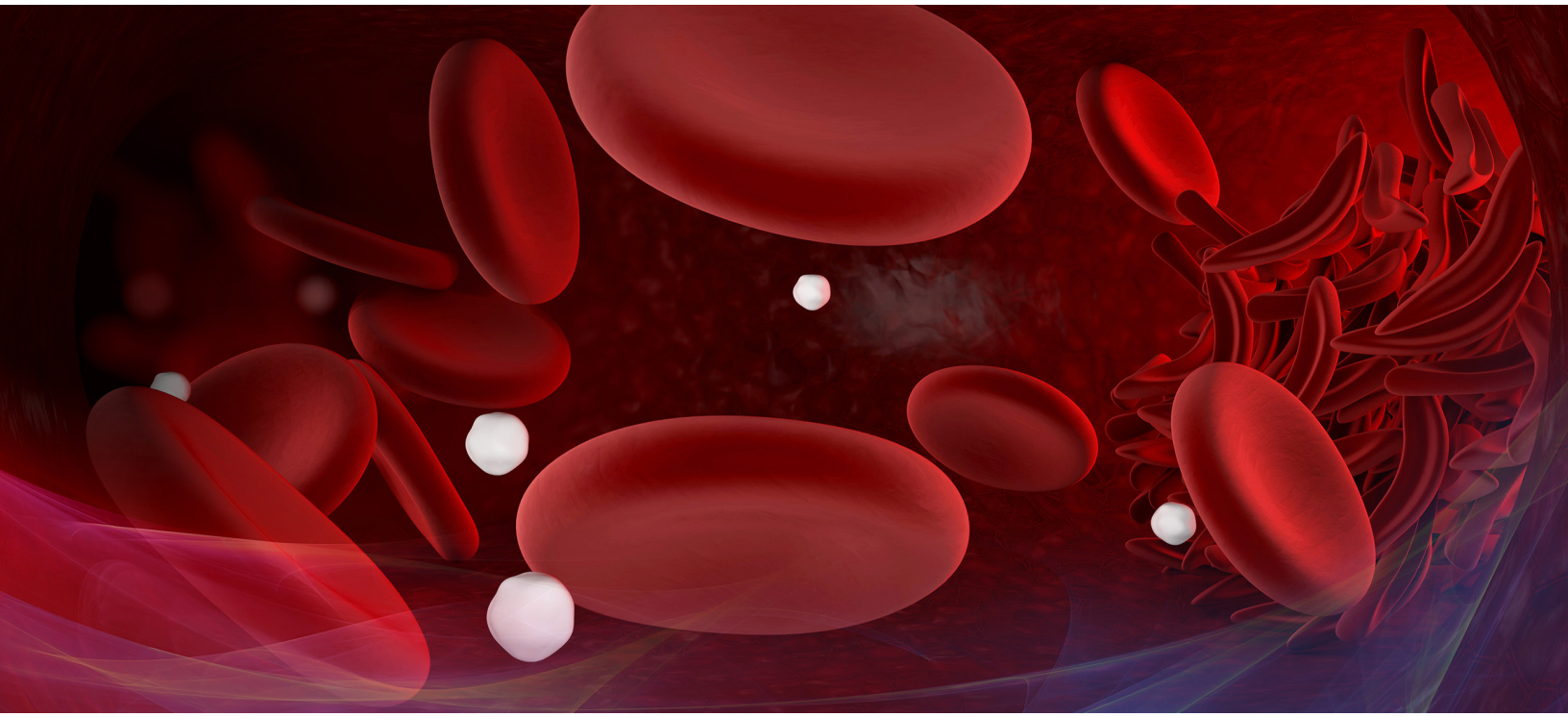
The 12th

SICKLE CELL IN FOCUS CONFERENCE



October 22 - 23, 2018

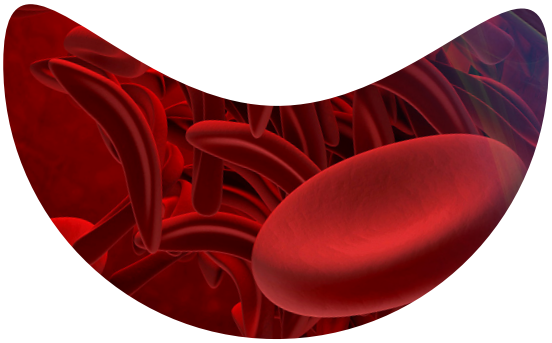
Natcher Conference Center

National Heart, Lung, and Blood Institute (NHLBI)
National Institutes of Health (NIH)
Bethesda, Maryland, USA

SCiF
SICKLE CELL IN FOCUS

sponsored by:

NIH National Heart, Lung, and Blood Institute



Welcome

Hello and a very warm welcome to the 12th annual Sickle Cell in Focus (SCiF) conference!

The National Heart, Lung, and Blood Institute (NHLBI) is excited to host the conference on the NIH Campus after a very successful conference in Kingston, Jamaica last year. Historically, investigators and physicians from Asia, Europe, Brazil, Africa, and the Caribbean have participated heavily in SCiF. We hope to continue expanding 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 SCD globally.

