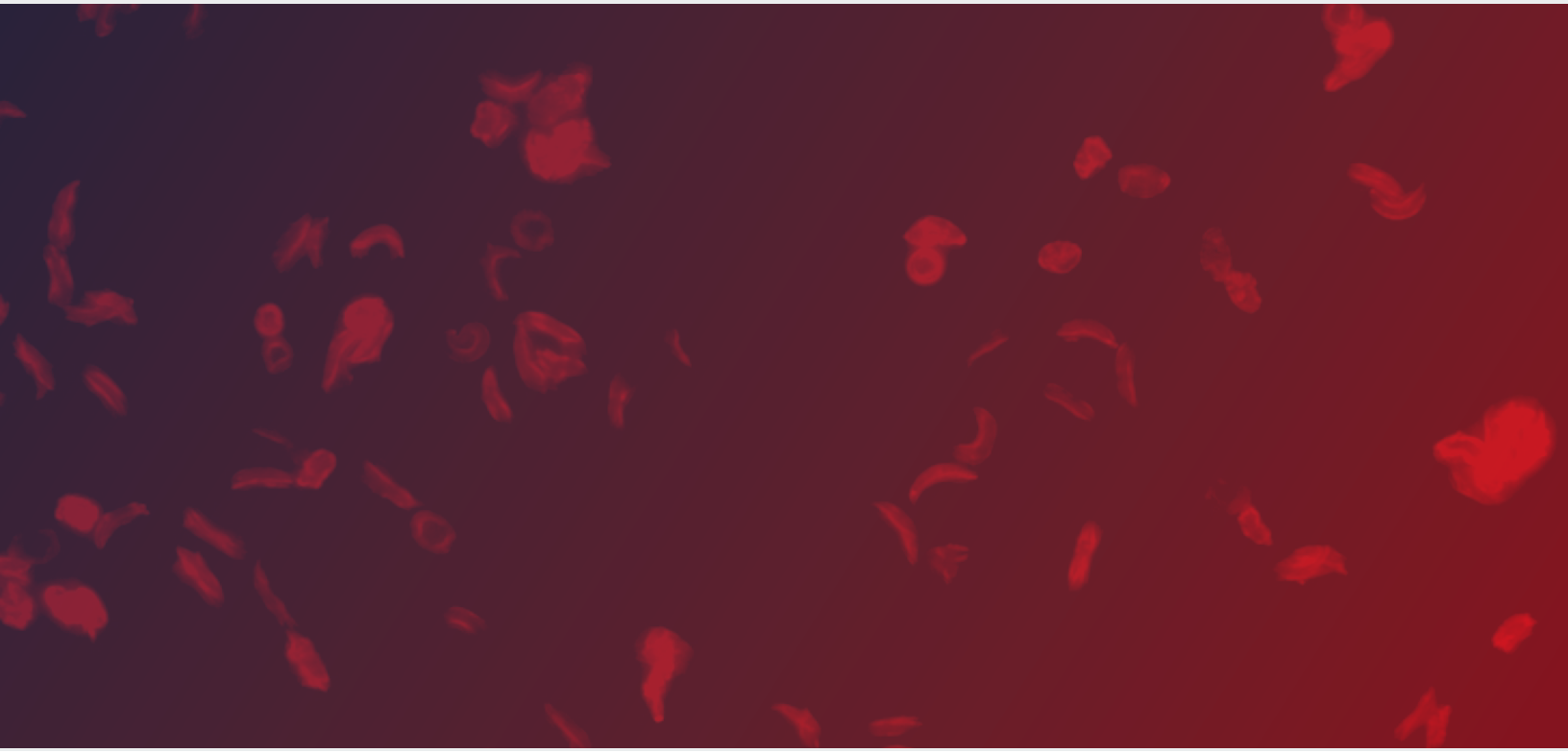
The 14th

SICKLE CELL IN FOCUS CONFERENCE



September 30 – October 1, 2021

HOSTED VIA ZOOM

SCiF
SICKLE CELL IN FOCUS

sponsored by:

NIH National Heart, Lung, and Blood Institute

Hello and a very warm welcome to the 14th annual Sickle Cell in Focus (SCiF) conference!

The National Heart, Lung, and Blood Institute (NHLBI) is excited tobe co-hosting the 2021 SCiF conference with the University of West Indies (UWI) once again. Historically, investigators and physicians from Asia, Europe, Brazil, Africa, and the Caribbean have participated heavily in SCiF in London and the USA. In co-hosting annually with the University of West Indies, we continue to move towards greater collaboration with researchers and health care professionals working with sickle cell disease around the world. We will successfully expand our capacity to provide consultants, trainee doctors, healthcare professionals, and academics interested in hemoglobin disorders with an opportunity for a comprehensive exploration of current medical trends and research results in sickle cell disease globally.

During this two-day, intensive, educational update on sickle cell disease, we will focus on updates on emerging clinical and management issues relating to sickle cell disease, emerging therapies and how we can use them in combination for effective treatment, and in light of the recent pandemic, we will also have a session on the impact of Covid-19 on patients with sickle cell disease. There will also be a panel discussion or debate at the end of each conference day on topical issues for which there are no clear answer. We received wonderful feedback on the quality and knowledge-level of speakers at last year's conference and are excited to have yet another great line up this year. These speakers are experts in their field and will highlight the latest in high-quality research in sickle cell disease. We thank them in advance for giving us their precious time to make SCiF successful.

Even though this year's conference is virtual due to the COVID-19 pandemic, we sincerely hope that you will enjoy SCiF 2021. We would like to thank you for supporting NHLBI and UWI with your attendance at SCiF; delegates are vital to the success of the conference . Your feedback is important and highly appreciated. Please keep an eye out following the event for an event evaluation form, as it helps us shape the program for next year.

Thank you to all our attendeesfor their participation in this year's conference. We hope to see you again next year for Sickle Cell in Focus 2022.

