

KNOWLEDGE BRIEF CAPACITY GENE THERAPY AND THE FUTURE OF SICKLE CELL CURE IN AFRICA

October 20, 2025

Based on the JCRC Webinar: "In Their Own Words: Two Africans Cured of Sickle Cell Disease by Gene Therapy"

THE BREAKTHROUGH



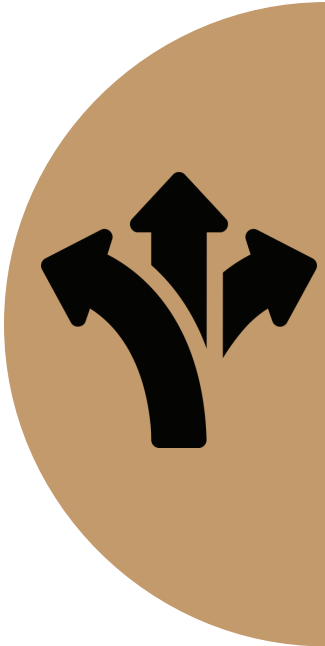
A CRISPR-based gene editing therapy, such as CASGEVY, provides a functional cure for Sickle Cell Disease by targeting the BCL11A gene regulator, permanently activating Fetal Hemoglobin (HbF) production, preventing red blood cell sickling, and restoring normal blood function.

"My hemoglobin level rose to 13 g/dL after therapy- no sickle crises." – Allan Byamukama, cured patient



"About 5 years ago I had the good fortune of participating in a crisper-based gene therapy for sickle cell disease which... I want to be careful with using the word cured but has uh functionally cured me."

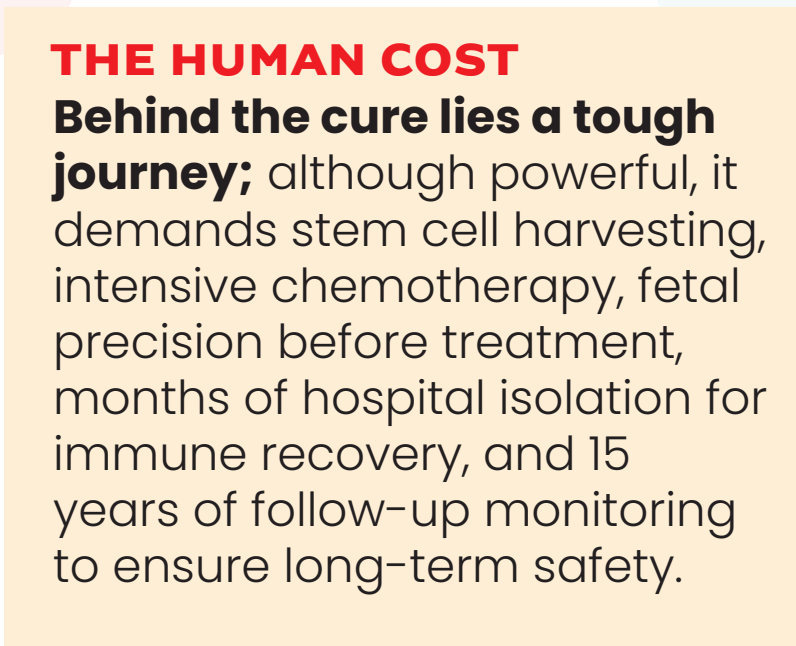
— Jimi Olaghere



WHAT MAKES IT DIFFERENT

Key Area Educational Insight

Molecular Precision Targets BCL11A, reactivating HbF safely and permanently. Clinical Proof Patients now show sustained normal hemoglobin levels. Preventive Impact Stops further organ and bone damage caused by sickling.



THE HUMAN COST

Behind the cure lies a tough journey; although powerful, it demands stem cell harvesting, intensive chemotherapy, fetal precision before treatment, months of hospital isolation for immune recovery, and 15 years of follow-up monitoring to ensure long-term safety.



THE MORAL DILEMMA

The \$2.2 million gene therapy, currently limited to high-income countries, poses deep moral and equity challenges for Africa, prompting critical questions: can African patients ever access such advanced cures, and what will it take to reach our hospitals?



THE AFRICAN CHALLENGE & ADVOCACY AGENDA

The science is ready, but Africa must catch up through fair pricing, infrastructure investment in centers like JCRC, global biotech partnerships for knowledge transfer, and awareness campaigns targeting policymakers and funders.



KEY TAKEAWAY

"The age of symptomatic SCD management in Africa must end. The only missing variable is political will." **To watch the entire webinar discussion, please scan the QR code provided here.**