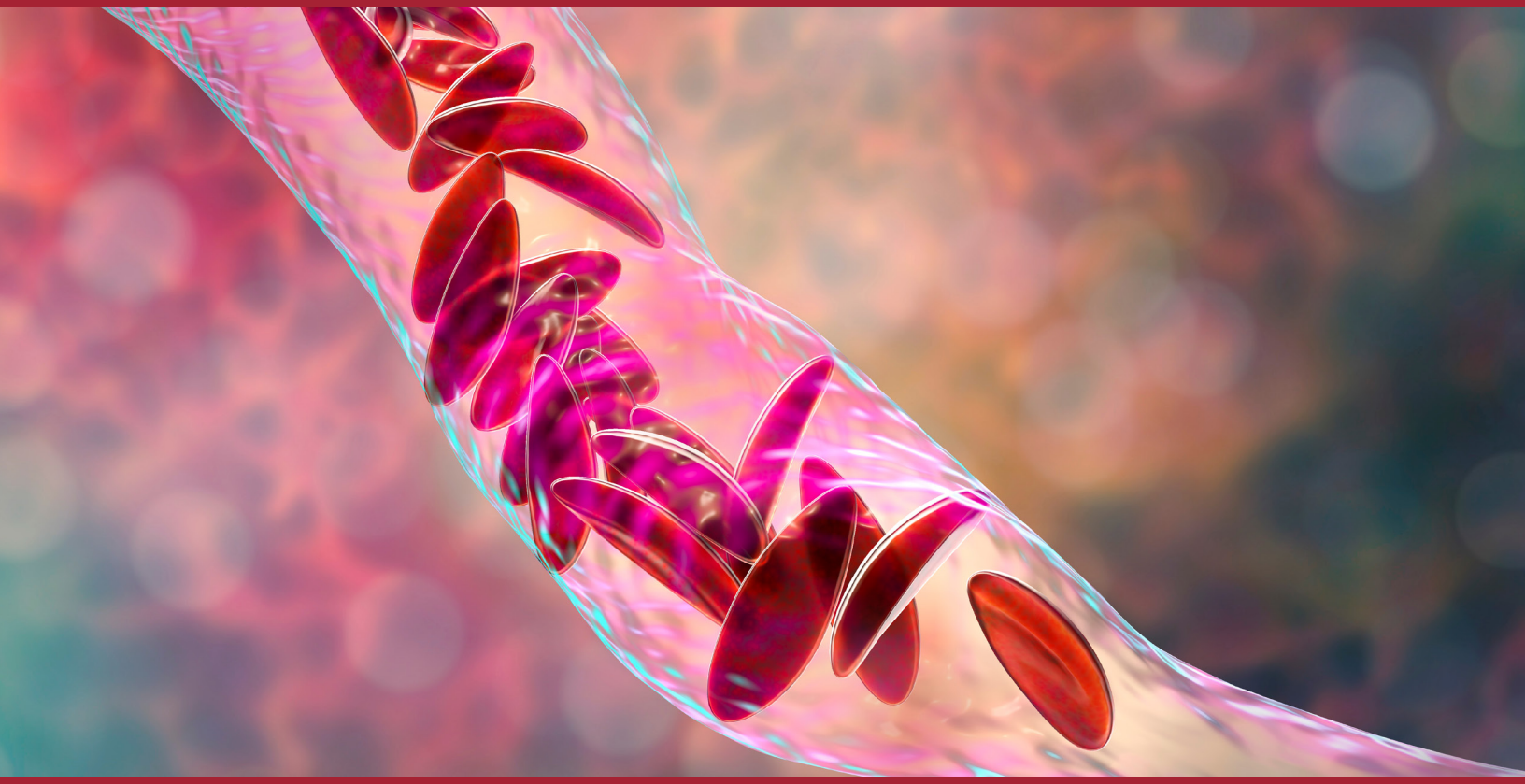
The 17th Annual

SICKLE CELL IN FOCUS

CONFERENCE



SEPTEMBER 19 - 20, 2024

The Jamaica Pegasus Hotel

Kingston, Jamaica

SCiF
SICKLE CELL IN FOCUS

sponsored by:

NIH National Heart, Lung, and Blood Institute

We are honored to welcome you to the 17th annual Sickle Cell in Focus (SCiF) Conference!

The National Heart, Lung, and Blood Institute (NHLBI) is excited to be co-hosting the 2024 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 in SCiF in London and the USA. By 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 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.

The goal of SCiF during these two rigorous days is to highlight updates on screening for sickle cell disease (SCD), complexities of common complications in SCD such as pain management, sleep and reproductive health, therapies for SCD both curative and non-curative, and ongoing challenges in the management of SCD complications. We will also include a panel discussion on where we are headed in the treatment of SCD, which will include perspective from both clinicians and patients. We are grateful to all our speakers for contributing their time and expertise to make this conference a success.

This year's conference is hybrid and being streamed from Kingston, Jamaica. We sincerely hope that you will enjoy SCiF 2024 whether in-person or virtually. For those attending in person, you will have the opportunity to interact with renowned experts, participate in engaging discussions, and network with fellow attendees who share your passion for sickle cell research and advocacy. 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.

Thank you to all of our attendees from all over the world for their participation in this year's conference. We look forward to seeing you again at Sickle Cell in Focus 2025!

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

Program Directors

Swee Lay Thein, MBBS, 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, MBBS, PhD
Professor, Pediatric Pulmonology & Clinical Research

Caribbean Institute for Health Research
The University of the West Indies

Monika Asnani, MBBS, DM, PhD
Professor, Family Medicine and Epidemiology
Director, Sickle Cell Unit

Caribbean Institute for Health Research
The University of the West Indies

AGENDA

Day One, THURSDAY, SEPTEMBER 19, 2024

8:00am Registration Check In

8:30am Opening Remarks from Prof. Minerva Thame, Dean-FMS, Jamaica
8:40am Remarks from RADM Richard Childs, MD, NHLBI

SESSION ONE: SCREENING FOR SICKLE CELL DISEASE
Chair: Monika Asnani, MBBS, DM, PhD

8:45-9:15am **Point of Care Testing: Promise and Role**
Daniel Dexter, MD
Imperial College Healthcare NHS Trust

NEWBORN SCREENING UPDATES

9:15-9:25am Caribbean
Jennifer Knight-Madden, MBBS, PhD
The University of the West Indies

9:25-9:35am Africa
Obiageli Nnodu, MD, MPH, FAAFP
University of Abuja

9:35-9:45am Well-Resourced Countries: Europe
Raffaella Colombatti, MD, PhD
University of Padova

9:45-9:55am Low- or Middle-Income Countries (LMIC): India, Brazil
Ana Cristina Silva-Pinto, MD, PhD
University of Sao Paulo

