

2023 Annual Sickle Cell Disease

Research Meeting

Agenda

August 14-16, 2023

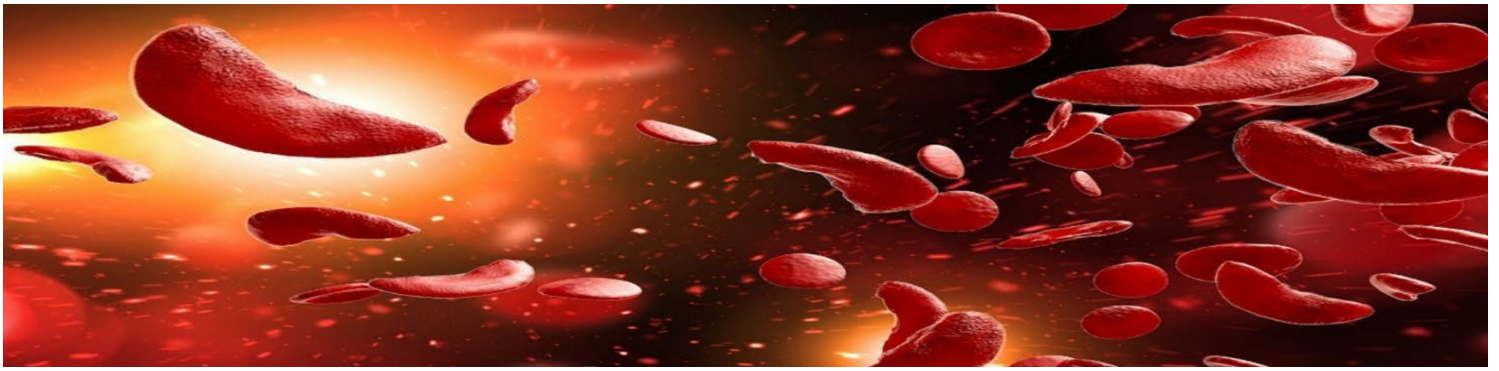
9:00 AM – 5:00 PM EDT

A Hybrid Meeting

**William H. Natcher Conference Center
Building 45, National Institutes of Health**



**National Heart, Lung,
and Blood Institute**



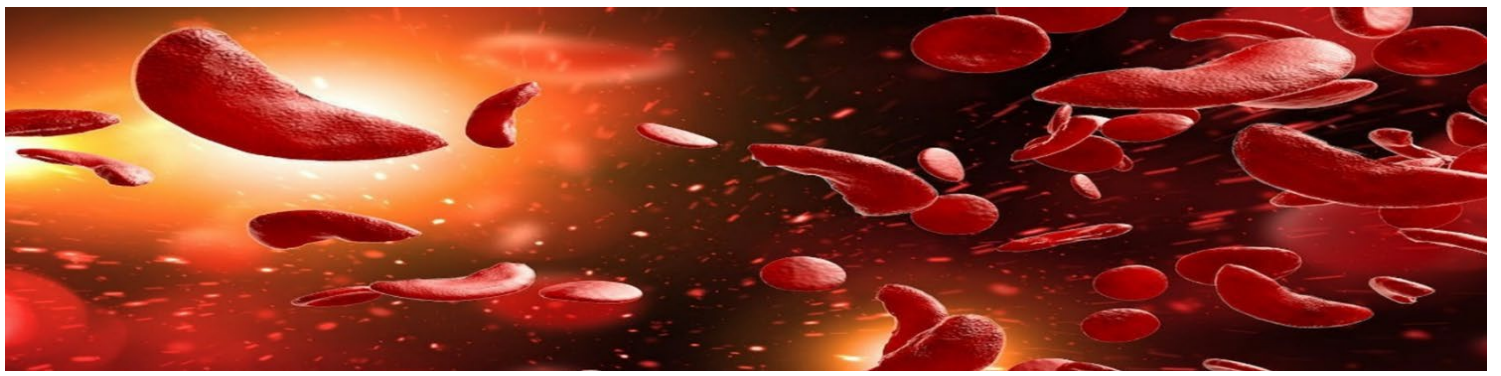
DAY 1 – Monday, August 14th

9:00 am-5:05 pm	MONDAY MEETING TIME (EDT) August 14th
9:00-9:10 am	<p>Welcome and Announcements <i>Dr. Julie Panepinto</i>, Director, Division of Blood Diseases & Resources, National Heart, Lung and Blood Institute, NIH</p> <p><i>Dr. Pankaj Qasba</i>, Division of Blood Diseases & Resources, National Heart, Lung and Blood Institute, NIH</p>
9:10 am-12:20 pm	SYMPOSIUM 1
9:10-10:40 am	<p>Curative Therapies for SCD- I Chair: <i>Dr. Lakshmanan Krishnamurti</i>, Yale University</p> <p>Parental Perspective on the Risk of Infertility and Fertility Preservation Options for Children and Adolescents With SCD Considering HCT <i>Dr. Cynthia B. Sinha</i>, Emory University</p> <p>Normalization of Cerebral Hemodynamics After HSCT in Children with SCD <i>Dr. Monica L. Hulbert</i>, Washington University</p> <p>Multimodal Phenotyping and Correlates of Pain Following Hematopoietic Cell Transplant in Children with Sickle Cell Disease <i>Dr. Nitya Bakshi</i>, Emory University</p> <p>Reduction in Vaso-Occlusive Events Following Stem Cell Transplantation in Patients with SCD <i>Dr. Alexis Leonard</i>, St. Jude Children’s Research Hospital</p> <p>Outcomes Following Posttransplant Virus-specific T-cell Therapy in Patients with SCD <i>Dr. Hannah Kinoshita</i>, Children’s National Hospital</p> <p>Long-Term Organ Function After HCT for SCD: A Report from the Sickle Cell Transplant Advocacy and Research Alliance <i>Dr. Elizabeth Stenger</i>, Emory University</p> <p>Increased Incidence of Hematologic Malignancies in SCD After HCT in Adults With Graft Failure and Mixed Chimerism <i>Dr. Rialnat A. Lawal</i>, National Heart, Lung and Blood Institute, NIH</p>

