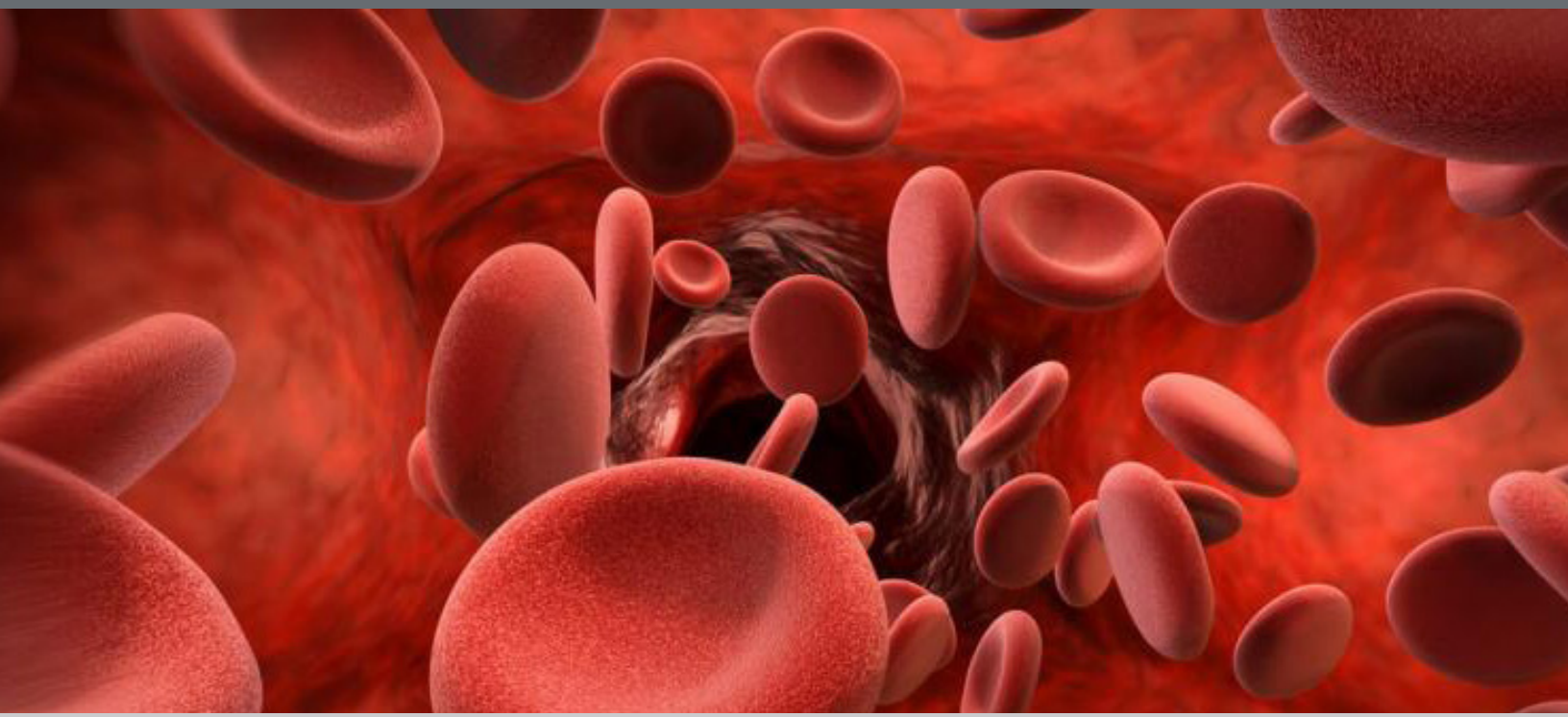
The 16th Annual

SICKLE CELL IN FOCUS CONFERENCE



SCiF
SICKLE CELL IN FOCUS

sponsored by:

NIH National Heart, Lung, and Blood Institute

It is our pleasure to welcome you to the 16th annual Sickle Cell in Focus (SCiF) Conference!

The National Heart, Lung, and Blood Institute (NHLBI) is excited to be co-hosting the 2023 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.

During this two-day, intensive, educational update on sickle cell disease, we will address the multifactorial aspects of sickle cell disease and foster the exchange of the latest scientific and clinical information related to sickle cell disease through innovative seminars and interactive panel debates. We received wonderful feedback on the quality and expertise of speakers at last year's conference and are excited to have yet another great lineup this year. These speakers 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 to make SCiF a success.

This year's conference is hybrid; we sincerely hope that you will enjoy SCiF 2023 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. Your feedback is important and highly appreciated. Please keep an eye out following the event for an event evaluation form, as it helps us shape the program for next year.

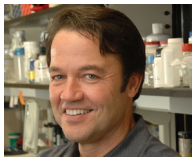
Thank you to all our attendees for their participation in this year's conference. We hope to see you again next year for Sickle Cell in Focus 2024.

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

Program Directors

Swee Lay Thein, MB, BS, FRCP, FRCPath, DSc
Senior Investigator and Chief
Laboratory of Sickle Cell Genetics and Pathophysiology

Sickle Cell Branch
NHLBI, National Institutes of Health



John F. Tisdale, MD
Senior Investigator and Chief
Cellular and Molecular Therapeutics Branch

NHLBI, National Institutes of Health

Jennifer Knight-Madden, MB, BS, PhD
Professor, Pediatric Pulmonology & Clinical Research
Director, Sickle Cell Unit

Caribbean Institute for Health Research
University of the West Indies

Monika Parshad-Asnani, MBBS, DM, PhD
Professor, Family Medicine and Epidemiology

Caribbean Institute for Health Research
University of the West Indies

AGENDA

TUESDAY, OCTOBER 10, 2023

8:00am Registration Check In

8:30am Opening remarks from Dr. Richard Childs
8:40am Remarks from Dr. Julie Panepinto

SESSION ONE : GLOBAL INITIATIVES TO ADVANCE CARE IN SCD

Chair: Jennifer Knight-Madden, MBBS, PhD

8:45-9:10am **ASH Initiatives in SCD: ASH Collaborative Network & CONSA**
Charles Abrams, MD
University of Pennsylvania

Alexis Thompson, MD, MPH
Children's Hospital of Philadelphia

9:10-9:25am **Global Network/Worldwide Coalition**
*Isaac Odame, MB ChB, MRCP (UK), FRCPCH, FRCPath, FRCPC
SickKids

9:25-9:40am **Caribbean Initiatives: CAREST**
*Marie-Dominique Hardy-Dessources, PhD
CAREST-Network

SESSION TWO: BLOOD TRANSFUSION

Chair: Kamille West-Mitchell, MD

9:40-10:05am **Should We Be Transfusing More?**
*Sean Stowell, MD, PhD
Harvard University Cancer Center

10:05-10:30am **Is Molecular Genotyping the Way Forward for Finding Compatible Antibodies?**
Stella Chou, MD
University of Pennsylvania

**Virtual*

10:30-10:55am **Why are SCD Patients More Prone to Making Allo-antibodies**
Neil Hanchard, MBBS, DPhil
National Human Genome Research Institute, NIH