Best Wishes,
Swee Lay Thein, John Tisdale, Jennifer Knight-Madden, and Monika Parshad-Asnani

Program Directors

Swee Lay Thein, MB, BS, FRCP, FRCPath, DSc
Senior Investigator and Chief, *Laboratory of Sickle Cell Genetics and Pathophysiology*
Sickle Cell Branch, NHLBI,
National Institutes of Health

John F. Tisdale, MD
Senior Investigator and Chief, *Cellular and Molecular Therapeutics Branch*
NHLBI, National Institutes of Health

Jennifer Knight-Madden, MB, BS, PhD
Professor, *Pediatric Pulmonology & Clinical Research*
Director, *Sickle Cell Unit*
Caribbean Institute for Health Research
University of the West Indies

Monika Parshad-Asnani, MBBS, DM, PhD
Professor, *Family Medicine and Epidemiology*
Caribbean Institute for Health Research
University of the West Indies

AGENDA

Day One, Thursday, September 30, 2021

8:45 am **Welcome Address**

SESSION ONE - COVID-19 and SCD

Chair: Dr. Subarna Chakravorty

9:00 am **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

COVID Experience Across the Globe

9:30 am **UNITED KINGDOM**
Subarna Chakravorty, PhD, FRCP, MRCPCH, FRCPath
Consultant Paediatric Haematologist
King's College Hospital
London, UK

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

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

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

10:25 am **BREAK**

SESSION TWO - Current and Emerging Therapeutics: Putting out the Fire in Sickle Pathophysiology

Chair: Dr. Kenneth Ataga

10:45 am **Reactivation of fetal hemoglobin in adult erythroid cells**
Bjorg Gudmundsdottir, PhD
Research Fellow, Cellular and Molecular Therapeutics Branch
National Heart, Lung, and Blood Institute

11:15 am **Activating PK-R, a therapy for hemolytic anemias**
Swee Lay Thein, MD
Chief, Sickle Cell Branch
National Heart, Lung and Blood Institute

11:45 am **Targeting Erythrocyte Hydration: from Sickle Cell Anemia to Familial Dehydrated Stomatocytosis**
Carlo Brugnara, MD
Professor of Pathology, Harvard Medical School
Director, Hematology Laboratory, Boston Children's Hospital
Boston, Massachusetts

12:15 pm **LUNCH**

SESSION THREE - Medical Emergencies in the Older Adult with SCD

Chair: Dr. Enrico Novelli

1:15 pm **Shortness of breath - causes and management [2 case scenarios - shunting and PE vs chest infection vs ACS]**
Parker Ruhl, MD
Associate Research Physician, Physiology Unit
National Institute of Allergy and Infectious Diseases

1:45 pm **SCD Nephropathy and Management**
Emily Limerick, MD
Staff Clinician, Laboratory of Early Sickle Mortality Prevention
National Heart, Lung, and Blood Institute

2:15 pm **Anticoagulation and Bleeding**
Arun Shet, MD
Associate Clinical Investigator, Sickle Cell Branch
National Heart, Lung, and Blood Institute

2:45 pm **Loss of Vision incl. Retinopathy and Management**
Prof. Sobha Sivaprasad
Professor, Institute of Ophthalmology
University College of London
London, UK

3:15 pm **Acute pain, a common presentation for many sickle cell complications**
Laurel Menapace, MD
Staff Clinician, Sickle Cell Branch
National Heart, Lung, and Blood Institute

3:45 pm **BREAK**

PANEL DISCUSSION - Beyond Hydroxyurea: How and when do we incorporate the new therapies?

Chair: Dr. Marvin Reid

4:00 pm **Overview of available Drugs**
Charles Quinn, MD
Medical Director, Pediatric Sickle Cell Program
Director, Erythrocyte Diagnostic Laboratory
Cincinnati Children's Hospital

4:15 pm **Panel Contributions from a Global Perspective**
(questions gathered in advance)

Members:

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

Kwaku Ohene-Frempong, MD
President, Sickle Cell Foundation of Ghana
Director, Comprehensive Sickle Cell Center
Children's Hospital of Philadelphia

AGENDA

Day Two, Friday, October 1, 2021

9:00 am **Welcome Address**

SESSION ONE - Application of New Technologies Part I

Chair: Dr. Marie-Dominique Hardy-Dessources

9:15 am **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

9:40 am **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

10:05 am **"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

10:30 am **BREAK**

SESSION TWO - Application of New Technology Part II

Chair: Dr. Swee Lay Thein

11:00 am **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

11:25 am **AI and machine learning to improve risk prediction**

Vandana Sachdev, MD
Director, Echocardiography Lab
National Heart, Lung, and Blood Institute

Xin Tian, PhD
Biostatician, Office of Biostatistic Research
National Heart, Lung, and Blood Institute

11:50 am **Mitochondrial DAMP in Sickle Cell Disease**

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

12:15 pm **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

12:40 pm **LUNCH**

SESSION THREE - Genetic / Genomic Therapies

Chair: Dr. John Tisdale

1:40 pm **Mechanisms of disease reversal after beta globin gene addition therapy**

Melissa Bonner, PhD
VP of Research
bluebird bio
Cambridge, Massachusetts

2:10 pm **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

2:40 pm **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

3:10 pm **Prospects for in vivo gene editing**
Sean Burns, MD
Vice President, Disease Biology
Intellia Therapeutics
Cambridge, Massachusetts

3:40 pm **BREAK**

DEBATE - Should we aim for curative therapies (HSCT and Genetic therapies) at all costs?

