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Seven Projects Pursuing Cures for Sickle Cell Disease Win \$6M in Research Support

The Doris Duke Charitable Foundation's Medical Research Program Awards Grants Through Inaugural Sickle Cell Disease/Advancing Cures Awards

New York, N.Y., September 14, 2017 – The Doris Duke Charitable Foundation today announced the seven projects receiving approximately \$6 million total through the inaugural [Sickle Cell Disease/Advancing Cures](#) awards competition. The researchers won support for the potential of their projects to address the underlying cause of sickle cell disease, attacking it at its core by restoring hemoglobin function through various approaches. These projects hold great promise to develop disease-modifying approaches to sickle cell disease and translate them into clinically feasible therapies, aiming to remove patients' need to manage the disease's acute, devastating and lifelong symptoms and ultimately to increase their life expectancy.

This announcement coincides with National Sickle Cell Awareness Month, which began in 1983 to foster public awareness about the genetic disease that researchers estimate affects between 90,000 to 100,000 Americans. It is the most common inherited blood disorder in the United States. Patients with sickle cell disease carry dysfunctional red blood cells that alter regular blood flow, which translates into pain, poor organ oxygenation and organ damage, and a life span of only about 40 years.¹

"Those with sickle cell disease often experience chronic pain and crippling side effects beginning as early as childhood," said Betsy Myers, program director for medical research at DDCF. "We applaud recent scientific progress and advances in drug development to address the disease, yet acknowledge that there is still significant work to be done at the foundational, curative levels of research. We are excited to be launching the Sickle Cell Disease/Advancing Cures awards in support of these outstanding research projects. We are hopeful and expectant that their findings will promote development of curative treatments and improve patients' lives."

The foundation has supported sickle cell disease research through a variety of grant-making mechanisms, including the Innovations in Clinical Research Award (ICRA), which began in 2009 and has helped enrich the field with projects on disease biology, management and treatment. The launch of the Sickle Cell Disease/Advancing Cures awards thus builds upon years of learning from these previously funded projects and seeks to capitalize on discoveries that allow for further investment in approaches that specifically target sickle cell disease's underlying cause.

¹ Lanzkron, S., Carroll, C.P., Haywood C., Jr. Mortality rates and age at death from sickle cell disease: U.S., 1979-2005. Public Health Rep. 2013 Mar-Apr;238(2):110-6.

Doris Duke, who endowed the foundation and for whom it is named, had a particular personal interest in supporting sickle cell disease research. She articulated this desire in her will, which in part guides the foundation's funding priorities.

For a list of this year's Sickle Cell Disease/Advancing Cures grant recipients and their projects, please see page 3.

About the Doris Duke Charitable Foundation

The mission of the Doris Duke Charitable Foundation is to improve the quality of people's lives through grants supporting the performing arts, environmental conservation, child well-being and medical research, and through preservation of the cultural and environmental legacy of Doris Duke's properties. The foundation's Medical Research Program supports clinical research that advances the translation of biomedical discoveries into new preventions, diagnoses and treatments for human diseases. To learn more about the program, visit www.ddcf.org.

2017 Sickle Cell Disease/Advancing Cures Awardees

Category of Research: Genome Modification

Eric E. Bouhassira, Ph.D.

Albert Einstein College of Medicine

Project name: Characterization of the Stem and Progenitor Cell Compartment in Sickle Cell Disease and Optimization of Gene Transfer at AAVS1 Site in CD49f+ LT-HSCs

Will build on CRISPR/Cas9 technology and on unique characteristics of blood-forming cells of patients with sickle cell disease to improve their genetic correction and potential for successful transplant back in patients so that hemoglobin function is restored.

Donald B. Kohn, M.D.

University of California, Los Angeles

Project name: Optimizing Gene Editing for Sickle Cell Disease

Will use CRISPR/Cas9 to optimize repair of the sickle cell disease mutation in blood-forming cells of patients with the disease to restore the cells' ability to produce normal hemoglobin.

Mitchell J. Weiss, M.D., Ph.D., and Shengdar Q. Tsai, Ph.D.

St. Jude Children's Research Hospital

Project name: Genome Editing of Bone Marrow and Plerixafor-mobilized CD34+ Cells to Raise Fetal Hemoglobin Levels in Sickle Cell Disease

Will use CRISPR/Cas9 to generate a safe, effective and practical clinical approach to achieve production of fetal hemoglobin and overcome effects of the abnormal sickle hemoglobin.

Category of Research: Drug Development

Stuart H. Orkin, M.D., and Daniel E. Bauer, M.D., Ph.D.

Boston Children's Hospital

Project name: Small Molecule Targeted Reactivation of HbF Expression for Sickle Cell Disease

Will use medicinal chemistry and CRISPR/Cas9 to direct drug development efforts targeting modulators of fetal hemoglobin production.

Patrick T. McGann, M.D., M.S.

Cincinnati Children's Hospital Medical Center

Project name: Maximizing Fetal Hemoglobin Responses to Hydroxyurea Using Precision Medicine

Will conduct a clinical trial to study a personalized regime to dose hydroxyurea, the only currently approved drug for sickle cell disease, and boost production of fetal hemoglobin in children with sickle cell disease.

Patrick M. Woster, Ph.D.

Medical University of South Carolina

Project name: Epigenetic Modulators for the Treatment of Sickle Cell Disease

Will refine the structure of new drug-like molecules to generate drug candidates that induce fetal hemoglobin production.

Category of Research: Bone Marrow Transplant

Allistair A. Abraham, M.D., and Robert S. Nickel, M.D.

Children's National Health System and the George Washington University School of Medicine and Health Sciences

Project name: Minimizing Toxicity in HLA-identical Sibling Transplantation for Children with Sickle Cell Disease

Will work to improve bone marrow transplant by reducing the toxicity of the treatment for children with a matched sibling.