10:55-11:00am: BREAK

SESSION THREE: PAIN MANAGEMENT

Chair: Patrick Carroll, MD

11:00-11:25am **Complexities and Management of Pain in SCD**
*Amanda Brandow, DO
Medical College of Wisconsin

11:25-11:50am **Cannabinoids vs Opioids**
Susanna Curtis, MD
Mount Sinai Icahn School of Medicine

11:50-12:15pm **Ketamine as an Adjunct to Opioids**
Zena Quezado, MD
National Institutes of Health

12:15-12:40pm **Buprenorphine Drug**
Patrick Carroll, MD
Johns Hopkins University

12:40-1:40pm LUNCH BREAK

SESSION FOUR: REPRODUCTIVE HEALTH

Chair: Monika Parshad-Asnani, MBBS, MSc, DM, PhD

1:40-2:20pm **Overview of Women's and Men's Reproductive Health**
Lydia Pecker, MD
Johns Hopkins University

2:20-2:45pm **Sperm Count and Motility**
*Belinda Morrison, MBBS, DM
The University of the West Indies

2:45-3:00pm BREAK

**Virtual*

SESSION FIVE: UTILIZATION OF EMERGING TOOLS AND THERAPEUTIC AGENTS IN MANAGEMENT OF SCD

Chair: Deepa Manwani, MD

3:00-3:25pm **Telemedicine**
*Lesley King
The University of the West Indies

3:25-3:50pm **Current and Emerging Therapeutic Agents in SCD**
Charles Quinn, MD, MS
Cincinnati Children's Hospital

3:50-4:15pm **Real-World Use of Current Therapeutics in SCD**
*Wally Smith, MD
Virginia Commonwealth University

4:15-4:40pm **Monocytes and Drug Efficacy in SCD**
Karina Yazdanbakhsh, PhD
New York Blood Center

4:40-5:00pm QUESTIONS

5:00pm EVENT CLOSURE

**Virtual*

AGENDA

WEDNESDAY, OCTOBER 11, 2023

8:00am Registration Check In

8:30am Opening remarks from Profs. Minerva Thame and Marshall Tulloch-Reid

SESSION ONE: GENETIC THERAPIES

Chair: John Tisdale, MD

8:45-9:10am **Micro-RNA (miRNA) for BCL11A**
*Erica Esrick, MD
Dana-Farber Cancer Institute

9:10-9:35am **Genetic Addition**
Alexis Thompson, MD, MPH
Children's Hospital of Philadelphia

9:35-10:00am **Base and Prime Editing**
*Nicole Gaudelli, PhD
Beam Therapeutics

10:00-10:10am BREAK

SESSION TWO: UNRAVELING MECHANISMS IN SCD USING NEWER TECHNOLOGIES

Chair: Swee Lay Thein, MD

10:10-10:35am **CHIP in SCD**
Lachelle Weeks, MD, PhD
Dana-Farber Cancer Institute

10:35-11:00am **OMICS and SCD**
*Angelo D'Alessandro, PhD
University of Colorado

**Virtual*

11:00-11:30am **Digital Health Platforms to Improve Patient Engagement and Self-Care**
Miguel Lara, MBA
Matthew Xu, BA
Microhealth

11:30-1:00pm LUNCH

SESSION THREE: ANIMAL AND CELLULAR MODELS OF SCD

Chair: Selami Demirci, PhD

1:00-1:25pm **HUDEP: Human Erythroid Cell Lines**
Jing Zeng, MD
Boston Children's Hospital

1:25-1:50pm **BEL-A: Bristol Erythroid Line-Adult and BEL-SS**
*Jan Frayne, PhD
University of Bristol

1:50-2:10pm **Mouse Model**
*Lucia De Franceschi, MD
University of Verona and AOUI Verona

2:10-2:35pm **Large Animal Models**
Irina Polejaeva, MS, PhD
Utah State University

2:35-2:40pm BREAK

*Virtual

SESSION FOUR: POST-CURATIVE TREATMENT IN SCD

Chair: Matt Hsieh, MD

2:45-3:10pm **Pain Post-Curative Treatment in SCD**
Alexis Leonard, MD
St. Jude Children's Hospital

3:10-3:35pm **Cerebral Dynamics**
*Andria Ford, MD
Washington University of Saint Louis

3:35-4:00pm **Post-Curative Malignancies Risk**
Lukasz Gondek, MD, PhD
Johns Hopkins University

DEBATE: WHO WILL FOLLOW UP THE CARE OF PATIENTS POST-TRANSPLANT?

Chair: Sophie Lanzkron, MD, MHS

4:00-4:15pm **"Hematologist should follow-up the care patients post-transplant"**
Lakshmanan Krishnamurti, MD
Yale School of Medicine

4:15-4:30pm **"Transplant doctors should follow-up the care patients post-transplant"**
Jane Little, MD
University of North Carolina-Chapel Hill

4:30-4:40pm **Patient perspective**
Nasir Mason
Clevetta Drew

4:40-5:00pm DISCUSSION AND QUESTIONS

5:00pm CONFERENCE CLOSING REMARKS

*Virtual

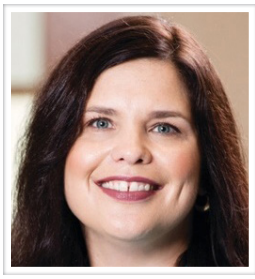
SPEAKER BIOGRAPHIES

Dr. Charles S. Abrams is the Francis C. Wood Professor of Medicine at the University of Pennsylvania School of Medicine. Dr. Abrams is the Director and Founder of the University of Pennsylvania/Children's Hospital of Philadelphia (PENN-CHOP) Blood Center for Patient Care & Discovery, and Vice Chair for Research and Chief Scientific Officer of the Department of Medicine at PENN. Over the past few years, he has also led an effort by the American Society of Hematology Research Collaborative to develop a National Clinical Trials Network which facilitate interventional trials that will lead to more rapid FDA approval of drugs designed to improve the quality of life for sickle cell patients and to increase the survival of individuals with this disorder.

Dr. Monika Asanani 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.



Dr. Amanda Brandow is a Professor of Pediatrics at the Medical College of Wisconsin in the section of hematology/oncology/bone marrow transplantation and the Director of the Comprehensive Sickle Cell Disease Clinical and Research Program. Dr. Brandow is a physician-scientist who provides care for children with sickle cell disease and other non-malignant hematologic conditions and conducts clinical and translational research focused on understanding the pathophysiology of acute and chronic pain in children and adults living with sickle cell disease. Specifically, her lab is investigating the underlying neurobiology of sickle cell pain with a focus on inflammation, the microbiome and nervous system sensitization. To support her research, Dr. Brandow was a past recipient of an NIH/NHLBI K23 Mentored Patient-Oriented Research Career Development Award, an American Society of Hematology Scholar Award and she is currently the principal investigator on an R01 from the NIH/NHLBI and an R61/R33 NIH/NINDS HEAL award in the Discovery and Validation of Biomarkers, Endpoints, and Signatures for Pain Conditions program. She is also and multi-principal investigator on a U01 form the NIH/NHLBI. Dr. Brandow is Co-Principal Investigator for the Sickle Cell Disease Clinical and Research Consortium of Southeast Wisconsin supported by the American Society of Hematology Sickle Cell Disease Clinical Trials Network. She currently serves as the Co-Chair for the development of the American Society of Hematology's Sickle Cell Disease Research Agenda. Dr. Brandow is also active in advocating for comprehensive pain management for individuals living with sickle cell disease at the national level where she served on the US Department of Health and Human Services Pain Management Best Practices Inter-Agency Task Force and as Chair of the American Society of Hematology Evidence Based Sickle Cell Disease Management Guidelines for Acute and Chronic Pain.