Chair: Dr. Jennifer Knight-Madden

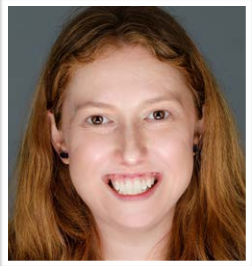
4:00 pm ***DEBATE FOR***
Lakshmanan Krishnamurti, MD
Professor, Pediatrics
Emory University School of Medicine
Atlanta, Georgia

DEBATE AGAINST - *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: Reginald Nicolas
Unsuccessful: Sundu Kargbo
Considering: Chynna Glaze

5:00 pm **Thank yous and close of conference**

SPEAKER BIOGRAPHIES



Melissa Bonner, PhD is VP of Severe Genetic Disease Research at bluebird bio where she oversees translational gene therapy projects across the portfolio. Prior to joining bluebird bio in 2014, she was at St. Jude Children's Research Hospital for her postdoctoral training in the laboratory of the late Dr. Brian Sorrentino working on HSC expansion and next-generation virus production for gene therapy applications.

Carlo Brugnara, MD is a Professor of Pathology at Harvard Medical School and the Director of Hematology Lab at Boston Children's Hospital. He received his MD from the University of Verona, Italy in 1979. In 1992, he joined the Department of Laboratory Medicine at Boston Children's Hospital as Director of the Hematology Laboratory. Dr. Brugnara's basic research interests are focused on transport of ions across cell membrane. His studies have identified the role of specific transport proteins in inducing erythrocyte dehydration in sickle cell disease and identified new therapeutic approaches for sickle cell anemia. His most recent research has focused on genetic determinant of erythrocyte hydration and genetic diseases of red cell hydration like dehydrated stomatocytosis.

Sean Burns, MD is Vice President of the Disease Biology and Pharmacology team at Intellia Therapeutics, a leading genome editing company based in Cambridge, MA. In addition, Sean is a practicing endocrinologist affiliated with Massachusetts General Hospital (MGH) and Nantucket Cottage Hospital. At Intellia, he leads the company's efforts to identify novel in vivo applications of CRISPR to treat genetic disease. Sean previously served at Intellia as medical director, and as director of Hematology and New Therapeutic Areas.

Before Intellia, Sean was a physician-scientist at MGH and the Broad Institute of Harvard and MIT. As an Assistant in Medicine within the Endocrine Division at MGH, he provided care for patients with diabetes and other metabolic disorders. In the laboratory, he was a fellow within the Department of Molecular Biology, applying human genetic discoveries and genome engineering to identify and validate potential drug targets.

Sean received his B.S. in Electrical Engineering from Cornell University and his MD with Honors from the New York University School of Medicine. He completed a residency in Internal Medicine at Beth Israel Deaconess Medical Center in Boston, followed by a fellowship in Endocrinology, Diabetes and Metabolism at MGH.

Subarna Chakravorty, PhD, FRCP, MRCPCH, FRCPath is a Paediatric Haematologist with a special interest in non-malignant haematology. She joined King's College Hospital as consultant in July 2015. Prior to that, Subarna led the Paediatric Haemoglobinopathy service at Imperial College Healthcare NHS Trust and the Imperial Paediatric Red Cell Disorders Network for
5 years, where she was also involved in the bone marrow transplant programme for paediatric haemoglobinopathy.

Subarna was the national lead for the UK Haemoglobinopathy Peer Reviews, 2018-2020. She is one of the clinical leads at the Southeast London and South East England Region Haemoglobinopathy Coordinating Centre for Sickle Cell disease and a member of the Clinical Reference Group for haemoglobinopathy, NHS England. She is a trustee of the British Society for Haematology and the UK Forum on Haemoglobin Disorders. Subarna is interested in clinical and molecular research in sickle cell disease and is involved in a number of projects at King's College London.

Philippe Connes, PhD obtained a PhD in Montpellier in 2003 in the field of exercise physiology and hematology.

After a one-year contract in Lyon where he served as a temporary Lecturer, he was recruited in 2004 as a Senior Lecturer in Exercise Physiology at the Laboratory ACTES - Department of Physiology (University of the French West Indies, Pointe-à-Pitre, Guadeloupe). He lead the team "Sickle Cell Trait and Exercise". In 2008, he obtained a 5 yrs Inserm National Research Contract to work as a researcher in the UMR Inserm 1134 "Integrative Biology of Red Blood Cell" at the Academic Hospital of Pointe-à-Pitre (Guadeloupe). He worked on the pathophysiological mechanisms of sickle cell disease, and more particularly on the role of blood rheology, vascular function, autonomic nervous system activity and inflammation.

He got its accreditation to supervise research (HDR) from the University of the French West Indies (Guadeloupe) in 2009. In 2013, he successfully applied to the French University Institute (IUF) as a junior member (5 yrs contract). In 2014, he was promoted on a Full Professor position at the University of Lyon (France). Since that time, he leads the "Vascular Biology and Red Blood Cell" team in the LIBM laboratory (EA7424).

He mainly works on the cellular/biological/physiological mechanisms involved in sickle cell disease complications and in other hematological disorders (hereditary spherocytosis, chronic mountain sickness…). He also investigates the acute and chronic effects of exercise on hematological adaptations and red blood cell physiology in healthy individuals and patients with chronic hematological disorders. Philippe obtained several local (Programme Hospitalier de Recherche Clinique locaux/Inter-régionaux, Contrat Plan-Etat Recherche, Bonus Qualité Recherche, Idex Lyon,…), national (French Research National agency, French Blood

Center,....), international (Eurostars H2020 program, German Research National Agency (DFG), International Training Network Marie-Curie, FAPESP,…) and private (Erytech Pharma, Hartis Pharma,…) grants/fundings.

He is internationally recognized in the field of blood rheology, microcirculation and sickle cell disease (ranked 1st and 10th in the field of sickle cell trait and sickle cell disease, respectively, on Expertscape website). He serves as Academic/Assistant Editor for several scientific journals: Clinical Hemorheology and Microcirculation, Frontiers in Physiology (section Red Blood Cell) and Plos One.

