



Collaborative Research Network

Dare to Dream of a Future without Lows

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2023 CHI Family Conference
April 14-16, 2023
Children's Hospital of Philadelphia



**Rare
As
One**

CHI CRN Mission and Cornerstones

Create a congenital hyperinsulinism (HI) collaborative research network (CRN) that puts patients at the center of a strategy that leads to faster and more accurate diagnosis, drives new evidence-based treatments and cures, standardizes clinical guidelines, and facilitates increased and improved access.

The HI CRN has a commitment to:

- supporting **collaboration** across the globe;
- elevating the **patient voice** and ensuring the patient perspective is central to our work;
- guaranteeing **access** to information, medical specialists, and treatments regardless of income and geography;
- engaging **new researchers and ideas** to find innovative concepts and foster additional leaders in the HI network; and
- **addressing diversity, equity, and inclusion** in our work and our community.



Dare to Dream of a Future without Lows



CHI Congenital Hyperinsulinism International
CRN Collaborative Research Network



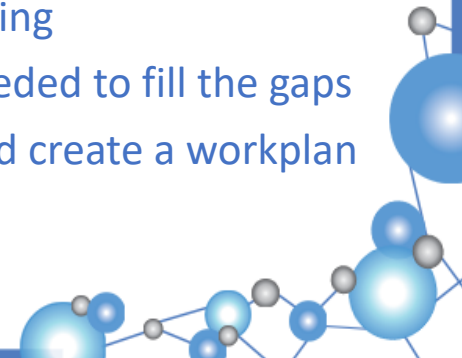
Workstreams

Themes

- What is HI?: Nomenclature and Inclusion
- Medical and surgical treatments
- Care Guidelines/centers of excellence
- Diagnostics
- Glucose monitoring
- Clinical trials/industry engagement
- Genetics

Activities and Deliverables

- Clearly defined workstream topics
- Brainstorm to generate a gap analysis document
- Identify the gaps in:
 - Research gaps or needs
 - Infrastructure/ Pooled Research Resources/ Collaboration
 - Knowledge/ Expertise
 - Dissemination/ Funding
- Describe the elements needed to fill the gaps
- Prioritize the elements and create a workplan



Workflow

Gap analysis and
research/ project
component identification



Completed by CRN Workstreams

Infrastructure
planning

Completed by CRN Core Team



CHI CRN
Convening

Prioritized
research agenda

Membership
structure

CRN
research
projects and
committee
work

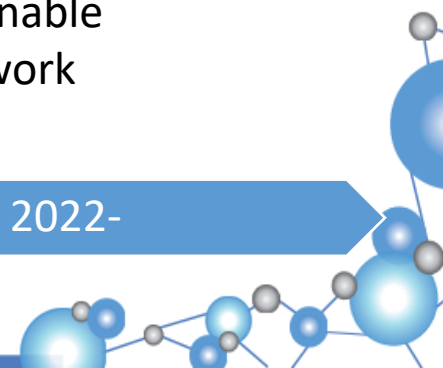
New research

Sustainable
network

Sustainable
Network

Phase 1: 2020- May 2022

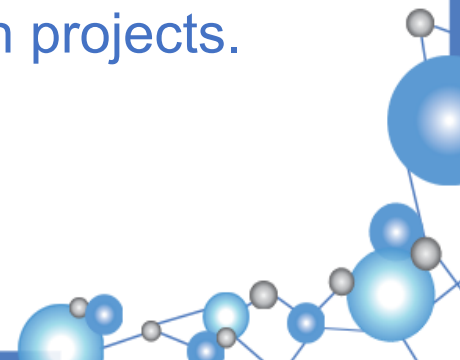
Phase 2: June 2022-



CHI CRN Convening Lisbon, Portugal May 20-22, 2022

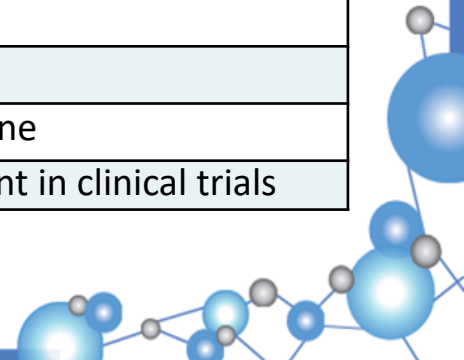
Meeting goals:

- (1) Finalize the first CHI prioritized research agenda;
- (2) Adopt a CRN membership structure and decide on communication strategies/ future meetings; and
- (3) Begin discussion of concrete collaborative research projects.



Top 12 Gaps/Needs Prioritized at Lisbon CRN Convening

Workstream	Gap/Need
All	HI Natural History Study
Diagnostics	Identification and adoption of newborn screening approach
Diagnostics	Glucose as a vital sign
Medical and Surgical Treatments	Knowledge of the cause of neurological damage
Care Guidelines & Centers of Excellence	Continually evolving global care guidelines
Clinical Trials & Industry Engagement	An expert group to develop novel clinical trials and outcome measures for regulatory approval
Genetics	Access to genetic testing
Diagnostics	A biomarker for hyperinsulinism
Clinical Trials & Industry Engagement	Meaningful glycemic endpoints
Glucose Monitoring	Device technology for HI patients
Medical and Surgical Treatments	Implementation of personalized medicine
Clinical Trials & Industry Engagement	Expand access and accelerate enrollment in clinical trials



Concrete Project Actions: Lisbon CHI CRN Convening

All projects will be mapped to the patient journey and will be led by goals and cornerstones

Identification and adoption of newborn screening approach/ Glucose as a vital sign

- Marketing campaign
- Newborn glucose and ketone pilot studies
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Knowledge of the cause of neurological damage

- Multi-center prospective cohort study utilizing CGMs and standardized outcome measures
- Basic science / lab studies for specific gene mutations and understanding mechanisms

Continually evolving global care guidelines

- Caregiver care guidelines
- Guidelines adapted for different languages/ cultures that reflect resource restraints
- Incorporation of the patient voice

An expert group to develop novel clinical trials and outcome measures for regulatory approval

- Assemble outside expertise
- PFDD FDA meeting
- Establish a workgroup and committee




Progress Since Lisbon

Explosion of Research

Did you know that...



 **79** articles about
congenital hyperinsulinism
were published over the
last year.

Search "congenital hyperinsulinism"
on pubmed.ncbi.nlm.nih.gov