Dr. Patrick Carroll is an internationally recognized expert in the multidisciplinary management of complex and high utilizing patients with sickle cell disease (SCD). After completing his graduate medical training at Washington University in St. Louis, he completed residency in the Johns Hopkins Department of Psychiatry and Behavioral Sciences. Thereafter he was a fellow in the Behavioral Pharmacology Research Unit, studying the behavioral pharmacology of opioids. Upon leaving the fellowship, he joined faculty as an associate medical director of Addiction Treatment Services, a leading addiction treatment center at Johns Hopkins Bayview Medical Center. In 2007, he assumed his current role in the Sickle Cell Center for Adults, combining his interests in chronic pain, opioid pharmacology, and the care of complex patients. He attends in the Department of Psychiatry's Pain Treatment Program, an intensive multidisciplinary treatment program for patients with refractory chronic pain or abnormal illness behavior. He has published a number of peer-reviewed papers on SCD pain and treatment utilization, in addition to his earlier work in behavioral pharmacology and addiction treatment. Along with the multidisciplinary team of the Johns Hopkins Sickle Cell Center for Adults, he has been consulted regionally and internationally regarding management of chronic pain, psychiatric illness, and treatment utilization in SCD.

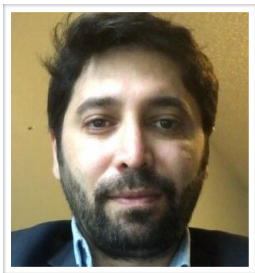
Dr. Stella Chou is an Associate Professor of Pediatrics at the Perelman School of Medicine at the University of Pennsylvania and Chief of the Division of Transfusion Medicine at The Children's Hospital of Philadelphia. She practices both Pediatric Hematology and Transfusion Medicine with a particular interest in patients with sickle cell disease. Her work has demonstrated that inheritance of variant Rh antigens in patients with sickle cell disease and in Black donors contributes to Rh alloimmunization. Ongoing work focuses on RH genetic matching of red cells, integrating molecular technologies into clinical transfusion medicine practice and developing novel tools for blood banks. Her laboratory uses induced pluripotent stem cells (iPSCs) to create customized iPSCs with rare blood group antigen combinations and explores methods to enhance their use as renewable sources of red cells. Her goal is to identify new approaches to minimize alloimmunization, reduce complications and improve therapy.

Dr. Susanna Curtis is Assistant Professor of Medicine at Icahn School of Medicine and Assistant Director of the Adult Sickle Cell Program at Mount Sinai Hospital. They received their medical degree from New York Medical College, completed residency in internal medicine at Montefiore Medical Center and fellowship in hematology and oncology at Yale University where they also completed a PhD in investigative medicine. Their research focuses on understanding the role dysfunctional inflammation plays in end organ damage development in people living with sickle cell disease, particularly in the development of chronic pain, and using this to develop targeted treatments. They are particularly interested in examining the utility of cannabis and cannabinoids for treating chronic pain and are currently studying this with support from the NHLBI. Doctorate in physics from the Free University Berlin, and an honorary Laurea in pharmaceutical chemistry and technology from the University of Parma.

Dr. Angelo D'Alessandro is a tenured Full Professor in the Department of Biochemistry and Molecular Genetics. He is the founder and Director of the Metabolomics Core of CU Anschutz School of Medicine and the director of the Mass Spectrometry Shared Resource for the University of Colorado Cancer Center. Age 39, he has published over 470 papers, research sponsored by NHLBI, NIDDK and NIGMS. He is the founder and CSO of Omix Technologies Inc, Altis Biosciences, and serves as an advisory board member for Hemanext Inc, and Macopharma. He is a Boettcher Investigator, AABB Hall of Fame, National Blood Foundation Scholar, Jean Julliard (ISBT) and RISE (AABB) awardee. He is also an affiliate investigator to the Vitalant Research Institute, the Linda Crnic Institute for Down syndrome, the Gates Grubstake Center for Regenerative Medicine.

Dr. Lucia de Franceschi - My scientific activity has focused on red cell pathologies, normal and pathologic erythropoiesis, hemoglobinopathies such as β-thalassemic syndromes and sickle cell disease (SCD) and hereditary rare anemias (i.e.: stomatocytosis, CDAII). An additional area of investigation is chorea-acanthocytosis and McLeod syndrome, which are neurodegenerative disorders involving also the erythroid compartment (*Blood* 118: 5652-63, 2011; *Blood* 128: 2976, 2016; *J Neurosci* 36: 12027, 2016). Through highly productive collaborations and keywork in my laboratory, my research has resulted in a number of breakthrough technologies and discoveries.

Clevetta Drew is a Life Coach and Patient Advocate. She is a former Federal Government employee for over 15 years as a Program Analyst. In 2019, she participated in a clinical trial using gene therapy at the National Institutes of Health (NIH) for Sickle Cell Anemia, which proved to be successful. She is now on the Internal Review Board at NIH. Clevetta now uses her personal experience and skills as a Life Coach to inspire the youth, empower women, and teach others with health challenges to manage mental health and everyday life.



Dr. Selami Demirci is a Stem Cell Researcher at the National Institutes of Health and started his career focusing on stem cell research for his doctoral research as they had become infatuated with the topic through the years. They studied various types of stem cells including adipose-, bone marrow-, and dental tissue-derived mesenchymal stem cells along with hematopoietic stem cells (HSCs) and their function, maintenance, differentiation, and roles in tissue regeneration using ex vivo manipulations and murine models. For his postdoctoral research, They have been working as a postdoctoral research fellow at the National Institutes of Health (NIH) which has allowed him to immerse himself in translational sciences and has given him the opportunity to meet with patients and see the primary effects of his research first hand.

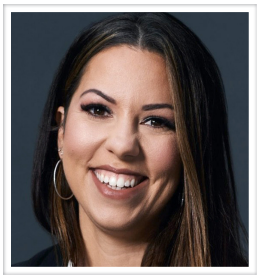
Dr. Erica Esrick is a pediatric hematologist-oncologist in Boston Children's Hospital affiliated with multiple centers in the area, including Dana-Farber Cancer Institute and Boston Medical Center. Dr. Erica is also an instructor in pediatrics at Harvard Medical School. She received her medical degree from Harvard Medical School and has been in practice between 11-20 years. Her primary clinical and research interest is in hemoglob-inopathies. She is the clinical lead for the Boston Children's Thalassemia Program, the clinical PI of BCH's sickle cell gene therapy study, and site PI for multiple other clinical studies of sickle cell disease and thalassemia.

