SICKLE CELL DISEASE PARTNERSHIP

April 29, 2024

Liz Fowler
Director
CMS Innovation Center
Department of Health and Human Services
7500 Security Blvd
Baltimore, MD 21244

cc: The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Blvd
Baltimore, MD 21244

Dear Director Fowler,

The Sickle Cell Disease Partnership, a multi-sector 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), expresses our sincere thanks and appreciation for CMS's announcements of further details about the CMS Innovation Center's first Cell and Gene Therapy (CGT) Access Model ("Access Model"). The Access Model recognizes that individuals with SCD have for far too long faced health disparities and inequities. The Access Model has the potential to increase access to treatment for Americans living with SCD in a meaningful way.

We appreciate how the Access Model builds on two initiatives from the Biden-Harris Administration to advance health equity: the "Strategic Vision for Medicaid and CHIP"[i] and CMS's Framework for Health Equity.[ii] The Strategic Vision emphasized that closing disparities in coverage, access, and innovation should be at the forefront of decisions across states and the health care system, and it underscored the racial disparities that persist in Medicaid. Likewise, CMS's framework committed the agency to identifying causes of disparities and helping health care stakeholders to overcome them. The Access Model presents one way in which CMS is working to meet these goals, though there is much work yet to be done.

Individuals living with SCD face severe health complications, including recurring pain crises, infection, acute chest syndrome, lung problems, severe and chronic pain, and stroke. Moreover, SCD primarily affects Black and Hispanic individuals, and individuals with SCD experience disparities and inequities in their care. Research shows that approximately half of the individuals with SCD in the United States are enrolled in Medicaid at some point within a year.[iii] Accordingly, state Medicaid programs play a disproportionately important role in meeting the needs of these individuals. Previous analysis of Medicaid T-MSIS claims has shown gaps in access to timely care and treatment for individuals with SCD.[iv]

The Partnership applauds the needed focus on SCD by the CMS Innovation Center. The Partnership recognizes that resources from across the agency are being put towards the SCD community to implement a monumental program that addresses access to a new and innovative treatment with a high associated cost for a disease population that has a history of being marginalized. The model is a thoughtful attempt to address barriers for rare diseases, particularly SCD.

The Partnership additionally appreciates CMS' efforts to address imbalances between Managed Care plans and Fee for Service beneficiaries; out-of-state access; variations in coverage policies across states; and variations in prior authorization and other utilization management tools. The model may help to establish a more consistent health care framework for individuals with SCD who choose to pursue gene therapy.

To inform the Innovation Center's work progression as it implements the model, the Partnership here provides some perspectives for your consideration based on the Access Model generally and the Request for Applications from Applicable Manufacturers as was released on March 7, 2024.

- We appreciate that the implementation timeline has been accelerated compared to the initial announcement. At the same time, while we recognize that CMS is implementing as quickly as it can, multiple contingent actions (including Innovation Center guidance, state decisions, and model implementation) should not worsen the access disparity between publicly and privately insured individuals seeking access to gene therapy. Because those eligible for gene therapy typically have the most advanced disease, time is of the essence. It would also be appropriate and helpful for CMS to remind states about what they can be *currently* doing to ensure access to gene therapies under current law, including through value-based payment arrangements and supplemental rebate agreements.

- Individuals who are eligible for and choose to pursue gene therapy will require a significant number of services – both inpatient and outpatient – in order for the treatment to be successful (e.g., 3-6 months of transfusions prior to receiving the gene therapy and a hospital stay of 30-40 days after receiving the therapy). As currently outlined in the Model, CMMI is leaving it up to the states to provide adequate coverage for these services. The Innovation Center should use its authority to ensure that States participating in the Model adequately cover all additional clinical services needed to make gene therapy successful.

- Fertility preservation is a major interest for individuals with SCD undergoing gene therapy. The Partnership applauds CMS for enabling participating manufacturers to cover some fertility preservation services for beneficiaries receiving treatment under the Model. However, we urge CMS to expand the scope of the defined fertility preservation services to cover in vitro fertilization (IVF) and storage fees for a longer period of time. On average, IVF costs over $60,000 per successful outcome, and almost all Medicaid programs do not cover IVF costs.[v] CMS' intent to assist individuals with SCD with the fertility barrier to obtaining gene therapies will not be reached without also covering the cost of IVF.

- The RFA from Manufacturers establishes that manufacturers should ensure the delivery of comprehensive beneficiary education on the provided fertility preservation services. While the RFA asks manufacturers to provide such materials in writing at a 5th grade or lower reading level, the RFA does not establish any language requirements. To ensure this information is as accessible as possible, especially for the populations most impacted by SCD, CMS should ask manufacturers to provide the educational materials in the native language of the gene therapy recipient.

- As you know, the Anti-Kickback Statute makes it difficult for biopharmaceutical manufacturers to provide assistance for fertility preservation for publicly insured patients. As currently proposed, manufacturers can only provide fertility preservation assistance to beneficiaries receiving treatment under the Model. To improve health equity and reduce barriers to treatment in every state, CMS should engage in dialogue regarding efforts to modify the Anti-Kickback Statute to enable fertility preservation coverage in states that do not choose to take up the Access Model.

- The language in the current Access Model guidance indicates that gene therapies approved by a specific date are eligible, which means that products approved in the future may not be included. The Innovation Center should use its authority to make changes to the model requirements to the extent that additional CGTs receive FDA approval in the future to treat SCD.

- Not all individuals with SCD are eligible for gene therapy, want to undergo such therapy, nor live in a state that will participate in the model. CMS should additionally work to address the health care needs of the majority of individuals with SCD who fall outside the model. It can do so by publicly engaging states and Medicaid Directors on their ability to comprehensively improve care and treatment for all individuals with SCD.

- There exists an opportunity for CMS to consider how to encourage states to reduce utilization management barriers to other forms of treatment (in addition to gene therapy) and ensure Medicaid beneficiaries with SCD have timely access to their most appropriate treatment option.

The Partnership appreciates the Administration's commitment to advancing health equity and remains optimistic that the CGT Access Model will bring long-awaited changes in access to treatment for individuals with SCD. We would value the opportunity to speak with you in coming months to further discuss the successes, concerns, and future guidance that we have identified in the CGT Access Model on SCD. We look forward to working with you and your colleagues on increasing equitable access to CGTs for those living with SCD.

Sincerely,

The Sickle Cell Disease Partnership

[i] https://www.healthaffairs.org/do/10.1377/forefront.20211115.537685/full/
[ii] https://www.cms.gov/about-cms/agency-information/omh/health-equity-programs/cms-framework-for-health-equity
[iii] Medicaid has been a key source of insurance coverage for those with SCD. According to older data, Medicaid nationwide covered 66 percent of SCD hospitalizations in 2004 and 58 percent of ED visits for the disease between 1999 and 2007
[iv] https://www.medicaid.gov/medicaid/quality-of-care/downloads/sickle-cell-disease-infographic.pdf, and https://www.medicaid.gov/medicaid/quality-of-care/downloads/scd-rpt-jan-2021.pdf
[v] https://www.kff.org/womens-health-policy/issue-brief/coverage-and-use-of-fertility-services-in-the-u-s/?utm_campaign=KFF-2020-Womens-Health-Policy-WHP&utm_medium=email&_hsmi=2&_hsenc=p2ANqtz-_tTirbZOqeIgjNSobzlIccF-uAC-R6Fp-K9I1PRTlVLROi3zK_STwRf4shqcKv0HvPCTIQxn9O-xudYlOwUtgHRlYYzw&utm_content=2&utm_source=hs_email