

Your Advocacy Organization: Support, COEs, CRN (advocacy and research), Clinical Research

Julie Raskin



CHI Support Programs

- Office Hours – Connecting individuals with HI and their families to the resources they need. Email jraskin@congenitalhi.org
- CHI Families and Individuals support each other: FB Group
 - FB Group link: <https://bit.ly/hi-fb-group>
- WEP Clinical – Helping source diazoxide when it is hard to come by.
- Special diazoxide country programs
- Family conference scholarships
- Hospital Grants
- Open Hyperinsulinism Genes Program (next page)



CHI Support Programs



Open Hyperinsulinism Genes Project

Genetic testing is necessary for patients to get proper treatment for hyperinsulinism.

CHI has funded genetic testing for 894 patients and family members from 61 countries through our program with the University of Exeter.

Program also contributes to research.



CHI Center of Excellence

Designation Program

The Centers of Excellence (COE) Program recognizes expert centers that provide the highest level of multi-disciplinary care to congenital hyperinsulinism patients and their families.





CHI Collaborative Research Network

A Research Collaborative for a Future Without Lows

Rare
As
One



The CHI CRN brings together 58 leading researchers, clinicians, and patients advocates from 16 countries working together on prioritized research and advocacy projects for earlier diagnosis, better access to care, and better treatments.





Universal Newborn Screening

- Working with Neonatologists
- Advocating for more stringent hypoglycemia guidelines
- Designing research projects that will generate data to change screening practice towards a universal screening program for hyperinsulinism



CHI Collaborative Research Network

A Research Collaborative for a Future Without Lows

Advocacy Track: Awareness

- Glucose as a Vital Sign Campaign
- More materials for people with HI

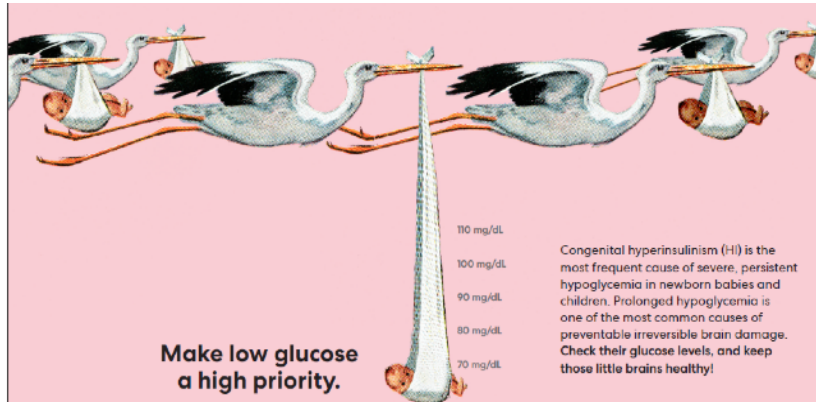


NEWBORN VITAL SIGNS
Temperature: Normal
Heartbeat: Normal
Breathing rate: Normal
Blood pressure: Normal
Oxygen saturation: Normal
Blood glucose: LOW

It's vital to check blood glucose in newborns. Prolonged hypoglycemia is one of the most common causes of preventable irreversible brain damage. A healthy brain depends on normal blood glucose.




CHI Congenital Hyperinsulinism International
www.congenitalhi.org



Make low glucose a high priority.

110 mg/dL
100 mg/dL
90 mg/dL
80 mg/dL
70 mg/dL

Congenital hyperinsulinism (HI) is the most frequent cause of severe, persistent hypoglycemia in newborn babies and children. Prolonged hypoglycemia is one of the most common causes of preventable irreversible brain damage. Check their glucose levels, and keep those little brains healthy!



CHI Congenital Hyperinsulinism International
www.congenitalhi.org



Check for signs of hyperinsulinism.
She'll thank you when she can talk.

It's vital to check blood glucose in newborns. Prolonged hypoglycemia is one of the most common causes of preventable irreversible brain damage.



CHI Congenital Hyperinsulinism International
www.congenitalhi.org





CHI Collaborative Research Network

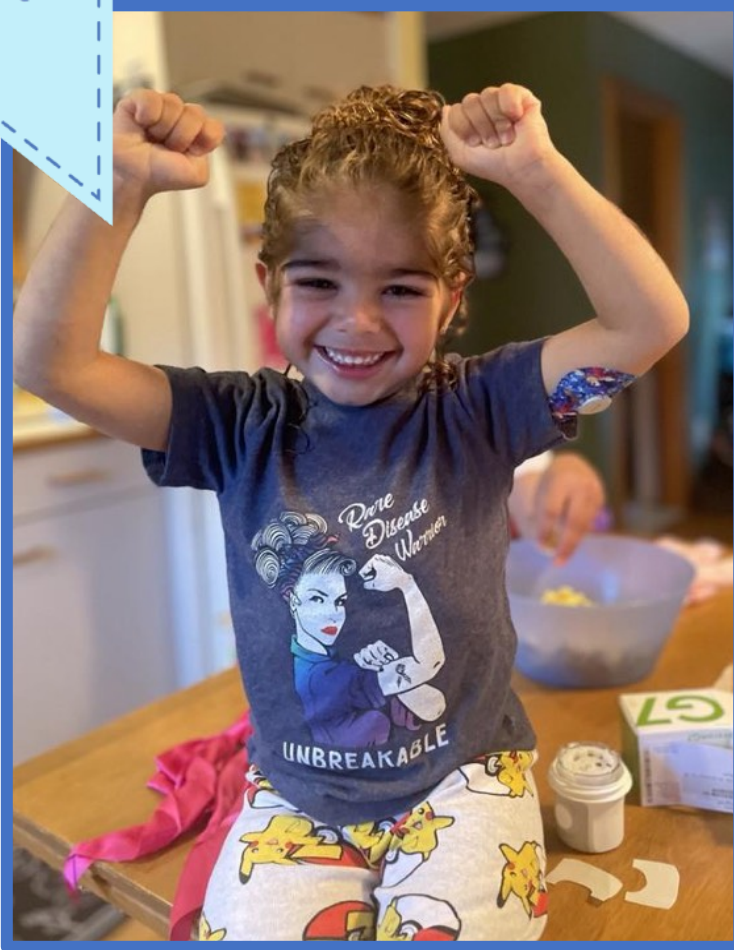
A Research Collaborative for a Future Without Lows



