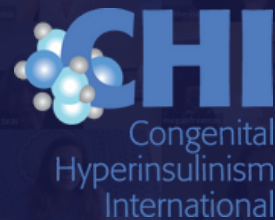


2021 Congenital Hyperinsulinism Research Conference



Saturday, September 18, 2021

10:00 AM EDT – 3:00 PM EDT

EDT – Eastern United States Daylight Time

9:00 - 10:00 AM

Booths Open

10:00 - 10:15 AM

Welcome and Introduction to the Collaborative Research Network (CRN)

- The CHI Staff: Julie Raskin, MA, Tai Pasquini, PhD, Jennifer Schmitt, Katherine Lewis, and Mahlet Mesfin
- CRN Core Team Members: Diva D. De León-Crutchlow, MD, Children's Hospital of Philadelphia (CHOP) and Paul Thornton, MD, Cook Children's Medical Center

10:15 - 10:40 AM

Diagnostics

- **Summary of Workstream Gap Analysis**
 - Workstream Chair: Katherine Lord, MD, CHOP
 - Parent Perspective: Lora Van Arsdell, PsyD, HI Parent
- **18F-FDOPA PET Imaging in Congenital Hyperinsulinism for Localization of a Focal Pancreatic Lesion: Do Medications Interfere With Radiotracer Uptake?**
Nithya Navarathna, CHOP
- **Birth Weight and Diazoxide Response Can Predict the Likelihood of KATP CHI**
Thomas Hewat, PhD Candidate, University of Exeter

10:40 - 11:10 AM

Genetics

- **Summary of Workstream Gap Analysis**
 - Workstream Co-Chair: Sarah Flanagan, PhD, University of Exeter
 - Parent Perspective: Sheila Bose, HI Parent
- **Genotype-Phenotype Correlation of Congenital Hyperinsulinism in Indian patients**
Radha Venkatesan, M.Sc, PhD, Madras Diabetes Research Foundation
- **Discovering New Genes in Congenital Hyperinsulinism**
Klaus Brusgaard, PhD, Odense University Hospital
- **Congenital Hyperinsulinism Diagnosed After 12 Months can be Genetic**
Jasmin Hopkins, PhD Candidate, University of Exeter

11:10 - 11:30 AM

Glucose Monitoring

- **Summary of Workstream Gap Analysis**
 - Workstream Co-Chair: Antonia Dastamani, MD, Great Ormond Street Hospital for Children
 - Parent Perspective: Cristina Pindado, HI Parent
- **Continuous Glucose Monitoring as a Phenotyping Tool: Expanding the Digital Phenotype of CHI**
Chris Worth, PhD Candidate, Royal Manchester Children's Hospital

11:30 - 12:00 PM

Medical and Surgical Treatments

- **Summary of Workstream Gap Analysis**
 - Workstream Co-Chair: Indi Banerjee, MD, Northern Congenital Hyperinsulinism Service
 - Parent Perspective: Irene Promussuas, PhD, HI Parent, Lobby4Kids
- **Outcomes of Feeding Interventions on Patients With Genetic HI**
Paul Thornton, MD, Cook Children's Medical Center
- **The Effectiveness of Low Dose Glucagon Infusions (<5mcg/kg/hr) in Preventing Hypoglycemia**
Paul Thornton, MD, Cook Children's Medical Center
- **Medical Treatment During Pregnancy of Women With Persistent Congenital Hyperinsulinism**
Klaus Mohnike, MD, Collaborative Alliance on Congenital Hyperinsulinism and Susann Empting, Otto-von-Guericke-Universität

12:00 - 12:30 PM

BREAK - See session information for description

12:30 - 1:00 PM

Care Guidelines and Centers of Excellence

- **Summary of Workstream Gap Analysis**
 - Workstream Co-Chair: Louise Conwell, MBBS, PhD, Queensland Children's Hospital
 - Parent Perspective: Sandra Melo, HI Parent
- **'Silver-lining' of a Pandemic for an Infant With Focal Congenital Hyperinsulinism: Transport of a Tracer Rather Than a Baby 924km Across Australian State Borders**
Louise Conwell, MBBS, PhD, Queensland Children's Hospital
- **Patient with CHI in Resource-Limited and Pandemic Settings**
Nino Kheladze, MD, M. Iashvili's Central Hospital
- **Duration of Fasting Studies to Demonstrate Cure in Focal HI**
Paul Thornton, MD, Cook Children's Medical Center

1:00 - 2:05 PM

Clinical Trials and Industry Engagement

- **Summary of Workstream Gap Analysis**
 - Workstream Co-chair: Julie Raskin, MA, CHI
 - Parent Perspective: Mike Ferguson, HI Parent
- **Congenital Hyperinsulinism Treatment Gaps**
Brian Roberts, MD, Rezolute Bio
- **Standardized CGM Time in Range Targets Needed for CHI**
Davelyn Hood, MD, MBA, Rezolute Bio
- **Single Dose Results From a Phase 1 Clinical Trial of CRN04777, an Orally Bioavailable SST5-Selective, Nonpeptide Somatostatin Agonist for the Treatment of Congenital Hyperinsulinism: Pharmacokinetics and Pharmacodynamics in Healthy Volunteers**
Chris Ferrara-Cook, MD, PhD, Crinetics Pharmaceuticals
- **Avexitide [Exendin (9-39)] for Treatment of Congenital Hyperinsulinism**
Colleen M. Craig, MD, Eiger BioPharmaceuticals
- **A Selective Nonpeptide Somatostatin Receptor 5 (SST5) Agonist Effectively Decreases Insulin Secretion in a KATPHI Mouse Model and in Human HI Islets**
Diva D. De León-Crutchlow, MD, MSCE, CHOP
- **The Effects of GDH Inhibitor on GDH Gain-of-Function Mutation (H454Y) Knockin Mice**
Changhong Li, MD, PhD, Nanjing AscendRare Pharmaceutical Technology Incorporation