9:55-10:05am Discussion and questions

10:05-10:15am BREAK

**Virtual*

SESSION TWO: CLINICAL TRANSLATION OF BIOENGINEERING AND BIOPHYSICS IN SCD

Chair: Swee Lay Thein, MD

10:15-10:45am **Clinical Translation of Microfluidics to Better Understand Blood Flow in SCD – Where Do We Go From Here?**
Wilbur A. Lam, MD, PhD
Emory University

10:45-11:15am **Dynamic Interplay Between Biomechanical and Biochemical Stimuli in SCD**
Manu O. Platt, PhD
National Institute of Biomedical Imaging and Bioengineering

11:15-11:45am **Role of RBC eNOS in Optimizing RBC Phenotype During Circulatory Transit**
Allan Doctor, MD
University of Maryland School of Medicine

11:45-12:45pm LUNCH

SESSION THREE: COMPLEXITIES OF THE COMMON COMPLICATIONS IN SCD

Chair: Zachary Ramsay, MBBS, MSc

12:45-1:15pm **Insomnia and Sleep Health**
Monika Haack, PhD
Harvard University

1:15-1:45pm **Pain: Many Mechanisms and Mysteries**
Cheryl Stucky, PhD
Medical College of Wisconsin

1:45-2:15pm **Managing Pain Beyond Opioids: Cannabinoids, Buprenorphine, Ketamine**
Deepika Darbari, MD, MS
Children's National Hospital

**Virtual*

SESSION FOUR: MECHANISM-BASED THERAPIES FOR HEMOGLOBINOPATHIES

Chair: Haydar Frangoul, MD, MS

2:15-2:35pm	**Overview** *Daniel E. Bauer, MD, PhD *Harvard Medical School & Boston Children's Hospital*
2:35-3:05pm	**Induction of Fetal Hemoglobin: Genetic Approaches** Haydar Frangoul, MD, MS *Children's Hospital at TriStar Centennial*
3:05-3:35pm	**Induction of Fetal Hemoglobin: Small Molecules and Pharmacological Approaches** Scott Peslak, MD, PhD *University of Pennsylvania*
3:35-3:45pm	BREAK
3:45-4:15pm	**Increase Anti-Sickling Potential- Genetic Approaches** John Tisdale, MD *National Heart, Lung, and Blood Institute*
4:15-4:45pm	**Increase Anti-Sickling Potential- Pharmacological Approaches** Swee Lay Thein, MD *National Heart, Lung, and Blood Institute*
4:45-5:15pm	**Emergence of Genetic Therapies for SCD: A Curative Treatment or Treatment with Curative Intent?** *Edward Benz Jr., MD *Dana-Farber Cancer Institute*
5:15-5:25pm	Discussion and Questions
5:25-5:30pm	Day One Closing Remarks
5:30pm	End of Day One

**Virtual*

AGENDA

Day Two, FRIDAY, SEPTEMBER 20, 2024

8:00am – Registration Check In

8:30am – Opening remarks from Prof. Marshall Tulloch-Reid, Director, CAIHR, Jamaica

SESSION ONE : HYDROXYUREA- A THERAPEUTIC STALWART IN SCD
Chair: Lesley King, MBBS, MRCP

8:40-9:10am **Revisiting Hydroxyurea Use in SCD (dosing, use in pregnancy, etc.,)**
Russell Ware, MD, PhD
Cincinnati Children's Hospital

9:10-9:40am **Improving Hydroxyurea Adherence: Understanding Barriers and Enablers**
Monika Asnani, MBBS, DM, PhD
The University of the West Indies

9:40-10:05am **Clinical and Hematopoietic Profiles Associated with Sustained Response**
Luisanna Sanchez Ventura, MD
Indiana Hemophilia and Thrombosis Center

10:05-10:35am **Re-examining the Utility of Absolute Neutrophil Count as MTD in the Context of *Duffy* Status**
Lauren Merz, MD, MSc
Brigham and Women's Hospital

10:35-10:45am BREAK

**Virtual*

SESSION TWO: ONGOING CHALLENGES IN THE MANAGEMENT OF SCD COMPLICATIONS

Chair: Angela Rankine-Mullings, MBBS, MRCP

10:45-11:25am **Fertility and Pregnancy**
*Lydia Pecker, MD, MHS
Johns Hopkins University

11:25-11:50am **Cardiac Disease**
Thomas d'Humières, MD, PhD
Henri Mondor Hospital, Assistance Publique Hôpitaux de Paris

11:50-12:15pm **Renal Disease**
Lori-Ann Fisher, MD
The University of the West Indies

12:15-12:40pm **Alloimmunization and Hyperhemolysis**
France Pirenne, MD, PhD
University Paris Est Créteil

12:40-1:40pm LUNCH

SESSION THREE: CURATIVE THERAPIES

Chair: John Tisdale, MD

1:40-2:05pm **The 5 Most Frequently Asked Questions**
Wynona Coles, MPH, CCRP
National Heart, Lung, and Blood Institute

HOW WE MEASURE THERAPEUTIC EFFICACY IN SCD

2:05-2:30pm **Improvements in Health-Related Quality of Life (to Include Pain)**
*Julie Kanter, MD
The University of Alabama at Birmingham Medicine

2:30-2:55pm **Cerebral Hemodynamics**
Melanie Fields, MD, MSCI
Washington University- St. Louis

2:55-3:20pm **Respiratory Revelations: Lung Function Post-Transplant in Sickle Cell Disease**
Parker Ruhl, MD, MHS
National Institute of Allergy and Infectious Diseases

3:20-3:30pm BREAK

**Virtual*

SESSION FOUR: MUTATIONAL/VARIANT SCREENING TO IMPROVE OUTCOME OF CURATIVE THERAPIES IN SCD

Chair: Swee Lay Thein, MD

3:30-3:55pm **Screening for Clonal Hematopoiesis**
Lachelle Weeks, MD, PhD
Dana-Farber Cancer Institute

3:55-4:20pm **Screening for Alpha Thalassemia**
John Pierciey, MSc
Bluebird Bio, Inc.

PANEL: WHERE ARE WE HEADED IN THE TREATMENT OF SCD?