As always, the conference will have a mix of updates on management and therapies, and exploring what's new in technology for translational diagnostics. With improved survival for affected newborns, physicians are faced with an increasing number of adults with SCD. We have focused on a few of the complications in older adults, including pain, VTE's, hepatopathy, red blood cell allo-immunization and iron overload. There will also be an update on the curative therapies, the in's and out's of gene therapy - gene correction and gene addition. We will revisit the cerebrovascular complications, and not just overt disease, in a whole session by 3 eminent speakers. Can systems medicine and big data contribute to care of SCD?? We wait to hear from 3 speakers who will address this issue. However, medical advances become meaningful only if they reach the majority of patients. An inequity of treatment in SCD exists not just between high- and low-income countries, but even within well-resourced settings. Here, 2 experts share their thoughts on how to implement coverage of patients taking hydroxyurea and how to translate clinical care to developing countries in Africa.

Each of the 2 days will be ended by Debates that are highly popular with our participants as they are interactive and very educational. As always, we chose topics for which there are no clear answers, but no doubt our speakers will engage you in their riveting one-on-one discourse to win your vote. We have a very exciting line-up of speakers who are experts in their fields and will highlight the latest in high-quality research in sickle cell disease. We thank them in advance for giving us their precious time.

Thank you, to all our attendees, both near and from far, for their participation in this year's conference. We sincerely hope that you will enjoy SCiF 2018 at the NIH Campus. Please take a moment to complete the evaluation form at the end of the conference. Your feedback is highly appreciated. Lastly, we would also like to thank NHLBI for supporting this endeavor to continue the progress and impact of SCiF.

Thank you for joining us and we hope to see you again in Kingston, Jamaica for Sickle Cell in Focus 2019.

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

Program Directors

Swee Lay Thein, M.B., B.S., F.R.C.P., F.R.C.Path., D.Sc.
Senior Investigator and Chief
Laboratory of Sickle Cell Genetics and Pathophysiology

Sickle Cell Branch
NHLBI, National Institutes of Health

John F. Tisdale, M.D.
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

Agenda

Day One: Monday October 22nd

8:00am **Registration**

9:00am **Opening Remarks**
Richard Childs
Clinical Director, National Heart, Lung, and Blood Institute

SESSION ONE

9:05am **Sickle Cell Disease in 2018**

From birth to adults, has survival improved?
Kathryn Hassell
University of Colorado School of Medicine, Aurora, CO, USA

Optimizing current approved therapies

9:35am **Implementation research: Improving coverage of patients taking HU**
Keith Hoots
National Institutes of Health, Bethesda, MD, USA

10:05am **Translating Clinical Care to Developing Countries**
Russell Ware
Cincinnati Children's Hospital Medicine Center, Cincinnati, OH, USA

10:35am **BREAK**

SESSION TWO: SCD in Adults

10:45pm **Optimal disease management & health monitoring in older adults**
Sophie Lanzkron
John Hopkins Medicine, Baltimore, MD, USA

11:15pm **How we manage VTE's in adults**
Theodore Wun
UC Davis Comprehensive Cancer Center, Sacramento, CA, USA

11:45pm **Understanding pain and its mechanisms**
Catherine Bushnell
National Institutes of Health, Bethesda, MD, USA

12:15pm **LUNCH**

SESSION TWO: SCD in Adults (cont.)

1:15pm **The many facets of sickle pain and its management**
Samir Ballas
Thomas Jefferson University, Philadelphia, PA

1:45pm **Management of liver complications in SCD**
Abid Suddle
King's College Hospital NHS Foundation Trust, London, UK

SESSION THREE: Blood transfusion and iron overload

2:15pm **Presentation of the untransfusable patient**
Arun Shet
National Institutes of Health, Bethesda, MD

2:45pm **Genotyping vs extended phenotyping**
Connie Westhoff
New York Blood Center, New York, NY, USA

3:15pm **Iron overload in SCD, are we making progress?**
John Porter
University College Hospital, London, UK

3:45pm **BREAK**

DEBATE 1: Biomarkers in SCD: Just a lot of noise and handwaving

4:00pm **Pro: David Rees**
King's College London, London, UK

4:20pm **Con: Marilyn Telen**
Duke School of Medicine, Durham, NC, USA

4:40pm **Debate**

5:00pm **DAY ONE CLOSE**

Agenda

Day Two: Tuesday, October 23rd

7:30am **Registration**

8:30am **Opening Remarks**
Gary H. Gibbons
Director, National Heart, Lung, and Blood Institute, Bethesda, MD, USA

SESSION FOUR: Unanswered questions and new frontiers in curative therapies

8:35am **Allo-HSCT: match related, match unrelated, cord blood, and haplo**
Mark Walters
Children's Hospital Oakland Research Institute, Oakland, CA

9:05am **Collecting HSCs, not a trivial business**
John Tisdale
National Institutes of Health, Bethesda, MD, USA

9:35am **Gene therapy-addition, correction**
Dan Bauer
Boston Children's Hospital, Boston, MA, USA

10:05am **Update on gene addition therapy in SCD**
Alexis Thompson
Northwestern University, Chicago, IL, USA

10:35am **BREAK**

SESSION FIVE: Cerebrovascular disease in SCD

10:45am **Cerebrovascular disease in SCD: In Children**
Lori Jordan
Vanderbilt University Medical Center, Nashville, TN, USA

11:15am **Neurocognitive outcomes in SCD**
Allison King
Washington University in St. Louis, St. Louis, MO, USA

11:45am **Cerebral Hemodynamics in sickle cell disease and sickle cell carriers**
Monica Hulbert
St. Louis Children's Hospital, St. Louis, MO, USA