	<p>Secondary Neoplasms After HCT for SCD <i>Dr. Mary Eapen, Medical College of Wisconsin</i></p> <p>Female Reproductive Health Outcomes After HCT for SCD: Is Reduced Intensity Better Than Myeloablative Conditioning? <i>Dr. Lillian Meacham, Emory University</i></p>
10:40-10:50 am	BREAK
10:50 am -12:20 pm	<p>Curative Therapies for SCD- II (Gene Therapy/Editing) <i>Chair: Dr. Punam Malik, Cincinnati Children's Hospital</i></p> <p>Antibody conditioning for curative approaches to sickle cell disease <i>Dr. John Tisdale, National Heart, Lung and Blood Institute, NIH</i></p> <p>RIC Lentiviral Gene Therapy with a Modified HbF: Future Directions <i>Dr. Punam Malik, Cincinnati Children's Hospital</i></p> <p>Induction of fetal hemoglobin with Cas9 Nuclease and Base editors <i>Dr. Mitch Weiss, St Jude Children's Hospital</i></p> <p>Preclinical results on Base editing to reactivate fetal hemoglobin <i>Dr. Annarita Miccio, Imagine Institute, Paris</i></p> <p>Preclinical results on base editing and prime editing to modify the SCD mutation <i>Dr. Gregory Newby, Johns Hopkins University</i></p> <p>In Vivo Gene Editing for SCD <i>Dr. Hans-Peter Kiem, The Fred Hutch Cancer Research Centre</i></p> <p>Update on HDR-Editing of Autologous CD34+ Cells to Treat Sickle Cell Disease <i>Dr. Matt Porteus, Stanford University</i></p>
12:20-1:05	LUNCH
1:05-5:05 pm	SYMPOSIUM 2
1:05-2:50 pm	Updates on Trials on SCD
1:05-1:30 pm	<p>Proof-of-concept Study of Nicotinamide and Oral THU/Decitabine to Treat High Risk Sickle Cell Disease <i>Dr. Yogen Saunthararajah, Cleveland Clinic, Case Western Reserve University</i></p>