Chair: Jennifer Knight-Madden, MBBS, PhD

4:20-4:40pm **Curative Therapies**
Mark Walters, MD
University of California, San Francisco

4:40-5:00pm **More Disease-Modifying Small Molecules (to include update on current therapies)**
Jane Little, MD
The University of North Carolina at Chapel Hill

Marilyn Telen, MD
Duke University School of Medicine

5:00-5:15pm **Perspective from Patients**

Ms. Morette Wright
Sickle Cell Support Foundation of Jamaica
Member, Sickle Cell Technical Working Group (SCTWG)

Mr. Patrick Onwuemene
NIH Patient

5:15-5:30pm Panel Discussion

5:30pm Conference Closing Remarks

SPEAKER BIOGRAPHIES

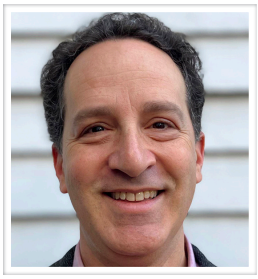
Monika Asnani, MBBS, DM, PhD

Monika Asnani 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 20 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; (ii) sickle nephropathy & iii) Maternal mortality in SCD . 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 continues to examine pain phenotypes in SCD including neuropathic pain and is currently leading a study examining barriers to hydroxyurea uptake in Jamaica. She is member of the WHO Guidelines Development Group currently writing guidelines for the management of pregnancy in sickle cell disease. She is also working with the PhenX team to develop standard measurement tools for use in biomedical research in pregnancy in sickle cell disease.

She is a graduate of the UWI and was awarded the M.B.B.S. 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.

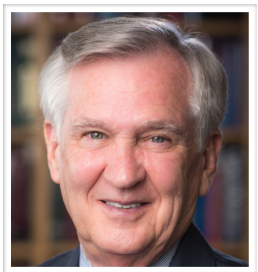
At the UWI, She is also a supervisor, examiner and member of Specialty Board in the Family Medicine Program. She is a member of the Caribbean College of Family Physician having served as its honorary secretary for a decade in the past. She also serves as a board member of the Caribbean Researchers in Sickle Cell Disease & Thalassemia (CAREST) group.

Daniel E. Bauer, MD, PhD



Daniel Bauer is a physician-scientist whose research focuses on functional genomics to dissect determinants of blood cell development and disease and develop innovative therapies. He discovered the gene editing approach targeting the *BCL11A* erythroid enhancer that was developed as exa-cel, now approved for sickle cell disease and β-thalassemia treatment. He has described mechanisms of fetal hemoglobin (HbF) repression, demonstrated how human genetic diversity alters therapeutic gene editing outcomes, and uncovered how the hematopoietic stem cell state constrains the efficiency and genotoxicity of nuclease, base and prime editing. He has sponsored investigator-initiated clinical trials of therapeutic gene editing. His clinical work focuses on non-malignant hematology. He is Donald S. Fredrickson, MD Associate Professor of Pediatrics at Harvard Medical School and Director of the Gene Therapy Program at Boston Children's Hospital.

Edward J. Benz Jr., MD



Dr. Benz graduated from Harvard Medical School in 1973 and received his training at Brigham and Women's Hospital and the National Institutes of Health. He is president and CEO emeritus of Dana-Farber Cancer Institute, director emeritus and principal investigator of Dana-Farber/Harvard Cancer Center, and a member of the Governing Board of Dana-Farber/Children's Cancer Center. He is also a clinical hematologist and an active NIH-funded investigator.

Wynona Coles, MPH, CCRP

Wynona Coles currently serves as Senior Technical Lab Manager for the Cellular and Molecular Therapeutics and Molecular and Clinical Therapeutics Branches within the National Heart Lung and Blood Institute (NHLBI) of the National Institutes of Health (NIH). She obtained her Associate Degree in Respiratory Care from Shenandoah University in 1993 and first started working as a Registered Respiratory Therapist for the Critical Care Medicine Department in the NIH Clinical Center in 1997. Ms. Coles furthered her education to include a Bachelor of Science in Clinical Research Administration from The George Washington University in 2005 and a Master of Science in Public Health from American Public University in 2010. Her career focus shifted from respiratory care to clinical research and for over 20 years, Ms. Coles has served as lead study coordinator for multiple therapeutic and non-therapeutic clinical trials in sickle cell disease research across multiple institutes and branches of the NIH. Her current role as transplant referral coordinator focuses on patient recruitment, education, and eligibility screening for transplant recipients and donors who may be eligible for enrollment onto a sickle cell transplant clinical trial within the NHLBI. Wynona enjoys reading, hiking, and spending time with family and friends.

Raffaella Colombatti, MD, PhD

Prof. Colombatti is pediatric hematologist-oncologist at the Pediatric Hematology Oncology Unit of the Azienda Ospedale -University of Padova. She is the co-founder and current director of the Veneto Region Reference Center for the Diagnosis, Treatment and Care of Sickle Cell Disease (SCD) in Childhood and the coordinator of the Red Blood Cell Disorders Subnetwork for the European Reference Network (ERN)-EuroBloodnet at the Azienda Ospedale – University of Padova (AOPD). She is member of the Scientific Committee of the Functional Department of Rare Diseases of AOPD, member of the Transition working Group of AOPD and process owner of the Transition process for the Transition of Adolescents with chronic and rare disorders into adulthood.

She is Associate Professor of Pediatrics at the University of Padova.

Since 2018 she is the Coordinator of the Red Cell Disorder Working Group of the Italian Association of Pediatric Hematology Oncology (AIEOP) which developed the first Italian National Guidelines for the Management of SCD in Childhood in Italy and promotes national studies in the field of red blood cell disorders.

Prof. Colombatti is PI in many research projects and clinical trials in SCD -with a specific interest in neurological and neurocognitive complications-, including a Natural History Study since 2007; she is involved in several European and International projects in SCD.

She has been working to strengthen the collaboration between European, American and African centers through, among others, the ERN on Rare Hematological Disorders (EuroBloodNet), the Medical and Research Advisory Committee (MARAC) of the SCD Association of America (SCDAA), the Marie Skłodowska-Curie Actions (MSCA) Research and Innovation Staff Exchange (RISE) H2020 Project "African Research and Innovative Initiative for Sickle cell Education: Improving Research Capacity for Service Improvement" and the organization of the Academy for Sickle Cell and Thalassaemia (ASCAT) annual meeting. She is also involved in projects regarding child health in European and African Countries, in collaboration with NGOs and international institutions. She is part of EU Horizon 2020 and Horizon Europe projects regarding Artificial Intelligence in Hematology (GenoMed4all and Synthema). She is member of the Steering Committee and Data Access Committee of the European platforms ENROL and RADEEP, related to the ERN EuroBloodNet. She is member of the European Affairs Committee and the Adolescents and Young Adults Task force of the European Haematology Association (EHA).

Thomas d'Humières, MD, PhD

I'm a French cardiologist and physiologist working at Henri Mondor teaching hospital (Créteil, France). Since 2018 and in close collaboration with the Sickle Cell National Referral Center (Pr. Bartolucci), I have developed a care and research program (DREPACOEUR registry) dedicated to the cardiac complications of red blood cell disorders, and sickle cell disease (SCD) in particular. After a PhD in cellular biology and a current post-doc (PARCC, Paris, Pr Tharaux's team), I'm trying to build an integrative, translational and systemic approach to cardiovascular disorders affecting SCD patients, from bench to bedside.