12:15pm **LUNCH**

SESSION SIX: Inflammation and vascular injury in SCD

1:15pm **The common ground between SCD and Malaria**
Tom Williams
KEMRI/Wellcome Trust Research Programme, Kilifi, Kenya

1:45pm **Elevated inflammatory markers - Do they mean anything in sickle patho physiology?**
Nicola Conran
University of Campinas, Sao Paulo, Brazil

SESSION SEVEN: System medicine and Big Data: Transforming care of SCD to the 21st century

2:15pm **Complex genetics for a simple mutation-getting to the heart of the matter**
Swee Lay Thein
National Institutes of Health, Bethesda, MD, USA

2:45pm **A short cut to modeling SCD**
George Karniadakis
Brown University, Providence, RI, USA

3:15pm **Cardiovascular phenotypes of SCD**
Vandana Sachdev
National Institutes of Health, Bethesda, MD, USA

3:45pm **BREAK**

DEBATE 2: Developing therapies for SCD-Makes more sense to target the root cause than all the downstream events

4:00pm **Pro: Yogen Saunthararajah**
Cleveland Clinic, Cleveland, OH, USA

4:20pm **Con: Jane Little**
UH Cleveland Medical Center, Cleveland, OH, USA

4:40pm **Debate**

5:00pm **CLOSE OF CONFERENCE**

Speaker Biographies

Biographies are alphabetical

Samir K. Ballas MD, FACP, HonDSc, FASCP, DABPM, FAAPM
Emeritus Professor of Medicine and Pediatrics
Former Director of Comprehensive Sickle Cell Center
Former Director Thomas Jefferson University Hospital Blood Bank
Cardeza Foundation for Hematologic Research
Thomas Jefferson University, Philadelphia PA USA

Samir K. Ballas, MD received his medical degree with distinction from the American University of Beirut-Lebanon. He completed his medical residency including being chief resident at the American University hospital of the American University of Beirut. He completed his fellowship training in Hematology at the Cardeza Foundation of Thomas Jefferson University in Philadelphia, Pennsylvania. He is board certified in Internal Medicine, Hematology, Transfusion Medicine/Blood Banking, Pain Medicine and Pain Management.

Dr. Ballas is currently Emeritus Professor of medicine and Pediatrics at Thomas Jefferson University and Honorary staff member of HEMORIO, the Hematology Institute in Rio de Janeiro, Brazil. He is Former Director of the adult Sickle Cell Program of the Commonwealth of Pennsylvania for the Philadelphia Region. He is Former Director of Jefferson's Sickle Cell Center, and Former Director of the Thomas Jefferson University Hospital Blood Bank. His major research interests include 1) Red cell disorders in general and the hemoglobinopathies in particular; 2) the pathophysiology and management of sickle cell pain; 3) molecular and cellular factors that affect the phenotypic expression of sickle cell disease; and 4) preventative and curative therapy of sickle cell disease.

Dr. Ballas is an elected member of the Alpha Omega Alpha Honor Medical Society. He is member of the Editorial Board of the American Journal of Hematology, Hemoglobin and Advances in Hematology. He is also a Hemoglobinopathies Editor of The Cochrane Review Database. He is Recipient of Life Time Achievement Award for Service, Research and Education for Sickle Cell Disease from Howard University, Recipient of Testimonial as an International Health Professional of the Year 2005 with Outstanding Contributions to Sickle Cell Disease, Recipient of Distinguished Service Award From Sickle Cell Thalassemia Patients Network, Brooklyn, NY and Recipient of Patient Advocacy Award from the American Academy of Pain Medicine. In addition, he has authored or co-authored over 800 articles and abstracts in a diverse number of publications. He has authored four books two of which are on Sickle Cell Pain.

Daniel E. Bauer, MD, PhD
Boston Children's Hospital
Dana-Farber Cancer Institute
Harvard Medical School

Daniel Bauer is a physician-scientist whose research utilizes genome editing to understand the causes of blood disorders and to develop innovative therapeutic strategies. His clinical work in pediatric hematology focuses on the care of patients with hemoglobin disorders. He received his ScB in Biology from Brown University and MD-PhD from the University of Pennsylvania. He completed clinical training in Pediatrics and Pediatric Hematology/Oncology at Boston Children's Hospital and Dana-Farber Cancer Institute. He is a Principal Investigator and Staff Physician at Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Assistant Professor of Pediatrics at Harvard Medical School, Principal Faculty at the Harvard Stem Cell Institute, and Associate Member of the Broad Institute of MIT and Harvard. His honors have included the American Society of Clinical Investigation Young Physician-Scientist Award (2014), NIH Director's New Innovator Award (2016), and Society for Pediatric Research's Young Investigator Award (2017).

M. Catherine Bushnell, PhD
Scientific Director
National Center for Complementary and Integrative Health
National Institutes of Health

Dr. Bushnell is the Scientific Director of the National Center for Complementary and Integrative Health at the NIH. She holds a Ph.D. in Experimental Psychology from the American University and received postdoctoral training in neurophysiology at the NIH. She was the Harold Griffith Professor of Anesthesia at McGill University before returning to NIH in 2012. Among her honors are the Lifetime Achievement Award from the Canadian Pain Society and the Frederick Kerr Basic Science Research Award from the American Pain Society. Her research interests include forebrain mechanisms of pain processing, psychological modulation of pain, and neural alterations in chronic pain patients.

