

AGENDA: DAY ONE, THURSDAY, SEPTEMBER 30, 2021

8:45AM Welcome Address

SESSION ONE - COVID-19 and SCD – Jo Howard (Invited)

9:00AM **COVID-19 in Individuals with Sickle Cell Disease or Sickle Cell Trait**
Julie Panepinto, MD
Deputy Director, Division of Blood Diseases and Resources
National Heart, Lung, and Blood Institute

9:30AM ***COVID Experience Across the Globe***

United Kingdom

Subarna Chakravorty, PhD, FRCP, MRCPCH, FRCPath
Consultant Paediatric Haematologist
King's College Hospital
London, UK

9:45AM **India**
Dipty Jain, MD
Professor and Head, Dept. of Pediatrics
Government Medical College
Nagpur, India

9:55AM **Brazil**
Clarisse Lobo, PhD
Clinical Research Specialist
HEMORIO
Rio de Janeiro, Brazil

10:10AM **Caribbean**
Jennifer Knight-Madden, MBBS, PhD
Professor, Pediatric Pulmonology and Clinical Research
Director, Sickle cell Unit of CAIHR
University of West Indies Mona

10:25AM **BREAK**

SESSION TWO – Current and Emerging Therapeutics: Putting out the Fire in Sickle Pathophysiology – Chair: Dr. Kenneth Ataga

10:45AM **Reactivation of Fetal Hemoglobin in Adult Erythroid Cells**
Bjorg Gudmundsdottir, PhD
Research Fellow, Cellular and Molecular Therapeutics Branch
National Heart, Lung, and Blood Institute

- 11:15AM** **Activating PK-R, a Therapy for Hemolytic Anemias**
Swee Lay Thein, MD
Chief, Sickle Cell Branch
National Heart, Lung and Blood Institute
- 11:45PM** **Improving Red Cell Hydration**
Carlo Brugnara, MD
Professor of Pathology, Harvard Medical School
Director, Hematology Laboratory, Boston Children's Hospital
Boston, Massachusetts

12:15PM **LUNCH**

SESSION THREE - Medical Emergencies in the Older Adult with SCD – Chair: Dr. Enrico Novelli

- 1:15PM** **Shortness of Breath - Causes and Management**
Parker Ruhl, MD
Associate Research Physician, Physiology Unit
National Institute of Allergy and Infectious Diseases
- 1:45PM** **SCD Nephropathy and Management**
Emily Limerick, MD
Staff Clinician, Laboratory of Early Sickle Mortality Prevention
National Heart, Lung, and Blood Institute
- 2:15PM** **Anticoagulation and Bleeding**
Arun Shet, MD
Associate Clinical Investigator, Sickle Cell Branch
National Heart, Lung, and Blood Institute
- 2:45PM** **Loss of Vision Incl. Retinopathy and Management**
Prof. Sobha Sivaprasad
Professor, Institute of Ophthalmology
University College of London
London, UK
- 3:15PM** **Acute Pain, a Common Presentation for Many Sickle Cell Complications**
Laurel Menapace, MD
Staff Clinician, Sickle Cell Branch
National Heart, Lung, and Blood Institute
- 3:45PM** **BREAK**

PANEL DISCUSSION – Beyond Hydroxyurea: How and when do we incorporate the new therapies? – Chair: Dr. Marvin Reid

4:00PM

Overview of Available Drugs

Charles Quinn, MD
Medical Director, Pediatric Sickle Cell Program
Director, Erythrocyte Diagnostic Laboratory
Cincinnati Children's Hospital

4:15PM

Panel Contributions from a Global Perspective

David Rees, MA, MBBS, FRCP
Professor of Paediatric Sickle Cell Disease
King's College Hospital
London, UK

Monkia Parshad-Asnani, MBBS, DM, PhD
Professor, Family Medicine and Epidemiology
University of West Indies, Jamaica

AGENDA: DAY TWO, FRIDAY, OCTOBER 1, 2021

9:00AM Welcome Address

SESSION ONE – Application of New Technologies Part I – Chair: Dr. Marie-Dominique Hardy-Dessources