Dr. Andria Ford is a Professor of Neurology and Radiology with Tenure at Washington University School of Medicine, with subspecialty training in Vascular Neurology. Dr. Ford specializes in stroke in the young adult population, sickle cell disease, and dementia due to both sporadic and inherited forms of cerebral small vessel disease. Dr. Ford cares for stroke patients in the Emergency Department and inpatient stroke service at Barnes-Jewish Hospital and the Outpatient Stroke Center at Washington University School of Medicine. She is Director of the Stroke Section in the Department of Neurology at Washington University School of Medicine. Dr. Ford leads an NIH-funded research program with a goal to understand and find new treatments and prevention strategies for patients with inherited and sporadic cerebral small vessel diseases including sickle cell disease and RVCL-S. Dr. Ford also directs the Stroke Patient Access Core (SPAC) overseeing participant enrollment into numerous clinical stroke trials conducted at Washington University School of Medicine. Dr. Ford is originally from Oklahoma City, but has raised her family in St. Louis, her 2nd home. She enjoys spending time with her husband and three children.

Jan Frayne - Originally researching into molecular aspects of sperm function and fertilization, in 2007 with funding from NHSBT I moved into the field of erythropoiesis, establishing a research group and now international reputation in erythrocyte biology in health and disease.

My research initially focused on the development and utilization of *in vitro* systems to generate human erythroid cells from different human stem cell sources (adult, cord blood, fetal liver, induced pluripotent [iPSC] and embryonic stem cells [ESC]), and the molecular analysis of these cells, along with innovative comparative proteomic approaches.

As available methods for the *in vitro* generation of red cells from adult and cord blood progenitors do not yet provide a sustainable supply, and current systems using iPSC and ESCs as progenitors do not generate viable red cells I took an alternative approach, pioneering creation of the first human immortalised adult erythroid cell line (BEL-A) which recapitulates normal erythropoiesis, demonstrating a feasible approach to the manufacture of red blood cells for clinical use. The approach is robust and reproducible with multiple further lines generated, including from patient cells. We subsequently developed a platform for CRISPR-Cas9 genome editing of the lines for gene knockout and introduction of specific mutations and tags. Using these approaches we have created sub-lines with single and multiple blood group antigens ablated, applicable for diagnostics and proof of principal for a 'Universal' blood product, and a catalogue of characterised model red blood cell disease lines for e.g. β-thalassemia, sickle cell disease, congenital dyserythropoietic anaemia I and IV, and for PFCP, utilising the lines to study underlying molecular defects of these diseases and as drug screening platforms. I am also interested in the transcription factor regulation of erythropoiesis and have identified novel factors involved in this process. A particular interest is the transcription factor KLF1, a key regulator of erythropoiesis, identifying the E325K KLF1 mutation that results in the severe human disease CDA IV, going on to determine how the mutation results in severe anaemia.



Nicole Gaudelli received her B.S. degree in biochemistry from Boston College in 2006 where she studied the structural and mechanistic underpinnings of neocarzinostatin biosynthesis and a non-heme iron oxygenase involved in vancomycin assembly. She earned her Ph.D. in chemistry from Johns Hopkins University where she studied monocyclic beta-lactam antibiotics and elucidated the mechanism through which they are biosynthesized. She completed her postdoctoral fellowship at Harvard University in the laboratory of Professor David R. Liu where she expanded the capabilities of base editing technology by creating the first adenine base editor (ABE) through 7 rounds of directed evolution and engineering. Both her doctoral and postdoctoral work culminated in prominent Nature publications in the fields of natural product chemistry and gene editing respectively.

Dr. Gaudelli is an inventor on numerous base editing patents and leads the gene editing technologies platform group at Beam Therapeutics where her team advances precision genetic medicines using base editors. Dr. Gaudelli is a recipient of the 2018 American Chemical Society's "Talented 12" award, recognized as a 2018 STAT News Wunderkind, a 2018 TEDMED Hive honoree, Genetic Engineering and Biotechnology News "Top 10 Under 40 of 2019", BioSpace's 2019 "10 Life Science Innovators Under 40 to Watch", and is MIT's 2019 Technology Reviews' 35 Innovators Under 35.

Dr. Lukasz Gondek is an Assistant Professor of Oncology at the Johns Hopkins University School of Medicine in the Division of Hematologic Malignancies. Dr. Gondek received his medical degree from the University of Silesia, Poland and his Ph.D. from the University of Warsaw, Poland. He completed his residency in internal medicine at the Cleveland Clinic and his fellowship training in hematology at the Johns Hopkins University. He joined the faculty in 2014.

Dr. Gondek's primary research is to better understand the role of molecular events in initiation and progression of myelodysplastic syndromes and leukemia as well as develop novel targeted therapies to prevent disease progression and relapse. Dr. Gondek has a clinical interest in hematologic malignancies and in particular myelodysplastic syndromes and acute myeloid leukemia.

Dr. Neil Hanchard received his medical degree MBBS with Honors from the University of the West Indies in Kingston, Jamaica, and his D.Phil. in Clinical Medicine from the University of Oxford, UK, where he was a Rhodes Scholar. Thereafter he completed his clinical training as a pediatrician at the Mayo Clinic in Rochester, Minnesota and as a clinical geneticist at Baylor College of Medicine (BCM) in Houston, Texas. Dr. Hanchard is currently a Senior Investigator and Stadtman Scholar in the Center for Precision Health Research within the National Human Genome Research Institute, where he heads the Childhood Complex Disease Genomics lab. His research uses quantitative and integrative genomics to better understand the

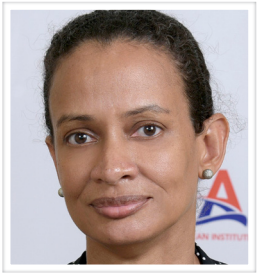
pathophysiology of complex pediatric diseases, with a particular emphasis on the interplay of population genetics and disease susceptibility in diverse populations. The lab's current research portfolio includes studies of pediatric hypertension, transfusion reactions in sickle cell disease, severe childhood malnutrition, and pediatric HIV. Dr. Hanchard is an adjunct Associate Professor at BCM, Chair of the Genome Analysis working group of the Human Health and Heredity in Africa (H3Africa) Consortium, Chair of the Diversity Equity and Inclusion Task Force of the American Society of Human Genetics, and a Distinguished Scholar of the NIH.

Dr. Marie-Dominique Hardy-Dessources is a biochemist who has worked at the French National Institute of Health and Medical Research (Inserm) for more than 30 years. She has been interested in sickle cell disease since her PhD, which she completed at the Inserm Unit 91 of the Henri Mondor Hospital in Créteil and defended at the Faculty of Pharmacy of the University of Paris Sud, Chatenay-Malabry. She joined the team of Dr Guy Mérault in Guadeloupe and participated with him in the creation of the first French Sickle Cell Centre in Guadeloupe.

In recent years, she has worked at Unit 1134 Inserm/Université de Paris/Université des Antilles, a research unit with two sites in Paris and Guadeloupe. Her research interests included basic laboratory studies and translational science, with a focus on the mechanisms of erythrocyte activation leading to vaso-occlusive crises and the relationships between the severity of sickle cell disease and factors such as changes in blood rheology, cellular inflammation and oxidation.

