Alliance for Regenerative Medicine

June 23, 2023

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
Hubert H. Humphrey Building
200 Independence Ave, SW
Washington, DC 20201

Submitted via http://www.regulations.gov

Proposed Rule on Ensuring Access to Medicaid Services (CMS–2442–P)

Dear Administrator Brooks-LaSure:

The Alliance for Regenerative Medicine (ARM) appreciates the opportunity to comment on the Centers for Medicare & Medicaid Services (CMS) Proposed Rule on Ensuring Access to Medicaid Services (the "Proposed Rule").[1]

ARM is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. As a community, ARM builds the future of medicine by convening the sector, facilitating influential exchanges on policies and practices, and advancing the narrative with data and analysis. We actively engage key stakeholders to enable the development of advanced therapies and to modernize healthcare systems so that patients benefit from durable, potentially curative treatments. As the global voice of the sector, we represent more than 475 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions, and patient organizations.

As of year-end 2022, 1,308 regenerative medicine and advanced therapies developers worldwide are sponsoring 1,200 clinical trials across dozens of indications, including rare monogenetic diseases, oncology, cardiovascular, central nervous system, musculoskeletal, metabolic disorders, ophthalmological disorders, and more.[2]

To date, the FDA has approved seven gene therapies – six for rare genetic diseases – and six CAR-T cell therapies for various blood cancer indications. Transformative cell and gene therapies (CGTs) have been approved for the rare genetic pediatric indications spinal muscular atrophy and cerebral adrenoleukodystrophy, and for the

[1] 88 Fed. Reg. 27960 (May 3, 2023).
[2] https://alliancerm.org/sector-report/2020-annual-report/

pediatric blood cancer indications acute lymphoblastic leukemia. These innovative therapies address high unmet medical needs; they can be lifesaving; and many have the potential to reduce the need for burdensome and costly chronic care.

The CGT pipeline for both rare and prevalent diseases is accelerating, with growing impacts on Medicaid. Regarding rare diseases, gene therapies for Duchenne muscular dystrophy, hemophilia A, and sickle cell disease could be approved by the FDA in 2023. There are also transformative therapies advancing in the pipeline for chronic diseases including Type 1 diabetes.

Ensuring Medicaid patients have timely access to the same transformative therapies that will become available to those with other forms of government and commercial insurance is critical to achieving CMS' goal of addressing health equity, including closing gaps in care for underserved populations and eliminating racial health disparities. Medicaid nationwide covered 66 percent of sickle cell disease hospitalizations in 2004 and 58 percent of emergency department visits for the disease between 1999 and 2007.[3] Not only does Medicaid pay for a majority of acute care for sickle cell disease patients, but those patients are overwhelmingly people of color.[4]

We commend CMS for its continued efforts to strengthen access to care, improve care quality and health outcomes, and address health equity issues for enrollees in the Medicaid program, across fee-for-service (FFS), managed care delivery systems, and programs for home and community-based services (HCBS). In this letter, we offer specific recommendations to protect medically fragile beneficiaries receiving care via fee-for-service (FFS) Medicaid programs and ensure access to medically necessary CGTs:

- CMS should support Medicaid enrollee access to CGTs by ensuring that states ensure representation from providers of CGTs, and patients treated with CGTs, in MAC and BAG appointments.
- CMS should adopt a consistent, nationwide credentialing standard to facilitate the provision of care by CGT providers across state lines.
- CMS should expand upon the proposed payment rate transparency provisions to ensure access to CGTs by Medicaid enrollees.

CMS should support Medicaid enrollee access to CGTs by ensuring that states ensure representation from providers of CGTs, and patients treated with CGTs, in MAC and BAG appointments, and by adopting a credentialing standard that can be used to facilitate the administration of CGTs to out-of-state patients.

[3] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8550393/.

[4] https://www.cdc.gov/ncbddd/sicklecell/data.html#:~:text=SCD%20affects%20approximately%20100%2C000%20Americans,sickle%20cell%20trait%20(SCT).

Consistent with the principles of Executive Order (E.O.) 14009 and E.O. 14070, the Proposed Rule seeks to help States "strengthen Medicaid and improve access to and quality of care."[5] Specifically, this Proposed Rule "is focused on addressing additional critical elements of access: (1) potential access, which refers to a beneficiary's access to providers and services, whether or not the providers or services are used; (2) beneficiary utilization, which refers to beneficiaries' actual use of the providers and services available to them; and (3) beneficiaries' perceptions and experiences with the care they did or were not able to receive."[6] ARM supports the Administration's efforts to ensure that Medicaid enrollees can access covered services, and we urge CMS to ensure these enrollees can access specialized providers of CGTs.

