Comprehensive Sickle Cell Center

Cincinnati Children's
changing the outcome together

The Comprehensive Sickle Cell Center at Cincinnati Children's is a national leader in caring for children, adolescents and young adults with sickle cell disease. The team includes six pediatric hematologists whose main areas of interest are sickle cell disease treatment and research. They collaborate with a multidisciplinary team of nurse practitioners, nurse care managers, psychologists, social workers, school intervention specialists, and others to help children with sickle cell disease thrive despite challenges to their health.

CONTACT US

For patient referrals:

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BEST CHILDREN'S HOSPITALS
U.S.News & WORLD REPORT
HONOR ROLL
2022-23

Cincinnati Children's is #3 in the nation among Honor Roll hospitals.

INNOVATIVE THERAPIES AND EXTENSIVE SUPPORT FOR CHILDREN WITH SICKLE CELL DISEASE

The Comprehensive Sickle Cell Center provides advanced, evidence-based care and novel treatments, many of which were developed at Cincinnati Children's. Our partnerships with national research organizations, including the National Institutes of Health (NIH), provide patients with access to innovative therapies.

- Patients benefit from careful monitoring and early interventions aimed at preventing pain and avoiding complications. These interventions include the pre-symptomatic, preventive use of hydroxyurea therapy, beginning as early as six months of age.
- More than 90% of our eligible patients are prescribed hydroxyurea, compared to the national average of less than 50%. We achieve this high level of treatment through intensive patient education by our medical team, with support from our social workers and behavioral medicine specialists.
- During the past five years, nearly 100% of our eligible patients have received transcranial Doppler screening by 24 months of age, in accordance with national guidelines, to prevent stroke. This very high rate of adherence reflects our commitment to prevent organ damage and improve outcomes.
- We partner with Hoxworth Blood Center in Cincinnati, one of the nation's premier blood centers, to offer state-of-the art methods of transfusion to reduce iron overload and minimize the long-term risks of transfusions.
- Patients requiring hospitalization are admitted to a dedicated inpatient unit at Cincinnati Children's and seen by many of the same care team members they see in clinic.
- Our center is the regional coordinating center for Sickle Treatment and Outcomes Research in the Midwest (STORM), a federally funded learning network for hematologists and primary care providers across 8 midwest states. As part of the STORM Network, the Comprehensive Sickle Cell Center hosts monthly ECHO telementoring for healthcare providers.
- Our Center is the Ohio Department of Health Sickle Cell Services Region 1 project that coordinates regional newborn screening follow-up testing, education and counseling for all hemoglobin disorders in southwestern Ohio.

LEADERSHIP

Punam Malik, MD
Director, Comprehensive Sickle Cell Center; Marjory J. Johnson Chair, Gene and Cell Therapy

Charles Quinn, MD, MS
Medical Director, Pediatric Sickle Cell Program; Medical Director, Erythrocyte Diagnostic Laboratory

15 countries

Our sickle cell team treats patients from 15 countries.

GLOBAL OUTREACH

In sub-Saharan Africa, many children born with sickle cell disease die before their fifth birthday and nearly 90% never reach adulthood. One reason is that few are properly diagnosed early in life, and even fewer have access to disease-modifying therapy. In 2014, **Russell Ware, MD, PhD**, initiated a prospective, multi-center trial called REACH (Realizing Effectiveness Across Continents with Hydroxyurea) to assess the feasibility, safety and benefits of hydroxyurea use in sub-Saharan Africa. The study, published in *The New England Journal of Medicine (January 10, 2019)*, demonstrated exciting results among 600 children from the Democratic Republic of Congo, Uganda, Kenya and Angola. Now, Ware and his colleagues at Cincinnati Children's are working with the NIH and partner organizations in Africa to expand access to this low-cost therapy while monitoring longer-term outcomes. Dr. Ware also led the NOHARM study (Novel use Of Hydroxyurea in an African Region with Malaria) in Uganda, and recently published in NEJM (June 25, 2020) that a higher dose of hydroxyurea was clinically superior to a lower fixed-dose. These findings set the stage for personalized dosing of hydroxyurea in Africa, which extends the global impact of the Comprehensive Sickle Cell Center.

ADVANCED DIAGNOSTIC AND THERAPEUTIC TESTING

The Erythrocyte Diagnostic Laboratory at Cincinnati Children's provides comprehensive, state-of-the-art testing, interpretation and consultation. Special testing includes:

- Capillary zone electrophoresis for the identification of hemoglobin and quantification of fetal hemoglobin
- ADVIA testing to assess cell size and density
- F-cell analysis to measure how fetal hemoglobin is distributed across all red blood cells
- Pharmacokinetic studies to determine a personalized dose of hydroxyurea
- Ektacytometry to measure red blood cell deformability, flexibility and point of sickling
- Whole blood viscosity to determine how well blood flows
- Gene-based testing to identify a patient's specific diagnosis and genetic modifiers of their disease course

Out partner, Hoxworth Blood Center in Cincinnati, provides extended RBC phenotyping and RBC genotyping to prevent alloimmunization in transfused patients.

CLINICAL AND LABORATORY RESEARCH TO IMPROVE PATIENT OUTCOMES

Cincinnati Children's researchers **Charles Quinn, MD, MS, Russell Ware, MD, PhD**, and **Alexander Vinks, PharmD, PhD** lead the Therapeutic Response Evaluation and Adherence Trial (TREAT). This research has successfully identified a method to determine a sickle cell patient's personalized dose of hydroxyurea in a matter of hours instead of common practice, which otherwise may take six to 12 months. Study participants—many of them under two years of age—experienced significantly higher treatment responses than those achieved with traditional weight-based dosing. Now in long-term follow-up, these patients have sustained, significant increases in fetal hemoglobin and they remain mostly asymptomatic.

Punam Malik, MD, is principal investigator of a groundbreaking clinical trial that is testing gene transfer therapy as a disease-modifying therapy and potential cure for sickle cell disease. Early data, presented at the American Society of Hematology's 2021 annual meeting and other research meetings, continue to be promising, showing near elimination of chronic pain and severe sickling events and improved anemia in the first two adult patients treated. Six patients have received this therapy to date.

Researchers at Cincinnati Children's are exploring a variety of clinical and translational research initiatives. **Charles Quinn, MD, MS**, and **Omar Niss, MD** are studying therapies that may prevent the acquisition and progression of heart and kidney disease in sickle cell disease. **Theodosia Kalfa, MD, PhD**, studies the structure and function of red blood cells in the laboratory to learn how they are affected by sickle cell disease and to develop ways to improve their function in a variety of blood diseases.

Luke Smart, MD is a hematologist trained in both pediatric and adult medicine who cares for people with sickle cell disease across the lifespan: children at Cincinnati Children's and adults at U.C. Health.

082022_BRV163497