Dr Hardy-Dessources is a founding member of the Caribbean Network for Research in Sickle Cell Disease and Thalassaemia (CAREST). She has been the President of the Board of Directors of this network since its inception in 2012.

Dr. Matthew Hsieh is a Senior Research Physician in the Cellular and Molecular Therapeutics Branch of the National Heart, Lung, and Blood Institute (NHLBI) in Bethesda, Maryland. He is active investigator in several intramural and extramural clinical trials for sickle cell disease, including hematopoietic cell transplantation (HCT) from matched related donors, industry sponsored clinical trials for gene therapy, late effects of HCT, and haploidentical transplantation. He is on the faculty for the combined NHLBI/NCI hematology and oncology fellowship. His expertise and research interests include stem cell mobilization, organ dysfunction related sickle and beta-globin disorders, iron overload, neutrophil disorders, and HCT conditioning regimens.



Dr. Lesley King - I am a Paediatrician and Head, Clinical Services, CAIHR, UWI, with 20-plus years of experience in clinical care for persons living with sickle cell disease (SCD). I have responsibility for overseeing the clinical services at CAIHR, primarily the Sickle Cell Unit (SCU). The SCU is a comprehensive care centre for persons living with SCD. Our clinical service is focused on health maintenance, but we also facilitate acute care in our day-care ward. We have ~ 8500 annual visits each year from ~ 2500 patients. Additionally, we facilitate training for students and other health care professionals (HCP) who do electives at the SCU. During the COVID-19 pandemic, I had the responsibility for ensuring continuity of care for patients and implemented a telehealth service to assist and complement our traditional service.

My research areas are focused on aspects of clinical care, including newborn screening (NBS) and hydroxyurea (HU) use in SCD. I am 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. I lead the Capacity Building for Health Care Workers sub-group, our focus being training activities to improve and standardize clinical care.

I believe there is a role for telehealth in management of SCD, as a tool for training and increasing access to care especially in low-resource settings where expert care is limited and poses access challenges for many patients.

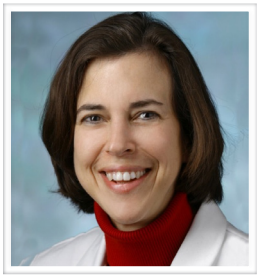
Professor Jennifer Knight-Madden is the Director of the Sickle Cell Unit, the Caribbean Institute for Health Research, The University of the West Indies (UWI). Professor Knight-Madden graduated from The UWI's medical school in 1988. She completed training in Pediatrics at the Hospital for Sick Children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

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

University of the West Indies Ethics Committee and the Advisory Panel on Ethics and Medico-Legal Affairs, MOHW (one year assignment). She is an examiner and member of Specialty Board of the Child and Adolescent Health Program and an honorary Consultant at the University Hospital of the West Indies.

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

Dr. Lakshmanan Krishnamurti - I am a pediatric hematologist oncologist of 25 years standing with a primary research focus interest in benign hematology, including sickle cell disease My main areas of interest are clinical and patient centered outcomes research include improving the delivery of care, newborn screening, international outreach, community outreach, hematopoietic stem cell transplantation and gene therapy. My research has been funded by NHLBI, NICHD, CDC, HRSA, PCORI, SCDAA and Cooley's Anemia foundation and Abraham J. and Phyllis Katz Foundation. I am also the currently the PI of an NHLBI U01 grant for a multicenter study of BMT for adults with SCD the first study comparative study of HCT to standard of care in SCD and the first to systematically capture patient reported and functional outcomes in an HCT for SCD study. I am also the PI of the Sickle Transplant Long term and Late effects Registry (STELLAR). I have had programmatic leadership roles for over 20 years as director of hematology at Children's Hospital of Pittsburgh, Director of Blood and Marrow Transplantation at Children's Healthcare of Atlanta, and most recently as section Chief of Pediatric Hem/Onc/BMT at Yale School of Medicine. In these roles I have a demonstrated track record in enhancing programs, improving delivery of care, and supporting faculty development while continuing to pursue NIH funded clinical and patient reported outcomes research.



Dr. Sophie Lanzkron is a professor of medicine at the Johns Hopkins University School of Medicine. Her areas of clinical expertise include hematology and sickle cell disease. Dr. Lanzkron is director of the Sickle Cell Center for Adults at Johns Hopkins.

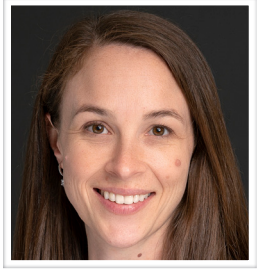
Dr. Lanzkron received her undergraduate degree in biology from Brandeis University. She earned her M.D. from Albert Einstein College of Medicine. She completed her residency at the University of Maryland Medical Center and performed her fellowship in hematology at Johns Hopkins. She joined the faculty of Johns Hopkins in 2000.

Her research focuses on sickle cell disease.

Miguel Lara - I am a digital health entrepreneur (my "what"). My focus is to serve people on their healing journey (my "why"). I use mobile software tools and devices to drive better outcomes for patients in the bleeding disorders community (the "how").

Formerly, my background was in finance and investment banking. I was the Managing Director for Healthcare and Consumer industries at BBVA USA in New York.

For the sickle cell disease project, I will be responsible for driving leadership in the planning, design, and implementation of the SCD Warrior app by defining vision, strategies, and tactics to address patient and provider needs in order to optimize user engagement with the platform and VOC predicting capabilities.



Dr. Alexis Leonard received her medical degree from Tufts University School of Medicine, and completed her pediatric residency training and pediatric hematology/oncology fellowship at Children's National Hospital. During her fellowship training, she joined the laboratory of Dr. John Tisdale at the National Institutes of Health and focused on understanding and improving gene therapy methods and outcomes for the treatment of sickle cell disease (SCD). After fellowship she became a faculty member in the department of hematology at Children's National while continuing her research at the NIH. In 2023 she joined St. Jude Children's Research Hospital as an Assistant Member in hematology with a focus on delivering and improving upon transformative therapies for patients with SCD.

Dr. Jane A Little - I have been the Director of the Sickle Cell Disease Program at the University of North Carolina in Chapel Hill, since March 2019. I have an interest in erythropoiesis and in the diagnosis, clinical management, and prognosis of sickle cell disease (SCD). I have participated in a number of collaborative multi-centered efforts, through which to better understand SCD, its complications, and best practices. I was a site Principal Investigator on the Walk-PHaSST prospective study of elevated tricuspid regurgitant jet velocities in SCD, through 2011. I was a site PI for the Escaped study (Comparison of Patient Centered Outcomes for People with SCD in the Acute Care Setting), completed in 2018.

