



NEWS RELEASE

For Immediate Release

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DORIS DUKE
CHARITABLE FOUNDATION

Medical Research Program

National Competition Attracts Innovative Researchers to Advance Treatments and Cures for Sickle Cell Disease

Five Projects to Receive Grants Totaling \$2.43 Million Over Three Years

NEW YORK, September 30, 2009—To reinvigorate funding and research collaborations targeting sickle cell disease and to tap into recent advances in biomedical research, the Doris Duke Charitable Foundation (DDCF) launched in April a national Innovations in Clinical Research Award competition to help catalyze new breakthroughs in sickle cell disease. DDCF announced today the five projects selected to receive three-year grants of \$486,000 each (see page 2 for a list of awardees).

The Doris Duke Innovations in Clinical Research Award (ICRA) provides seed funding for early-stage, multi-disciplinary clinical research projects. The 2009 ICRA grants will support a range of approaches to improving the health of patients with sickle cell disease, including:

- Identifying new drugs to more effectively treat sickle cell disease;
- Testing new strategies to identify and treat patients at risk for severe neurologic complications;
- Understanding the genetic basis for variations in the severity of sickle cell disease to better predict its clinical course and improve treatment options;
- Advancing efforts to find a cure by using gene correction to repair the sickle mutation in the gene that causes the disease.

In the 1980s and early 1990s, management of sickle cell disease improved greatly with advances such as screening of newborns, the use of antibiotics to prevent infections in infants and young children, and treatment with the anticancer drug hydroxyurea – which remains the only FDA-approved drug available for treating sickle cell disease. The 2009 ICRA grants aim to spur new advances by enabling researchers to apply expertise and innovations from other fields like genetics, chemistry and cardiology to improve the health of sickle cell patients.

Many of the more than 70,000 people living with sickle cell disease in the United States – most of whom are African-American – face a lifetime of painful, debilitating and expensive health problems, with a much-shortened life expectancy. Sickle cell disease takes an even heavier toll in Africa, where approximately 200,000 babies are born with the disease each year.

“Sickle cell anemia is a hugely important problem worldwide. It is exciting and fitting that the Doris Duke Charitable Foundation is taking a strong stand in an attempt to find a solution to this scourge,” said Dr. David Nathan, physician-scientist and chair of the DDCF Medical Research Program’s Scientific Advisory Council. Dr. Nathan was among the first to demonstrate that hydroxyurea significantly reduces sickle cell disease symptoms and complications.

“We are excited to support the investigators leading these five research projects, which hold great promise for developing improved treatments and cures for sickle cell patients,” said Ed Henry, president of the Doris Duke Charitable Foundation. “We hope the results of this competition and the innovative work of our grantees over the next three years will help revitalize interest in pursuing new breakthroughs in sickle cell disease.”

The foundation received 81 applications from 52 teams of investigators for the 2009 Innovations in Clinical Research Award competition. A panel of six experts reviewed the proposals and recommended the strongest candidates for funding. The panel was co-chaired by Dr. Edward J. Benz, President of the Dana-Farber Cancer Institute; and Dr. David Ginsburg, Professor at the University of Michigan and member of the DDCF Medical Research Program’s Scientific Advisory Council.

Since 1998, the foundation’s Medical Research Program has committed approximately \$185 million to strengthen and support clinical research, which advances the translation of basic biomedical discoveries into new treatments, preventions and cures for human diseases. To learn more about the program or to receive competition announcements, visit www.ddcf.org/mrp.

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, medical research and the prevention of child maltreatment, and through preservation of the cultural and environmental legacy of Doris Duke’s properties.

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2009 Doris Duke Innovations in Clinical Research Award Recipients
(listed alphabetically by last name)

James E. Bradner, MD
Dana-Farber Cancer Institute
Selective Inhibition of HDAC1 and HDAC2 in Sickle Cell Disease

Michael M. Dowling, MD, PhD
University of Texas Southwestern Medical Center at Dallas
PFAST: Patent Foramen Ovale and Stroke in Sickle Cell Disease

Benjamin L. Ebert, MD, PhD and Maureen Okam, MD, MPH
Brigham and Women’s Hospital
Clinical Development of Histone Deacetylase Inhibitors for the Treatment of Sickle Cell Disease

Donald B. Kohn, MD and Philip Gregory, PhD
University of California, Los Angeles and Sangamo Biosciences, Inc.
B-globin Gene Correction in Hematopoietic Stem Cells for Sickle Cell Disease

Guillaume Lettre, PhD and Joel N. Hirschhorn, MD, PhD
Montreal Heart Institute and Children’s Hospital Boston
Whole-exome Re-sequencing in Sickle Cell Disease Patients with Extremely Mild Clinical Courses