

Developing a Multi-Disciplinary Collaborative Research Network to Accelerate the Understanding and Treatment of the Rare Disease Congenital Hyperinsulinism

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CHI CRN Congenital Hyperinsulinism International Collaborative Research Network

- Congenital Hyperinsulinism International (CHI) is an international nonprofit organization focused on improving the lives of patients and families living with HI.
- 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.
- The CRN plays a vital role in CHI's mission by fostering a multidisciplinary environment to drive sustainable research excellence.
- **The objective of this poster is to report on the formation of the HI CRN, to present our prioritized research agenda, and to report on current projects.**

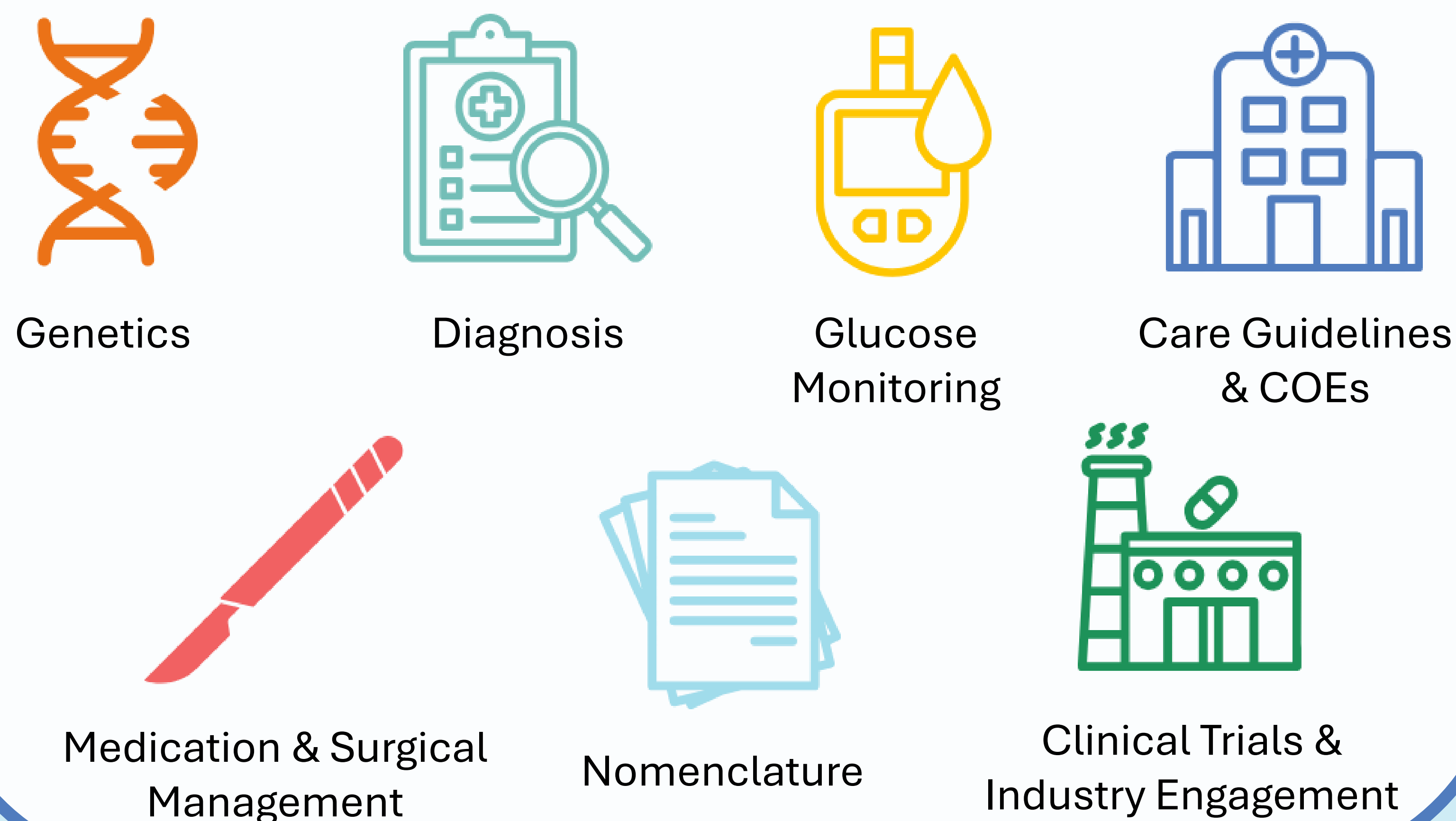
CONGENITAL HYPERINSULINISM

- Congenital Hyperinsulinism (HI) is a life-threatening condition that results in severe hypoglycemia (low blood sugar) and can lead to neurological damage (brain damage).
- Despite advances in understanding HI in recent decades, long-term neurologic outcomes have not significantly improved.

GAP ANALYSIS

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

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



PRIORITIZED RESEARCH AGENDA



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

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

New HI drug receives **PDUFA** date

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

CRN members authored **43% of all HI research** papers since 2020



ACHIEVEMENTS & CURRENT WORK



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

One **drug trial** in Phase 3

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

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

Adoption of **CRN priorities** by EU Endocrinology Society

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



Learn more about the **CHI CRN!**