Together with collaborators at Johns Hopkins, CWRU, OSU, Einstein, Oakland/UCSF, the Medical College of Wisconsin, and the University of Alabama, I helped to develop the multi-centered HIPAA-compliant prospective database (Globin Research Network for Data and Development, GRNDaD) through which to study the natural history of SCD in the modern era. Because of improvements in care in the US for people with SCD over the last 4-5 decades, the population of adults with SCD has steadily grown; and our knowledge of this emerging population is limited. GRNDaD arose out of long-simmering frustration amongst colleagues with short-lived databases in SCD, which have deprived our community

of the benefits of a long view on disease modifying therapies, like hydroxyurea, transfusions, and recently emerged 'novel' therapies.

With Umut Gurkan, a bioengineering colleague at CWRU, I have published a number of studies seeking to expand point-of-care in SCD, encompassing both diagnosis (the hemechip) and management (the SCD Biochip). Together, we have studied cellular adhesion in adult patients (at CWRU in Cleveland) and in pediatric patients (with Deepa Manwani at Children's Hospital at Montefiore in NYC). We have described associations with clinical comorbidities, such as right to left shunts and priapism, which we now wish to pursue further.

With Dr. Lanzkron at Johns Hopkins, I have participated in a previous successful PCORI award, entitled 'Comparing Patient Centered Outcomes in the Management of Pain between Emergency Departments and Dedicated Acute Care Facilities for Adults with Sickle Cell Disease (ESCAPED)', from 2014-2018. In that important study, we learned that infusion center-based care was superior to emergency department-based care for people with SCD.

Dr. Deepa Manwani completed her residency at North Shore University Hospital, and she received her sub-specialty in pediatric hematology and oncology at Mount Sinai Hospital. Under the supervision of James Bieker, professor of developmental and regenerative biology at Mr. Sinai School of Medicine, in 1997 she initiated basic science research on hemoglobin gene regulation. Her primary areas of research focused on exploring mechanisms of fetal hemoglobin reactivation with the ultimate goal of designing novel therapies for sickle cell disease and thalassemia. She served as Director of the Thalassemia Program (2006-2008) and Sickle Cell Program (2007-2008) at Schneider Children's Hospital in New Hyde Park, NY before coming to Montefiore.

Dr. Manwani's research interests focus on fetal hemoglobin activating agents, adhesive cellular interactions in vaso-occlusion in SCD and contribution of neutrophils to SCD pathophysiology. She is currently participating in studies aimed at increased adherence to hydroxyurea and improved transition to adult care. In March 2015, her contributions have been recognized by the Sickle Cell Thalassemia Patient Network of New York in the form of the "Distinguished Service Award."

Dr. Belinda Morrison is a Consultant Urologist at the University Hospital of the West Indies. She is currently Lecturer and Head of the Division of Urology at the University of the West Indies. A proud graduate of the Wolmer's Girls School, she completed undergraduate and postgraduate training at the University of the West Indies, Mona campus.

Her areas of academic interest include prostate cancer and andrology, where she has published several articles in peer-reviewed journals and book chapters.

Dr. Morrison has made many local, regional and international presentations to audiences in her field of urology. She is extensively involved in research, particularly as it related to describing the treatment and epidemiology of prostate cancer in Jamaica. Dr. Morrison has received research awards from the University of the West Indies as well as the American Urological Association.

Dr. Morrison is a volunteer at the Jamaica Cancer Society where she performs regular monthly prostate screening. In addition, she travels across the island to many churches, public and private organizations and small groups to give men's health talks. Dr Morrison is passionate about health education to the public and regularly engages the media to facilitate this.

She is the current President of the Jamaica Urological Society and member of the Medical Association of Jamaica, American Urological Association and Sexual Medicine Society of North America. She is a Christian and worships at the Webster Memorial United Church.

Dr. Isaac Odame is the Hematology Section Head, Division of Hematology/ Oncology, The Hospital for Sick Children (SickKids), Toronto. He is Professor and Director of the Division of Adult and Pediatric Hematology in the Departments of Medicine and Pediatrics, University of Toronto. He holds the Alexandra Yeo Endowed Chair in Hematology at the University of Toronto.

Currently, Odame is the Medical Director of the Global Sickle Cell Disease Network based at the SickKids Centre for Global Child Health that is building enduring collaborations between clinicians/scientists worldwide to further research and advance care of patients with sickle cell disease, particularly in LMICs that shoulder the heaviest disease burden. As a member of Steering Committees, Data Safety Monitoring Boards, and site Principal Investigator, he is actively involved in several clinical studies aimed at developing novel disease-modifying and curative therapies for SCD. Odame is currently Associate Editor of *Pediatric Blood & Cancer* journal and serves on the Editorial Board of the *Lancet Child & Adolescent Health*.

Dr. Lydia H. Pecker is an Assistant Professor of Medicine at Johns Hopkins University. She received her undergraduate degree in Africana Studies from Brown University and her MD from The University of Pennsylvania Medical School. She also holds a Masters in Health Sciences from the Johns Hopkins Bloomberg School of Public Health. Dr. Pecker's primary research is focused on reproductive health in girls and women with SCD, with particular focus on infertility risks and pregnancy. 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.

Dr. Pecker is the founding Director of the Young Adult Clinic at the Sickle Cell Center for Adults at Johns Hopkins, which aims to provide developmentally appropriate, expert care for young adults living with sickle cell disease.

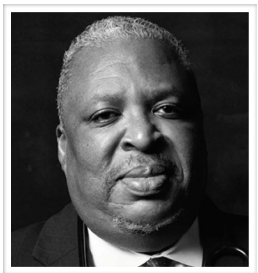
Dr. Irina Polejaeva is currently a Professor of Developmental Biology in the Department of Animal, Dairy and Veterinary Sciences at Utah State University (USU) and is also a member of the USU Veterinary Diagnostics and Infectious Diseases Team. Her primary research interests are: 1) the development of genetically engineered large animal models for biomedical and agricultural applications using Somatic Cell Nuclear Transfer (SCNT) and CRISPR/Cas9 genome editing techniques; and 2) cloning efficiency improvement through understanding the molecular mechanism of epigenetic reprogramming.

Prior to joining USU, Irina worked as the Project Manager for the Porcine Nuclear Transfer Program at PPL Therapeutics, Inc. (Blacksburg, VA, USA) and then served for eight years as the Chief Scientific Officer at ViaGen, Inc. (Austin, TX, USA). Her research led to the generation of the world's first cloned pigs by SCNT and the birth of the first 1,3-galactosyltransferase deficient pigs. More recently her laboratory was the first to report on efficient gene knockouts in goats using the CRISPR/Cas9 and generate novel animal models including sheep models for Cystic Fibrosis, goat model for Atrial Fibrillation, the first trans-chromosomic goats for human polyclonal antibody production and sheep model for sickle cell disease (SCD). Dr. Polejaeva has served on the Board of Governors for the International Embryo Technology Society (2015-2018). Since 2016, Irina has been also serving as the Chair of the Organizing and Scientific Committee for the Biannual International Large Animal Genetic Engineering Summit that brings together reproductive biologists, geneticists, molecular biologists, animal model developers, and representatives of federal research and regulatory agencies.