<p>1:30-2:10 pm</p>	<p>CRISPR SCD Gene Editing Trial Update <i>Dr. Mark Walters, University of California</i></p> <p>Post-transcriptional Silencing of BCL11A Using a Lentivirus shmiR Vector, the Phase 2 GRASP Trial <i>Dr. David Williams, Boston Children’s Hospital</i></p>
<p>2:10-2:50 pm</p>	<p>A Phase IIb Randomized, Double-Blind, Placebo-Controlled Multi-Center Study to Assess the <u>S</u>afety, <u>T</u>olerability, and <u>E</u>fficacy of <u>R</u>iociguat in Patients with <u>S</u>ickle <u>C</u>ell <u>D</u>isease: The Primary Results of the STERIO-SCD Trial <i>Dr. Mark Gladwin, University of Maryland School of Medicine</i> <i>Dr. Kaleab Abebe, University of Pittsburgh School of Medicine</i></p>

<p>2:50 – 3:00 pm</p>	<p>BREAK</p>
<p>3:00-3:20 pm</p>	<p>Enhancing Emergency Department Care for Children with SCD: An Implementation Science Approach <i>Dr. David Brousseau, Nemours Children’s Health, Delaware & Sidney Kimmel Medical College at Thomas Jefferson University</i></p>
<p>3:20-4:05 pm</p>	<p>Making the Case for Palliative Care in Sickle Cell Disease <i>Dr. Eberechi Nkele Nwogu-Onyemkpa, Baylor College of Medicine</i> <i>Dr. Stephanie Kiser, Harvard University</i> <i>Dr. Ashley Allen, Duke University</i></p>
<p>4:05-4:25 pm</p>	<p>HIV-1 Infection in Sickle Cell Disease <i>Dr. Sergei Nekhai, Howard University</i></p>
<p>4:25-4:45 pm</p>	<p>Panel of Multiple Urinary Biomarkers for the Early Detection of Chronic Kidney Disease in Sickle Cell Anemia SCORE: <i>Dr. Marina Jerebtsova, Howard University</i></p>
<p>4:45-5:05 pm</p>	<p>Stepping Up Into Adult Care: An Update on the ST3P UP Study <i>Dr. Ify Osunkwo, Novo Nordisk Rare Disease</i></p>
<p>5:05 pm</p>	<p>MEETING ADJOURNED FOR THE DAY</p>



DAY 2 – Tuesday, August 15th

9:00 am-5:15 pm	TUESDAY MEETING TIME (EDT)-August 15th
9:00-9:05 am	Welcome and Announcements
9:05-11:25 am	SYMPOSIUM 3
9:05-10:35 am	<p><u>Global SCD:</u></p> <p>SCD: Indian Scenario <i>Dr. Rabindra Kumar Jena, SCB Medical College & Hospital, Cuttack-India</i></p> <p>Implementation of Massive National SCD Programme in Indian Health Care System; Challenges and Solutions <i>Dr. Bontha V. Babu, National Institute for Implementation Research on NCDs-Jodhpur, India</i></p> <p>Translation of Research to Improve Health in Sickle Cell Disease: Anemia, Genomics, and Gene Therapy <i>Dr. Julie Makani, MUHAS, Dar-es-Salaam, Tanzania, Africa</i></p> <p>Recent Progress in Brazilian SCD <i>Dr. Carla Dinardo, Fundação Pró-Sangue São Paulo Hemocenter and University of São Paulo School of Medicine-Brazil</i></p>
10:25-10:35 am	Q & A
10:35-10:55 am	<p>The Global Gene Therapy Initiative (GGTI): Sickle Cell Disease as a Global Community <i>Dr. Jennifer Adair, Fred Hutchinson Cancer Center</i></p>
10:55-11:05 am	BREAK
11:05-11:25 am	<p>H3 Africa The SickleGenAfrica Cohort: Hemolysis Cytoprotective Phenotypes <i>Dr. Solomon Ofori-Acquah, University of Ghana</i></p>
11:25 am-5:00 pm	SYMPOSIUM 4
11:25 am-12:55 pm	<p><u>Building a TRIAD to Improve Transition in SCD</u> <i>Co-chairs: Dr. Payal Desai, Levine Center, Atrium Health and Dr. Elna Saah, Emory University School of Medicine</i></p> <p>The Issues Around Transition <i>Peggy Magnus, MHS, Dr. Patience White, and Jennifer Cornette- The Got Transition Team</i></p> <p>Practical Implementation Standard of Care in Transition <i>Dr. Elna Saah, Emory University School of Medicine</i></p> <p>TRIAD Fishbowl Engagement Discussion Between Pediatric/Adults/CBO: What Barriers are Identified to Building a TRIAD? <i>Drs. Raymona Lawrence, India Sisler, Jane Hankins, & Wanda Whitten-Shurney- Community Based Organization</i></p>
12:55-1:40 pm	LUNCH

