Congenital Hypoglycemia Disorders: Hyperinsulinism and GSD

April 14 and 15, 2016

Ruth and Tristram Colket, Jr. Translational Research Building at The Children’s Hospital of Philadelphia

Sponsored by The Congenital Hyperinsulinism Center Division of Endocrinology and Diabetes

www.chop.edu/cme

The Children’s Hospital of Philadelphia®
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Session 1: Congenital Hyperinsulinism (CHI)  
8 – 10 a.m.  Chair: Paul Thornton, M.D.

8 a.m.  Welcome and Introductions  
Divá D. De León, M.D., M.S.C.E.

8:15 a.m.  History of Hyperinsulinism (HI) in Pediatrics and Overview of Diagnostic/Therapeutic Algorithms  
Charles A. Stanley, M.D.

8:30 a.m.  Diazoxide-responsive Hyperinsulinism  
- GDH-HI  
- KATP-HI  
- Perinatal Stress HI  
- HNFs-HI  
Charles A. Stanley, M.D.

9 a.m.  Diazoxide-unresponsive Hyperinsulinism  
- KATP-HI: dominant vs. recessive, focal vs. diffuse  
- Atypical – LINE  
- GCK  
Divá D. De León, M.D., M.S.C.E.

9:30 a.m.  Syndromic Hyperinsulinism  
BWS – Jennifer Kalish, M.D., Ph.D.  
Kabuki Syndrome – Jean Baptiste Arnoux, M.D., M.Sc.

10 a.m.  Break and Exhibits

Session 2: Other Causes of Hyperinsulinemic Hypoglycemia  
10:30 – 11:20 a.m.  Chair: Paul Thornton, M.D.

10:30 a.m.  Short Communications (10 minutes each)  
UCP2 Hyperinsulinism — Christine Ferrara, M.D., Ph.D.  
PGM1 Hyperinsulinism — Amanda Ackerman, M.D., Ph.D.  
Hyperinsulinism in Turner Syndrome — Christopher Gibson, M.D.

11 a.m.  Insulinomas and HI Mimickers  
Andrew Palladino, M.D.

11:20 a.m.  Lunch — Exhibits and Scientific Posters

Session 3: Diagnosis and Treatment of Hyperinsulinism  
12:30 – 2:20 p.m.  Chair: Mark A. Sperling, M.D.

12:30 p.m.  Approach to the Diagnosis and Management of Hyperinsulinism  
Divá D. De León, M.D., M.S.C.E.

12:50 p.m.  Genotype/Phenotype Correlation in KATP Hyperinsulinism  
Arupa Ganguly, Ph.D.

1:05 p.m.  The Role of Genetic Testing for Hyperinsulinism: New Frontiers in Genetic Diagnosis  
Sian Ellard, Ph.D., F.R.C.Path.

1:25 p.m.  Surgical vs. Nonsurgical Decisions and Options  

continued >
2 p.m.  Long-acting Somatostatin Analogs  
Jean Baptiste Arnoux, M.D., M.Sc.

2:20 p.m.  Break, Exhibits and Scientific Posters

Session 4: Investigational Therapies for Hyperinsulinism  
2:40 – 4:10 p.m.  Chair: Mark A. Sperling, M.D.

2:40 p.m.  Investigational Therapies: Exendin-(9-39)  
Divia D. De León, M.D., M.S.C.E.

2:55 p.m.  Investigational Therapies: Sirolimus  

3:10 p.m.  Investigational Therapies: Subcutaneous Glucagon  
Susann Empting, M.D.

3:25 p.m.  Investigational Therapies: Insulin Receptor Antibody  
Divia D. De León, M.D., M.S.C.E.

3:40 – Oral Communications (3 – 10 min. each)

4:10 p.m.  The Molecular and Pathological Features of Insulinomas  
Tricia Bhatti, M.D.  
The Children's Hospital of Philadelphia

Search for Allosteric Regulators of Glutamate Dehydrogenase: Treatment of the Hyperinsulinism/Hyperammonemia Disorder  
Thomas J. Smith, Ph.D.  
University of Texas Medical Branch at Galveston

Incidence, Phenotype and Current Outcomes of Infants with Prolonged/Protracted Hyperinsulinemic Hypoglycemia in a Women’s and Children’s Hospital  
KK Women’s and Children’s Hospital  
Duke-NUS Medical School, Singapore

Session 5: Surgical Management Approaches to Hyperinsulinism  
4:10 – 5 p.m.  Chair: Mark A. Sperling, M.D.

