PRESS RELEASE

DORIS DUKE CHARITABLE FOUNDATION

Medical Research Program

For Immediate Release

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Doris Duke Charitable Foundation Announces $4.4 Million in New Grants to Support Innovative Clinical Research in Sickle Cell Disease

New York, NY, Dec. 16, 2011 – The Doris Duke Charitable Foundation's Medical Research Program today announced $4.4 million in new grants to research projects with the potential to accelerate breakthroughs in the treatment of sickle cell disease—an often painful and life-shortening genetic blood disorder affecting one in 400 African Americans. These grants are the product of the 2011 competition for the Doris Duke Innovations in Clinical Research Award (ICRA), which invited proposals for cutting edge, clinical research focused on making advancements in sickle cell anemia. Nine projects were selected and will receive $486,000 each over three years.

"We are pleased to support these exciting projects, which have the potential to improve the lives of patients with sickle cell disease and advance our knowledge of other genetic diseases," said Betsy Myers, director of the Medical Research Program. "While the clinical characteristics and the genetic basis of this disease have been known for decades, few advances have been made in its treatment or the prevention of its life-threatening complications. With no widely available cure, we believe that new research into this disorder merits this investment."

The selected projects seek to develop new therapies or to increase understanding of how to treat the debilitating and devastating symptoms that accompany sickle cell disease. The investigators come from a wide variety of fields, including adult and pediatric hematology, nephrology, radiation oncology and pathology. Project highlights include:

- Developing a human model for red-cell sickling using induced stem cells, led by George Q. Daley, Children's Hospital Boston;
- Delivering a correction of the sickle cell gene defect using nanotechnology, led by Peter M. Glazer, Yale University and;
- Identifying DNA markers to better match blood donors for transfusions, which is the primary treatment for sickle cell disease complications, led by co-principal investigators Stella T. Chou of Children's Hospital of Philadelphia and Connie M. Westhoff, of the New York Blood Center.

A complete list of the 2011 ICRA awardees is on page 3.

About the Innovations in Clinical Research Award (ICRA)

ICRA, a competitive grant program that began in 2000, provides seed funding for early-stage, multi-disciplinary clinical research projects. Through ICRA, the Medical Research Program has funded 56 projects, ranging from HIV diagnostic development to cardiovascular disease research, with a total commitment of approximately $16 million.

For the 2011 ICRA competition, the Medical Research Program received 85 applications from investigators at 60 research institutions, which were reviewed by a panel of 12 experts in blood disorders. The 2009 ICRA competition also focused on sickle cell disease.

About Sickle Cell Disease

Sickle cell disease is both a national and global health concern. Many of the more than 70,000 people living with sickle cell disease in the United States face a lifetime of painful, debilitating and expensive health problems, with a much-shortened life expectancy. Sickle cell disease takes an even heavier toll abroad, where an estimated 230,000 children are born with the disease each year in sub-Saharan Africa alone.

Symptoms originate, through mechanisms that are not entirely understood, from the circulation of abnormally shaped sickle red blood cells throughout the body. Sickle-shaped red blood cells clump in blood vessels thereby obstructing normal blood flow to vital organs including the brain, lungs—resulting in strokes and a high susceptibility to respiratory and other types of infections. Obstructed blood supply to tissues also results in episodes of extreme pain.

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, medical research and the prevention of child abuse and neglect, and through preservation of the cultural and environmental legacy of Doris Duke's properties. Since 1998, the foundation's Medical Research Program has sought to advance the prevention, prediction, diagnosis and treatment of human disease by strengthening and supporting clinical research.

To learn more about the Medical Research Program or to sign up to receive competition announcements, visit www.ddcf.org/mrp.

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2011 Doris Duke Innovations in Clinical Research Award Recipients
(listed alphabetically by last name)

Seth L. Alper, M.D., Ph.D.
Beth Israel Deaconess Medical Center
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

Jen-Tsan A. Chi, M.D., Ph.D. & Marilyn J. Telen, M.D.
Duke University
NRF2 Induction as Novel Treatment for Sickle Cell Disease

Stella T. Chou, M.D. & Connie M. Westhoff, Ph.D.
Children's Hospital of Philadelphia/New York Blood Center
Genomic Approaches to Prevent Red Blood Cell Alloimmunization in Patients with Sickle Cell Disease

George Q. Daley, M.D., Ph.D.
Children's Hospital Boston
Modeling Sickle Cell Anemia with Induced Pluripotent Stem Cells

Joshua J. Field, M.D., MS & Jonathan Lindner, M.D.
The Medical College of Wisconsin, Inc/ Oregon Health Sciences University
Effects of the Adenosine 2A Receptor Agonist Regadenoson on Sickle Cell Vaso-occlusion and Inflammation

Peter M. Glazer, M.D., Ph.D.
Yale University
Nanoparticle-Mediated Correction of the Sickle Cell Disease Mutation

Antonio Guasch, M.D. & Marianne E.M. Yee, M.D., M.Sc.
Emory University
Reversal of Sickle Cell-Related Chronic Kidney Disease

Carolyn C. Hoppe, M.D., M.P.H.
Children's Hospital and Research Center Oakland
Effect of Simvastatin Treatment on Vaso-occlusive Pain in Sickle Cell Disease

Michel Sadelain, M.D., Ph.D. & Patricia A. Shi, M.D., MS
Memorial Sloan-Kettering Cancer Center/New York Blood Center
Preclinical Evaluation of Globin Gene Transfer in Mobilized SCD Patient CD34+ Cells

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