



# Congenital Hyperinsulinism Collaborative Research Network (CRN) Convening December 5-6, 2020

The schedule is set in the Eastern Standard Time (EST, GMT-5)

## SATURDAY, December 5

9:00 AM Event Space Opens (Booths, Patient Information, Lobby space, Lounge)

### 11:00 AM **Opening Session**

You may enter session beginning at 10:45 AM EST. The program begins promptly at 11 AM EST.

#### **Welcome from your CRN Team**

- Julie Raskin
- Tai Pasquini, PhD
- Jennifer Schmitt
- Diva D. De León-Crutchlow, MD, CHOP
- Paul Thornton, MD, Cook Children's
- Davelyn Hood, MD

11:10 AM **What is a CRN? Why a CRN?** CHI CRN Mission, Goals, and Cornerstones

11:35 AM **Histories** - *Patients and their caregivers will describe the kind of HI that affects them:*

- Diazoxide Responsive Negative Genetics Diffuse
- ABCC8/KCNJ11 Non Diazoxide Responsive
- ABCC8/KCNJ11 Diazoxide Responsive
- HIHA
- GCK HI
- Focal HI
- HNF1a and 4a Defects
- MCT-1 HI
- SCHAD-HI
- UCP-2 HI
- Kabuki Syndrome
- Turner's Syndrome
- Beckwith-Wiedemann Syndrome
- Sotos Syndrome

- Rubinstein-Taybi syndrome
- HI -> Ketotic Hypoglycemia
- HI -> Diabetes
- Unknown Type

12:00 PM

**Guided Stretching and Relaxing**

12:15 PM

**Virtual Breakout Rooms - By HI Type for patients and led by Members of the CHI Scientific Advisory Group:**

- Diazoxide Responsive Negative Genetics Diffuse
- ABCC8/KCNJ11 Non Diazoxide Responsive
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- HIHA
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- Focal HI
- HNF1a and 4a Defects
- MCT-1 HI
- SCHAD-HI
- UCP-2 HI
- Kabuki Syndrome
- Turner's Syndrome
- Beckwith Weidemann Syndrome
- Sotos Syndrome
- Rubenstein Taybi Syndrome
- HI -> Ketotic Hypoglycemia
- HI -> Diabetes
- Special topics for industry and physicians

12:45 PM

**CHI Research: How Far we Have Come**

- Research Timeline (accomplishments and breakthroughs in treatment and diagnostics)  
Diva D. De León-Crutchlow, MD, CHOP  
Paul Thornton, MD, Cook Children's
- Recognizing the HI Greats (including biotech researchers)

1:15 PM

**Break**

1:30 PM

**Clinical Research**

- Participation in Clinical Research - Davelyn Hood
- Commercial Pre-Clinical and Clinical Trials Timeline - Julie Raskin
- Presentations from Biotechs on current trials and pre-clinical programs (Background, goal, inclusion and exclusion criteria, and locations/map)

2:15 PM

**Community Check-In/ Open discussion**

2:30 PM-4:00 PM

**Time to Visit the Networking Lounge or Visit the Exhibit Hall**

4:00 PM-5:00 PM **Virtual Happy Hour**

## **SUNDAY, December 6**

11:00 AM

### **Second Day Begins**

You may enter session beginning at 10:45 AM EST. The program begins promptly at 11 AM EST.

#### **Welcome to Day 2**

Recap and HI International Stories

11:15 AM

#### **Introducing the CRN Methods:**

##### **Concept of Gap Analysis**

- Identifying the gaps in structure, knowledge, expertise (human resources) tools, and funding to treat HI more effectively and ultimately find a cure
- Project goal is to address gaps; prioritize research; and fund research
- The Workstream committees will come together at a convening in 2022, after they have each completed their work, at which point a prioritized research agenda will be discussed and agreed upon by the project members with input from the whole CHI Community.

##### **Workstreams Introduction**

- Medical and Surgical Treatments
- Care Guidelines/Centers of excellence
- Diagnostics
- Glucose monitoring
- Clinical trials/ industry engagement
- Genetics
- Infrastructure/ sustainability

11:45 AM

#### **Questions and Answers**

12:00 PM

#### **What is your vision for HI by topic? (First Breakout Room)**

- Medical and Surgical Treatments
- Care Guidelines/Centers of excellence
- Diagnostics
- Glucose monitoring
- Clinical trials/ industry engagement
- Genetics
- Infrastructure/ sustainability

12:45 PM

#### **Break**

- 1:00 PM **What is Your Vision for HI by Topic? (Second Breakout Room)**  
Topics as above
- 1:45 PM **Report Back and Introduce “What is HI?” Session**
- 2:10 PM **What is HI? (Interactive Small Group Session)**  
In this interactive session members will have an opportunity to talk about how they define HI. Is it just a blood sugar disease? Are there other health issues that need to be addressed with clinical trials and endpoints? Should we have more agreement on how we abbreviate the name of the condition? Should “congenital” be part of the name? How do we define transient disease?
- 2:40 PM **Breakout Rooms Report Back**
- 3:00-3:30 PM **Next Steps and Timeline**