

## **“Sickle Cell Disease and Gene Therapies: A Two-Part Webinar Series”**

### **Part 1—Trust, Clinical Trials, and Transformative Therapies: Ethical Pathways in Gene Therapy and Sickle Cell Disease**

Thursday, September 18, 2025, 1-2:30 PM ET

**Background:** Sickle cell disease (SCD) is an inherited blood disorder that affects approximately 100,000 Americans and is characterized by severe pain crises, multi-organ damage, and decreased life expectancy. Although the FDA approved two breakthrough, potentially curative, SCD gene therapies in 2023, uptake of these therapies has been modest.

One issue has been historical mistrust among the SCD community coupled with inconsistent education regarding federal safeguards and the integrity of medical research. Another concern is their high cost (\$2M-\$3M per treatment course), which is a focus of the Centers for Medicare & Medicaid Services (CMS) Cell and Gene Therapy Access Model.

This two-part webinar series will highlight strategies, best practices, and federal programs designed to enhance SCD gene therapy education and access. Part 1 of this series will also identify ethical considerations for SCD research.

Part 1 is hosted by the HHS Office for Human Research Protections. (OHRP).

Part 2 is hosted by the HHS Office of Minority Health (OMH).

#### **Agenda**

#### **Part 1—Trust, Clinical Trials, and Transformative Therapies: Ethical Pathways in Gene Therapy and Sickle Cell Disease**

**Thursday, September 18, 2025, 1-2:30 PM ET (90 minutes)**

<b>Welcome</b>	<i>Jonathan Green, MD, MBA (Moderator)</i> Director, Office of Human Subjects Research Protections National Institutes of Health
<b>Opening Remarks</b>	HHS Principal TBD
<b>Talk #1 “Parent/Caregiver Perspective on Gene Therapy”</b>	<i>Antuan Sartin</i> Father of 12-year-old daughter with SCD recently approved for gene therapy Louisville, KY
<b>Talk #2 “Responsibilities for the Ethical Conduct of SCD Research”</b>	<i>Wally Smith, MD</i> Florence Neal Cooper Smith Professor of Sickle Cell Disease and Vice-Chair for Research Division of General Internal Medicine Virginia Commonwealth University School of Medicine Richmond, VA

<b>Panel Discussion</b>	<p>Dr. Green, Mr. Sartin, Dr. Smith, and 2 additional panelists</p> <p><i>Lakshmanan Krishnamurti, MD</i>  Professor of Pediatrics  Chief of the Section of Pediatric Hematology/Oncology/Bone Marrow Transplantation  Yale School of Medicine  New Haven, CT</p> <p><i>Megha Kaushal, MD, MSc</i>  Branch Chief, Division of Clinical Evaluation Hematology  Office of Therapeutic Products  Center for Biologics Evaluation and Research  Food and Drug Administration</p>
<b>Q&amp;A</b>	All
<b>Closing</b>	<p><i>Natalie Klein, PhD</i>  Acting Director, Office for Human Research Protections  U.S. Department of Health and Human Services</p>