As noted in the Proposed Rule, the regulations at 42 CFR § 431.12 require states to have a Medical Care Advisory Committee (MCAC) to advise Medicaid agencies about health and medical care services. CMS is proposing to revise the current MCAC requirements with a new framework to ensure the Medicaid program is efficiently administered and that care is provided in a manner that aligns with enrollees' best interests. If finalized, states would be required to enact a Medicaid Advisory Committee (MAC) and a Beneficiary Advisory Group (BAG), which would provide bi-directional feedback between stakeholders and the state regarding program administration matters.

State Medicaid agency directors or higher state authorities would appoint MAC and BAG members. CMS in the Proposed Rule encourages states to consider the demographics of their Medicaid populations when selecting members and explains how states can consider the demographic representation of MAC members by including members "representing or serving Medicaid beneficiaries in the following categories: (1) children's health care; (2) behavioral health services; (3) preventive care and reproductive health services; (4) health or service issues pertaining specifically to people over age 65; and (5) health or service issues pertaining specifically to people with disabilities."[7] The MAC must include representation from clinical providers or administrators with familiarity of Medicaid beneficiaries' health and social needs, among other representation. CMS proposes several types of providers to include in the clinical category, including primary care providers; behavioral health providers; reproductive health providers; pediatric providers; dental and oral health providers; community health, rural health clinic or Federally Qualified Health Center (FQHC) administrators; long-term care services and supports providers; and direct care workers.[8] Additionally, CMS proposes to require that 25 percent of MAC members have "lived Medicaid beneficiary experience" from the BAG, defined as either having been or currently being a Medicaid beneficiary or having "direct experience supporting Medicaid beneficiaries."[9]

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[5] 88 Fed. Reg. 27960, at 27961.
[6] *Id.*
[7] *Id.* at 27968.
[8] *Id.* at 27969.
[9] *Id.*

To ensure these new regulations guarantee access to care for patients with complex medical conditions who may seek treatment with CGTs, ARM urges CMS to include specialty providers of CGTs among the required categories of providers, such as members with experience in health or service issues for persons with disabilities, to be appointed to the MAC. These providers have extensive experience treating the most vulnerable patients with innovative therapies that are complex to administer; by sharing their experience and feedback with the state Medicaid agency, these providers can ensure their patients' needs are considered. Furthermore, ARM appreciates CMS' assessment that establishing a BAG will provide the state with "increased access to the beneficiary perspective."[10] CMS should therefore ensure that BAG members, including members who have served on the MAC and BAG, have lived Medicaid beneficiary experience related to seeking treatment via CGTs, either as patients or caregivers. BAG members with experience seeking treatment with CGTs can offer unique insights on access issues and areas for improvement.

To further improve access to CGTs, ARM urges CMS to adopt a consistent, nationwide credentialing standard to facilitate the provision of care by CGT providers across state lines.

Because of the specialization required for the administration of CGTs, manufacturers generally contract with providers in a limited number of states that have the appropriate experience and facilities necessary for the administration of their therapies. For this reason, patients seeking CGT treatments, who in many cases tend to be critically ill with medically complex conditions, often are required to travel beyond their home states to obtain care. Providers seeking to treat nonresident Medicaid beneficiaries must become enrolled in, and credentialed by, the program in the patient's home state. Currently, since each state Medicaid program establishes and administers its own credentialing program, the rules and procedures for credentialing can vary from state to state, resulting in a patchwork of state-specific credentialing requirements. These requirements can be onerous, complex, and time-consuming. As a result, certain providers qualified to administer CGTs may be reluctant to complete necessary credentialing procedures to allow the treatment of nonresident beneficiaries, creating avoidable barriers to care for medically complex patients seeking treatment with CGTs.

Consistent with CMS's authority under section 1902(a)(16) of the Social Security, Act (the "Act"), and similar to the standards CMS has already adopted for medically-fragile children,[11] the establishment of a consensus-based credentialing standard for CGT providers treating patients of all ages that state Medicaid agencies may opt to use will help facilitate access to care among some of the nation's most vulnerable patients.

CMS should expand upon the proposed payment rate transparency provisions to ensure access to CGTs by Medicaid enrollees.

[10] 88 Fed. Reg. 27960, at 27969.

[11] https://www.medicaid.gov/federal-policy-guidance/downloads/smd22004.pdf.

Under Section 1902(a)(30)(A) of the Act, states must "assure that payments are consistent with efficiency, economy, and quality of care and are sufficient to enlist enough providers so that care and services are available under the plan at least to the extent that such care and services are available to the general population in the geographic area." CMS explains in the Proposed Rule that § 447.203 requires states to develop an access monitoring review plan (AMRP) for a core set of services and submit this information to CMS.[12] Noting how compliance with the AMRP process has been burdensome on states, CMS proposed to rescind and replace the AMRP requirements with a more streamlined process.[13] Specifically, CMS seeks to replace the old process with "new requirements to ensure FFS Medicaid payment rate adequacy, including a new process to promote payment rate transparency."[14] Additionally, in the case of a bundled fee schedule payment rate, CMS proposes to require that the state identify each service included in the bundle.[15] Further, CMS proposes requiring the state to identify how much of the bundled rate is allocated to each service included in the bundle.