Dr. Zenaide Quezado is a Senior Investigator and the Chair of Pediatric Anesthesiology and Anesthesia Research sections in the Department of Perioperative Medicine at the National Institutes of Health Clinical Center in Bethesda, MD, USA. Dr. Quezado received her MD from the Universidade Federal do Ceara, Brazil and is trained in Internal Medicine, Critical Care Medicine, Anesthesiology and Pediatric Anesthesiology. As a physician scientist, Dr. Quezado's clinical practice focuses on pediatric anesthesiology and pain management. Dr. Quezado's scholarship focuses on the study of pain associated with pediatric diseases including sickle cell disease (SCD) and syndromes associated with intellectual disabilities. To address questions in these areas Dr. Quezado's preclinical work includes innovative mechanistic studies of the pathobiology of pain and behavior deficits in SCD and pre-clinical drug testing in parallel with human studies. Dr. Quezado's preclinical work focuses on the study of SCD pathobiology using model systems of the disease, preclinical evaluation of novel therapies to treat SCD, and the study of opioid-sparing therapies to treat acute and chronic pain. Dr. Quezado aims to elucidate the mechanisms underlying SCD-related co-morbidities to inform the development of safer, targeted, and effective therapies for those co-morbidities in patients with SCD. Dr. Quezado's research has been funded by multiple grants from the NIH, the Sheik Zayed Institute for Pediatric Surgical Innovation, and by grants from private industry (Pfizer Foundation). She has published in leading clinical journals including *JAMA, JAMA*

Pediatrics, The Lancet Child and Adolescent Health, Pediatric Research, Annals of Internal Medicine, Circulation, Blood, Anesthesiology, Anesthesia and Analgesia. Dr. Quezado's bibliography contains over 105 publications and her research is frequently cited in clinical reviews and guidelines and has prompted many changes in the practice of pediatric anesthesia. Dr. Quezado has served as an Associate Examiner of the American Board of Anesthesiology since 2006. She is an innovator and holds a patent on an apparatus and method for human algometry and other provisional patent applications. Additionally, faculty development and mentorship has been a central focus of Dr. Quezado's career and she has mentored numerous trainees who have gone on to outstanding academic careers. She particularly focuses on advancing the careers of women and underrepresented minorities in her Department and to create an environment where everyone is welcomed and has an opportunity to advance.

Dr. Charles Quinn is a pediatric hematologist and Professor of Pediatrics at Cincinnati Children's Hospital Medical Center. He is the medical director of the pediatric sickle cell program and co-director of the Erythrocyte Diagnostic Laboratory. He is also the medical director of the regional newborn screening program for hemoglobinopathies. Dr. Quinn conducts clinical and translational research in sickle cell disease with focus on identifying mechanisms of disease, novel treatments, and advanced diagnostic testing.



Dr. Wally Smith is a Florence Neal Cooper Smith Professor of Sickle Cell Disease at Virginia Commonwealth University. He has personally cared for patients with SCD for 40 years. He is best known as the Principal Investigator of PiSCES, (R01 HL 64122, Pain in Sickle Cell Epidemiology Study, the first study to show that in adults acute pain and VOEs were rare in comparison to chronic SCD pain, which affected over half of patients. It was the first to relate ED and hospital use, health-related quality of life, alcohol use, depression and anxiety, catastrophizing, somatization, and coping to daily pain measured using diaries. These 16 publications, most notably *Ann Intern Med* 2008 Jan 15, 148(2):94-101, led to an NIH Request for Proposals on the Neurobiology of Pain in SCD. PiSCES methods were replicated in Europe (van Tuijn CF, et al. *Am J Hematol*. 2017). PiSCES led to Dr. Smith and colleagues' development of published national consensus research definitions of acute (Field JJ, et al. J Pain. 2018 Dec 19), and chronic (Dampier et al. *J Pain*. 2017 May;18(5):490-498) SCD pain. It demonstrated three phenotypes of daily SCD pain prevalence/severity (Bakshi N, et al. *Pain*. 2022 Jun 1;163(6). Under review is a PiSCES daily diary research definition of high-impact chronic pain (Jagtiani A, et al).

Dr. Smith also collaborated with colleagues to design and implement the NIH-funded IMPROVE trial as part of the SCD Clinical Research Network (1U10HL083732). IMPROVE was a multi-center phase III clinical trial comparing two alternative opioid PCA dosing strategies. Dr Smith was PI of the VCU Basic and Translational Research Program in SCD (U54HL090516) which also trained several sickle

cell scholars. He was a member of the lnteragency Pain Research Coordinating Committee for DHHS, which published the first National Pain Plan. He is VCU site PI for SCD Treatment Demonstration Program Regional Collaborative for the North East Region. He has twice served on the NHLBI SCD Advisory Committee. Since 2012, He has been Pl of likely the first-ever randomized controlled trial of implementation science in SCD, Start Healing in Patients with Hydroxyurea (SHiP HU, R18HL112737). He is an expert on the PhenX Toolkit panel to define a manageable SCD pain battery to appropriately measure the pain experience in patients with SCD. He is a member of the Multidisciplinary Work Group overseeing the Helping to End Addiction Long-term (HEAL) initiative, in response to the opioid epidemic. He was co-chair of the July 21-22, 2021 NIH Workshop, Approaches to Effective Therapeutic Management of Pain for People With SCD, an NIH-wide (7 ICs and Centers) Workshop whose themes for advancing research on pain in SCD helped lead to this RFA-AT-24-001.

Dr. Smith is a Co-Investigator and Steering Committee Member of the Patient-Centered Outcomes Research Institute: HSR Project Number: HSRP20181364 NCT Number: NCT03593395 **Comparative Effectiveness of Enhanced Peer Navigation versus Structured Education** in SCD, whose major outcome is VOE visits. Dr Smith is a Co-investigator and core advisor on UG3/UH3 multi-site SCD RCT of behavioral/ integrative therapy, Peer suppoRt for adolescents and Emerging adults with Sickle cell pain: promoting ENgagement in Cognitive behavioral thErapy (PRESENCE), PI Jonassaint, Charles, 1UG3HL165839-01A1.

Dr. Sean Stowell is the Medical Director of Apheresis at Brigham and Women's Hospital and the Associate Director of the National Center for Functional Glycomics at Harvard Medical School. He is an Associate Professor in the Department of Pathology, Brigham and Women's Hospital at Harvard Medical School, where he runs a research program that examines the development and consequences of immune responses to blood group antigens. He obtained his MD and PhD degrees from Emory, where he graduated Summa Cum Laude followed by additional training in laboratory and transfusion medicine.

Dr. 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 organizing 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.

Dr. Alexis A. Thompson is Chief of the Division of Hematology at Children's Hospital of Philadelphia. She holds the Elias Schwartz, MD, Endowed Chair in Hematology. She served as the president of the American Society of Hematology (ASH) in 2018 and is a member of the ASH CONSA steering committee. She received her medical degree from Tulane University School of Medicine and has been in practice 34 years. She specializes in pediatric anemia and red blood cell hematology and is experienced in thalassemias, sickle-cell anemia, anemia, and sickle cell anemia. She has more than 150 publications and over 500 citings.

