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## Six Projects Pursuing Cures for Sickle Cell Disease Win \$5M in Research Support

### The Doris Duke Charitable Foundation Awards Second Round of Grants Through *Sickle Cell Disease/Advancing Cures* Program

**New York, September 10, 2019** – The Doris Duke Charitable Foundation today announced the six projects receiving approximately \$5 million through the second Sickle Cell Disease/Advancing Cures grant competition. Four of the researchers who won support for their projects will study aspects of gene editing that could improve its successful clinical application in patients with sickle cell disease. The other two researchers were awarded grants to investigate new drug-like molecules and potential new drug targets to promote expression of functional hemoglobin. Adult hemoglobin is a protein in red blood cells that is mutated in sickle cell disease, which disrupts its ability to transport oxygen through the body.

“With the rise of CRISPR gene editing and many other promising developments, this is an exciting time for sickle cell disease research and an opportunity to make important strides toward delivering a cure,” said Betsy Myers, Program Director for Medical Research at the Doris Duke Charitable Foundation. “We are thrilled to support these clinical researchers as they harness the power of recent progress in the field in efforts to develop new ways to advance gene therapies and restore red blood cell function.”

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, and approximately 300,000 people worldwide are born with sickle cell disease each year. 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.<sup>1</sup>

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 Sickle Cell Disease/Advancing Cures awards program was first launched in 2017 to build upon years of learning from these previously funded projects and seek to capitalize on discoveries that allow for further investment in approaches that specifically target sickle cell disease’s underlying causes. The second peer reviewed competition of the Sickle Cell Disease/Advancing Cures awards is meant to further build upon this investment by supporting research that explores aspects of gene modification and drug discovery that could accelerate cures.

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<sup>1</sup> 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](http://www.ddcf.org).

## 2019 Sickle Cell Disease/Advancing Cures Awardees

### *Category of Research: Gene Editing*

**Daniel E. Bauer, M.D., Ph.D. and Alex Kentsis, M.D., Ph.D.**

Assistant Professor of Pediatrics and Assistant Member, Assistant Professor, Attending Physician  
*Dana-Farber/Boston Children's and Memorial Sloan Kettering Cancer Center*

Project name: Enhanced nuclease delivery for therapeutic gene editing of hematopoietic stem cells in sickle cell disease

**James J. Bieker, Ph.D. and Jeffrey A. Glassberg, M.D.**

Professor and Associate Professor

*Icahn School of Medicine at Mount Sinai*

Project name: Quantitative modulation of an erythroid regulator as a novel genetic target for sickle cell disease

**Punam Malik, M.D.**

Professor, Division of Hematology, Director, Cincinnati Children's Comprehensive Sickle Cell Center  
*Cincinnati Children's Hospital Medical Center*

Project name: Reducing error-prone repair for therapeutic correction of sickle cell anemia

**Matthew H. Porteus, M.D., Ph.D.**

Professor of Pediatrics

*Stanford University School of Medicine*

Project name: Optimization of gene correction using genome editing in human hematopoietic cells

### *Category of Research: Promoting Expression of Fetal Hemoglobin*

**Stuart H. Orkin, M.D.**

David G. Nathan Professor of Pediatrics, Harvard Medical School, Investigator, Howard Hughes Medical Institute

*Dana-Farber/Boston Children's*

Project name: Structure-based small molecules for HbF reactivation

**Shuaiying Cui, Ph.D.**

Assistant Professor of Medicine, Boston University

*Boston Medical Center*

Project name: Inhibition of LSD1 by small molecule inhibitors stimulates fetal hemoglobin synthesis for sickle cell disease