CMS proposes to require that only certain services—specifically, primary care services, obstetric and gynecological services, and outpatient behavioral health services—are included in the comparative rate analysis. The analysis will compare the Medicaid FFS payment rates for these services to the most recently published Medicare payment rates (effective for the same time period) for the evaluation and management (E/M) codes[16] applicable to the category of service. This analysis must be updated every two years. According to CMS, "these categories of services are critical preventive, routine, and acute medical services in and of themselves, and that they often serve as gateways to access to other needed medical services, including specialist services, laboratory and x-ray services, prescription drugs, and other mandatory and optional Medicaid benefits that States cover."[17] CMS further explains how including these services in the comparative rate analysis would require states to ensure their Medicaid FFS payment rates conform with Section 1902(a)(30)(A) of the Act.

CMS notes how these proposed transparency requirements will provide insights into how state Medicaid rates compare with Medicare rates. According to CMS, few physicians opt out of the Medicare program, therefore Medicare payment rates are consistent with a high level of physician willingness to accept new Medicare patients. CMS proposes to use Medicare non-facility payment rates as the national benchmark for states to compare Medicaid rates because CMS believes Medicare rates "are likely to serve as a reliable benchmark for a level of payment sufficient to enlist providers to furnish the relevant services to an individual."[18]

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[12] *Id.* at 27965.
[13] *Id.* at 27997.
[14] *Id.* at 27998.
[15] *Id.* at 27999.
[16] Current Procedural Technology (CPT®) codes or Healthcare Common Procedure Coding System (HCPCS) codes.
[17] *Id.* at 28002.
[18] *Id.* at 28011.

ARM greatly supports CMS' efforts to ensure payment rate analysis and transparency and agrees that beneficiary access is inextricably tied to provider payment rates. However, we urge CMS to go further to ensure access to CGTs and related items and services for Medicaid enrollees.

First, ARM respectfully urges CMS to expand the list of services for the comparative rate analysis to include the CGT administration and related items and services. As CMS notes, higher provider payment rates are associated with provider willingness to treat Medicare beneficiaries, and lower provider payment rates under Medicaid could lead to reduced investment in emerging technology among providers that serve large numbers of Medicaid beneficiaries. CGTs are some of the newest technologies and involve significant resources to acquire and administer. In addition, as noted above only certain provider types are qualified to administer these therapies. Without adequate payment, Medicaid beneficiaries are likely to have limited access to these promising new lines of treatment.

Second, while we appreciate CMS' proposal to require states to identify each service included in a bundle and identify how much of the bundled amount was used per service, we urge CMS to adopt a comparative rate analysis other than comparison with Medicare payment rates with respect to CGTs and other inpatient-administered therapies. Many CGTs are administered in the inpatient setting. Under Medicare, this means that the hospital is paid a bundled rate for all items and services furnished during the hospital stay, the amount of which is determined based on the Diagnosis Related Group (DRG) assigned at discharge. Notably, this bundled rate is generally inadequate to cover the hospital's cost of purchasing innovative new therapies, including DRGs. As a result, hospitals can be strongly disincentivized to purchase and administer newly available CGTs products because of significant financial losses.

Further, ARM urges CMS to adopt specific standards to ensure payment adequacy for administration of CGTs.

We note that a limited number of states have begun to pay hospitals separately (i.e., outside of the bundle) for their acquisition cost of CGTs through state plan amendments (SPAs) or administrative policies. These policies can significantly advance access to innovative new therapies. CMS should therefore encourage the adoption of such policies by state Medicaid programs in an effort to bolster access to these emerging technologies.

With the rapid adoption of alternative payment methodologies, including diagnosis and episode-based fees, global payments, bundled payments, sub-capitation, performance incentives, and shared saving arrangements, it is increasingly challenging to hold state Medicaid programs accountable for assuring access, particularly for complex therapies covered under State Plans or EPSDT. Medicaid plans often indicate how a provider receives an all-inclusive fee. At the same time, the hospitals and physicians maintain the fixed fee is either inadequate or does not envision the particular treatment.

Thank you, in advance, for considering our recommended improvements to the proposed revisions in CMS–2442–P. We look forward to working with CMS and state Medicaid agencies to establish policies that promote equitable and appropriate access to CGTs. Please feel free to contact me at ecischke@alliancerm.org with any questions.

Sincerely,

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Alliance for Regenerative Medicine