4:10 p.m.  [18F]-DOP A PET Imaging of Focal Hyperinsulinism: “GPS” for the Surgeon  
Lisa J. States, M.D.

4:30 p.m.  Surgery for Hyperinsulinism  
N. Scott Adzick, M.D., M.M.M., F.A.C.S., F.A.A.P.

5 p.m.  Adjourn

5:30 p.m.  Symposium Dinner  
Keynote Speaker: Franz M. Matschinsky, M.D.
Program – Friday, April 15, 2016

Session 6: Prevention of Brain Damage
8 – 9:40 a.m. Chair: Charles A. Stanley, M.D.

8 a.m.                  Hypoglycemia Guidelines
                       Paul Thornton, M.D.

8:20 a.m.             Neurodevelopmental Outcomes
                       Diva D. De León, M.D., M.S.C.E.

8:50 a.m.             Management after Pancreatectomy: Persistent Hypoglycemia and Hyperglycemia
                       David Langdon, M.D., F.A.A.P.

9:10 – 9:40 a.m.    Oral Communications (3 – 10 min. each)

9:40 a.m.             The Hypoglycemic Phonotype Is Islet Cell-autonomous in Short-chain Hydroxyacyl-CoA Dehydrogenase (SCHAD) Deficient Mice
                       Anders Molven, Ph.D.
                       University of Bergen, Norway

Syndromic Hypoketotic, Hypoinsulinemic Hypoglycemia Due to a Mosaic Activating Phosphatidylinositol 3-kinase (PI3K) Mutation
                       Sebastian Hummer, M.D.
                       University Children's Hospital, Duesseldorf, Germany

Diagnostic Performance of [18F]-DOPA PET/CT and 68Ga*Dotanoc PET/CT Scan on Focal vs. Non-focal Type of Congenital Hyperinsulinism
                       Henrik T. Christesen, M.D., Ph.D.
                       Hans Christian Andersen Children's Hospital, Odense University Hospital, Odense, Denmark

9:40 a.m.             Break, Exhibits and Scientific Posters

Session 7: Islet Pathophysiology in Hyperinsulinism
10 – 11:30 a.m. Chair: Charles A. Stanley, M.D.

10 a.m.                  ß-cell KATP Channels in Health and Disease
                       Show-Ling Shyng, Ph.D.

10:20 a.m.            Insulin Secretion and Fuel Metabolism in Human HI Islets
                       Changhong Li, M.D., Ph.D.

10:40 a.m.            Beyond the ß-cell
                       Mark Dunne, Ph.D.

11 – 11:30 a.m.    Oral Communications (3 – 10 min. each)

11:30 a.m.           Characterization of a Novel Murine Model of Glycogen Storage Disease Type VI
                       Tayoot Chongsupanimit, B.S.
                       Glycogen Storage Disease Program, Department of Pediatrics, University of Florida

Gene Therapy for Murine Glycogen Storage Disease Type Ib
                       Youngmok Lee, Ph.D.
                       Glycogen Storage Disease Program, Department of Pediatrics, University of Florida

11:30 a.m.            Lunch — Exhibits and Scientific Posters

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Session 8: Hyperketotic Hypoglycemia
1 – 2:50 p.m. Chair: Diva D. De León, M.D., M.S.C.E.

1 p.m. Overview of Ketotic Hypoglycemia Disorders (Glycogenosis, MCT1)
Joseph Wolfsdorf, M.D., B.Ch.

1:30 p.m. Natural History of GSD
David Weinstein, M.D., M.M.Sc.

2 p.m. Current and Investigational Therapies for GSD
David Weinstein, M.D., M.M.Sc.

2:30 p.m. PGM1 in the Regulation of Glucose Metabolism and Insulin Secretion
Eva Morava-Kozicz, M.D., Ph.D.