Philippe Connes is also the Scientific Research referent of the Constitutive Reference Center on Red Blood Cell disorders in Lyon. October 2020, Philippe published 215 international scientific articles, several book chapters, coordinated an international handbook in Exercise Physiology and supervised 20 PhD/post-doc. He is currently an Executive Board member in the European Society of Clinical Hemorheology and Microcirculation (ESCHM), the French club of Red Blood Cell and Iron (Club du Globule Rouge et du Fer) and the International Society for Clinical Hemorheology (ISCH). Philippe is also the vice-dean of the Doctorate School EDISS 205 (Université Claude Bernard Lyon 1) since September 2019.

Deepika Darbari, MBBS, MS

Children's National Hospital, The George Washington University School of Medicine and Health Sciences, Washington, DC.

Dr. Darbari is a Professor of Pediatrics and a Pediatric Hematologist-Oncologist at Children's National Hospital in Washington DC. Dr. Darbari studies complications of sickle cell disease with emphasis on pain. She has been conducting clinical and translational studies directed towards better understanding of mechanisms of sickle cell pain and its management using pharmacologic and non-pharmacologic integrative modalities. Her research has contributed to improved understanding of variability of pain experience and risk factors associated with high pain burden in patients with sickle cell disease. Her team has described changes in brain connectivity and volume associated with pain in patients with sickle cell disease. She has also contributed to development of diagnostic criteria for acute and chronic pain in sickle cell disease and PhenX Toolkit for pain in sickle cell disease. As an active participant of diversity, equity and inclusion efforts at Children's National and ASH she is also committed to promoting diversity in medicine and hematology.

Deepika Darbari, MBBS, MS
Attending Physician, Division of Hematology
Children's National Hospital
Professor of Pediatrics
George Washington University School of Medicine and Health Sciences

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William Allen Eaton, MD, PhD was born and educated in Philadelphia, earning a B.A. degree in Chemistry in 1959, an MD degree in 1964, and a Ph.D. degree in Molecular Biology in 1967, all at the University of Pennsylvania. During 1959-60 he studied biophysics at the Free University of Berlin, as the first "Willy Brandt Exchange Student." Eaton's Ph.D. thesis research on single crystal optical spectroscopy of heme proteins was carried out under the supervision of Robin M. Hochstrasser. He then moved to the National Institutes of Health (NIH) in Bethesda, Maryland, to fulfill his military service obligation as a Medical Officer in the US Public Health Service. Eaton's entire subsequent career has been spent carrying out research in protein biophysics at NIH, apart from a semester as a Visiting Professor teaching physical chemistry at Harvard University, Cambridge. Since 1986 he has served as Chief of the Laboratory of Chemical Physics, NIDDK, the principal laboratory at NIH carrying out research in the biophysical sciences, and as Scientific Director of the NIH Intramural AIDS Targeted Ant-viral Program until 2018. In 2007, Eaton was named an NIH Distinguished Investigator. He is a Fellow of the American Physical Society, the Biophysical Society, the American Academy of Arts and Sciences, a member of the National Academy of Sciences, and a foreign member of the Accademia Nazionale dei Lincei, Rome. His numerous honors include the Henry M. Stratton Medal for Basic Research from the American Society of Hematology, the Max Delbruck Prize in Biological Physics of the American Physical Society, the Founders Award of the Biophysical Society, the Hans Neurath Award of the Protein Society, the John Scott Award of the City of Philadelphia, the Weizmann Memorial Lectures, an honorary Doctorate in physics from the Free University Berlin, and an honorary Laurea in pharmaceutical chemistry and technology from the University of Parma.

Erica Esrick,MD is a pediatric hematologist at Boston Children's Hospital in Boston MA. Her primary clinical and research interest is in hemoglob-inopathies. She is the clinical lead for the Boston Children's Thalassemia Program, the clinical PI of BCH's sickle cell gene therapy study, and site PI for multiple other clinical studies of sickle cell disease and thalassemia.

Courtney Fitzhugh, MD received her BS magna cum laude from the University of California, Los Angeles in 1996, and her MD from the University of California, San Francisco in 2001. During medical school, Dr. Fitzhugh participated in the NIH Clinical Research Training Program, where she studied with Dr. John Tisdale at the NHLBI. After receiving her MD, Dr. Fitzhugh completed a joint residency in internal medicine and pediatrics at Duke University Medical Center, and in 2005 she did a combined adult hematology and pediatric hematology-oncology fellowship at the NIH and Johns Hopkins Hospital. Dr. Fitzhugh returned to the NHLBI in 2007 and was appointed as Assistant Clinical Investigator in 2012 and Clinical Tenure Track Investigator in 2016. She is a member of the American Society of Hematology and American Society of Transplantation and Cellular Therapy.

Eleni Frangos, PhD is a postdoctoral fellow in the Pain and Integrative Neuroscience Branch the National Center for Complementary and Integrative Health (NCCIH) institute at the NIH. Her research focuses on behavioral and neural pain mechanisms in healthy and chronic pain populations as well as non-invasive and non-pharmaceutical methods of pain modulation. Dr. Frangos attended Rutgers University – Newark and received her Ph.D. in Psychology-Behavioral Neuroscience in 2014, M.A. in Psychology in 2012, and B.A. in Biology in 2009. Her work at Rutgers, funded by the NIH Initiative for Minority Biomedical Research Support (MBRS) Maximizing Student Development (IMSD) fellowship program, provided the first functional magnetic resonance (fMRI) evidence that the vagus nerve is accessible non-invasively via the surface of the ear and the neck, which has therapeutic implications in pain, depression, and epilepsy.

Bjorg Gudmundsdottir MBA, PhD is a Research Fellow in The Cellular and Molecular Therapeutics Laboratory at the NHLBI, NIH. Dr. Gudmundsdottir performed her dissertation research within the NIH graduate partnership program and obtained her PhD at the University of Iceland. Her research focuses on the transcriptional and epigenetic regulation of erythroid cell development and characterizing novel regulators of fetal hemoglobin expression.