Dr. John Tisdale received his medical degree from the Medical University of South Carolina in Charleston after obtaining his B.A. in Chemistry from the College of Charleston. He completed an internal medicine and chief residency at Vanderbilt University Medical Center in Nashville and then trained in hematology in the Hematology Branch, National Heart, Lung and Blood Institute (NHLBI), where he served as a postdoctoral fellow. He joined the Molecular and Clinical Hematology Branch of NHLBI in 1998 and is now the Chief of the Cellular and Molecular Therapeutics Branch. In 2020 the College of Charleston recognized Dr. Tisdale as one of their Top 25 History makers in honor of the schools 250-year anniversary and was Samuel J. Heyman Service to America Medal finalist. He was recently elected to the American Society for Clinical Investigation and is a member of the American Society of Hematology. He serves as an

editorial board member of the journals Stem Cells, Experimental Hematology, and Molecular Therapy Methods & Clinical Development. He is a frequent ad hoc reviewer for Blood, New England Journal of Medicine, Human Gene Therapy, and Nature Medicine Experimental Hematology Molecular Therapy to name a few. He has served on the NIDDK/ NIAMS Institutional Review Board for over 15 years, is a founding member of the NIH Bone Marrow Transplant Consortium, and is an active member of the NIH Intramural Gene Therapy Task Force. Dr. Tisdale's research and clinical work center on sickle cell disease. His group focuses on developing curative strategies for sickle cell disease through transplantation of allogeneic or genetically modified autologous bone marrow stem cells. He has published over 200 first- and co-author publications.

Dr. Lachelle Weeks - I am a Physician at Dana-Farber Cancer Institute, an Associate Physician at Brigham and Women's Hospital and Instructor in Medicine at Harvard Medical School. As an independent investigator in the Division of Population Sciences and Department of Medical Oncology at Dana-Farber, my research is focused on the chronic inflammatory conditions, genomic features and clinical factors that influence risk of myeloid malignancy in patients with clonal hematopoiesis (CH). I have a strong research background in cancer biology and have produced several peer-reviewed, first authored publications. As a postdoctoral trainee, I acquired significant skill in secondary data analysis and human genomics in myeloid malignancy and myeloid precursor conditions such as clonal hematopoiesis (CH). As PI of a Harold Amos Medical Faculty Development Award from the American Society of Hematology and Robert Wood Johnson Foundation, I developed the clonal hematopoiesis risk score (CHRS), the first clinically validated model that estimates the risk of progression from the leukemia precursor, CH, to blood cancers such as acute myeloid leukemia (AML). The CHRS helps clinicians categorize CH as low risk (where follow-up is not needed), high risk (where close follow-up is definitely needed) and intermediate risk for progression to AML. As a logical extension of my published work, the scope of my present research program covers 3 distinct domains: 1) resolving large genomic databases with deep clinical phenotyping to determine the features that influence risk of myeloid malignancy at a population-level; 2) computational modeling to generate, refine and validate clinically relevant predictive tools in myeloid malignancy precursor states; and 3) designing and implementing therapeutic and non-therapeutic interventions that prevent adverse outcomes in patients with myeloid precursors.

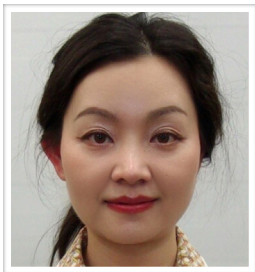
Dr. Kamille West-Mitchell is the Chief of the Blood Services Section at the NIH Clinical Center Department of Transfusion Medicine.

Dr. West-Mitchell graduated from medical school at the University of the West Indies in her home country of Jamaica in 2005; she then completed residency in Anatomic and Clinical Pathology in New York in 2013 and a 2-year-fellowship in Transfusion Medicine at the NIH in 2015.

As Blood Services Section Chief, she oversees blood donor collections at the NIH Blood Donor and Apheresis Centers to provide transfusion support for Clinical Center patients, and the NIH research blood donor program, which supports research at multiple laboratories at the NIH and FDA. She is the Medical Director of the Dowling Apheresis Clinic, which serves adult and pediatric patients requiring apheresis procedures to support hematopoietic stem cell transplantation and novel cell and gene therapies. She has authored and co-authored books chapters and peer-reviewed journal articles on various topics in Transfusion Medicine.

Matthew Xu - I am the Director of Product Management at MicroHealth, where I oversee product vision, strategy, development, and execution of new features and projects. My work spans our mobile application and web products in Hemophilia and Sickle Cell Disease (SCD), covering both patient-facing and provider-facing offerings. Prior to joining MicroHealth, I was the Product Manager for Provider Experience at Pear Therapeutics, the former industry leader in Prescription Digital Therapeutics (clinically validated, FDA-regulated software-based medicines), as well as the Product Manager for Oncology Prevention and Wellness at Cancer Treatment Centers of America. Within my work at Pear Therapeutics, I also served as product lead for a number of successful clinical and Real World Evidence studies, developing features and managing backend administration for single-site, multi-site, and decentralized studies. In each of these roles, I have built, deployed, and scaled highly engaging, user-centered digital health products that drive positive behavioral change and support positive patient-provider interactions. Furthermore, I have experience developing technology products in highly regulated environments, including both Software as a Medical Device and HIPAA guidelines.

Dr. Karina Yazdanbakhsh is Vice President and Director of Research at the Lindsley F. Kimball Research Institute of the New York Blood Center. Her current research is focused on understanding mechanisms of transfusion associated alloimmunization and immune pathophysiology of blood disorders including sickle cell disease and immune cytopenias. She is a recipient of several US National Institutes of Health grants, including an Outstanding Investigator Award from the NHLBI. She received her Ph.D. in molecular biology from the National Institute for Medical Research (MRC) at Mill Hill London and her postdoctoral training in molecular and cellular immunology at Columbia and Rockefeller Universities before joining the New York Blood Center in 1996.



Dr. Jing Zeng - Eight years of studying at Medical School has given me the opportunity to understand medicine and diseases. Three years of clinical service at Hospital has provided me the opportunity to take care of patients and ensure patient safety. I realized that the mechanism of many diseases was still unknown and there were no ideal curative therapies for many diseases. I became interested in research. Half a year of working in the laboratory at Brigham and Women's Hospital gave me experimental experience and opened my mind to stem cell therapy to cure diseases. To pursue my interest, I chose Dr. Bauer's lab at Boston Children's Hospital where I had the opportunity to develop cutting-edge gene editing technologies in hematopoietic stem cells. In our initial studies, we achieved highly efficient gene editing using CRISPR/Cas9 in hematopoietic stem cells, generated humanized mouse models of disease and demonstrated the therapeutic potential of gene editing. In the next two years we applied base editor technology to hematopoietic stem cells and demonstrated that base pair conversion could be sufficient to cure disease. I have devoted myself to investigating the potential efficacy and safety of these novel gene-modified cell therapies to promote their clinical translation, including conducting IND enabling preclinical and cell product manufacturing process development studies. We have demonstrated that human genetic diversity modifies therapeutic gene editing off-target potential. My current work is focusing on optimizing clinically relevant approaches to hematopoietic stem cell gene editing to maximize stem cell potential and minimize genotoxicity of edited HSCs. My career goal is to develop innovative approaches for therapeutic gene editing.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