Nicola Conran, PhD
University of Campinas, Brazil

Nicola Conran is a Biochemist (University of Birmingham, UK, and Ph.D., University of Nottingham, UK). She leads the Vascular Inflammation Laboratory at the Haematology and Haemotherapy Centre, University of Campinas where she has been involved in investigating aspects of mechanisms of vaso-occlusion, inflammation and cell adhesion in sickle cell disease, in collaboration with Fernando Costa.

Kathryn Hassell, MD Professor of Medicine, *Division of Hematology*
Director, *Colorado Sickle Cell Treatment and Research Center*
University of Colorado Denver

Dr. Kathryn Hassell is a Professor of Medicine and Director of the Colorado Sickle Cell Center and inpatient and outpatient pharmacy-directed anticoagulation services at the University of Colorado Denver. She completed her Bachelor's of Art in Medical Technology at the College of St. Scholastica and her medical education at University School Minnesota School of Medicine, internal medicine residency at University of North Carolina-Chapel Hill and fellowship in Hematology/Oncology at the University of Colorado Denver. Her interest and expertise includes academic specialty practice, clinical and translational research in hemoglobinopathies, thrombotic disorders and anticoagulation. Dr. Hassell is an active member of the American Society of Hematology, International Society of Thrombosis and Hemostasis, and the American Pain Society. She co-chairs and serves on a number of national steering committees for multicenter clinical trials and federal projects sponsored by NIH, HRSA and CDC, and is the founder of the Sickle Cell Adult Provider Network, an organization which seeks to enhance research collaboration and communication across the adult sickle cell provider community. She has published numerous articles and other work in her field and participated in guideline and registry development. Dr. Hassell lectures nationally and internationally on sickle cell disease, thrombotic disorders and anticoagulation. In her spare time, she enjoys the mountains of Colorado and medical missionary work in Central America and Africa.

W. Keith Hoots, MD
Director, *Division of Blood Diseases and Resources*
National Institutes of Health

W. Keith Hoots, M.D., is director of NHLBI's Division of Blood Diseases and Resources. Dr. Hoots received his A.B. in English and chemistry and his M.D. from the University of North Carolina (UNC) at Chapel Hill, North Carolina. While a senior at UNC, he worked in the hemostasis laboratory of Kenneth Brinkhous, M.D. He then completed his pediatric internship and residency at Children's Medical Center, Parkland Memorial Hospital, in Dallas, Texas. He returned to UNC for his fellowship in pediatric hematology oncology and worked in the laboratory of Harold Roberts, M.D. Dr. Hoots then joined the faculty at MD Anderson Cancer Center.

Dr. Hoots' major interests involve the management and diagnosis of congenital and acquired bleeding disorders and clotting disorders. His work includes the creation of longitudinal follow-up of hemophilia cohorts with HIV and hepatitis, gene therapy trials for hemophilia A and B, clinical trials of new clotting concentrates for hemophilia A and B, and the impact of care and clotting factor product on hemophilia patient outcome. He also has a 20-year interest in the diagnosis and treatment of diffuse intravascular coagulation (DIC), particularly DIC in head trauma. He has been intimately involved in the development of safe coagulation factor products, having completed his training as the HIV epidemic was evolving in hemophilia patients. By the late 1980s, he was able to return to the hemostasis focus that initially attracted him to the field, and he has continued to be a productive investigator and collaborator. He has a strong interest in global collaborations and in developing public–private partnerships, and he recently completed his sabbatical in Belgium.

Dr. Hoots is a past member of the U.S. Department of Health and Human Services Blood Safety and Availability Advisory Committee to the secretary of health, past chair of the Medical and Scientific Advisory Committee for the National Hemophilia Foundation, and subcommittee co-chair of the DIC Subcommittee of International Society on Thrombosis and Hemostasis. He has also been an associate editor for Seminars in Thrombosis and Hemostasis and served on the editorial boards of Haemophilia, Haemophilia Forum, and the International Monitor on Hemophilia. Dr. Hoots is a past president of the Hemophilia Research Society of North America.

Monica L. Hulbert, MD
Associate Professor of Pediatrics, *Washington University School of Medicine*
Director, *Sickle Cell Disease Program, St. Louis Children's Hospital*

Dr. Hulbert is the director of the Pediatric Sickle Cell Disease Program at St. Louis Children's Hospital and an Associate Professor of Pediatrics at Washington University School of Medicine. As program director, she developed and implemented a primary hematologist care model to improve continuity of care and oversaw the program's expanded hydroxyurea use for primary prevention of sickle cell disease complications. Working with a collaborative group of neurologists and neuroimaging specialists at Washington University, her research interests are focused on strokes and cerebral vasculopathy related to sickle cell disease, and the impact of hematopoietic stem cell transplant on these complications.

Lori Jordan, MD, PhD
Associate Professor of Pediatrics and Neurology
Director, *Pediatric Stroke Program*
Vanderbilt University Medical Center

Lori Jordan, MD, PhD is an Associate Professor of Pediatrics and Neurology at Vanderbilt University Medical Center in Nashville, TN. She completed medical school at the University of Oklahoma and residency in Pediatrics and Child Neurology at the Johns Hopkins Hospital in Baltimore. Dr. Jordan became interested in stroke in children and young adults during her child neurology residency. She completed a fellowship in Vascular Neurology (Stroke) at Johns Hopkins as well as a PhD in Clinical Investigation at the Johns Hopkins Bloomberg School of Public Health. At Vanderbilt, Dr. Jordan directs the pediatric stroke program. She developed an acute stroke team for children at Monroe Carell Jr. Children's Hospital. Dr. Jordan's research is focused on understanding predictors of recovery after stroke in children and on primary and secondary stroke prevention in children and adults with sickle cell anemia. She leads imaging research on altered cerebral hemodynamics in sickle cell anemia that may predispose to stroke and works as part of multidisciplinary teams on a number of studies focused on stroke prevention in children and adults with sickle cell disease. She has research funding from the National Institutes of Health and recently completed a Collaborative Science Award from the American Heart Association.

