SICKLE CELL DISEASE
PARTNERSHIP

Via Electronic Submission

May 6, 2025

Dr. Mehmet Oz
Administrator, Centers for Medicare and Medicaid Services
U.S. Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244

Dear Administrator Oz,

The Sickle Cell Disease Partnership[i] is a public policy and advocacy collaboration of patient advocates, health care providers, biopharmaceutical manufacturers, and other health care stakeholders committed to advancing policies that will improve the lives of those living with Sickle Cell Disease (SCD). In 2022, the Sickle Cell Disease Partnership ("Partnership") was founded under the leadership of Trump Administration former HHS Assistant Secretary for Health, Brett Giroir, M.D.

The goal of the SCD Partnership is to support the SCD community in translating the Trump Administration-funded blueprint for SCD[ii] into concrete policy improvements by working with Congress and the Administration to effectuate policy and program improvements. The Partnership is pleased to see the significant progress made to improve access to care and treatment for Americans with SCD,[iii] thanks to bold leadership under President Trump's first Administration.

The Partnership appreciates the clinical expertise you bring to your role at the Centers for Medicare and Medicaid Services (CMS) and notes your experience providing care for individuals with SCD. We also appreciate your personal recognition that SCD is "a devasting disease" and your acknowledgement that the agency's Cell and Gene Therapy (CGT) Access Model is an innovative treatment model.[iv]

As you may know, various authorities at CMS work together to play a critical role in ensuring quality care for Americans living with SCD. In particular, Medicaid is the primary insurer for more than half of all individuals with SCD,[v] providing essential coverage that enables access to comprehensive care, pain management, and potentially transformative treatments through the CMS Innovation Center's Gene Therapy Access Model. As you examine how to leverage these authorities to improve care and boost outcomes, the Partnership looks forward to working with you and your team over the next four years.

<u>Medicaid Covers Half of Americans with SCD</u>

As you know, SCD is a chronic, debilitating disease that is also the most common inherited blood disorder in the United States.[vi] Although it is a rare disease, SCD affects approximately 100,000 Americans. Individuals living with SCD face severe health complications, including recurring and life-altering pain crises, repeat infection, acute chest syndrome, lung problems, severe and chronic pain, leg ulcers, organ damage, and stroke. Due to inadequacies in comprehensive care management, only about

1 in 4 patients with SCD receive a standard of care described in guidelines,[vii] resulting in individuals with SCD needing to visit the emergency department far more often than the average population.[viii]

These and other factors increase the cost of care for Americans with SCD and on the system overall. As a result of systemic inadequacies and the severity of the disease, individuals with SCD have an estimated life expectancy that is dozens of years shorter than the average expected life expectancy in the nation.

The importance of continuous Medicaid coverage for SCD cannot be overstated. Approximately 52,524 Medicaid enrollees had SCD in 2021,[v] representing about half of all Americans with SCD. Further, about 49 percent of these individuals have severe forms of the disease requiring more frequent medical care.[ix] When coverage is inconsistent, individuals with SCD face barriers to accessing the medical care they need, leading to heightened health care costs and higher emergency room utilization. For example, without continuous coverage, SCD patients become reliant on emergency services, contributing to $2.4 billion in avoidable annual ED costs.[x]

CMS Innovation Center's Cell and Gene Therapy Access Model

The Partnership appreciates the Administration's continued interest in and support for including SCD as the first therapeutic focus of the Cell and Gene Therapy (CGT) Access Model ("Access Model"). As you know, SCD was selected as the initial focus for this Model following the FDA's December 2023 approval of two gene therapies (Casgevy and Lyfgenia), marking the first approvals in this new therapeutic class. Under the Access Model, CMS directly negotiates with drug manufacturers for increased cost savings primarily through outcomes-based agreements between manufacturers and states who opt-in to the Model. The Access Model holds great promise to increase access to potentially transformative treatments, better support value and budget predictability, and provide needed support to state Medicaid programs.

For individuals with SCD who already face significant health challenges and shortened life expectancies, the potential of the Access Model represents a hopeful opportunity to improve care delivery and health outcomes by leveraging historic advancements in gene therapy. We understand implementation of the model continues to be underway at CMS, with many states having opted in. The Partnership looks forward to continuing to work with CMS to be a resource throughout Model implementation.

Proposed CMS Quality Measure

Given inadequacies in comprehensive health care and treatment, individuals with SCD tend to visit the Emergency Department (ED) more often than the average population.[xi] From 1999-2020, Medicaid and Medicare covered 72 percent of Emergency Department (ED) visits of individuals with SCD.[xii] And 75 percent of the yearly average ED visits are due to a complaint of pain.[xiii]

Mindful of the frequency of ED visits and the lack of any metrics to assess the timeliness of ED care, the Partnership recently expressed its support[xiv] for the American Society of Hematology's (ASH) new quality measure in the Hospital Outpatient Quality Reporting Program and the Rural Emergency Hospital Quality Reporting Program, entitled: *Median Time to Pain Medication for Patients with a Diagnosis of Sickle Cell Disease with Vaso-Occlusive Episode (VOE)*.[xv] CMS has been undergoing a review of the measure, which ASH proposed in partnership with a panel of clinical and patient experts. Using Digital-Electronic Health Record Data, ASH's proposed quality measure would assess the median time, in minutes, from when an individual with SCD arrives to the ED until the individual is first administered pain medication during the ED encounter.