Dipty Lalit Jain, MD was the former Professor and Head, Department of Pediatrics, Govt. Medical College, Nagpur and she is a Consultant for WHO (UIP), USAID (Domestic Violence against women and children and scientific advisory committee members, Indian Council Medical Research (ICMR).

She has a teaching experience of 35 years and was awarded the 'Best Teacher Award' in the Year 2011

She has done her Under Graduation and Post Graduation from Nagpur University and M.Sc (Clinical Epidemiology) from Mc. Master University, Canada.

She is very active in the field of Research and she been awarded the Best paper award for "Hydroxurea in SCD " at second International meet on Sickle Cell Disease "Natural History of Sickle Cell Disease" at National Pediatric Hematology and also for the Paper Fixed Low Dose Hydroxurea in Children with Sickle Cell Disease. Her pro-activeness for research also translates into the multiple International and National Publications that are to her credit.

She has been awarded the 'Rockefeller Fellowship' for the year 1991-92. MSc Master University, Canada for Masters in Med (Clinical Epidemiology) with special course on Health Economics and also is a Member of International Clinical Epidemiology Network (INCLEN) Philadelphia, USA.

Apart from the astounding academical achievements, her contribution towards society in the middle income country like in India is astonishing . Her case Control study , "Physical Abuse during pregnancy and Low birth weight Infant" was awarded the Best Paper award at a Conference held at Trivandrum. India and is the President INSPACAN-Indian Society for the Prevention of Child Abuse and Neglect, affiliated by ISPCAN-International Society for the Prevention of Child Abuse and Neglect.

Her innovative and unique work in the field of Sickle Cell Disease is unparalleled to anyone in the Country and she is member of the Global Sickle Cell Disease Network (GSCDN) International Advisory Council, ICMR and Government of India Policy on Sickle Disease is determined by her and has trained faculty of Medical and Paramedical on Sickle Cell Disease in the entire country , also she is the Reviewer for Clinical Evidence on Sickle Cell Disease in British Medical Journal (BMJ).

What she gives back to the Society is what defines her. She has aptly been awarded "Woman of the year in 2002-2003 by The United States of America."

Jennifer Knight-Madden MB, BS, FRCP-C, PhD

Professor Jennifer Knight-Madden is the Director of the Sickle Cell Unit, the Caribbean Institute for Health Research, The University of the West Indies (UWI). Professor Knight-Madden graduated from The UWI's medical school in 1988. She completed training in Pediatrics at the Hospital for Sick Children in Toronto) and Fellowship training in Pediatric Pulmonology at Duke University Medical Center (DUMC) in Durham, NC. She completed an MSc in Biometry (DUMC), a PhD in Clinical Research (King's College London), a Certificate in Implementation Science (University of California San Francisco) and a short course in Strategic Health Planning. She is Professor of Pediatric Pulmonology and Clinical Research

Her PhD at King's College London examined pulmonary complications of sickle cell disease; she also has interests in newborn screening, asthma, clinical trials and implementation science. She has published several book chapters and more than 60 papers in peer reviewed journals. She is active in the national Sickle Cell Technical Working Group (Co-Chair); the Caribbean Network of Researchers in Sickle Cell Disease and Thalassemia (Vice President); the SickKids Caribbean Initiative; Scientific Advisory Committee, Global Alliance of SCD Organizations (GASCDO); two National Institutes of Health Data Safety Monitoring Boards for studies in Africa; National Institute for Health Research (NIHR) Global Health Research Centre Call 1; National Committee on Non-Communicable Diseases (NCDs); Chair- Surveillance, M&E and Research Sub-Committee; The Mona Campus Research Ethics Committee (one year break); the UWI Mona Academic Board Sub-Committee for Student Disciplinary Matters; The University of the West Indies Ethics Committee and the Advisory Panel on Ethics and Medico-Legal Affairs, MOHW (one year assignment). She is an examiner and member of Specialty Board of the Child and Adolescent Health Program and an honorary Consultant at the University Hospital of the West Indies.

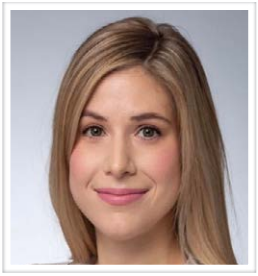
Professor Knight-Madden is a proud St Andrew High School for Girls Old Girl. A recipient of the St. Andrew High School Trailblazer Award in 2016, she remains actively engaged with school life. As well as being a parent , she is on the Board of Management and leads the Excellence in Education sub-committee of the Parent Teacher Association. She is a member of the Christian Life Fellowship, where she is in Children's Ministry.

Lakshmanan Krishnamurti, MD is the Chief of Pediatric Hematology and Oncology at Yale School Medicine Pediatric Department and Yale New Haven Children's Hospital. Dr. Krishnamurti received his MBBS from University of Poona, Armed Forces Medical College, India and completed a pediatrics residency at University of Bombay, India. He attended University of Minnesota for his pediatric residency and fellowship in pediatric hematology, oncology and BMT. He is an accomplished pediatric hematologist oncologist and an international leader in bone marrow transplant and the treatment of hemoglobinopathies.

Emily Limerick, MD is a staff clinician n NHLBI working with Dr. Courtney Fitzhugh in the Laboratory of Early Sickle Mortality Prevention. She completed her pediatric hematology/oncology fellowship at Johns Hopkins and the NIH. Dr. Limerick's research focus has been haploidentical stem cell transplantation for sickle cell disease as well as biomarkers of organ function in sickle cell disease, specifically sickle nephropathy with a burgeoning interest in sickle cardiomyopathy. She is the principal investigator of a study exploring markers of sickle nephropathy which is currently ongoing. Dr. Limerick is currently pursuing her Masters in Clinical Research through the combined Duke-NIH Clinical Research Training Program; her thesis work explores renal function in patients with sickle cell disease after stem cell transplantation.

