Alliance for Regenerative Medicine

March 11, 2024

Daniel Tsai
Deputy Administrator and Director
Center for Medicaid and CHIP Services
Centers for Medicare & Medicaid Services
7500 Security Blvd.
Baltimore, MD 21244

Dear Deputy Administrator Tsai:

In recent years, transformative new cell and gene therapies (CGTs) have arrived on market to treat debilitating diseases, offering potentially transformative impacts to Americans living with different conditions. The innovation in this sector is accelerating, offering significant promise to patients, including many Medicaid beneficiaries.

Given the specialized nature of these treatments, including the new gene therapies for sickle cell disease, gaining access to CGTs will require many Medicaid beneficiaries to seek care across state lines. This expectation will create barriers to access without intervention by CMS and state Medicaid programs. The Alliance for Regenerative Medicine (ARM) therefore urges CMS to take steps now to facilitate credentialing by Medicaid providers across state lines. Specifically, building on the steps CMS has taken to facilitate cross-state care for pediatric Medicaid beneficiaries, ARM encourages CMS to consider:

- Establishing a consensus-based credentialing standard for providers involved in the administration of CGTs;
- Issuing guidance to states to improve the process of provider screening and enrollment.

We encourage CMS to take these steps as soon as possible to ensure that no Medicaid beneficiaries – children or adults – experience disruption in access to CGTs due to bureaucratic delays. At the request of Congress, CMS has issued guidance specific to providers offering care to children with complex health needs who live outside their state of practice. However, CMS does not need to wait for express direction from Congress to issue similar guidance for adults who could benefit from similar policies, particularly as new CGTs impacting adults living with conditions like sickle cell disease are coming to market.

ARM stresses that critical, out-of-state credentialing is but one of many continuing barriers to access for Medicaid beneficiaries. Further outlined below, CMS should continue to work with state Medicaid programs to ensure states comply with their coverage obligations and take steps to ensure timely access to innovative new CGTs. The Agency has the legal authority to proactively

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take steps to ensure these and other administrative barriers do not delay or deter patients' access to critical treatments.

**About the Alliance for Regenerative Medicine**

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 400 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions, and patient organizations.

**CGT Pipeline**

To date, the Food and Drug Administration (FDA) has approved 35 CGTs. These innovative therapies have been approved to treat rare genetic pediatric indications including Duchenne muscular dystrophy, spinal muscular atrophy, and cerebral adrenoleukodystrophy, acute lymphoblastic leukemia, Hemophilia, Sickle Cell Disease, and solid tumors. They address high unmet medical needs, can be lifesaving and have the potential to reduce the need for burdensome and costly chronic care.

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.

The CGT pipeline for both rare and more prevalent diseases is accelerating, with growing impacts on Medicaid. Notably, two gene therapies for sickle cell disease were approved by the FDA in late 2023. We've recently seen approval of a gene therapy for Hemophilia A and a cell therapy for type 1 diabetes. Many patients afflicted by conditions with newly available or soon-to-become available CGTs are children or individuals living with disability who rely on Medicaid for their health coverage. For example, Medicaid covers two-thirds of all hospitalizations for sickle cell disease, and 58 percent of all children with spinal muscular atrophy, and 30 percent of people with a bleeding disorder are enrolled in Medicaid.[1]

**Potential for Access Issues**

Because of the specialization required for the administration of CGTs, manufacturers generally contract with a limited number of providers that have the appropriate experience and facilities necessary for the administration of their therapies. These providers are often located in only a

[1] Grady A, Fiori A, Patel D, Nysenbaum J. Profile of Medicaid enrollees with sickle cell disease: A high need, high cost population. PLoS One. 2021 Oct 27;16(10):e0257796. doi: 10.1371/journal.pone.0257796. PMID: 34705847; PMCID: PMC8550393.

limited number of states. 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.

To give an example, as noted above, the FDA in December 2023 announced the approval of both Casgevy and Lyfgenia, "two milestone treatments […] representing the first cell-based gene therapies for the treatment of sickle cell disease in patients 12 years and older."[2] The Casgevy biotechnology company, Vertex, subsequently announced that the therapy would initially be available from only nine authorized treatment centers – one in Massachusetts, one in the District of Columbia, one in California, two in Texas, one in Tennessee, two in Ohio, and one in Illinois – meaning that residents of forty-four states would have to travel out-of-state to receive that treatment.[3] Similarly, Lyfgenia's biotechnology company, bluebird bio, announced that Lyfgenia would be offered from thirteen authorized treatment centers – one in Massachusetts, two in New York, one in New Jersey, one in Pennsylvania, one in Maryland, one in North Carolina, one in Illinois, one in Missouri, one in Tennessee, one in Kansas, and two in Texas. Therefore, to receive treatment with Lyfgenia, residents of forty states, including any non-Texas patient living west of Houston, will have to travel out-of-state.[4]

These specialized 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 proper verification 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.