George Karniadakis, PhD
The Charles Pitts Robinson and John Palmer Barstow Professor of Applied Mathematics, *Brown University*

George Karniadakis received his S.M. (1984) and Ph.D. (1987) from Massachusetts Institute of Technology. He was appointed Lecturer in the Department of Mechanical Engineering at MIT in 1987 and subsequently he joined the Center for Turbulence Research at Stanford / Nasa Ames. He joined Princeton University as Assistant Professor in the Department of Mechanical and Aerospace Engineering and as Associate Faculty in the Program of Applied and Computational Mathematics. He was a Visiting Professor at Caltech (1993) in the Aeronautics Department. He joined Brown University as Associate Professor of Applied Mathematics in the Center for Fluid Mechanics on January 1, 1994. He became a full professor on July 1, 1996. He has been a Visiting Professor and Senior Lecturer of Ocean/Mechanical Engineering at MIT since September 1, 2000. He was Visiting Professor at Peking University (Fall 2007 & 2013). He is a Fellow of the Society for Industrial and Applied Mathematics (SIAM, 2010-), Fellow of the American Physical Society (APS, 2004-), Fellow of the American Society of Mechanical Engineers (ASME, 2003-) and Associate Fellow of the American Institute of Aeronautics and Astronautics (AIAA, 2006-). He received the Ralf E Kleinman award from SIAM (2015), the J. Tinsley Oden Medal (2013), and the CFD award (2007) by the US Association in Computational Mechanics. His h-index is 73 and he has been cited over 26,000 times.

Allison King, MD, MPH, PhD
Associate Professor of Occupational Therapy
Pediatrics and Medicine, Washington University School of Medicine

Dr. King is a pediatric and young adult hematologist investigating cognitive function and educational attainment of children and adults with sickle cell disease. Her team is focused on conducting interventions to prevent cognitive decline.

Dr. King conducts dissemination and implementation research to facilitate dissemination of NHLBI and ASH clinical guidelines and improve the care of people with sickle cell disease.

Sophie Lanzkron, MD, MHS
Director, *Sickle Cell Center for Adults*
Associate Professor of Medicine and Oncology
Johns Hopkins School of Medicine

Dr. Lanzkron is an Associate Professor of Medicine and Oncology in the Division of Hematology at the Johns Hopkins University School of Medicine and is the Director of the Sickle Cell Center for Adults at Johns Hopkins which delivers state-of-the art, multidisciplinary care to over 500 patients. She is internationally recognized for her pioneering research on the optimal care and management of patients with sickle cell disease. She has served on the National Institutes of Health, Expert Panel in the Management of Sickle Cell Disease and serves on the American Society of Hematology's Sickle Cell Guideline Panel. Her research focus is on improving the quality of care provided to this historically underserved population and she is considered an expert in health services research in sickle cell disease. The Johns Hopkins Sickle Cell Infusion Center, which opened in 2008, provides urgent care to patients in crisis so that they can bypass the emergency department. This remarkable innovation has led to numerous improvements in outcomes including decreases in admissions, 30 day readmissions and most importantly rapid relief of pain in a patient centered environment. This innovative model of care is currently being emulated throughout the country and she has a $4 million grant from PCORI to systematically compare outcomes from infusion models in four states to usual emergency department care for the treatment of vaso-occlusive crisis.

Jane Little, MD
Director, *Adult Sickle Cell Disease Program*
University Hospitals Cleveland Medical Center.
Professor, *Case Western Reserve School of Medicine*

Jane Little MD is director of the Adult Sickle Cell program at University Hospitals/Seidman Cancer Center in Cleveland, Ohio. She is a Professor of Medicine at Case Western Reserve University School of Medicine. She has participated in a number of NIH- or PCORI-funded natural history, treatment, and observational studies in SCD, including Walk-PHaSST and ESCAPED. With Umut Gurkan PhD at Case School of Engineering and Deepa Manwani MBBS at Children's Hospital at Montefiore (Einstein College of Medicine), Dr. Little has an RO1 entitled, "Standardized Monitoring of Cellular Adhesion to Improve Clinical Care in Sickle Cell Disease". Dr. Little/CRWU are also founding members (with Johns Hopkins, Oakland-UCSF Benihoff, and Medical College of Wisconsin) of The Globin Research Network of Data and Discovery or GRNDaD, a multi-centered prospective database.