The Partnership encourages CMS to adopt the quality measure as an essential first step in enabling stakeholders to assess the national trends in management of SCD in the ED. Assessing the timing to administration of pain management for adult and pediatric patients presenting to the ED with SCD will not only impact pain management, but has the potential to also impact admission rates, length of ED stay, hospital length of stay, and patient satisfaction.[xvi]

Legislative Proposal to Enhance Comprehensive Care Through Medicaid Health Homes

As CMS considers effective and efficient policies to support individuals with SCD, the Partnership believes CMS should be aware of a bipartisan Congressional proposal that improves access to comprehensive, coordinated, and quality care for individuals with SCD. The Partnership is advocating[xvii] for the *Sickle Cell Disease Comprehensive Care Act*, S. 721,[xviii] sponsored by Sens. Cory Booker (D-NJ) and Tim Scott (R-SC) in the 119th Congress. This legislation would incentivize states to use Medicaid Health Homes to provide coordinated care to enrollees with SCD, regardless of whether such enrollee previously participated in a Health Home. Medicaid Health Homes are a proven and effective tool to reduce unnecessary expenditures and improve care outcomes in Medicaid.[xix]

The Congressional Budget Office evaluated the policies included in the legislation and concluded the policies would improve care and reduce federal Medicaid expenditures. CBO ultimately found that the net effect of the policies would produce federal and state Medicaid savings given lower hospital utilization with better care coordination for beneficiaries with SCD. The Partnership urges you to support a Medicaid Health Home model for individuals based on their SCD status alone, along with other policies to improve care coordination for SCD patients.

CDC's Sickle Cell Data Collection Program and Its Importance to CMS' Access Model

The Partnership also notes the recent reduction in force (RIF) at the Centers for Disease Control and Prevention's (CDC) Division of Blood Disorders and Public Health Genomics (the "Blood Division"). The Blood Division oversees the Sickle Cell Data Collection Program (Data Collection Program), which is the only national surveillance mechanism for SCD.[xx] First funded by Congress in 2019 under the leadership of sickle cell champions in the first Trump Administration, the Data Collection Program has since grown to include 16 states, including many states among those with the highest prevalence of SCD.

We understand the breadth of data provided by the Data Collection Program indirectly supports CMS' evaluation of programs for SCD, including the Access Model. For example, the Data Collection Program identifies geographic concentrations of SCD patients and indicates who might be qualified to undergo gene therapy – which is critical to the success of the Model. Currently, there is uncertainty surrounding the Data Collection Program, as personnel with historical knowledge of the program and communities it serves were placed on administrative leave. The Partnership asks that CMS work with CDC and HHS leadership to ensure the longevity of the Data Collection Program to enable the best usage of its data, including those that are relevant to the Access Model and at CMS generally.

Contact Information

We look forward to collaborating with CMS and the Trump Administration to continue to improve access to care and treatment for Americans with SCD. Please reach out to Advisors to the Partnership, Josh Trent (Josh.Trent@LeavittPartners.com), Clay Alspach (Clay.Alspach@LeavittPartners.com), or Liz Hassett (Elizabeth.Hassett@LeavittPartners.com) with any questions.

Sincerely,

The Sickle Cell Disease Partnership

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[i] https://www.sicklecellpartnership.org/

[ii] https://www.nationalacademies.org/our-work/addressing-sickle-cell-disease-a-strategic-plan-and-blueprint-for-action

[iii] https://www.sicklecellpartnership.org/wp-content/uploads/2024/06/SCD-Progress-Report.pdf

[iv] https://www.finance.senate.gov/imo/media/doc/responses_to_questions_from_the_record_to_mehmet_oz.pdf

[v] https://www.norc.org/research/library/spotlight-new-analysis-of-sickle-cell-disease-prevalence-among-medicaid-enrollees.html

[vi] https://www.hematology.org/education/patients/anemia/sickle-cell-disease

[vii] https://minorityhealth.hhs.gov/news/coming-together-confront-sickle-cell-disease

[viii] https://www.jscimedcentral.com/public/assets/articles/hematology-3-1037.pdf

[ix] https://pmc.ncbi.nlm.nih.gov/articles/PMC9898623/

[x] https://www.cms.gov/About-CMS/Agency-Information/OMH/Downloads/Opioid-Prescription-in-Medicare-Beneficiaries-Report.pdf

[xi] https://www.jscimedcentral.com/public/assets/articles/hematology-3-1037.pdf;

[xii] https://www.ajpmfocus.org/article/S2773-0654(23)00095-0/fulltext

[xiii] https://www.ajpmfocus.org/article/S2773-0654(23)00095-0/fulltext

[xiv] https://www.sicklecellpartnership.org/wp-content/uploads/2025/01/SCDP-Letter-on-MUC-2024-074.pdf

[xv] https://p4qm.org/sites/default/files/2025-02/PRMR-2024-2025-MUC-Recommendations-Report-Final.pdf; https://p4qm.org/sites/default/files/2024-12/PRMR-PA-MUC2024_074.pdf

[xvi] https://ashpublications.org/blood/article/142/Supplement%201/7201/504559/Delayed-Management-of-Sickle-Cell-Disease-Vaso; https://pubmed.ncbi.nlm.nih.gov/25834959/

[xvii] https://www.sicklecellpartnership.org/wp-content/uploads/2024/10/SCDP-Statement_SCDCCA-Senate-Introduction_10.02.2024.pdf

[xviii] https://www.congress.gov/bill/119th-congress/senate-bill/721?q=%7B%22search%22%3A%22S.721%22%7D&s=1&r=1

[xix] https://www.medicaid.gov/state-resource-center/medicaid-state-technical-assistance/health-home-information-resource-center/downloads/medicaidhomehealthstateplanoptionrtc.pdf

[xx] https://archive.cdc.gov/#/details?q=Sickle%20cell%20data%20sharing&start=0&rows=10&url=https://www.cdc.gov/ncbddd/hemoglobinopathies/data-reports/2018-summer/documents/sickle-cell-data-to-action-h.pdf