Deepika Darbari, M.B.B.S., M.S.

Dr. Darbari is Pediatric Hematologist-Oncologist at Children's National Hospital and the Professor of Pediatrics at the George Washington University 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 with the American Pain Society and more recently development of PhenX Toolkit for pain in sickle cell disease. As an active participant of diversity, equity, and inclusion efforts at Children's National and the American Society of Hematology (ASH), she is also committed to promoting diversity in medicine and hematology specially as it relates to trainees from underrepresented in medicine background. Her efforts have been recognized by the 2021 ASH Honorific Award for Leadership in Promoting Diversity.

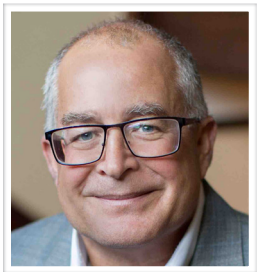
Daniel Dexter, MD

Dr Daniel Dexter is training in pediatric hematology, and is currently a hematology registrar at Kings College Hospital, London, UK. His experiences providing care for children with sickle cell disease informed his decision to pursue sub-specialty training in pediatric hematology. He also performs research, with an interest in widening access to screening and comprehensive care for sickle cell disease globally.

Previously, Daniel has researched the epidemiology of sickle cell disease at the Central Public Health Laboratory in Kampala, Uganda. He is currently leading on a UK national audit of vascular access for those patients receiving elective red cell exchange transfusions. He is also clinical advisor for the NHS Race & Health Observatory on the Sickle Cell Comparative Research to Inform Policy.

He has published several papers, including a review in the British Journal of Haematology on point-of care-testing in sickle cell disease.

Allan Doctor, MD



Dr. Allan Doctor is Professor of Pediatrics and Bioengineering at the University of Maryland School of Medicine (UMSOM). He is a Pediatric Intensivist and previously led Pediatric Critical Care at Washington University and St Louis Children's Hospital for 10 years, before transitioning to UMSOM to focus on a rapidly expanding research program and on development of a novel bio-synthetic artificial red cell (ErythroMer). He is a co-Founder and Chief Scientific officer for KaloCyte, which is developing ErythroMer for commercial use. His laboratory studies the role of red blood cell-based signaling in the control of regional blood flow, related pathophysiology arising from acquired red cell injuries, blood substitute design, and on translational transfusion medicine in critical illness. Dr. Doctor has led two translational research networks: NIH/NICHD Collaborative Pediatric Critical Care Research Network and BloodNet, an international group that pursues questions common to Transfusion & Critical Care Medicine. Currently, he directs the University of Maryland Center for Blood Oxygen Transport and Hemostasis (CBOTH), which addresses fundamental, challenging questions related to O2 transport and

hemostasis from a systems perspective, and is the Principle Investigator for CONCERT: the US DoD/ DARPA funded Consortium for Optimized Integration of Bio-artificial Blood Components for Adaptive Resuscitation.

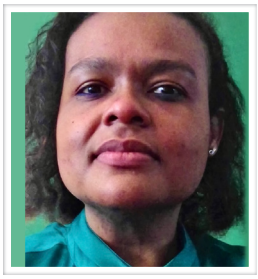
Melanie Erin Fields, MD, MSCI

I am a board-certified pediatric hematologist with the long-term goal of using advanced neuroimaging to define the impact of sickle cell anemia on neurologic and cognitive development in children.

Through the support of my K12 award (K12 HL087107), I used MR measurements of cerebral blood flow and oxygen extraction fraction to improve our understanding of stroke pathophysiology in pediatric sickle cell disease patients. We found that children with sickle cell disease have globally increased cerebral blood flow and oxygen extraction fraction when compared to healthy siblings without sickle cell disease, and that the region of greatest oxygen extraction falls within the border zone of the brain, co-localizing with the region of the brain at greatest risk for stroke. Additionally, we have found that primary disease modification with chronic transfusion therapy and hydroxyurea decreases ongoing metabolic stress in the brains of children with sickle cell disease, as measured by oxygen extraction fraction. I presented the results of our research as a poster at the American Society of Hematology's annual meeting in 2014 and platform oral presentations at the American Society of Hematology's annual meetings in 2015, 2016 and 2017, and published these results in Blood (2018, 2019) and Neurology (2018).

I expanded the scope of my research to explore the effects of sickle cell disease on cognition through my ASH Fellow Scholar and K23 awards (K23HL136904). In a prospective imaging study of children with sickle cell disease and unaffected, healthy siblings without significant differences in cognitive testing, we found diminished functional connectivity within specific, anatomically-contiguous and clustered networks in children with sickle cell disease compared to controls. Additionally, increased metabolic stress, as measured by oxygen extraction fraction, was selectively associated with decreased connectivity within specific networks. These data suggest that elevated oxygen extraction and disrupted functional connectivity are potentially pre-clinical neuroimaging biomarkers for cognitive decline in sickle cell disease. These data were presented at the American Society of Hematology's annual meeting in 2019, and were published in the Annals of Neurology (2020). Currently, I am investigating the intersection of metabolic stress with structural and functional connectivity in sickle cell disease (R01HL157188).

Lori-Ann Fisher, MD



Dr Lori-Ann Fisher is a Consultant Nephrologist at the University Hospital of the West Indies and an Honorary Lecturer in the Epidemiology Research Unit of the Caribbean Institute of research (CAIHR).After completing residency and chief residency in Internal Medicine at Medstar Good Samaritan Hospital, she pursued a clinical fellowship at Johns Hopkins Hospital in Baltimore in Nephrology and a critical care fellowship at Montefiore, Medical Centre in New York. She returned to Jamaica subsequent to training as a consultant nephrologist and intensivist at the University Hospital of the West Indies. She is currently the deputy chair of the North America and Caribbean board of the International society of nephrologists (ISN) and is a member of the ISN council. Her research interests include the epidemiology of kidney disease and its risk factors in the Caribbean, as well as sickle cell nephropathy and has published on Acute Kidney Injury and Chronic Kidney Disease in Jamaica.

Haydar Frangoul, MD, MS

Haydar Frangoul is the Director of the Pediatric Stem Cell Transplant program at the Sarah Cannon Research Institute and Tristar Centennial Children's Hospital in Nashville Tennessee. Dr Frangoul completed his MD degree at the American University of Beirut followed by Pediatric residency at Duke University. He then went to complete a fellowship in Pediatric Hematology/ Oncology and Stem Cell Transplant at the University of Washington and the Fred Hutchinson Cancer Center. He was the Carolyn Perot Rathjen Endowed Professor of Pediatric Hematology/Oncology at Vanderbilt University until 2015 when he joined Sarah Cannon Research Institute to lead the Pediatric Stem Cell Transplant Program.

