

Blood, Bias, and Breakthroughs: Rethinking the Sickle Cell Experience

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Guiding Questions for Reflection

Use these questions to spark discussion or personal reflection before, during, or after the lecture.

Rare Disease Research

- What factors influence how research funding and resources are allocated across different diseases and conditions, including sickle cell disease?
- How can differences in research investment affect patient outcomes, access to care, and the pace of medical innovation?
- What historical, cultural, or structural factors have shaped the experience of people who are living with sickle cell disease?

Advancements in Treatment

- What role can health professionals, educators, researchers and institutions play in advancing awareness, research and support for people affected by rare diseases?
- How can health systems, policymakers, and communities help ensure that emerging treatments are accessible and affordable for those who need them most?
- What responsibilities do we have to ensure that advances in medicine reduce, rather than widen, existing health disparities?
- What does patient-centered care look like for individuals living with chronic conditions such as sickle cell disease?

This guide accompanies the lecture “Blood, Bias, and Breakthroughs: Rethinking the Sickle Cell Experience” presented as part of the Health C.A.R.E (Cultural Awareness, Research, and Education) Series. We invite you to reflect on the evolution of treatment for sickle cell disease, and explore how health disparities have affected research and clinical care.

Recommended Resources

Explore these articles, podcasts, and tools to deepen your understanding:

Reducing Health Care Disparities in Sickle Cell Disease: A Review

As an orphan disease—one that affects <200 000 persons nationwide—sickle cell disease (SCD) does not receive the research funding and pharmaceutical investment directed to other orphan diseases. For example, cystic fibrosis affects fewer than half the number of persons but receives 3.5 times the funding from the National Institutes of Health and 440 times the funding from national foundations. In this review, we discuss the health inequities affecting persons with SCD, describe programs intended to improve their care, and identify actions that could be taken to further reduce these inequities, improve care, control treatment costs, and ease the burden of disease.

[Read more at Public Health Reports](#)

Medicaid Has a New Way to Pay for Costly Sickle Cell Treatment: Only If It Works

New treatment offers a potential cure for many of the 100,000 primarily Black Americans with sickle cell disease, which is estimated to shorten lifespans by more than two decades. But the treatment's cost presents a steep financial challenge for Medicaid, the joint state-federal government insurer for people with low incomes or disabilities.

[Read more at NPR](#)

The Dawn of a Cure for Sickle Cell Disease through CRISPR-Based Treatment

Sickle cell disease (SCD) is an inherited monogenic red blood cell disorder that affects hemoglobin, the protein that carries oxygen through the body. Globally, the African continent carries the highest burden of SCD with at least 240,000 children born each year with the disease. Recently, the UK medical regulator approved its cure through clustered regularly interspaced short palindromic repeat (CRISPR)-based treatment, whereas the US Food and Drug Administration has equally approved two SCD gene therapies. This CRISPR-based treatment is expensive and therefore, a need for an ambitious action to ensure that they are affordable and accessible where they are needed most and stand to save millions of lives.

[Read more at Annals of Human Genetics \(UVM login required\)](#)