John Porter, MA, MD, FRCP, FRCPath
Professor and Consultant Hematologist
University College London Hospitals

Dr. Porter is a Professor of Haematology and Consultant Haematologist at the University College London Hospitals in London, UK and head of the joint Red Cell Unit for UCLH and Whittington Hospitals. His clinical and research focus has been treatments of thalassaemia and sickle cell disorders, with particular reference to iron overload, his particular focus has been the interface of clinical and basic laboratory research on the mechanisms of iron chelation, the speciation and uptake of non-transferrin-bound iron (NTBI) species, the molecular basis of iron homeostasis in health and disease, and the actions and toxicities of mixed-ligand chelation therapies. Dr. Porter has received funding from many sources including the Medical Research Council (MRC), the Welcome Foundation and National Institutes of Health (NIH) for this work. He has been the principal UK investigator in numerous multicentre clinical trials on iron chelation and am currently the principal UK investigator on ongoing trials with Luspatercept for correcting anaemia in Thalssaemias (Celgene) and Gene therapy for Thalassaemia (Bluebird Bio). He has published more than 350 peer-reviewed articles with over 10,000 citations and an H index of over 54, and also made numerous contributions to books, as well as clinical guidelines and other medical articles. Dr. Porter has served as scientific adviser to the British Society of Haematology, the UK Thalassaemia Society, the Thalassaemia International Federation (TIF), and to grant review and advisory panels at the NIH. In 1999, he was awarded the Lionel Whitby Medal for MD of exceptional merit by Cambridge University. In 2008 he received the Prix Gallien at the houses of parliament on behalf of Novartis for 'Deferasirox as outstanding orphan drug'. In 2015 he received the SITA international award for outstanding international clinical thalassaemia centre at UCLH. In 2017 I received the International Bioiron society (IBIS) Marcel Simon award for excellence in research on Non-transferrin bound iron (NTBI). I am the recent recipient of the British Society of Haematology Gold Medal award 2018.

David C. Rees, MA, MBBS
Pediatric Hematologist
King's College Hospital, London

David Rees is a paediatric haematologist at King's College Hospital, London. He trained in various places around the UK, including Bath and the Channel Island of Jersey. He is a medical adviser to the Sickle Cell Society in the UK.

Vandana Sachdev, MD
Senior Research Clinician and Director of the Echocardiography Laboratory
Division of Intramural Research
Heart, Lung, and Blood Institute, National Institutes of Health

Vandana Sachdev is a cardiologist and Staff Clinician in the Cardiology Branch of the NHLBI Division of Intramural Research. She received her M.D. from the University of Michigan and trained in cardiology at the University of Maryland. She joined NHLBI in 1998 and is now the Director of the Echocardiography Laboratory. She is a member of the American Society of Echocardiography and is active in various committees and task forces. Dr. Sachdev's lab served as the core lab for the Walk-Phasst study and for other studies supported by NHLBI. Her research area of interest is cardiac imaging and she has worked on phenotyping the sickle cell cohort here as well as numerous other rare disease groups.

Yogen Saunthararajah, MD
Professor of Medicine, Staff, *Co-Leader Developmental Therapeutics Program*
Taussig Cancer Institute of Cleveland Clinic and
Case Comprehensive Cancer Center

Yogen Saunthararajah is a Professor of Medicine, Staff Physician and Co-Leader of the Developmental Therapeutics Program at the Taussig Cancer Institute of Cleveland Clinic and Case Comprehensive Cancer Center, and founding-scientist of EpiDestiny. His research and drug development efforts focus on development of non-cytotoxic drugs to inhibit specific corepressor enzymes implicated in epigenetic repression of the fetal hemoglobin gene, to thereby activate fetal hemoglobin expression in an accessible, sustainable way that is feasible for life-long modification of sickle cell disease and beta-thalassemia around the world. A co-focus is the use of such non-cytotoxic epigenetic drugs to exploit a fundamental and common distinction between normal and malignant self-replication, for selective termination of malignant but not normal self-replication even if p53 is mutated. The same treatments can moreover trigger immune-recognition of cancers (convert tumors from 'cold' to 'hot') while sparing immune-effectors, and are therefore a rational platform for increasing the spectrum of cancers that respond to immune checkpoint blockade.

Arun Shet, MD
Staff Clinician/Senior Research Physician
Sickle Cell Branch, Division of Intramural Research
National Heart, Lung, and Blood Institute, National Institutes of Health

Dr. Shet is a Staff Clinician/Senior Research Physician in the Sickle Cell Branch/NHLBI, a position involving patient care and translational research centering on adults with sickle cell disease. He serves as the Principal Investigator on two active protocols and as Associate Investigator on four other active research protocols. His clinical interest applies a fundamental understanding of the regulation of erythropoiesis and basic biochemistry to the diagnosis and management of patients with inherited and acquired anemia, and reflects his translational research interest in hematology. He has extensive experience conducting patient oriented research in understanding the vascular pathobiology of sickle cell disease. Some of his work has evaluated novel drug therapeutics and tested their role in the pre-clinic setting using murine models of sickle cell disease. His current interests include a more fundamental understanding of the hypercoagulable state in sickle cell disease. More recently, his research group, along with international collaborators, developed an educational intervention seeking better public health control of childhood anemia that we evaluated in a randomized trial.

Abid Suddle
Consultant Hepatologist and Liver Transplant Physician at the Institute of Liver Studies
King's College Hospital, London, United Kingdom

Research and clinical interests include liver cancer, liver transplantation, and liver disease in patients with Sickle cell anaemia. I run a joint Liver-Sickle clinic with Haematology colleagues.