Jane Little, MD is a professor of Hematology and director of the UNC comprehensive sickle cell disease program. Dr. Little has an interest in red blood cells, registries, and outreach. Dr. Little has actively participated in GRNDaD, GRNDaD speaks, virtual red cell meetings, and NASCC. Dr. Little is often asked to take the 'anti-cure for SCD' position at SCIF, but she is **totally pro-cure!**

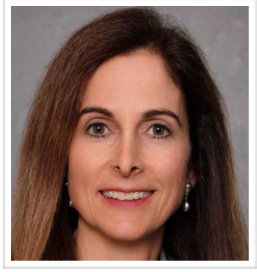
Clarisse Lobo, MD, PhD has been caring for patients with sickle cell disease for almost 30 years and has considerable experience leading and conducting translational and clinical trials in this field. She has held leadership positions in organizations such as the Health Foundation of the State of Rio de Janeiro and Brazil's Institute of Hematology Arthur de Siqueira Cavalcanti – HEMORIO. She has also participated as Effective Member as part of the Brazilian Ministry of Health's Commissions for the elaboration of laws, training and treatment manuals with a strong impact on the evolution of awareness of Sickle Cell Disease nationally and internationally. Dr. Lobo is currently in charge of Research and Studies in Sickle Cell Disease at HEMORIO and provides Medical/Technical Consultancy and training for private companies and hematology practices. She is recognized nationally and internationally as a member of the scientific community of Sickle Cell Disease with numerous scientific papers published in the area. Dr. Lobo graduated in Medicine from the State University of Rio de Janeiro (UERJ) and obtained her PhD in Medical Sciences at Universidade Federal Fluminense (UFF).



Laurel Menapace, MD is a Hematologist and Staff Clinician in the Sickle Cell Branch at the National Heart, Lung, and Blood Institute (NHLBI). She received her MD from the University of Rochester in New York, completed residency at Strong Memorial Hospital in Rochester and trained in Hematology at the Cleveland Clinic. She provides comprehensive clinical care to adult patients with sickle cell disease and is involved in clinical protocols exploring novel drug therapies as an associate investigator.

Gregory Newby, PhD received his BS in Biological Sciences from Carnegie Mellon University in 2009, and following one year conducting research at the University of Zurich as a Fulbright Scholar, beginning his PhD in the Biology Department at MIT. Studying under Susan Linquist, Dr. Newby developed a genetic circuit to detect protein aggregation and select for factors that modulated prion formation. In 2017, he joined the laboratory of David Liu at Harvard University and the Broad Institute as a postdoctoral fellow. His current research is focused on developing base editors and prime editors to treat genetic disease.

Kwaku Ohene-Frempong, MD is the director emeritus of the Comprehensive Sickle Cell Center at Children's Hospital of Philadelphia (CHOP). After graduating from the Yale School of Medicine in 1975, he trained in Pediatrics at the New York Hospital - Cornell Medical Center, and in Pediatric Hematology-Oncology at the Children's Hospital of Philadelphia (CHOP). Dr. Ohene-Frempong is a world leader in the treatment of sickle cell disease (SCD) and has established many of the current practice standards. For years, he has led the development of SCD treatment centers in the U.S. and globally, including Ghana, his native country, as the president of the Sickle Cell Foundation of Ghana and the national coordinator for the ASH Consortium on Newborn Screening in Africa (CONSA). One of Dr. Ohene-Frempong's key research achievements was his observation of the frequency of strokes in young children with SCD, and his work was instrumental in establishing bone marrow transplant as a cure for SCD. ived his BS in Biological Sciences from Carnegie Mellon University in 2009, and following one year conducting research at the University of Zurich as a Fulbright Scholar, beginning his PhD in the Biology Department at MIT. Studying under Susan Linquist, Dr. Newby developed a genetic circuit to detect protein aggregation and select for factors that modulated prion formation. In 2017, he joined the laboratory of David Liu at Harvard University and the Broad Institute as a postdoctoral fellow. His current research is focused on developing base editors and prime editors to treat genetic disease.

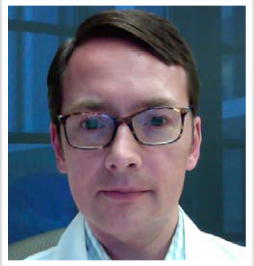


Julie Panepinto, MD, MSPH is the Deputy Director of the Division of Blood Diseases and Resources at the National Heart, Lung, and Blood Institute of the National Institutes of Health.

Prior to joining the NHLBI in June, 2021, Dr. Panepinto was a tenured Professor of Pediatrics, Hematology, Vice Chair of Value in Pediatrics, and the Director of the Center for Clinical Effectiveness Research of the Children's Research Institute at the Medical College of Wisconsin and Children's Wisconsin. As a clinician researcher with a background in public health, Dr. Panepinto focused her academic career on understanding and improving the health outcomes of patients and families with chronic disease. Her research focused on integrating the perspective of the patient and family in health care through the use of patient reported outcomes (PROs) and understanding systems of care and acute care utilization for those individuals living with sickle cell disease.

Dr. Panepinto completed her fellowship in Pediatric Hematology/Oncology/Bone Marrow Transplant at the University of Colorado Health Sciences in Denver, Colorado, where she also earned a Master's of Science in Public Health. She served as a past member on the NHLBI Data and Safety Monitoring Board (DSMB) for the Sickle Cell Disease Clinical Research Network (2006-2010), the NHLBI's Sickle Cell Disease Advisory Committee from 2016-2019, and the Advisory Council for the NHLBI from 2020-2021. Dr. Panepinto was active in training researchers and served as the mentor for mentees' NHLBI funded K23 career development awards and as the co-director of the American Society of Hematology's Clinical Research Training Institute from 2011-2013. Dr. Panepinto is a past Chair of the Guideline Oversight Subcommittee of the American Society of Hematology and former associate editor for the Blood Advances journal. Dr. Panepinto is a member of AOA and a past Hedwig van Ameringen Executive Leadership in Academic Medicine (ELAM) Fellow.

