

February 20, 2020
Institute for Clinical and Economic Review
Two Liberty Square
Boston, MA 02109

RE: The Draft Evidence Report for the Treatment of Sickle Cell Disease

Dear Dr. Pearson,

On behalf of the tens of thousands of individuals living with sickle cell disease (SCD) in the United States, we write to provide public comment on the Sickle Cell Disease: Draft Evidence Report. We appreciate ICER bringing attention to SCD and the drastic need for treatment, with great assistance from the sickle cell disease community. We acknowledge and thank the organization for seeking input from patients and patient advocacy organizations, and gathering the patient perspective on this devastating disease. However, given the sparsity of evidence currently available, we recommend for ICER to suspend their review. This will acknowledge the significant shortcomings of the analysis and the need for additional time to ensure adequacy of the report. We encourage your organization to act responsibly and avoid producing a report that may unintentionally introduce, perpetuate or exacerbate inequities. We recommend rescheduling the final report to take place in line with [the timeline proposed by the National Institute for Health and Care Excellence \(NICE\)](#) for review of these sickle cell disease treatments.

Sickle cell disease is a devastating condition robbing people of their lives every single day, a battle that is made worse by stigma and prejudice towards those who are impacted. While racism and discrimination are acknowledged in the report as monumental barriers to care for sickle cell patients, the economic model fails to incorporate these important factors. Without access to real-world data on how these new treatments impact vulnerable populations, your report will inaccurately measure benefit and in turn can result in inequitable denial of access. Equity needs to be more systematically considered by incorporating these complex historical and sociological processes into the base case model. We reiterate our comments from previous letters that ICER is conducting this review without ample long-term or patient experience data. Conversely, simplifying the model without inclusion of these factors will underestimate the magnitude of health and economic burden associated with sickle cell disease.

Pain is a difficult outcome to measure due to its multifaceted and subjective nature. Societal factors and bias influence beliefs, perceptions, and behaviors, all of which have important implications on treatment and care. The way an individual interprets the pain, or learns to cope with its unpredictability, can greatly moderate the way pain affects his or her life. Given these challenges to measure chronic pain, the measurement of acute vaso-occlusive crises (VOCs) are often the focus of clinical trials. These utilize available data in medical records to document patients' encounters with the health system for treatment of pain. However, VOCs are only one of the many complex complications that impact patients' quality and longevity of life. Likewise, patients report that VOCs are sometimes not the most impairing parts of their sickle cell disease, rather other complications like chronic pain, fatigue, and sleep disturbances have greater impacts. Real-world data that capture how these drugs impact not only VOCs but other effects beyond the restrictive design of clinical trials are critical in order to assess the true value of these treatments. We hope that you consider this recommendation to suspend the review of these sickle cell disease

treatments. In doing so, our community would look forward to furthering our collaboration with your organization and partnering to ensure the most accurate real-world data are available prior to finalizing this assessment.

Sincerely,

Axis Advocacy

Cayenne Wellness Center and Children's Foundation

Dreamsickle Kids Foundation, Inc.

Hope for SCD

International Association of Sickle Cell Nurses and Professional Associates

Kids Conquering Sickle Cell Disease, Inc.

The Martin Center Sickle Cell Initiative

Maryland Sickle Cell Disease Association

MTS Sickle Cell Foundation

New York State Sickle Cell Advocacy Network

Ohio Sickle Cell Affected Families Association

A Precious Organization for Sickle Cell

RedMoon Project, Inc.

SCD Forum

Sick Cells

Sickle Cell Association of St. Louis

Sickle Cell Awareness 365

Sickle Cell Community Consortium

Sickle Cell Disease Association of America, Michigan Chapter, Inc

Sickle Cell Disease Association of America Ohio Sickle Cell and Health Association

Sickle Cell Disease Association of America, Philadelphia/Delaware Valley Chapter

Sickle Cell Disease Association of America, St. Petersburg Chapter

Sickle Cell Disease Association of Illinois

The Sickle Cell Experience

Sickle Cell Foundation of Minnesota

Sickle Cell Thalassemia Patient Network

Sickled Not Broken Foundation of Nevada

Supporters of Families with Sickle Cell Disease, Inc.

SWGA Sickle Cell Awareness

Tova Community Healthy, Inc.

Uriel Owen Sickle Cell Disease Association of the Midwest

Individual Community Stakeholders:

Pat Corley, RN, Adult Sickle Cell Disease Nurse

Dr. Lewis Hsu, MD, Pediatric Hematology/Oncology, Chicago, IL

Dr. Charles Otieno, MD, Emergency medicine physician, California

Dr. Ahmar U. Zaidi, MD, Pediatric Hematology/Oncology Physician, Detroit, Michigan