Marilyn Telen, MD
Wellcome Professor of Medicine, *Division of Hematology*
Director, *Duke Comprehensive Sickle Cell Center*
Duke University School of Medicine

Dr. Telen is the Wellcome Professor of Medicine at Duke University, where she has been since 1980. Dr. Telen has focused both her research and clinical efforts on red cell membrane proteins, the antigens carried by them, and the disorders associated with abnormalities of red blood cells. Her research identified the biochemical and genetic bases of a number of blood group antigen systems, as well as the physiologic role of several of the proteins bearing those antigens. Her current work focuses on the role of red cell adhesion molecules in sickle cell disease (SCD), as well as on the conduct of genetic and clinical translational research in SCD and transfusion medicine. Her genetic work has focused on the role of genetic polymorphisms in the variability of SCD and in identification of the genetic mechanisms leading to sequelae of SCD. In addition, she served as Chief of Hematology at Duke for >17 years and has been Director of the Duke Comprehensive Sickle Cell Center for 20 years.

Swee Lay Thein, MB, BS, FRCP, FRCPath, DSc
Chief, *Sickle Cell Branch*
Division of Intramural Research
National Heart, Lung, and Blood Institute, National Institutes of Health

Swee Lay Thein 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.

Alexis Thompson, MD, MPH
Sarah and A. Watson Armour Chair in Childhood Cancer and Blood Disorders, Hematology Section Head; *Professor of Pediatrics, Feinberg School of Medicine at Northwestern University*

Alexis A. Thompson, MD, MPH, is the head of the hematology section of the Division of Hematology Oncology Transplantation at the Ann and Robert H. Lurie Children's Hospital of Chicago, where she also serves as the A. Watson and Sarah Armour Endowed Chair for Childhood Cancer and Blood Disorders. Dr. Thompson is also Associate Director of Equity and Minority Health at the Robert H. Lurie Comprehensive Cancer Center of Northwestern University. She earned her medical degree from Tulane University School of Medicine. After completing her residency at the Children's Hospital of Los Angeles, Dr. Thompson earned her Master of Public Health at the University of California – Los Angeles School of Public Health, followed by additional postgraduate training at the Children's Hospital of Philadelphia. She is board-certified in Pediatric Hematology-Oncology.

Immediately prior to her election as ASH Vice President, Dr. Thompson served as an ASH councilor from 2010-2014. She is currently a member of the ASH Sickle Cell Task Force and a reviewer for ASH's journal, Blood. Dr. Thompson has also served on the ASH Committee on Government Affairs, as a mentor for the Society's Minority Medical Student Award Program for more than 10 years, and she has co-chaired the ASH Annual Meeting Education Program. In addition to her service to ASH, Dr. Thompson has served on the Board of Directors of the National Marrow Donor Program and on the U.S. Department of Health and Human Services Secretary's Advisory Committee on Heritable Disorders in Newborns and Children.

Dr. Thompson's major fields of interest include sickle cell disease, thalassemia, transfusional iron overload, and stem cell transplantation for pediatric patients. Her research has focused on developmentally regulated genes in early hematopoiesis, and she is an investigator on NIH-funded multi-center clinical trials as well as her own institutional clinical studies. In addition to her academic endeavors, Dr. Thompson is interested in enhancing the trainee pipeline in hematology, particularly in non-malignant hematology, and fostering international collaborations in hematology in regions with unmet medical need.

Dr. Thompson has received several prestigious awards, including the Robert Wood Johnson Foundation Minority Medical Faculty Development Award as well as the Minority Scholar Award in Cancer Research from the American Association for Cancer Research. She recently received the Frank A. Oski Memorial Award from the American Society of Pediatric Hematology Oncology in recognition of her contributions to the field.

John Tisdale, MD
Chief, *Cellular and Molecular Therapeutics Branch*
Division of Intramural Research
National Heart, Lung, and Blood Institute, National Institutes of Health

John Tisdale received his M.D. degree from the Medical University of South Carolina in Charleston, where he also received his B.A. in Chemistry. 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 2011 the College of Charleston recognized Dr. Tisdale with the Alumni of the Year Award and the Pre-Medical Society's Outstanding Service Award in Medicine. He was recently elected to the American Society for Clinical Investigation and is a member of the American Society of Hematology. 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.

Mark C. Walters, MD
Jordan Family Director, *Blood and Marrow Transplant Program*
Professor of Pediatrics
University of California, San Francisco

Mark C. Walters, MD, is the Jordan Family Director of the Blood and Marrow Transplantation Program at UCSF Benioff Children's Hospital, Oakland and Professor of Pediatrics at UCSF. He is Program Director of the CIRM alpha stem cell clinic at UCSF. Dr. Walters received his A.B. with honors in Genetics from the University of California, Berkeley and his MD from the University of California, San Diego. He completed pediatric residency training at the University of Washington and hematology/oncology fellowship training at the University of Washington and the Fred Hutchinson Cancer Research Center in Seattle. He was a junior faculty member in Seattle before matriculating to Oakland in 1999. He has been active in cooperative clinical transplantation trials and has led several NIH-supported investigations of hematopoietic cell transplantation for sickle cell anemia and thalassemia. He has authored or co-authored many publications with a focus on hematopoietic cell transplantation for hemoglobin disorders, and he has a research interest in extending transplantation to young adults with hemoglobin disorders and other novel cellular therapies for hemoglobin disorders. Currently, research interests are focused on genomic editing and gene addition therapies as a strategy to extend curative therapy in all patients who inherit a clinically significant hemoglobinopathy.

