

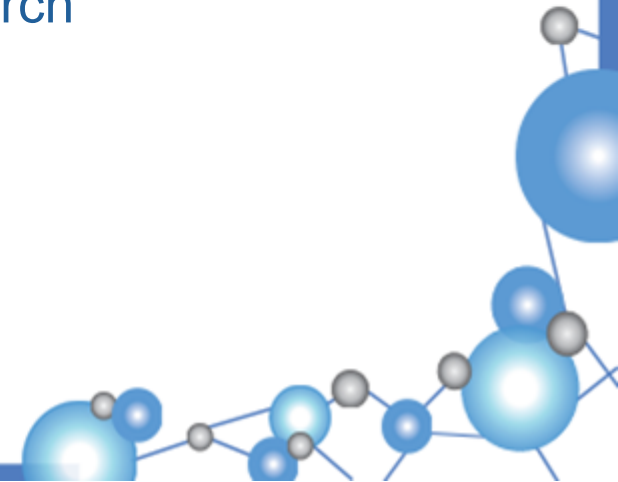


# CHI Collaborative Research Network



## Congenital Hyperinsulinism International **Collaborative Research Network**

- CHI established a Collaborative Research Network (CRN) for HI in 2020 thanks to a grant from the Rare As One program from the Chan-Zuckerberg Initiative.
- The CHI CRN is a diverse group of 60 academic and industry researchers, clinicians, and expert patient advocates from 19 countries.
- To accelerate tomorrow's breakthroughs, the CRN has extended research collaboration far beyond the traditional boundaries.



# Gap Analysis for Research Progress

CRN members worked virtually over 2 years to identify gaps in knowledge & resources

Identified >360 individual deficits across **7 key areas:**



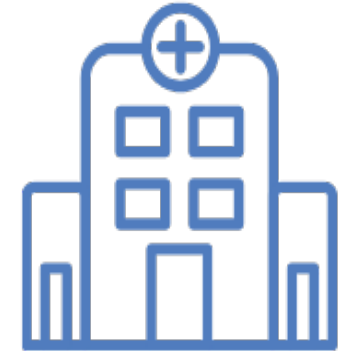
Genetics



Diagnosis



Glucose Monitoring



Care Guidelines & COEs



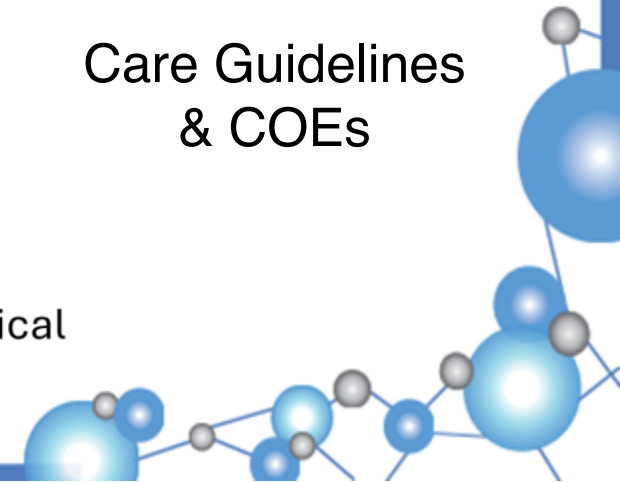
Clinical Trials & Industry Engagement



Nomenclature



Medication & Surgical Management

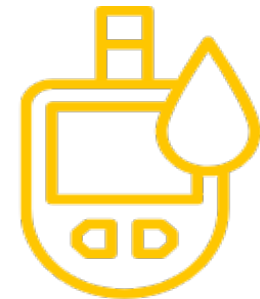


# Prioritized Research Agenda



# Current Work

- Newborn Screening (Diagnose all babies with congenital hyperinsulinism in a timely manner)
- Glucose as a Vital Sign (Increase awareness and timely diagnosis of HI)
- Care Guidelines (Create and disseminate continually evolving global care guidelines)
- Natural History (Build a robust registry that collects patient-reported, physician, and real-world data to provide a foundation for HI natural history)
- Continuous Glucose Monitoring (Create principles for Draft guidelines on CGM use in HI patients)



# Achievements

Active discussions to reform **newborn screening** for HI

Developed a consensus statement for global **advocacy and access needs**

One **drug trial** in Phase 3, one trial in Phase 2, and several in the preclinical phase

New HI drug receives **PDUFA** date

Developing guidelines for **Continuous Glucose Monitoring** in HI



Expert updates to the **Wikipedia** page for HI

Two in-person **convenings** and many virtual meetings

Expanded the HI Global Registry to **6 new languages**

One drug has FDA **breakthrough** designation

Wrote & published **international guidelines** to help diagnose and treat HI

Received **EU funding** for new HI **treatment** and development of **patient-reported** outcome measures

