HI centers
- Core Data Set rigorously reviewed/approved by HIGR Steering Committee, CHI R&D Committee
- Beta-tested registry prior to launch
- Data Management Plan to monitor and protect data quality
  - ⟶ Initial participant review for HI diagnosis
  - ⟶ Review for consistent answers on similar questions
  - ⟶ Regular review of “free text” responses

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COLORING BOOK

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BENEFITS TO PATIENTS

- Better understanding of how the whole community of HI patients is affected by the condition
- Individualized comparison of participant to the HI patient community
- Becoming part of the HI community
- Notification of new clinical studies
- Patient community driving change
- De-identified information shared with researchers dedicated to improving treatments and patient outcomes

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HI GLOBAL REGISTRY

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BENEFITS TO RESEARCHERS/CLINICIANS

- Connection with truly global set of HI patients
  - Broadest compiled set of HI data
  - Study participation recruitment
- At least annual reporting:
  - Natural history of HI
  - Status of current, global HI management
- Fertile ground for future research projects

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KEY PERFORMANCE INDICATORS

- Enrollment
  - At least 100 participants (by October 2019)
  - At least 300 participants (by October 2021)
- Reporting
  - Ongoing evaluation of data, at least quarterly (2019)
  - General reporting, at least annually (2020)
- Research
  - At least 1 successful research recruitment cycle (by early 2021)
  - Proof of concept for use of registry to design prospective clinical trial (by end of 2021)

https://www.higlobalregistry.org/

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HI Global Registry

Patient-Powered Research for a Brighter Future!

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 disaease community.

Patients »

Join the Registry

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

Register »

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CHI Congenital Hyperinsulinism International

powered by NORD National Organization for Rare Disorders

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https://www.higlobalregistry.org/Home/About

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Welcome and Introduction to the HI Global Registry (HIGR)

CHI and the HI Global Research team would like to extend our warmest welcome, and thank you for supporting this important patient-powered research initiative. We couldn't do this without you; together we will connect the dots to advance knowledge and research for better patient outcomes.

The HI Global Registry is a patient-reported online research study that consists of a series of 13 surveys, which ask questions about the patient's experience with hyperinsulinism over his or her lifetime. These surveys include questions about contact details and demographics (such as, age, sex and country of birth), as well as questions about diagnosis, medication management, diet & feeding, surgical procedures, other diagnoses, developmental and quality of life for the parent/guardian and participant (patient). The questions have been carefully developed by a team of HI experts, including family members of children with HI, advocates, clinicians and researchers.

More information about the registry can be found on the HI Global Registry webpage located on CHI's website at https://congenitalhi.org/higlobalregistry/. Information includes the registry study's objectives; members of the Registry Steering Committee; communication material, like a brochure and fact sheet; a Q&A; and best practice guidelines.

We encourage anyone who has received a diagnosis or is suspected of having congenital hyperinsulinism to complete the surveys in the registry. Patients (referred to as participants in the registry) who are eighteen years old or older should enroll themselves and may work with parents or legal guardians on answering survey questions.Parents or legal guardians of participants under the age of 18, or the legally authorized representative of participants who are 18 and over, may report on behalf of the participant.

Before joining it's helpful to understand key words and terminology and the different roles in participating in the registry:

- **HIGR** is the shortened name for the HI Global Registry Study.
- The **Participant** is the patient (the person with congenital hyperinsulinism).
- The **Respondent** is the individual who completes the surveys, and can either be the participant or his or her parent, guardian or legally authorized representative (LAR).
- **LAR** is the shortened name for legally authorized representative who is legally authorized to input the data on behalf of the participant.
- The **Sponsor** (CHI) is the organization responsible for the initiation, management, and financing of the HI Global Registry.
- The **Principal Investigator** (PI) is the researcher overall in charge of conducting the HI Global Registry Study, including producing de-identified reports, evaluating requests for de-identified data from third parties (such as, researchers, advocacy partners, and biotechnology firms doing relevant work), and ensuring the safety and ethics of the HI Global Registry, in accordance with the Institutional Review Board (IRB) approved study documents.
- **Co-investigators** are researchers who work with the PI to conduct the HI Global Registry Study, including producing de-identified reports, evaluating requests for de-identified data from third parties (such as, researchers, advocacy partners, biotechnology firms doing relevant work), and ensuring the safety and ethics of the HI Global Registry, in accordance with the Institutional Review Board (IRB) approved study documents.
- **The HIGR Research Team** includes the Research Director, Investigators and other personnel who support the operations of the Registry.
- The**Registry Steering Committee** is a collection of international HI representatives that provides oversight and expertise to the design and operation of the Registry from the patient, parent and clinical professional perspectives [https://congenitalhi.org/hi-gr-steering-committee/].
- **An Institutional Review Board** (IRB) is an appropriately constituted group 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).

In order to join the Registry, respondents will need to complete the following steps; they will see red asterisks which indicates a required field:

1. Register with the registry by creating an online account.
2. Start by clicking on the green Register button.
3. Fill in the required registration information, such as name, username and password.
4. Select contact preferences, which gives the Respondent an opportunity to choose why he or she would like to be contacted by the HIGR research team. For example, the Respondent can select to be contacted about potential clinical trials that he or she may be interested in; or being put in touch with other respondents who are reporting similar experiences.
5. Read Terms and Conditions of entering the NORD (National Organization of Rare Disorders) *I am Rare*Registry Platform, and agree to them by clicking "I have read and agree to the terms and conditions".
6. Confirm registration by copying the Confirmation Token from the confirmation email that will be automatically sent after accepting the terms and conditions.
7. The Respondent then needs to enroll himself or herself as the participant (patient) or add someone else as the participant. If the respondent adds someone else as the participant, then the relationship to the participant needs to be established. For example, if a parent is adding his or her child's information to the registry study, then as the Respondent the parent will need to select "parent (biological, adoptive, or step)" from the drop-down menu of responses. Or if the Respondent is another family member, such as the grandparent, then the Respondent would select "Grandparent" from the drop-down menu. Information about the participant, like his or her name and birthdate, will also be asked.
8. After enrolling, the respondent will need to provide consent to provide the participant's data in HIGR study. The conditions of participating in the study will be outlined clearly in the consent form. In order to proceed to take the study surveys the Respondent will need to agree to all the conditions of consent.

HIGR STEERING COMMITTEE

International Patient Advocates

Davelyn Hood, Chair
Isabel Calderón, Vice Chair
Julie Raskin
Ulrike Seyfarth
Maria Paz Oviedo
Michelle Walkley
Sarah Dearman

HI Patient Representative

Rianna Sommers

Scientific Advisors

Jean-Baptiste Arnoux
Indi Banerjee
Diva De León
Sian Ellard
Klaus Mohnike
Pratik Shah
Charles Stanley
Paul Thornton

Registry Manager

Jacqui Kraska

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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.