Russell E. Ware, MD, PhD
Director, *Division of Hematology*
Institute Co-Director, *Cancer and Blood Diseases Institute*
Director, *Global Health Center*
Marjory J. Johnson Chair of Hematology Translational Research
Professor, University of Cincinnati Department of Pediatrics

Russell Ware, MD, PhD, has been involved with a wide variety of clinical and translational hematology research projects for over 25 years, but his primary interests have focused on sickle cell disease. Dr. Ware has substantial personal experience with directing patient-oriented research, and he currently runs an NIH-funded laboratory effort that investigates genetic modifiers of sickle cell disease. The main focus of his lab research is to understand the phenotypic variability that occurs with hydroxyurea treatment, through the study of hydroxyurea pharmacokinetics, pharmacodynamics, pharmacogenetics, and pharmacogenomics. Dr. Ware is also the national principal investigator for several NIH-funded multicenter sickle cell clinical trials, including the recently completed Stroke With Transfusions Changing to Hydroxyurea (SWiTCH), and the current TCD With Transfusions Changing to Hydroxyurea (TWiTCH) and Sparing Conversion to Abnormal TCD Elevations (SCATE) studies that include non-US clinical sites. Most recently, Dr. Ware has moved his research efforts into the international arena, starting SCD pilot screening programs in Angola, and now conducting clinical trials to determine the safety and efficacy of hydroxyurea in developing countries.

Connie Westhoff, SBB, PhD
Executive Scientific Director
Immunohematology and Genomics, New York Blood Center

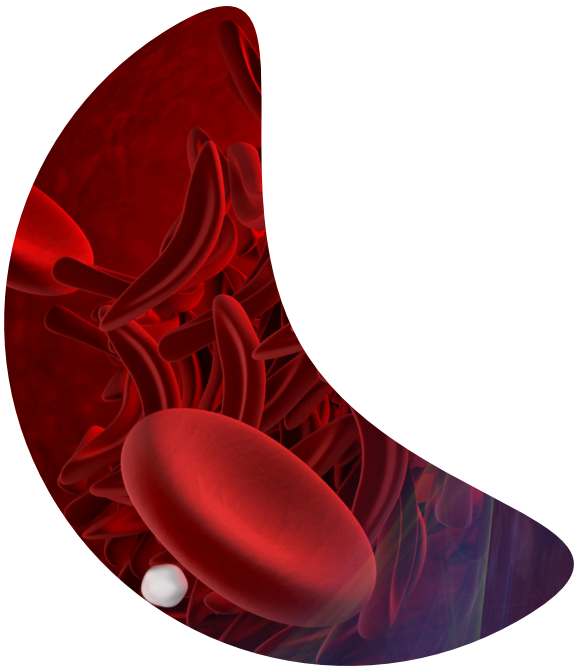
Dr. Westhoff is executive scientific director of the Laboratory for Immunohematology and Genomics at the New York Blood Center and the National Center for Blood Group Genomics. The primary focus of her research is to improve patient care and transfusion safety through the use of genomics with an emphasis on patients with Sickle Cell Disease. She is considered an expert on the Rh system, and has published more than 100 scientific papers and authored numerous book chapters. Her current funded research projects include RH genotype matching and expanding cultured red cells in the laboratory for use as laboratory reagents. She is an associate editor for the Genomics section of the journal *Transfusion*, an editor of the AABB technical manual, and has served on numerous AABB committees including the board of directors, and has received numerous awards. Also active in ASH and ISBT, she has lectured nationally and internationally and is a reviewer for numerous journals and abstracts for U.S. and international meetings in hematology and transfusion medicine.

Tom Williams, MD, PhD
Chair of Hemoglobinopathy Research, *Imperial College, London*
Pediatrician and Clinical Investigator, *KEMRI/Wellcome Trust Research programme*

Tom Williams is a pediatrician and clinical investigator with more than 25 years of scientific experience. He has worked at the KEMRI/Wellcome Trust Research Programme (KWTRP) in Kilifi, Kenya (**http://www.kemri-wellcome.org**) since 2000, while holding parallel appointments at UK Universities. Professor Williams is the Chair of Hemoglobinopathy Research at Imperial College, London. His main focus is on genetic conditions that affect red cell structure and function. He runs a program of research on the epidemiology and basic science of hemoglobin disorders in Kenya, and a specialist research clinic serving >700 children with SCD. Dr. Williams has published extensively on the burden and natural history of SCD on local and global scales, and is active in the national dialogue for the development of treatment guidelines in Kenya.

Ted Wun, MD, FACP
Professor of Medicine
Associate Dean, Research PI and Director
UC Davis Clinical and Translational Science Center
Chief, *Division of Hematology Oncology*
University of California, Davis School of Medicine

Ted Wun, M.D., is Associate Dean for Research, PI/Director of the UC Davis Clinical and Translations Science Center, and Chief, Division of Hematology Oncology at the UC Davis School of Medicine. He has been involved in sickle cell disease clinical research his entire career and served as Chair of the NHLBI Sickle Cell Disease Advisory Committee. Over the last 12 years his team at UC Davis has been using California Cancer Registry (CCR), and California Office of Statewide Health Planning and Development (OSHPD) discharge and emergency department utilization data to describe the epidemiology of cancer-associated thrombosis and complications of other hematological and oncologic conditions. More recently, using OSHPD alone and linked to the CCR, the UC Davis team has derived a sickle cell disease cohort to describe the epidemiology of complications such as venous thromboembolism, osteonecrosis of the femoral head, and malignancies in the California sickle cell disease population.



National Heart, Lung,
and Blood Institute