12:55-1:40 pm	<p style="text-align: center;">LUNCH SESSION SPECIAL (Room A/B)</p> <p>Warrior Fishbowl - Lunch session special - only in breakout room A/B Patient/family panelist to discuss Transition Opportunities Facilitator: <i>Raymona Lawrence</i>, MPH Presenters: <i>Michelle Harlan, Nana Bilkisu Habib, Ugonna Anyadike, & Ayesha Habib</i></p>
1:40-2:00pm	<p>ASH Research Collaborative: Where are we now? <i>Dr. Charles Abrams</i>, University of Pennsylvania <i>Dr. Alexis Thompson</i>, Children’s Hospital of Philadelphia</p>
2:00-3:00pm	<p>A Study of Sickle Cell Disease and Spleen Function through Modeling and Experiments Integrating <i>In Silico</i>, <i>In Vitro</i>, <i>Ex Vivo</i> and <i>In Vivo</i> Approaches <i>Dr. George Karniadakis</i>, Brown University <i>Dr. Pierre Buffet</i>, Institute Pasteur <i>Dr. Ming Dao</i>, MIT</p>
3:00-3:10 pm	BREAK
3:10-3:50 pm	<p><u>TOPMed Updates</u> Unbiased Plasma Proteomics Analysis Uncovers Potential Biomarkers for Renal Disease and Pulmonary Hypertension in Patients With Sickle Cell Disease. <i>Dr. Allison Ashley-Koch</i>, Duke University</p> <p>Blood Gene Expression Profile of Sickle Cell Trait <i>Dr. Mari Johnson</i>, The Fred Hutch Cancer Research Centre</p>
3:50-4:10 pm	<p>Development of Biomarkers for Acute and Chronic Pain in SCD Using Omics <i>Dr. Vivien Sheehan</i>, Emory University</p>
4:10-4:30 pm	<p>Endothelialized Microfluidic Assays for Emerging Therapies in Sickle Cell Disease <i>Dr. Umut Gurkan</i>, Case Western Reserve University</p>
4:30-4:50 pm	<p>Cost-Effectiveness of Gene Therapy for Sickle Cell Disease: Accounting for Impacts Beyond Healthcare <i>Dr. Scott Ramsey</i>, Hutchinson Institute for Cancer Outcomes Research</p>
4:50-5:10 pm	<p>Heterogeneity of Magnetic Susceptibility, and Correspondingly, Oxygen Saturation, of RBC in Non-Transfused, SS, SC, And SB+ Patients <i>Dr. Jeffrey Chalmers</i>, Ohio State University</p>
5:10 pm	MEETING ADJOURNED FOR THE DAY