Dr Frangoul had leadership positions in the Children Oncology group, and Pediatric Blood and Marrow Transplant Consortium. His primary interest is allogeneic stem cell transplant using an alternative donor sources especially for non-malignant diseases. Dr Frangoul has authored or co-authored more than 140 peer reviewed manuscripts. He has been an invited speaker at numerous national and international conferences. He is the lead investigator for the clinical trial using CRISPR-Cas9 gene editing for patients with sickle cell disease.

Monika Haack, PhD

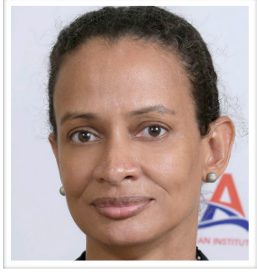
Dr. Monika Haack is an Associate Professor of Neurology at Harvard Medical School and leads the Sleep & Pain Laboratory at Beth Israel Deaconess Medical Center in Boston. Her research goals are directed at discovering the mechanisms through which sleep disturbances increase disease risk in humans, in particular chronic pain. Over the last fifteen years, she as developed an interdisciplinary research program linking sleep disturbances with inflammatory, neuroendocrine, and central pain modulatory networks. Her team is testing the impact of sleep disturbances on health through complex laboratory models that are representative of sleep problems in many medical conditions, expecially in those characterized by chronic pain. Her group also studies the role of sleep disturbances on inflammation, pain, and well-being in patients suffering from insomnia disorder or Long COVID. Dr. Haack is a Preceptor of the Sleep, Circadian, and Respiratory Neurobiology T32 program in the Division of Sleep Medicine at Harvard Medical School and is continously mentoring students joining her research group through various national and international programs.

Julie Kanter, MD

Julie Kanter is Professor of Medicine and Pediatrics at the University of Alabama at Birmingham School of Medicine. She leads the adult sickle cell disease program and co-leads the Lifespan Comprehensive Sickle Cell Center. She has two main research focuses: advancing novel therapies through improved understanding of biology and clinical endpoints and using dissemination and implementation science to improve access to care in sickle cell disease.

Dr. Kanter has received funding from HRSA, CDC and the NIH/NHLBI including two R01 grants to improve implementation and dissemination in sickle cell disease. Currently, Dr. Kanter is also the president of the National Alliance of Sickle Cell Centers (NASCC), the first network of sickle cell centers dedicated to reducing barriers and improve access to quality care for people living with sickle cell disease.

Lesley King, MBBS, MRCP



Dr. Lesley King is a paediatrician and Head, Clinical Services at Caribbean Institute for Health Research (CAIHR) with 20-plus years of experience in clinical care for persons living with sickle cell disease (SCD). She received her MB.BS from University of the West Indies in 1992. She later trained at the Royal Hospital for Sick Children, Yorkhill, Glasgow and its neighbouring hospitals, and was awarded the MRCP (UK) in 1997. She returned to Jamaica in 2000 to work as a paediatrician at the Sickle Cell Unit, TMRI.

Her research areas are focused on aspects of clinical care, including newborn screening (NBS) and hydroxyurea (HU) use in SCD. She is a member of the Sickle Cell Technical Working Group, commissioned by the Ministry of Health and Wellness (MOHW), Jamaica in 2012 to implement island-wide NBS and now focused on improving access and standard of care for persons living with SCD. She leads the Capacity Building for Health Care Workers sub-group, its focus being training activities to improve and standardize clinical care across the island. She also serves on the National Health Fund Expert Panel of Physicians with the responsibility to provide a clinical opinion regarding drugs under review related to sickle cell disease.

She has co-authored papers on NBS, Hydroxuyrea, renal and vasculopathy disorders in SCD.

Memberships include:

1. Medical Association of Jamaica
2. Paediatric Association of Jamaica
3. Royal College of Paediatrics and Child Health (UK)
4. CAREST

In her spare time, she tinkers with orchids and enjoys road cycling with friends.

Jennifer Knight-Madden, MBBS, FRCP-C, PhD

Professor Jennifer Knight-Madden was the Director of the Sickle Cell Unit, the Caribbean Institute for Health Research, The University of the West Indies (2014-2021). 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; 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; 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 (Chair). 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. She is a member of the Christian Life Fellowship, where she is in Children's Ministry.

Wilbur A. Lam, MD, PhD

Wilbur A. Lam is a physician-scientist-engineer who is the W. Paul Bowers Research Chair and Professor (with tenure) of Pediatrics and Biomedical Engineering at Emory University and Georgia Institute of Technology and Associate Dean of Innovation at the Emory University School of Medicine as well as a physician at the Aflac Cancer and Blood Disorders Center of Children's Healthcare of Atlanta, where he also serves as Chief Innovation Officer of the Pediatric Technology Center. Dr. Lam obtained his B.A. from Rice University, his M.D. from Baylor College of Medicine, and his bioengineering Ph.D. from the University of California, Berkeley and University of California, San Francisco, where he also completed his clinical training in pediatrics and pediatric hematology/oncology. Dr. Lam's interdisciplinary laboratory serves as a unique "one-stop shop" in which they develop microsystems- and smartphone-based platforms to study and diagnose pediatric and blood diseases and then directly translate those technologies to his patients. His lab's basic science interests involve developing and leveraging microfluidic devices as *in vitro* models of hematologic diseases, including sickle cell disease and bleeding/clotting disorders. With an interest in home-based and smartphone-based diagnostics, the Lam Lab is also dedicated to further developing their technologies as novel "cheap tech" solutions to enable and empower pediatric patients to more easily monitor their own diseases at home and in the global health and rural settings. Dr. Lam has also co-founded and serves/served as chief medical officer for three medical device startups based on his laboratory's research. In addition, Dr. Lam is a principal investigator of the Atlanta Center for Microsystems Engineered Point-Of-Care Technologies (ACME POCT), which is part of the NIH's Point-of-Care Technologies Research Network and currently serves as the Test Verification Core of the NIH RADx initiative for COVID-19 diagnostic testing. Among other honors, Dr. Lam has been elected into the National Academy of Medicine and the American Society of Clinical Investigation, is a Fellow of the American Institute for Medical and Biological Engineering and the National Academy of Inventors, is a Kavli Fellow of the National Academy of Sciences, named an Emerging Investigator by the journal *Lab-on-a-Chip* published by the Royal Society of Chemistry, and is recipient of an NSF Career Award as well as the American Society of Pediatric Hematology/Oncology's Frank A. Oski Memorial Lectureship Award, the Lab on a Chip and Dolomite Pioneers of Miniaturization Lectureship Award, and an Emerging Investigator Award by the NHLBI of the NIH. His laboratory's research is funded by the NIH, NSF, FDA, the American Heart Association, the Coulter Foundation, the Department of Defense, and the Georgia Research Alliance.