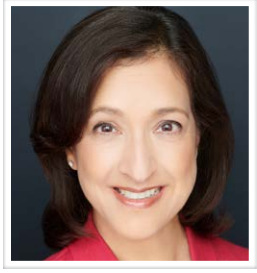
Monika Parshad-Asnani,MBBS,DM,PhD is a Professor of Family Medicine and Epidemiology and the Director of Sickle Cell Unit of the Caribbean Institute for Health Research (CAIHR) at the University of the West Indies (UWI) in Jamaica. She joined CAIHR in 2003 and has over 18 years' experience of providing clinical care for persons with sickle cell disease (SCD). Her focus of research is (i) psychosocial outcomes and determinants in SCD and (ii) sickle nephropathy. Her work has advanced understanding of predictors of renal function decline in SCD and has sought to validate methods to determine presence of early sickle nephropathy. She has validated and tested various tools to examine quality of life in children, adolescents and adults with SCD in Jamaica. She is currently leading a project to assess the prevalence of neuropathic pain in SCD and is the local PI for the Gene Therapy in SCD project. She is a graduate of the UWI and was awarded the MBBS. degree in 1992. Her thesis work leading up the award of D.M. Family Medicine in 2006 examined the Quality of Life of Patients with Sickle Cell Disease (SCD). She completed in 2014 her PhD in Epidemiology with a focus on the Epidemiology of Sickle Nephropathy. At the UWI, She is also a course tutor, examiner and member of Specialty Board in the Family Medicine Programme. She is a board member of the Cochrane Caribbean which is led by CAIHR. She holds a term graduate faculty appointment at Duke University Graduate School in the US. She has been an Academic Editor for Plos One journal since 2014. She is a member of the Caribbean College of Family Physician having served as its honorary secretary for a decade in the past. She also serves as a board member of the Caribbean Researchers in Sickle Cell Disease & Thalassaemia (CAREST) group.



Charles Quinn, MD is a Pediatric Hematologist at Cincinnati Children's Hospital Medical Center (CCHMC) and Professor of Pediatrics at the University of Cincinnati College of Medicine. His clinical focus is sickle cell disease and other hemoglobinopathies. He is the medical director of the Pediatric Sickle Cell Disease Program and the medical director of the Erythrocyte Diagnostic Laboratory at CCHMC. He is also the medical director of the Ohio Department of Health Regional Sickle Cell Services Program – Region 1. He conducts patient-oriented and translational research in sickle cell disease, with recent studies recent of newborn screening, genetic diagnosis, dis-ease-modifying medications, gene therapy, and the pathophysiology and treatment of sickle cell cardiomyopathy and nephropathy.

David Rees is professor of paediatric sickle cell disease at King's College London and consultant in paediatric haematology at King's College Hospital. He has research and clinical interests in sickle cell disease, and other red cell abnormalities, including porphyrias, thalassaemia and enzymopathies. He has previously worked in Jersey, Bath, Milton Keynes, Oxford and Sheffield. He is involved in the care of more than 1000 children with sickle cell disease and thalassaemia in southern England. He is a medical adviser to the Sickle Cell Society in the UK.

A. Parker Ruhl, MD, MHS completed her training in Pulmonary Medicine at Johns Hopkins Hospital and Critical Care Medicine at the NIH Clinical Center. She is an Associate Research Physician in the Physiology Unit of the Laboratory of Malaria and Vector Research at the National Institute of Allergy and Infectious Diseases and the Pulmonary Branch of the National Heart, Lung, and Blood Institute. Her current research is focused on pulmonary and vascular disease related to sickle cell disease, alpha thalassemia, and malaria.



Vandana Sachdev MD, is a cardiologist and Senior Research Clinician in the Cardiology Branch of the NHLBI Division of Intramural Research. She received her MD from the University of Michigan and trained in cardiology at the University of Maryland. She joined NHLBI in 1998 and is the Director of the Echocardiography Laboratory. She is a member of the American Society of Echocardiography Board of Directors and is active in various ASE committees and task forces. Dr. Sachdev's research areas of interest include heart failure, cardiac imaging, and artificial intelligence. Her lab served as the echo core lab for the Walk-Phasst study and other studies supported by NHLBI, and they have been phenotyping the NIH sickle cell cohort for many years.

Arun Shet,MD is currently Senior Research Physician and Assistant Clinical Investigator in the Sickle Cell Branch at the National Heart Lung and Blood Institute. The main focus of Dr Shets' research is the crosstalk between sickled red cells, vascular endothelial cells, and leucocytes that mediates inflammation and thrombosis and lead to the vascular pathobiology characteristic of Sickle Cell Disease. Dr. Shet actively investigates the role for novel therapeutics to prevent thrombosis in early phase clinical trials. He currently investigates the potential utility of flavonoid Isoquercetin in a phase 2 clinical trial. The goal of his research is to identify anticoagulation therapy that maximizes antithrombotic efficacy with minimal to no bleeding side effects.

Professor Sivaprasad is a Consultant Ophthalmologist at Moorfields Eye Hospital and King's College Hospital, London. She is also a Reader at King's College London.

She has extensive training and experience in managing the entire breadth of Medical Retina conditions. Her busy clinical practice is complemented by her active involvement in several multicenter clinical trials investigating new treatment options in retinal vascular diseases.

She has been well funded in her research and has collaborated with many esteemed centres around the world. She has over 100 peer-reviewed publications to her credit and has been an invited speaker in several international conferences. She is a member of several prestigious societies including the Macular Society. Professor Sivaprasad is the Associate Editor of Eye.