9:15AM **Potential Role Of Non-Invasive Point Of Care (POC) And Wearable Optical Diagnostics In Sickle Cell Disease**

Bruce Tromberg, PhD
Director, National Institute of Biomedical Imaging and Bioengineering
National Institutes of Health

9:40AM **Measuring Sickling of SS and Trait Cells by Slow Deoxygenation**

William Eaton, MD, PhD
Chief and Distinguished Investigator, Laboratory of Chemical Physics
National Institute of Diabetes and Digestive and Kidney Diseases
National Institutes of Health

10:05AM **"Oxygen Gradient Ektacytometry (Oxygenscan) and Deformability Measurements Using the Laser Optical Rotational Red Cell Analyzer"**

Philippe Connes, PhD
Professor, Universite Claude Bernard Lyon 1
Head, Vascular biology and Red Blood Cell, LIBM Laboratory
Region de Lyon, France

10:30AM **BREAK**

SESSION TWO – Application of New Technology Part II – Chair: Dr. Swee Lay Thein

11:00AM **fMRI and Dissection of Pain in SCD**

Deepika Darbari, MD Pediatric Hematologist-Oncologist Children's National Medical Center Washington, DC	Eleni Frangos, PhD Post-Doctoral Fellow National Center for Complementary and Integrative Health
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11:25AM **AI And Machine Learning to Improve Risk Prediction**

Vandana Sachdev, MD Director, Echocardiography Lab National Heart, Lung, and Blood Institute	Xin Tian, PhD Biostatistician, Office of Biostatistic Research National Heart, Lung, and Blood Institute
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11:50AM **Mitochondrial DAMP in Sickle Cell Disease**

Lax Tumburu, PhD
Staff Scientist, Sickle Cell Branch
National Heart, Lung, and Blood Institute

12:15PM **Leukemia and SCD**
Courtney Fitzhugh, MD
Tenure Track Investigator, Laboratory of Early Sickle Mortality Prevention
Cellular and Molecular Therapeutics Branch
National Heart, Lung, and Blood Institute

12:40PM **LUNCH**

SESSION THREE – Genetic / Genomic Therapies – Chair: Dr. John Tisdale

1:40PM **Mechanisms of Disease Reversal After Beta Globin Gene Addition Therapy**
Melissa Bonner, PhD
VP of Research
bluebird bio
Cambridge, Massachusetts

2:10PM **Update on shmirbcl11a Gene Transfer in Patients with SCD**
Erica Esrick, MD
Attending Physician, Dana-Farber/Boston Children's Cancer and Blood Disorders Center
Instructor in Pediatrics, Harvard Medical School
Boston, Massachusetts

2:40PM **Base Editing Rescues SCD in Human and Mouse Hematopoietic Stem Cell Models**
Greg Newby, PhD
Postdoctoral Fellow
Broad Institute of MIT and Harvard
Boston, Massachusetts

3:10PM **Prospects for in vivo Gene Editing**
Sean Burns, MD
Vice President, Disease Biology
Intellia Therapeutics
Cambridge, Massachusetts

3:40PM **BREAK**

DEBATE – Should We Aim for Curative Therapies (HSCT And Genetic Therapies) At All Costs? – Chair: Dr. Jennifer Knight-Madden

4:00PM **Debate**
Lakshmanan Krishnamurti, MD
Professor, Pediatrics
Emory University School of Medicine
Atlanta, Georgia

Debating for Continued Excellent Care in SCD as Curative Therapies are Refined

Jane Little, MD

Professor of Medicine, Division of Hematology and Oncology

Director, UNC Comprehensive Sickle Cell Disease Program

UNC School of Medicine

Chapel Hill, North Carolina

Patient Perspective Session

Successful (TBD)

Unsuccessful - (Jarmanni Cormeaux)

Considering - (Aminu Alibe)

5:00PM

Thank Yous and Close of Conference

Jennifer Knight-Madden, MBBS, PhD

Professor, Pediatric Pulmonology and Clinical Research

Director, Sickle cell Unit of CAIHR

University of West Indies Mona