2:50 p.m. Break, Exhibits and Scientific Posters

Session 9: Breakout Sessions — Q&A with the Experts
3:10 – 4 p.m.

Group A:
Management of the Diazoxide-responsive Children

Group B:
Management of the Diazoxide-unresponsive Children

Group C:
Management of Diabetes after Pancreatectomy

Group D:
Management of Children with GSD

4 p.m. Closing Remarks
Charles A. Stanley, M.D.

4:15 p.m. Adjourn

Acknowledgement
The “Congenital Hypoglycemia Disorders: Hyperinsulinism and Glycogen Storage Disease (GSD) Symposium” is supported by an educational grant provided by the Orphan Disease Center at the University of Pennsylvania. The Congenital Hyperinsulinism Center at The Children’s Hospital of Philadelphia gratefully acknowledge this support.

Please note: This meeting will be followed by a two-day conference for patients, parents and families of children with HI on Saturday, April 16 and Sunday, April 17. Co-sponsored by Congenital Hyperinsulinism International (CHI), more information will be available on CHI’s website, www.congenitalHI.org, and will also be posted on our Web page, hyperinsulinism.chop.edu. Please encourage families of any patients you follow to consider joining us. Professional staff are also welcome to join us for this more informal informational exchange.
Program Overview
This two-day international symposium will be an update on recent exciting advances in research related to congenital hyperinsulinism and other inherited hypoglycemia disorders, particularly disorders of glycogenoses. The symposium is intended to enhance the clinical management skills and practices of endocrinologists, metabolism specialists, neonatologists, surgeons, pathologists, radiologists, geneticists and nurses, as well as basic scientists interested in pancreatic islet biology and regulation of glucose metabolism. The symposium faculty, chosen from 10 institutions in the United States, France, Great Britain, and Germany, will present 27 plenary lectures on all aspects of these disorders. Participants may register via mail or fax by completing the Registration Form attached to the brochure and indicating their form of payment. To register online go to www.chop.edu/cme.

General Information
The symposium will be held on Thursday, April 14 and Friday, April 15, 2016, at the Ruth and Tristram Colket, Jr. Translational Research Building, located at 3614 Civic Center Blvd. on the campus of The Children's Hospital of Philadelphia (www.chop.edu). Registration will begin each day at 7:30 a.m. The registration fee includes continental breakfast, lunch, breaks, parking and syllabus materials. Participants are invited to attend a reception and dinner on Thursday, April 14 for an additional fee of $75. If you wish to attend, please check this option on the registration form and add this fee to your total registration fee.

Learning Objectives
Upon completion of this live activity, participants should be able to:
• Provide an overview of the clinical manifestations, genetics and natural history of congenital hyperinsulinism and disorders of glycogenoses
• Review current knowledge of the normal regulation of insulin secretion and the pathophysiology of disorders causing hyperinsulinism
• Discuss recent advances and current controversies in the surgical and medical management of diffuse and focal hyperinsulinism

Abstract Submissions
The program includes three sessions at which attendees may present posters and short oral communications of their own clinical or laboratory research. Abstracts must be submitted electronically using the registration form on the Congenital Hyperinsulinism Symposium website (www.chop.edu/cme). The deadline for abstract submission is Friday, Jan. 8, 2016. Authors will be notified of abstracts accepted for poster or oral presentations by Monday, Feb. 1, 2016. A limited number of travel grants will be available to young investigators wishing to apply. Young investigators must apply at the time they register for the symposium and submit their abstract.

ACCME Accreditation Statement
The Children’s Hospital of Philadelphia is accredited by the Accreditation Council for Continuing Medical Education (ACCME) to provide continuing medical education for physicians.

AMA Credit Designation Statement
The Children’s Hospital of Philadelphia designates this live activity for a maximum of 13.0 AMA PRA Category 1 Credit(s)™. Physicians should claim only the credit commensurate with their participation in the activity.

Nursing Accreditation
This activity has been submitted to the PA State Nurses Association for approval to award contact hours. The PA State Nurses Association is accredited as an approver of continuing nursing education by the American Nurses Credentialing Center’s Commission on Accreditation.