Swee Lay Thein,MD is a hematologist and clinical investigator with more than 30 years of clinical and translational hematology research and extensive personal experience in laboratory research – molecular biology, genetics and genomics. Dr. Thein joined the National Heart, Lung and Blood Institute / NIH in spring 2015 as Senior Investigator and Chief of the Institute's Sickle Cell Branch. Prior to this, she was Professor of Molecular Hematology and consultant hematologist at King's College London (KCL), where she served as clinical director of the Red Cell Centre in King's College Hospital. At the hospital, she was involved in the care of 800 adult patients with sickle cell disease and other red blood cell disorders, and also provided a comprehensive diagnostic service for red blood disorders including antenatal and newborn screening, and prenatal diagnosis of the hemoglobin disorders.

Swee Lay Thein completed her specialist training in general medicine and hematology at the U.K. Royal Postgraduate Medical School, Hammersmith, and the Royal Free Hospital, London.

She has also worked in Oxford at the Weatherall Institute of Molecular Medicine (Medical Research Council Molecular Hematology Unit) where she held various positions, including MRC clinical training fellow, Wellcome Senior Fellow in Clinical Science, senior MRC clinical scientist, and the John Radcliffe Hospital as honorary consultant hematologist before she moved to KCL, London, in 2000.

Dr. Thein runs a program on Sickle cell genetics and pathophysiology with an objective of identifying plasma and genetic markers to allow early detection and monitoring of severe complications. Using the hemoglobinopathies as genetic models, her research has contributed significantly to the understanding of genetic modifiers and complex traits and DNA diagnostics in hemoglobinopathies, and unravelling the loci contributing to the control of fetal hemoglobin, a major ameliorating factor in these disorders.

Developing evidence base for management of sickle-related complications and education is another focus of her work. Since 2006, she has directed and hosted an annual 2-day international conference in sickle cell disease, in KCL (London) and the NHLBI/NIH. She has also been previously involved in planning and organising various educational meetings (national and international), and working with the European School of Hematology and European Hematology Association. She is the feature editor of Blood Hub on sickle cell anemia in BLOOD, and Associate Editor of Haematologica. She was elected to the Fellowship of the UKs Academy of Medical Sciences in 2003.

Xin Tian, PhD

Mathematical Statistician, Division of Intramural Research
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Xin Tian, PhD, is a senior mathematical statistician at Office of Biostatics Research, Division of Intramural Research, National Heart, Lung, and Blood Institute, NIH. She is also an adjunct professor of statistics at the George Washington University, and an adjunct professor of medicine in School of Medicine, University of Maryland. She joined NHLBI after receiving Ph.D. in statistics from Rutgers University in 2003. Her research interests include design and analysis of clinical trials, longitudinal data analysis, disease risk tracking, and machine learning with applications to big medical studies in cardiovascular, pulmonary, and hematological diseases as well as clinical trials of stem cell transplantation.

John Tisdale, MD received his medical degree from the Medical University of South Carolina in Charleston after obtaining his B.A. in Chemistry from the College of Charleston. He completed an internal medicine and chief residency at Vanderbilt University Medical Center in Nashville and then trained in hematology in the Hematology Branch, National Heart, Lung and Blood Institute (NHLBI), where he served as a postdoctoral fellow. He joined the Molecular and Clinical Hematology Branch of NHLBI in 1998 and is now the Chief of the Cellular and Molecular Therapeutics Branch. In 2020 the College of Charleston recognized Dr. Tisdale as one of their Top 25 History makers in honor of the schools 250-year anniversary and was Samuel J. Heyman Service to America Medal finalist. He was recently elected to the American Society for Clinical Investigation and is a member of the American Society of Hematology. He serves as an editorial board member of the journals Stem Cells, Experimental Hematology, and Molecular Therapy Methods & Clinical Development. He is a frequent ad hoc reviewer for Blood, New England Journal of Medicine, Human Gene Therapy, and Nature Medicine Experimental Hematology Molecular Therapy to name a few. He has served on the NIDDK/ NIAMS Institutional Review Board for over 15 years, is a founding member of the NIH Bone Marrow Transplant Consortium, and is an active member of the NIH Intramural Gene Therapy Task Force. Dr. Tisdale's research and clinical work center on sickle cell disease. His group focuses on developing curative strategies for sickle cell disease through transplantation of allogeneic or genetically modified autologous bone marrow stem cells. He has published over 200 first- and co-author publications.

Bruce J. Tromberg, Ph.D. is the Director of the National Institute of Biomedical Imaging and Bioengineering (NIBIB) at the National Institutes of Health (NIH) where he oversees a portfolio of research programs focused on developing, translating, and commercializing engineering, physical science, and computational technologies in Biology and Medicine. In addition, he leads NIBIB's Rapid Acceleration of Diagnostics (RADx Tech) innovation initiative to increase SARS-COV-2 testing capacity and performance. Prior to joining NIH in January 2019, he was a professor of Biomedical Engineering and Surgery at the University of California, Irvine (UCI). During this time he served as director of the Beckman Laser Institute and Medical Clinic (BLIMC) (2003-2018) and the Laser Microbeam and Medical Program (LAMMP), an NIH National Biomedical Technology Center at the BLIMC (1997-2018). Dr. Tromberg specializes in the development of optics and photonics technologies for biomedical imaging and therapy. He has co-authored more than 450 publications and holds 23 patents in new technology development as well as bench-to-bedside clinical translation, validation and commercialization of devices.

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Dr. Laxminath Tumburu, PhD, is a Staff Scientist in the Laboratory of Sickle Cell Genetics and Pathophysiology at Sickle Cell Branch, NHLBI. Dr. Tumburu received his doctorate from Wright State University (Ohio). He was a National Research Council Postdoctoral Fellow at the National Academies, before joining the National Institutes of Health as a Visiting Fellow in 2013, to pursue his research career in erythropoiesis, fetal hemoglobin regulation, and the sickle cell disease pathophysiology. His current research interests include understanding the role of mitochondria vis-à-vis heteroplasmy and red cell mitochondrial retention in sickle cell disease pathophysiology.

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