2:05 - 2:35 PM

What is HI? Nomenclature and Inclusion

- **Summary of Workstream Gap Analysis**
 - Workstream Co-chair: Charles Stanley, MD, CHOP
 - Parent Perspective: Ulrike Seyfarth, HI Parent, Kongenitaler Hyperinsulinismus e.V.
- **Persistent Hyperinsulinemic Hypoglycemia due to an Insulinoma in a Two-Year-Old Child**
Julia Crowley, MD, CHOP, Melinda Danowitz, DO, MS, CHOP, and Diva D. De León-Crutchlow, MD, MSCE, CHOP
- **HI Global Registry**
Tai Pasquini, PhD and Mahlet Mesfin, CHI

2:35 - 2:45 PM

Final Words

Julie Raskin

2:45 - 4:00 PM

Exhibit Booths Open

Confirmed Panelists, Presenters, and Moderators



Indi Banerjee, MBBS, MD, MRCPCH

Pediatric Endocrinologist, Clinical Lead, Northern Congenital Hyperinsulinism Service (NORCHI)
Royal Manchester Children's Hospital, Manchester, UK

Sheila Bose

HI Parent, President, CHI Board of Directors
MA, USA

Klaus Brusgaard, PhD, MSc

Associate Professor, Department of Clinical Genetics
Odense University Hospital, Odense, Denmark

Louise Conwell, MBBS PGCert Med FRACP PhD

Head of Children's Health
Queensland Children's Hospital, University of Queensland, Australia

Colleen M. Craig, MD

Vice President, Metabolic Diseases
Eiger BioPharmaceuticals, CA, USA

Julia Crowley, MD

Fellow in the Division of Endocrinology and Diabetes
Children's Hospital of Philadelphia (CHOP), PA, USA

Melinda Danowitz, DO, MS

Fellow in the Division of Endocrinology and Diabetes
CHOP, PA, USA

Antonia Dastamani, MD, PhD

Pediatric Endocrinologist
Great Ormond Street Hospital (GOSH), London, UK

Diva D. De León-Crutchlow, MD, MSCE

Chief of the Division of Endocrinology and Diabetes, Director of the Congenital Hyperinsulinism Center
CHOP, PA, USA

Susann Empting

Otto-von-Guericke-Universität
Magdeburg, Germany

Mike Ferguson

HI Parent, Biopharma Strategist
Toronto, Canada

Chris Ferrara-Cook, MD, PhD

Senior Medical Director, Endocrinology Clinical Development
Crinetics Pharmaceuticals, CA, USA

Sarah Flanagan, PhD

Associate Professor of Genomic Medicine and Sir Henry Dale Fellow
University of Exeter, Exeter, UK

Thomas Hewat

PhD Candidate
University of Exeter, Exeter, UK

Davelyn Hood, MD, MBA

Director of Scientific & Patient Affairs
Rezolute Bio, TX, USA

Jasmin Hopkins

PhD Candidate
University of Exeter, Exeter, UK

Christine Juliana, PhD

Scientist at the Congenital Hyperinsulinism Center
CHOP, PA, USA

Nino Kheladze, MD

Pediatric Endocrinologist
M. Iashvili Children's Hospital, Tbilisi, Georgia

Katherine Lewis

Communications and Engagement Associate
CHI, VA, USA

Changhong Li, MD, PhD

Associate Director
Nanjing AscendRare Pharmaceutical Technology Incorporation, Jiangsu, China

Katherine Lord, MD

Pediatric Endocrinologist
CHOP, PA, USA

Sandra Melo

HI Parent
Setubal, Portugal

Mahlet Mesfin

Research and Policy Associate
CHI, NJ, USA

Klaus Mohnike, MD

Pediatric Endocrinologist, Professor
Collaborative Alliance on Congenital Hyperinsulinism (COACH), Otto-von-Guericke-Universität
Magdeburg, Germany

Nithya Navarathna

Undergraduate Student, Research Intern
CHOP, PA, USA

Tai Pasquini, MPA, PhD

Research and Policy Director
CHI, MA, USA

Cristina Pindado

HI Parent, President, La Asociación de Afectados por Hiperinsulinismo Congénito (AFHICO)
Barcelona, Spain

Irene Promussas, PhD

HI Parent, Chairwoman, Lobby4Kids
Vienna, Austria

Julie Raskin, MA

Executive Director

CHI, NJ, USA

Brian Roberts, MD

Head of Clinical Development

Rezolute Bio, CA, USA

Jennifer Schmitt

Conference Co-Director

CHI, NJ, USA

Ulrike Seyfarth

HI Parent, President, Kongenitaler Hyperinsulinismus e.V.

Charité, Berlin, Germany

Charles Stanley, MD

Founder of the Congenital Hyperinsulinism Center, Professor of Pediatrics

CHOP, PA, USA

Paul Thornton, MB, BCh, MRCPI, DCh

Medical Director, Endocrine and Diabetes Program

Cook Children's Medical Center, TX, USA

Lora Van Arsdell, PsyD LCSW

HI Parent

Saint Martin's University Counseling and Wellness Center, WA, USA

Radha Venkatesan, M.Sc, PhD

Head, Molecular Genetics

Madras Diabetes Research Foundation, Chennai, India

Chris Worth

PhD Candidate

Royal Manchester Children's Hospital, Manchester, UK