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Improving Life for Kids With Sickle Cell Anemia

Watertown, MA, February 2, 2016 – New England Research Institutes (NERI) has been awarded a federal research contract from the National Heart, Lung and Blood Institute (NHLBI) to complete a study with promising treatment for sickle cell anemia in children that will address gaps in the best possible care. The contract is co-funded by NHLBI and the National Institute of Child Health and Human Development (NICHD) for $3.6 million over two years. With more than 30-years of experience in sickle cell disease, NERI was sought after because of its expertise in conducting research with rare diseases, pediatric clinical trials, and, specifically, with pediatric patients suffering from sickle cell disease.

The study NERI has undertaken will look at the long-term effects of using the drug hydroxyurea in children that could provide practice changing results. Hydroxyurea is the only treatment for sickle cell anemia shown to modify the actual disease process (rather than simply treating symptoms). Although it has been FDA-approved for use in adults with sickle cell disease since 1998, this study could provide a labeling expansion for use in young children. Existing studies suggest that if all patients with sickle cell disease used hydroxyurea, the number of hospitalizations for the disease could be cut in half.[1]

"This new award aligns with NERI's extensive expertise in clinical research on rare diseases, pediatric trials, and regulatory strategy to benefit children with sickle cell disease, who are now underserved by existing treatment options," says Sandra Siami, MPH, NERI's VP of Clinical & Regulatory Affairs.

Approximately 155 children with sickle cell anemia are involved in the research. The children were screened at study entry for signs of abnormal brain, kidney, pulmonary, and splenic function, and developmental milestones. They were randomly assigned to receive either hydroxyurea or placebo and followed yearly to assess the drug's effects, if any, on major organ systems.

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[1] Moore RD. et al. Cost-effectiveness of hydroxyurea in sickle cell anemia. Investigators of the Multicenter Study of Hydroxyurea in Sickle Cell Anemia. *Am J Hematol.* 2000;64(1):26–31.

NERI scientists Susan Assmann, PhD, and Julie Miller, MPH, will serve as co-Principal Investigators on the new research.

About Sickle Cell Disease

Sickle cell disease is a collection of genetic diseases that alter the structure of hemoglobin, the oxygen-carrying molecule in red blood cells. The name of the disease reflects the tendency of red blood cells to take on characteristic sickle shapes under certain conditions. When red blood cells take the sickle form, they can clump together, blocking blood vessels and producing a wide range of health problems, depending on where in the body the sickling occurs. For example, sickling in the lungs causes a condition known as acute chest syndrome while sickling in the long bones causes a condition known as a painful crisis. About 72,000 people in the U.S. have sickle cell disease.

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About NERI

NERI is a global, privately-held specialty Contract Research Organization providing customized clinical trial solutions and patient registry services to pharmaceutical, biotechnology, biomaterial and medical device companies. NERI also has extensive experience collaborating on federally-funded research with organizations like the National Institutes of Health. Since its founding in 1986, NERI has earned widespread recognition for its scientific credibility, efficiency, and expertise in conducting clinical trials in a variety of medical specialties. For more information, visit www.neriscience.com.