



**DORIS DUKE CHARITABLE FOUNDATION
INNOVATIONS IN CLINICAL RESEARCH AWARD PROGRAM
CATALIZING BREAKTHROUGHS IN SICKLE CELL DISEASE RESEARCH, DIAGNOSIS AND TREATMENT**

2009 grantees

Selective Inhibition of HDAC1 and HDAC2 in Sickle Cell Disease

James E. Bradner, MD

Dana Farber Cancer Institute

PFAST: Patent Forament Ovale and Stroke in Sickle Cell Disease

Michael M. Dowling, MD, PhD

University of Texas Southwestern Medical Center at Dallas

Clinical development of histone deacetylase inhibitors for the treatment of sickle cell disease

Benjamin L. Ebert, MD, PhD and Maureen Okam, MD, MPH

Brigham and Women Hospital

B-globin gene correction in hematopoietic stem cells for sickle cell disease

Donald B. Kohn, MD and Philip Gregory, PhD

University of California, Los Angeles and Sangamo Biosciences, Inc.

Whole-exome re-sequencing in sickle cell disease patients with extremely mild clinical course

Guillaume Lettre, PhD and Joel N. Hirschhorn, MD, PhD

Montreal Heart Institute, and Children's Hospital Boston

2010 grantees

Erythrocyte Hydration Pathways as Modifiers in Sickle Cell Disease

Patrick G. Gallagher, MD

Yale University School of Medicine

Identification of Novel Factors and Mechanisms Influencing Expression of Fetal Hemoglobin

Derek A. Persons, MD, PhD and Andy C. Wilber, PhD

St. Jude Children's Research Hospital and Southern Illinois University of Medicine

Genetic Predictors of Cerebrovascular Disease in Sickle Cell Anemia

Russell E. Ware, MD, PhD

St. Jude Children's Research Hospital

2011 grantees

Molecular identification and inhibition of the deoxygenation-activated, calcium-permeable cation channel of the sickle erythrocyte, Psickle, a novel therapeutic target for treatment of sickle disease

Seth L Alper, MD, PhD

Beth Israel Deaconess Medical Center

NRF2 induction as novel treatment for sickle cell disease

Jen-Tsan A. Chi, MD, PhD & Marilyn J. Telen, MD

Duke University

Genomic Approaches to Prevent Red Blood Cell Alloimmunization in Patients with Sickle Cell Disease

Stella T Chou, MD & Connie M. Westhoff, PhD

Children's Hospital of Philadelphia and New York Blood Center

Modeling Sickle Cell Anemia with Induced Pluripotent Stem Cells

George Q Daley, MD, PhD

Children's Hospital Boston

Effects of the Adenosine 2A Receptor Agonist Regadenoson on Sickle Cell Vaso-occlusion and Inflammation

Joshua J Field, MD, MS & Jonathan Lindner

The Medical College Of Wisconsin and Oregon Health Sciences University

Nanoparticle-mediated correction of the sickle cell disease mutation

Peter M. Glazer, MD, PhD

Yale University

Reversal of Sickle Cell-Related Chronic Kidney Disease

Antonio Guasch, MD and Marianne E.M. Yee, MD, MSc

Emory University

Effect of Simvastatin Treatment on Vaso-occlusive Pain in Sickle Cell Disease

Carolyn C Hoppe, MD, MPH

Children's Hospital & Research Center Oakland

Preclinical evaluation of globin gene transfer in mobilized SCD patient CD34+ cells

Michel Sadelain, MD, PhD & Patricia A. Shi, MD, MS

Memorial Sloan-Kettering Cancer Center and New York Blood Center

2012 grantees

Targeted derepression of fetal hemoglobin in sickle cell disease

James E. Bradner, MD

Dana Farber Cancer Institute

Translating genetic discoveries to improve sickle cell disease prognosis and treatment

Guillaume Lettre, PhD and Joel N. Hirschhorn, MD, PhD

Montreal Heart Institute, Children's Hospital Boston

Genomic and Functional Analyses of Erythrocyte Hydration Pathways as Modifiers in Sickle Cell Disease

Patrick G. Gallagher, MD

Yale University School of Medicine

2013 grantees

Genome editing of the GWAS-marked BCL11A enhancer: an approach to HbF reactivation in sickle cell disease

Daniel E. Bauer MD, PhD and Stuart H Orkin, MD

Children's Hospital Boston

Harvard Medical School

Examination of human samples with somatic mutations in hematopoietic stem cells to inform the biology of fetal hemoglobin induction

Benjamin L. Ebert MD, PhD and Maureen M. Okam MD, MPH

Brigham and Women's Hospital

Harvard Medical School

'SCD Biochip': Towards a Simple and Reliable Way to Monitor Sickle Cell Disease

Umut A. Gurkan, PhD and Jane A. Little, MD

Case Western Reserve University

Feasibility and efficacy of a home-based, computerized cognitive training program in pediatric sickle cell disease

Steven J. Hardy, PhD and Kristina K. Hardy, PhD

Children's National Medical Center

Novel use Of Hydroxyurea in an African Region with Malaria

Chandy C. John, MD

Indiana University

The effects of hypoxia on red blood cell-dependent thrombin generation in sickle cell disease

Nigel S. Key, MB ChB and Kenneth Mann, MD

University of North Carolina at Chapel Hill and University of Vermont

Site-Specific Gene Modification in Hematopoietic Stem Cells for Sickle Cell Disease

Donald B. Kohn, MD

Regents University Of California Los Angeles

Gene Therapy for Sickle Cell Anemia

Punam Malik, MD

Cincinnati Children's Hospital Medical Center

Targeting Neutrophil Extracellular Traps in Sickle Cell Disease

Leslie V. Parise, PhD and Bruce A. Sullenger, PhD

University of North Carolina at Chapel Hill and Duke University Medical Center

Accurate and Inexpensive Point-of-Care Diagnosis of Sickle Cell Anemia

Rebecca R. Richards-Kortum, PhD

William Marsh Rice University

Risk Stratification for Clinical Severity of Sickle Cell Disease in Nigeria and Assessment of Efficacy and Safety during Treatment with Hydroxyurea

Bamidele Tayo, PhD and Victor R. Gordeuk, MD

Loyola University of Chicago and University of Illinois at Chicago

2015 grantees

High-resolution and high-throughput genome editing to determine minimal repressive sequences within the beta-globin gene cluster and establish lead compounds for therapeutic re-induction of fetal hemoglobin in SCD

Daniel E. Bauer, MD, PhD and Stuart S. Orkin, MD

Children's Hospital Boston

Expanding genomic approaches to transfusion therapy for patients with sickle cell disease

Stella T. Chou, MD and Connie M. Westhoff, PhD

Children's Hospital of Philadelphia and New York Blood Center

High-throughput Metabolite Profiling and Genetic Analyses to Identify Novel Predictive Biomarkers of SCD-related Complications

Marilyn J. Telen, MD and Guillaume Lettre, PhD

Duke University and Montreal Heart Institute

Genetic variants influencing the phenotypic expression of sickle cell anemia

Russell E. Ware, MD, PhD

Cincinnati Children's Hospital Medical Center