



ASH Research Collaborative and Reagan-Udall Foundation for the FDA Launch Sickle Cell Disease Real-World Data Project

The Real-World Evidence Consortium for Sickle Cell Disease will develop consensus on how to use data collected from electronic health records for research and to improve care

(WASHINGTON, April 17, 2024) –The ASH Research Collaborative (ASH RC) and the Reagan-Udall Foundation for the Food and Drug Administration (FDA Foundation) are launching the Real-World Evidence Consortium for Sickle Cell Disease (SCD) to develop consensus recommendations on clinical outcomes important to treating people with SCD and apply those standards to real-world data sets. The goal of the Consortium is to improve the lives of individuals living with SCD through real-world evidence generation and cutting-edge research.

“Real-world evidence holds tremendous promise to accelerate research by overcoming the limitations of traditional clinical trials,” said Mark A. Crowther, MD, MSc, FRCPC, FRSC, chair of the ASH RC Board of Directors and chair of the Department of Medicine at McMaster University. “Sickle cell disease research and treatments have been historically underfunded and overlooked, and it’s time we better understood clinical outcomes.”

SCD is the most common inherited red blood cell disorder in the United States, affecting an estimated 100,000 people. According to the Centers for Disease Control and Prevention, SCD affects one out of every 365 Black or African American births and one out of every 16,300 Hispanic American births.

“Driving innovation and working toward the creation of new clinical data standards and approaches to evidence generation is a hallmark of the Foundation’s work,” said Richard L. Schilsky, MD, FACP, FSCT, FASCO, Chair of the FDA Foundation Board of Directors. “We’re eager to begin this collaboration that has the potential to lead to better health outcomes for this historically poorly served patient population.”

The consortium will establish consensus on the use of electronic health record (EHR) data to better understand the natural history and outcomes of people with SCD through a collaboration among SCD researchers, clinicians, patients, industry representatives, federal regulators, and informaticians.

There are over 300,000 codes that define different conditions and symptoms in EHRs. Unlike a clinical trial that observes patients and collects a limited set of standardized data accordingly, the codes used to define a condition in the real world may be more difficult to identify, standardize and interpret. By defining which codes indicate clinical characteristics of SCD, researchers will be able to more quickly and accurately use EHR data to accelerate SCD research.

The ASH RC and FDA Foundation represent the intersection of clinical research, clinical care, regulatory affairs, and industry partnerships, which uniquely positions these organizations to drive change. The ASH RC's Data Hub currently contains data from over 25,000 individuals living with SCD and will be used to validate the standards once established.

The ASH RC is working to accelerate change by making it more efficient to conduct research, from increasing access to high-quality clinical data to making it easier for individuals with hematologic conditions to participate in studies.

The FDA Foundation is an independent 501(c)(3) organization that aims to advance the mission of the FDA to modernize product development, accelerate innovation, and enhance product safety.

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About the American Society of Hematology

The American Society of Hematology (ASH) (hematology.org) is the world's largest professional society of hematologists dedicated to furthering the understanding, diagnosis, treatment, and prevention of disorders affecting the blood. Since 1958, the Society has led the development of hematology as a discipline by promoting research, patient care, education, training, and advocacy in hematology.

The *Blood* journals (<https://ashpublications.org/journals>) are the premier source for basic, translational, and clinical hematological research. The *Blood* journals publish more peer-reviewed hematology research than any other academic journals worldwide.

About the ASH Research Collaborative

The ASH Research Collaborative (ASH RC) is a non-profit organization established by the [American Society of Hematology](http://hematology.org) (ASH) to improve the lives of people affected by blood diseases by fostering collaborative partnerships to accelerate progress in hematology. The foundation of the ASH RC is its [Data Hub](#) and [Clinical Trials Network](#). Through the ASH RC's state-of-the-art data-sharing platforms and patient-centric approach to enrollment, design, and execution of clinical trials for SCD, the ASH RC is making it quicker and more efficient for companies to develop new treatments to help those suffering from hematologic conditions, and to maximize the value of those treatments post-approval. The ASH RC aims to transform research and practice in malignant and classical hematologic diseases throughout the world, for the benefit of patients and the hematology community.

About the Reagan-Udall Foundation for the FDA

The Reagan-Udall Foundation for the FDA (FDA Foundation) is an independent 501(c)(3) created by Congress to advance regulatory science to help the U.S. Food and Drug Administration accomplish its mission. The FDA Foundation works to improve health and safety through stakeholder engagement and

public-private partnerships that facilitate innovation, foster the use of real-world evidence, and identify modern tools and policies to keep pace with today's rapidly evolving science.