Jane Little, MD

Jane Little MD, is a Professor of Medicine at UNC-Chapel Hill and Directs the Adult Sickle Cell Disease Program there. She is interested in prospective registries, such as GRNDaD, through which to examine the manifestations of disease over time in sickle cell disease, and in occult or night-time hypoxemia. She is committed at all times to supporting community in sickle cell disease.

Lauren Merz, MD, MSC

Dr. Lauren Merz obtained an MD from the University of Michigan Medical School and a MSc in Clinical Research from the University of Michigan School of Public Health. She then completed Internal Medicine residency at Brigham and Women's Hospital, and is currently a fellow in hematology/oncology at Dana-Farber Cancer Institute/Mass General Brigham. Her research interests include healthy equity in classical hematology with a focus on women's health and the Duffy null phenotype, a common variant mostly seen in those of African descent that results in lower circulating neutrophil counts. While in residency, she studied the impact of sickle cell trait on outcomes after COVID-19 infection and developed a "heme team" embedded into a general medicine team to improve resident education and outcomes for patients with sickle cell disease. She is currently designing a multi-institute pragmatic trial investigating VTE prophylaxis in postpartum patients with sickle cell disease. Her work on the Duffy null phenotype has resulted in an update in the terminology used to describe this variant, a Choosing Wisely recommendation, and new ICD-10 codes for Duffy phenotypes. She also established Duffy-null specific ANC reference ranges while a resident at Brigham and Women's Hospital, and is currently the principle investigator Doris Duke Foundation Grant through the American Society of Hematology to accelerate this work across the United States.

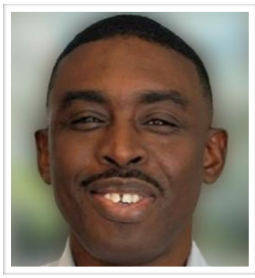
Obiageli Nnodu, MD, MPH, FAAFP

Obiageli Nnodu is Professor of Haematology and Blood Transfusion, Director, Centre of Excellence for Sickle Cell Disease Research and Training, University of Abuja (CESRTA), National Coordinator, American Society of Hematology Consortium for Newborn Screening in Africa (CONSA), the Nigerian Principal Investigator for SPARCO, Abuja Coordinator for SickleGenAfrica, Co-PI for the UK NIHR Patient centred management of sickle cell disease in Sub Saharan Africa, MPI for mAnaging siCkle CELL diseasE through incReased AdopTion of hydroxyurEa in Nigeria (ACCELERATE) and Contact PI for SPARC-TRAIN.

Prof Nnodu, a UK Foreign Office Chevening Scholar, has carried out many multi-institutional, multi-national research projects including a multilateral DFID DeLPHE Project. She serves as expert on

technical committees on non-communicable diseases with the Nigerian government and as consultant for international agencies including the WHO AFRO. Between 2018-2019, she carried out an assessment of the level of implementation of the WHO AFRO Strategy in SCD in sub-Saharan Africa high-burden countries and the findings highlighted the need for increased global funding for SCD. She recently collaborated with AFRO in articulating strategic pathways for the development of gene therapy as cure for SCD in Africa. Prof Nnodu is a Fellow of the Nigerian Academy of Medicine and Chairs the largest SCD network in Africa i.e., the Sickle Cell Support Society of Nigeria. She led the section on Screening and Prevention in the Lancet Haematology Commission on Sickle Cell Disease and has been appointed the Special Adviser to the Coordinating Minister of Health and Social Services on Sickle Cell Disease.

Mr. Patrick Onwuemene



I am a patient at NIH Clinical Center, Bethesda, Maryland under the care of Dr. Swee Lay Thein. I've dealt with sickle cell disease all my life. As a young adult till the point I was accepted into the experimental therapy called mitapivat (AG-348), it seemed to be a losing battle on my end. Growing up as a child with four very athletic brothers and neighbors as competitive as they were, I had a drive to play soccer, tennis, and other track and field activities. Becoming an adolescent, I felt so left behind, watching my brothers and friends develop and having fun while I had to deal with sickle cell disease. I began experiencing the effects of sickle cell. Among these were fatigue, severe joint pains, swelling of the joints, jaundice, and stunted growth. Occasionally, I would have a full blown crisis that would last for hours at a time during the cold months we call harmattan in west Africa.

Eventually, my parents brought my siblings and I to the United States, where I received better care for my blood disorder. Even with the close care I got from my Oncologist, I fell into crisis more often due to the much colder weather experienced in the mid-eastern United States.

My saving grace came when I saw on 60 Minutes, a news magazine tv program, a profile of a young lady by the name of Jenelle Stevenson who was experimented on and "cured" of sickle cell disease. I began searching out health clinics that offered such experiments till I landed on an NIH website sometime in October of 2019. Since been in the program, my health has come a long way. It's like coming from the edge of death to living a life full of vigor and I have God and the clinical trial team at NIH to thank.

Lydia H. Pecker, MD, MHS

Dr. Lydia H. Pecker is an Associate Professor of Medicine at Johns Hopkins University. She earned her Bachelors degree with honors in Africana Studies from Brown University, MD from The University of Pennsylvania Medical School and MHS from the Johns Hopkins Bloomberg School of Public Health. Her NIH and foundation-funded research focuses on reproductive health in girls and women with SCD, particularly defining and mitigating infertility risks and management of high risk pregnancy. Dr. Pecker is the Interim Director at

the Sickle Cell Center for Adults at Johns Hopkins where she founded the Young Adult Clinic. She is a member of the American Society of Hematology Maternal Health Committee, Foundation for Women and Girls with Blood Disorders' Medical Advisory Committee, and a co-Founder of the Sickle Cell Reproductive Health Education Directive.

Scott Peslak, MD, PhD

Dr. Scott Peslak graduated from the University of Scranton in 2006 with a double major in Biochemistry and Philosophy. He received his MD/PhD from the University of Rochester School of Medicine in 2014 and trained in the laboratory of Dr. James Palis studying injury and recovery of the red blood cell lineage and mechanisms of stress erythropoiesis. He subsequently completed his Internal Medicine Residency and Hematology/Oncology Fellowship at the Hospital of the University of Pennsylvania in 2021, during which he completed his postdoctoral work in the laboratory of Dr. Gerd Blobel at the Children's Hospital of Philadelphia studying cellular signaling pathways in the regulation of fetal hemoglobin for treatment of sickle cell disease. He is a physician-scientist and Assistant Professor of Medicine at the University of Pennsylvania, and cares for patients in the UPenn Comprehensive Sickle Cell Program and Comprehensive Adult Thalassemia Program. In addition to his clinical focus on red cell disorders, his laboratory at the University of Pennsylvania studies novel regulators of fetal hemoglobin and new genetic and pharmacologic therapies for the treatment of sickle cell disease and beta-thalassemia.