DAY 3 – Wednesday, August 16th

9:00 am-5:00 pm	WEDNESDAY MEETING TIME (EDT)-August 16 th
9:00-9:05 am	Welcome and Announcements
9:05 am-1:10 pm	SYMPOSIUM 5
9:05-9:25am	Leveraging the SCDIC Registry Population and Infrastructure for Ancillary Studies <i>Dr. Tabitha Hendershot, RTI International</i>
9:25-9:45am	REAL ANSWERS: A Target Trial Emulation Study Built on the SCDIC Registry Data Architecture <i>Dr. Jeffrey Glassberg, Icahn School of Medicine at Mount Sinai</i>
9:45-10:00 am	Program: Inviting Blood Related Clinical Trials to NHLBI <i>Dr. Andrei Kindzelski, Division of Blood Diseases & Resources, National Heart, Lung and Blood Institute, NIH</i>
10:00- 11:30	<u>National Alliance of Sickle Cell Centers Consensus Recommendations for Screening for People Living with Sickle Cell Disease</u> Co-chairs: <i>Dr. Julie Kanter, University of Alabama, Birmingham</i> and <i>Dr. Deepa Manwani, The Children’s Hospital at Montefiore</i> Consensus Recommendations for Children with SCD ages 0-2 <i>Dr. Seethal Jacob, Indiana University</i> Consensus Recommendations for Children and adolescents with SCD ages 2-18 <i>Dr. Melissa Frei-Jones, South Texas Comprehensive Sickle Cell Program</i> Consensus Recommendations for Adults with SCD <i>Dr. Andrew O’Brien, Indiana Lifespan Sickle Cell Center at Indiana University Health</i>
11:30:11:40	BREAK
11:40 am-1:10 pm	<u>Integrative Interventions Targeting Peripheral and Central Mechanisms of Pain in SCD</u> Co-chairs: <i>Dr. Kalpna Gupta, and Dr. Shiu-Lin Tsai</i> Disease Modifying and Nociceptive Mechanisms Underlying Sickle Cell Pain. Promising Interventions Waiting for Clinical Translation <i>Dr. Kalpna Gupta, University of California</i> Acupuncture for Pediatric Sickle Cell Pain Management: A Promising Non-Opioid Therapy <i>Dr. Shiu-Lin Tsai, Columbia University</i> Musculoskeletal Pain in SCD: Underlying Mechanisms and Challenges for Treatment <i>Dr. Jahnvi Gollamudi, University of Cincinnati</i> Music-Based Interventions for Pain <i>Dr. Sonya Wang, University of Minnesota</i>
1:10-1:55 pm	LUNCH
1:55-5:00 pm	SYMPOSIUM 6

1:55-2:35 pm	<p><u>ALIGN</u> Improving Emergency Department Care of VOE by Embedding Individualized Pain Plans in the Electronic Health Record for Providers AND Patients: A Report from the ALIGN Trial <i>Dr. Allison King, Washington University</i></p> <p><u>COMPARE</u> A Comparison of Patient Specific vs. Weight Based Opioid Pain Plans to Treat VOE in the ED: Results From the COMPARE-VOE Trial <i>Dr. Paula Tanabe, Duke University</i></p>
2:35-2:55 pm	<p>Prevalence and Risk Factors of Thromboembolism in Women with Sickle Cell Disease During Pregnancy <i>Dr. Shreya Agarwal, University of California San Francisco</i></p>
2:55-3:15 pm	<p>Sickle Cell Disease Treatment with Arginine Therapy (STArT) <i>Dr. Claudia R. Morris, Emory University</i></p>
3:15-3:35 pm	<p>Emerging Therapeutics for Sickle Cell Disease <i>Dr. Modupe Idowu, UTHealth Houston McGovern Medical School</i></p>
3:35-3:45 pm	BREAK
3:45-4:05 pm	<p>PhenX toolkit: a Resource for Sickle Cell Disease Research <i>Dr. Wayne Huggins, RTI International</i> <i>Dr. Carol Hamilton, RTI International</i></p>
4:05-4:50 pm	<p>Sickle Cell Data Collection Updates <i>Dr. Mary Hulihan, Center for Disease Control and Prevention</i></p> <p>Sickle Cell Data Collection Program: Overview, Approach, and Progress <i>Dr. Matt Smeltzer, The University of Memphis</i></p> <p>Pregnancy And Disease-Related Morbidity Among Medicaid Enrollees With Sickle Cell Disease <i>Dr. Mariam Kayle, Duke University School of Nursing</i></p> <p>The Impact of COVID-19 on People With Sickle Cell Disease <i>Dr. Sarah Reeves, Susan B. Meister Child Health Evaluation and Research Center (CHEAR), University of Michigan</i></p> <p>Sickle Cell Data Collection Program: Moving Forward <i>Dr. Mary Hulihan, Centers for Disease Control and Prevention, Division of Blood Disorders</i></p>
4:40-4:50 pm	Q & A
4:50-5:00 pm	Closing Remarks
5:00 pm	MEETING ADJOURNED