Advocacy Track: Access

- Universal statement on needs of every person with HI
- Advocate to increase access





Continuous Glucose Monitoring

- Tools for assessing value to individuals with HI
- Materials for medical professionals and families
- Better CGMs
- Access to CGMs



CHI Collaborative Research Network

A Research Collaborative for a Future Without Lows

Natural History

- Increase understanding of hyperinsulinism throughout a lifetime
- Grow and increase diversity of HIGR to reflect all patients and caregivers worldwide
- Add glucometer and CGM collection to HIGR
- Create Biobank



Our Community Goal: Future Without Lows

The HI community needs new therapies and approaches so people with HI can live life even more fully.



Willow loves to swim,
but her mom worries about blood sugar drops
when she does.

Learn about HI clinical trials.
#FutureWithoutLows

CHI



CHI's Role in Clinical Research

- Patient Voice: Convey the experience of living with HI to drug developers and ensure they try and develop treatments that are what patients want.
- One of CHI's important roles is to inform the patient community of clinical research opportunities in order to help raise awareness of and increase participation in clinical research and clinical trials
- CHI's role does not include endorsing specific studies
- CHI strongly recommends discussing clinical research participation for each specific study with your physician specialist or healthcare provider



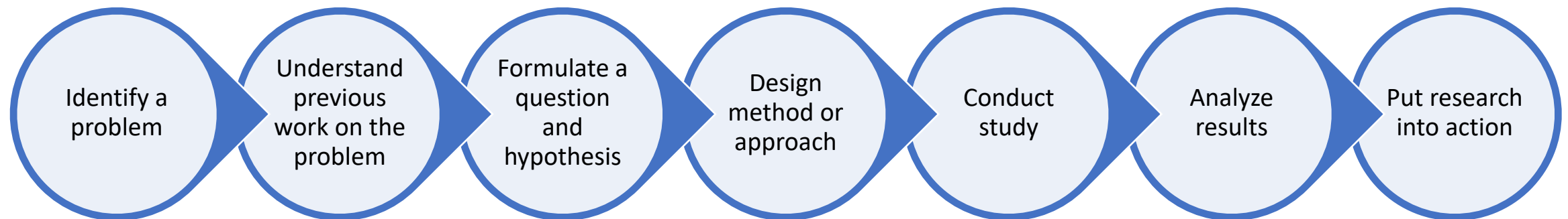
Current Challenges: Why are New Treatments Needed?

- Long hospital stays before baby can go home
- Many with HI still have significant hypoglycemia on therapy
- From HI to diabetes: subtotal pancreatectomies
- Some respond to diazoxide but can't take it because of adverse effects or want an alternative
- Restricted activities and lifestyle because of hypoglycemia or fear of it/Exhausted or stressed parents
- More independence for children with HI
- Feeding issues: Not wanting to eat or needing to eat often



What is Clinical Research to Develop New Treatments?

- Research project conducted with people to learn more about new potential treatments
- Every new therapy or device must go through carefully monitored studies in people before being available at the pharmacy
- Clinical trials that safeguard the health of study participants are necessary for the development of new HI treatments
- Conducted by industry or academic centers



Drug Development Timeline



Goal: Focus on safety, finding the proper dose range, and identify any side effects

Participants: A small group of healthy volunteers

Goal: Focus on effectiveness and side-effects

Participants: A relatively small group of individuals with the disease of interest

Goal: Compare the safety and effectiveness of the new treatment against the current treatment

Participants: A larger group of individuals with the disease of interest



Why Participate in Clinical Research?

- Research increases our knowledge of HI and potentially leads to new and better treatments with fewer undesirable effects
- More treatments available for HI allow for more personalized approach to treatment
- Allows access to specialist care for those who may not have regular access to HI specialists
- Personal satisfaction of being part of scientific innovation as a member of the research team and process



How to Participate?

- If you hear about a clinical trial that you may be eligible for, you can talk to your doctor, or your doctor may recommend a trial to you
- Most studies will have a screening survey to confirm your eligibility
- Participating in research is a partnership
 - You can ask questions before, during, and after the study
- Study staff will explain what they are hoping to learn from the study and ensure you know what to expect
 - This is called informed consent
 - If your child is participating, they will be given kid-friendly documents to provide assent as well



Find Clinical Trial Opportunities

- CHI Clinical Trials Webpage
 - <https://congenitalhi.org/clinical-research-trials/>
- U.S. Clinical Trials
 - www.clinicaltrials.gov
- EU Clinical Trials Register
 - www.clinicaltrialsregister.eu/ctr-search/search
- WHO International Clinical Trials Registry
 - <https://www.who.int/clinical-trials-registry-platform>



Thank You!!!!