John Pierciey, MSc

John Pierciey is currently the head of research at bluebird bio. Over the past thirteen years, he has led the process development and characterization of gene therapies for the treatment of rare diseases, with an emphasis on hemoglobinopathies such as β-thalassemia and sickle cell disease (SCD). His expertise and research interests include stem cell mobilization, cell collection & processing, hematopoiesis/erythropoiesis, and exploratory clinical assay development. SCD research at bluebird bio is currently focused on understanding how the pathophysiology of SCD impacts the manufacture of autologous gene therapy products and how genotypic variation impacts gene therapy outcomes.

France Pirenne, MD, PhD

France Pirenne is MD, and professor of Hematology/Transfusion at the University Paris-Est-Créteil, France. First, she did research on transplant immunology at Columbia University, USA, then served as a medical doctor at the Blood Group National Reference Center in Paris, developing the first blood group molecular laboratory in France. She has been the medical director of the French Blood Agency (EFS) of the Parisian area, based in the H.Mondor Hospital

(Créteil), working closely with the SCD Reference Center (3000 patients). At her current position at the EFS, she is deputy director of the International Affairs. She also run an INSERM research team, working on haemolytic transfusion reaction in SCD patients. She is the President of the French Society of Blood Transfusion.

Manu Platt, PhD

Dr. Manu Platt became the inaugural director of the NIH-wide Center for Biomedical Engineering Technology Acceleration (BETA Center) housed within NIBIB, as a new NIH campus model for accelerating technology-driven interdisciplinary research and clinical translation and to bring engineering, clinicians, and basic scientists together in February 2023,. Dr. Manu Platt earned his B.S. in Biology from Morehouse College and Ph.D. from Georgia Tech/Emory in Biomedical Engineering. After a postdoc at MIT, he returned to Georgia Tech/Emory's joint department as an Assistant Professor where he worked up to promotion to full Professor. His research program focuses on proteolytic mechanisms of disease, translational approaches to reduce strokes in people affected by sickle cell disease, and harnessing proteolytic networks and systems biology tools to predict disease progression. Among other awards, Dr. Platt was awarded the Biomedical Engineering Society Diversity Award, is a Fellow of American Institute for Medical and Biological Engineering (AIMBE), Fellow of Biomedical Engineering Society, the Root 100 in 2019, and AAAS Mentor Award in 2021.

Zachary Ramsay, MD, MSc

Dr. Zachary Ramsay is a medical doctor and epidemiologist who has been working at the Sickle Cell Unit since 2017. His initial roles were as a study physician and sub-investigator on the Voxelotor, HUPK and EXTEND trials. In 2019, he conceptualized and conducted research on important studies assessing neuropathic pain and Universal healthcare among patients with sickle cell disease. His work on neuropathic pain was the subject of his MSc in Epidemiology that he completed in 2020, with Distinction.

In 2021, he began working as a Lecturer teaching in undergraduate and graduate Epidemiology courses. During this period, he was also awarded the Annual Research Award from the Foundation for Women & Girls with Blood Disorders (FWGBD) to assess the relationship between menstrual cycles, female hormones and pain among women with sickle cell disease. Subsequently, he was awarded a Chevening scholarship to study a MSc in Health Informatics at King's College London. This will include learning important skills in machine learning, artificial intelligence, bioinformatics (interpretation and data quality in genome analysis), spatial epidemiology and prediction modelling. His future PhD work will employ these skills to model and predict sickle cell pain.

Angela Rankine-Mullings, MBBS, MRCP

Dr Angela Rankine-Mullings is a graduate of the University of the West Indies. She is a Pediatrician and Senior Research Fellow at the Sickle Cell Unit, Caribbean Institute for Health Research. Dr Rankine-Mullings is an experienced researcher and has 13 years of experience in investigator led research and clinical trials and has a notable number of first author publications. She has generated over 1.5 million United States Dollars in Research funding.

Her research focuses on stroke prevention in children with sickle cell disease and hydroxyurea management. Dr Rankine-Mullings is in her final year of her PhD work that addresses questions about arterial stiffness and Transcranial Doppler defined stroke categories in children with sickle cell anemia. She has been at the forefront of clinical trials in hydroxyurea since being employed to CAIHR, including the NIH sponsored, SCATE (Sparing Conversion to Abnormal Transcranial Doppler velocity Elevation) trial (NCT01531387), the EXTEND trial (NCT02556099), in collaboration with Cincinnati Children's Hospital Medical Centre, and was Chief Investigator and local Principal Investigator for the clinical trial ''A prospective open label, pharmacokinetic study of an oral hydroxyurea solution in children with sickle cell anaemia'' (NCT03763656) all of which have advanced the use of hydroxyurea as non-transfusion disease modifying therapy.

Dr Rankine-Mullings is a Member of the Royal College of Pediatrics and Child Health (MRCPCH), Associate Member of the American Society of Hematology (ASH), a patron of the Organization of Sickle Cell Research (OSCAR), Bristol. She currently holds full registration with the General Medical Council with a license to practice having worked in the United Kingdom previously with additional international work experience in Belize, Central America and Grenada.

A. Parker Ruhl, MD, MHS

Dr. Ruhl 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.

Luisanna M. Sanchez Ventura, MD

I received my education and initial clinical experience in a country where the prevalence of sickle cell disease (SCD) is four times greater than in the United States. Witnessing many patients with SCD suffer from often treatable complications motivated me to find ways to improve healthcare systems and reduce health disparities. As a result of my personal and training experiences, improving access to early screening and treatment therapies for hematologic disorders has become one of my long-term career goals. Under the mentorship of international leaders in pediatric hematology, I gained extensive clinical experience while broadening my scholarly pursuits and deepening my interest in clinical research. I am part of several national committees and have collaborated with other researchers, producing numerous peer-reviewed publications from various projects. As the principal investigator of this current study, my goal is to understand the clinical and molecular profiles of children with SCD associated with robust and sustained induction of hemoglobin F in response to hydroxyurea treatment. I am dedicated to uncovering the marked variability in hydroxyurea response among patients, thereby facilitating a more personalized treatment approach for both pediatric and adult individuals with SCD.

Ana Cristina Silva-Pinto, MD, PhD

Dr. Silva Pinto completed her medical degree in 1999 and a PhD in Clinical Medicine, Hematology in 2011. She's currently the coordinator of the Sickle Cell Disease Program at General Hospital of the Ribeirão Preto Medical School, University of São Paulo and member of the Advisory Committee for Sickle Cell Disease of the Brazilian Ministry of Health.