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Course Faculty

Symposium Course Directors

Diva D. De León, M.D., M.S.C.E.
Director, Congenital Hyperinsulinism Center
Associate Professor of Pediatrics

Charles A. Stanley, M.D.
Founder, Congenital Hyperinsulinism Center
Emeritus Professor of Pediatrics

Faculty

N. Scott Adzick, M.D., M.M.M., F.A.C.S., F.A.A.P.
Surgeon-in-Chief
C. Everett Koop Professor of Pediatric Surgery

Arupa Ganguly, Ph.D.
Director, Genetic Diagnostic Laboratory
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Associate Professor, Department of Genetics

Jennifer Kalish, M.D., Ph.D.
Attending Physician
Division of Human Genetics and Metabolism
Instructor of Pediatrics

David Langdon, M.D., F.A.A.P.
Director, Inpatient Endocrine Service
Clinical Associate Professor

Changhong Li, M.D., Ph.D.
Division of Endocrinology and Diabetes
Research Assistant Professor of Pediatrics

Franz M. Matschinsky, M.D.
Benjamin Rush Professor of Biochemistry and Biophysics

Lisa J. States, M.D.
Director of Outpatient Satellite Imaging
Department of Radiology
Associate Professor of Radiology

Guest Faculty

Jean Baptiste Arnoux, M.D., M.Sc.
Senior Consultant in Inherited Metabolic Disorders and Congenital Hyperinsulinism
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Unless otherwise noted, faculty is from The Children’s Hospital of Philadelphia and the Perelman School of Medicine at the University of Pennsylvania.
David Weinstein, M.D., M.M.Sc.
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Joseph Wolfsdorf, M.D., B.Ch.
Associate Chief, Division of Endocrinology
Clinical Director, Division of Endocrinology
Professor of Pediatrics
Boston Children’s Hospital
Boston, Mass.

Hotel Information
The Children’s Hospital of Philadelphia has reserved blocks of rooms at the Sheraton University City at 3549 Chestnut St., in Philadelphia. The Sheraton University City (www.philadelphiasheraton.com) is located in the heart of the University of Pennsylvania campus, adjacent to Philadelphia’s prestigious medical, education and corporate centers, and just blocks away from the Colket Building. The group overnight room rate is $179 single and double occupancy plus a 15.5 percent room tax. Please contact the hotel directly at 215-387-8000 to make your reservation. When making your reservation at the hotel, please mention the Congenital Hyperinsulinism Symposium. For a complete listing of lodging in Philadelphia, please visit the Philadelphia Convention and Visitor’s Bureau website at www.pcvb.org.

Cancellation and Refund Policy
The Children’s Hospital of Philadelphia reserves the right to cancel or postpone any conference due to any unforeseen circumstances. In the event of cancellation or postponement, Children’s Hospital will refund any registration fees, less a $30 service charge, but is not responsible for any related costs, charges or expenses to participants, including cancellation charges assessed by airlines/travel agencies. In order to process refunds for course withdrawals, cancellation must be received in writing by Friday, April 1. No refunds will be issued thereafter.

Services for People with Disabilities
If arrangements are required for an individual with a disability to attend this meeting, please contact the Continuing Medical Education Department at 215-590-5263.
Registration

Congenital Hypoglycemia Disorders: Hyperinsulinism and GSD
April 14 and 15, 2016 – Registration Deadline: Friday, April 1

Name _____________________________ Credentials _____________
Hospital/Professional Affiliation ____________________________________________
Home Address __________________________________________________________________
City ____________________________ State _____ ZIP __________
Phone ________________________ Fax _______________________
Email ___________________________________________________

Conference Registration Fees
- M.D.s: $350
- Non-M.D.s and Physicians-in-training: $250
- Reception and Dinner, Thursday, April 14: $75

Total Registration Fee: $____________

Registration will not be processed without full payment. Faxed registrations will be accepted for credit card payments only. Please contact Micah Holliday (hollidaydm@email.chop.edu) if you do not receive a confirmation letter.

Method of Payment
- Personal check
- Institutional check
- Discover
- MasterCard
- American Express
- VISA

Credit Card # _____________________________
Exp. Date _____________________________

Mail/fax the completed registration form and payment to:
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