



MEMO

Date: May 31, 2023
To: External
From: Sick Cells

RE: May 2023 Policy Memo

Though we moved through appropriations season quickly this year, the topic of federal budgeting continues to dominate policy conversations. Regardless, Sick Cells has continued to work on various initiatives at the federal level to raise awareness for sickle cell disease and ensure federal agencies and the Hill are providing support where it's needed. This month's highlights include touchpoints with the FDA, CMCS, CDC, and CMMI, and a Kentucky Medicaid meeting.

1. Sick Cells **participated in a Patient Listening Session (PLS)** with the U.S. Food and Drug Administration (FDA) alongside the [Sickle Cell Disease Foundation](#), the [SCDAA](#), [SC RED](#), and 6 Warrior/caregiver representatives.
 - a. President & Co-Founder Ashley Valentine spoke on what matters most to patients with SCD in clinical trials and research, developing community partnerships, and future opportunities for the FDA. Sick Cells recommendations included:
 - i. Help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation
 - ii. Encourage use of community partnerships for collecting and communicating data
 - iii. Collaborate across agencies to improve patient access to FDA-approved therapies
 - iv. Recognize the need for increased linguistically diverse populations in clinical trials
 - v. Increase clinical trial opportunities for older individuals with SCD
 - b. A formal read-out of the event will be shared in next month's memo.

2. Sick Cells **met with staff at the Center for Medicare & Medicaid Innovation (CMMI)** to discuss the [Cell & Gene Therapy Access Model](#) and community engagement. Our comments and recommendations focused on:
 - a. Disease Management, Cost, Access, Metrics & Measurements, Stakeholder Collaboration
 - b. We provided additional agency-related recommendations including, but not limited to:
 - i. CMS Centers should continue developing models for existing SCD treatments to address continuing issues in access and affordability.
 - ii. CMS Centers should ensure that models for gene therapy address patient-important outcomes typically excluded from clinical trial reporting.



3. Sick Cells **joined a meeting with staff from the Center for Medicaid & CHIP Services (CMCS)** to introduce the [Sickle Cell Disease Partnership](#), highlight opportunities for state Medicaid solutions, and discuss current federal legislation.
 - a. Emma Andelson, Senior Program Manager - Government Affairs & External Relations, led a portion of the Q&A with CMCS staff to learn more about cross-agency collaboration.
4. Emma Andelson **presented at a Centers for Disease Control & Prevention (CDC) [Sickle Cell Data Collection \(SCDC\) Program](#) meeting** on “Social Media for Advocacy & Education.”
 - a. The presentation focused on streamlining advocacy messaging, digital visual cohesion, and effectively communicating complex data or policy topics to wide audiences.
5. Sick Cells **trained one advocate** to participate in a [Kentucky P&T Advisory Committee](#) Meeting.
 - a. Representing the [Sickle Cell Association of Kentuckiana](#), this advocate shared their personal experience as a caregiver to many SCD Warriors and community leaders. They requested that all SCD treatments be listed as preferred and that prior authorization and step therapy be removed.
 - b. The committee recommended that Endari™ be preferred (with prior authorization and step therapy) and Oxbryta® be non-preferred (with prior authorization and step therapy). Other drugs in the class were not reviewed.

State Highlights

1. **Louisiana** | INTRODUCED: [HR201](#) | “Creates a subcommittee to study the effectiveness of sickle cell healthcare treatment practices in this state”
 - a. Sponsor: Rep. Adrian Fisher (D-LA-16)
 - b. The first meeting is to be held by September 15, 2023
 - c. A recommendations & findings report will be submitted to the House Committee on Health & Welfare by February 1, 2024
2. **Louisiana** | INTRODUCED: [HCR92](#) | “Requests study by Louisiana Department of Health to consider Medicaid coverage of gene therapy” (for sickle cell disease)
 - a. Sponsor: Rep. Jason Hughes (D-LA-100)
 - b. Requests that the LA DoH study “innovative financing models” to provide access to potential gene therapies by the end of 2023
 - c. Report to be submitted no later than 30 days before the next Assembly session