Cheryl Stucky, PhD

The Stucky lab studies neuronal and non-neuronal cells' contributions to pain in various diseases. Our NIH funded research projects are focused on: 1) understanding pain mechanisms in a lysosomal disorder called Fabry disease. 2) describing mechanisms that lead to steady state and episodic pain associated to Sickle Cell Disease. 3) determining how non-neuronal cells, such as keratinocytes and Schwann cells, contribute to the development on chronic pain associated with a variety of diseases, such as chemotherapy-induced neuropathy and traumatic nerve injury.

To address these questions, the Stucky lab uses a multi-disciplinary approach including : 1) evoked and non-evoked sensory behavior assays; 2) electrophysiology including whole cell, single channel patch clamp, and teased fiber recordings: 3) *in vivo* and *in vitro* calcium imaging; 4) tissue specific opto- and chemogenetic interventions; and 5) molecular techniques including qPCR, immunohistochemistry, and

RNAscope. Our work in rodent models is guided by human research, and human samples are used to complement our studies when possible.

Marilyn J. Telen, MD

Dr. Telen is an international authority on SCD and other hematologic disorders, as well as on blood group antigens and erythrocyte alloimmunization. Her scientific vision has contributed to the evaluation of new methodologies and provided fresh ideas for improving critical areas of health care research, especially relating to transfusion medicine, SCD, and other hemoglobinopathies. Her early work was responsible for the identification of the biochemical and genetic basis of five blood group antigen systems. Her current basic and clinical research explores the role of red cell adhesion molecules in sickle cell disease (SCD) and downstream pathogenetic pathways, including inflammation, through both experimental science as well as genetic and clinical translational research in SCD and transfusion medicine. Since 2001 she has conducted genetic and multi-omic work aimed at understanding the role of genetic polymorphisms in the variability of SCD and at identifying the genetic and 'omic mechanisms contributing to clinical sequelae of SCD. In addition, she has participated in and led both early and late phase clinical trials in SCD and has developed methods for risk stratification for end-organ damage in SCD. She served as Chief of Hematology at Duke University for >17 years and as Director of the Duke Comprehensive Sickle Cell Center for >20 years.

Swee Lay Thein, MD

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.

John Tisdale, MD

John Tisdale, MD, is a senior investigator and chief of the Cellular and Molecular Therapeutics Branch of the National Heart, Lung, and Blood Institute (NHLBI) at NIH. Dr. Tisdale's research focuses on developing curative strategies for sickle cell disease through transplantation of allogeneic or genetically modified autologous hematopoietic stem cells. He received his medical degree from the Medical University of South Carolina (MUSC) and trained in internal medicine at Vanderbilt University, where he also completed a chief residency. After a postdoctoral fellowship in NHLBI's Hematology Branch, he joined the Molecular and Clinical Hematology Branch of NHLBI in 1998.

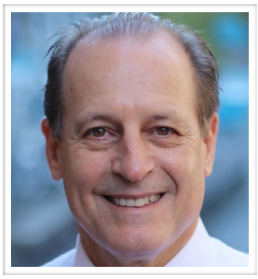
An elected member to the American Society for Clinical Investigation, Dr. Tisdale is the recipient of the Ernest Beutler Lecture and Prize of the American Society of Hematology. His awards include the American Society of Gene and Cell Therapy's Jerry Mendell Award for Translational Science, the George C. Marshall Innovation Leadership Award, the Public Health Service Outstanding Service Medal, the Annual Bipartisan Congressional Silver Innovator Award, the American College of Physicians' Richard and Hinda Rosenthal Award #1, and the College of Charleston's Pre-Medical Society's Outstanding Service Award in Medicine. He was a finalist of the Samuel J. Heyman Service to America Medal and is one of four clinicians featured in Discovery Channel's "First in Human" documentary on clinical trials conducted at NIH.

Dr. Tisdale has over 250 publications. He was the Principal Investigator on the gene therapy trial of lovo-cel for SCD that was FDA approved in 2023.

Mark C. Walters, MD

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 and Chief, Hematology division in the Department of Pediatrics at University of California, San Francisco School of Medicine. He is Program Director of the California Institute of Regenerative Medicine alpha clinic at UCSF. He has been active in cooperative clinical transplantation trials and has participated in NIH-supported investigations of hematopoietic cell transplantation for sickle cell disease and thalassemia and in industry-sponsored gene therapy clinical trials 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 such as sickle cell disease.

Russell Ware, MD, PhD



Dr. Ware is an internationally recognized expert in pediatric hematology with special interest in sickle cell disease. He trained at Duke University, receiving both MD and PhD degrees there. He joined Cincinnati Children's Hospital in 2013 and directs the Division of Hematology and the Global Health Center. His NIH-funded laboratory studies hydroxyurea therapy and genetic modifiers of sickle cell disease. He has led many NIH-funded national and international clinical trials using hydroxyurea, and currently has research partnerships in the Caribbean and sub-Saharan Africa. He has also conducted sickle surveillance studies in Angola, Uganda, Tanzania, and Malawi. Dr. Ware has published over 400 scientific papers and chapters and was an expert panel member for the NHLBI Evidence-Based Guidelines for Sickle Cell Disease.

Lachelle D. Weeks, MD, PhD

Dr. Lachelle D. Weeks is a physician-scientist at Dana-Farber Cancer Institute in the Department of Medical Oncology, an Assistant Professor of Medicine at Harvard Medical School. She earned her MD and PhD degrees from Case Western Reserve University School of Medicine. Dr. Weeks subsequently completed her medical training at Brigham and Women's Hospital Internal Medicine Residency and Dana-Farber Cancer Institute Hematology/Oncology Fellowship. Dr. Weeks is the director of the CHIP Clinic in the Dana-Farber Centers for Early Detection and Interception where she counsels patients who have clonal hematopoiesis. As an independent investigator in the Division of Population Sciences, Dr. Weeks's translational laboratory focuses on identifying risk factors for clonal hematopoiesis, understanding clinical and molecular features that predict risk of progression to overt malignancy, and designing strategies for early intervention to prevent adverse outcomes. She led the development of the clonal hematopoiesis risk score (CHRS), a clinical algorithm to estimate the risk of transformation to myeloid malignancy in clonal hematopoiesis. Currently, along with PI Dr. Coleman Lindsley, she is co-leading a large international NHLBI funded effort to determine the prevalence and gene distribution of CH in SCD. Her work is supported by the American Society of Hematology, Robert Wood Johnson Foundation, Edward P. Evans Foundation for MDS, and Breakthrough Cancer.

Fulcrum Therapeutics

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