Ensuring Medicaid patients have timely access to the same transformative therapies that will become available to those with other forms of public or 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. Any delays in the availability of new CGTs for the Medicaid population relative to those with other coverage could exacerbate existing disparities.

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[2] FDA, FDA Approves First Gene Therapies to Treat Patients with Sickle Cell Disease, Dec. 8, 2023, *available at*: https://www.fda.gov/news-events/press-announcements/fda-approves-first-gene-therapies-treat-patients-sickle-cell-disease#:~:text=FDA%20Approves%20First%20Gene%20Therapies%20to%20Treat%20Patients%20with%20Sickle%20Cell%20Disease,-Share&text=Today%2C%20the%20U.S.%20Food%20and,patients%2012%20years%20and%20older.

[3] Vertex and CRISPR Therapeutics Announce FDA Approval of Casgevy for the Treatment of Sickle Cell Disease, Dec. 8, 2023, *available at*: https://news.vrtx.com/news-releases/news-release-details/vertex-and-crispr-therapeutics-announce-us-fda-approval.

[4] bluebirdbio, Find a Qualified Treatment Center, Feb. 2024, *available at*: https://www.lyfgenia.com/find-a-qualified-treatment-center.

Stat News recently reported on the experience of an infant with an ultra-rate genetic disorder residing in Texas who needed to receive CGT treatment in Minnesota. This story exemplifies the concern that patients may experience avoidable complications.[5] The infant suffers from metachromatic leukodystrophy, which is typically irreversible and fatal once symptoms appear. The CGT biotechnology company, Orchard Therapeutics, offered its treatment, Libmeldy, free of charge to the boy's parents but, as is frequently the case with CGTs, receiving the treatment required travel from the boy's home state of Texas to work with specialists in Minneapolis. In negotiating with the Texas Medicaid agency to cover the incidental costs of the therapy (such as bloodwork and hospital stays), the boy's parents spent months arguing over credentialing issues. Although these issues were able to be resolved in time for the infant to receive the treatment, in other cases these delays can have devastating consequences that CMS can and should work to avoid.

**Previous Legislation and Regulatory Guidance**

The concerns regarding out-of-state provider credentialing are not new to CMS.

In 2019, Congress passed the ACE Kids Act, which was intended to improve access to care for children with chronic conditions. The law established a new health home state plan option for children with complex medical conditions. As part of implementation, the law required CMS to issue guidance to state Medicaid directors on processes for screening and enrolling out-of-state providers involved in furnishing care to children in these health homes, "including efforts to streamline such processes or reduce the burden of such processes on such providers."

Issued by CMS in October 2021, among the recommendations, the guidance:

- Suggested state Medicaid agencies "consider an expedited screening and enrollment process with respect to out-of-state providers, to help ensure that beneficiaries, including Medicaid-eligible children with medically complex conditions, who need out-of-state-care can receive it promptly."
- Restated instances in which state Medicaid agencies can reimburse otherwise payable claims from out-of-state providers.
- Recommended states develop "standard agreements with other states governing coverage and payment for service furnished to Medicaid-eligible children with medically complex conditions."

[5] Megan Molteni, Texas Medicaid Agrees to Fully Cover Gene Therapy for Afghan Refugees' Infant, Statnews, Feb. 5, 2024, *available at*: https://www.statnews.com/2024/02/05/orchard-gene-therapy-libmeldy-texas-medicaid-afghan-refugees/?utm_campaign=dc_diagnosis&utm_medium=email&_hsmi=292896217&_hsenc=p2ANqtz-9C2czGfCVxqqEG136gq85aSDSGC64s62fMhK6HSQnP3h5NPXSFZKYOS90gggaLNQTgMkrAPSKid7B7hkG6Tf-SWtfYJw&utm_content=292896217&utm_source=hs_email.

More recently, CMS announced that it would soon launch the first phase of the Cell and Gene Therapy Access Model and will begin in earnest in 2025, with a focus on sickle cell disease.[6] HHS Secretary Xavier Becerra noted that the Model "gives us a chance to streamline a cumbersome process and put transformative cell and gene therapies within reach for Americans with sickle cell disease." Secretary Becerra likewise noted that "many of the more than 100,000 Americans with [sickle cell disease] today face difficulty accessing effective health care and groundbreaking treatments." ARM shares CMS' hope that the Model will improve access to gene therapies for Medicaid beneficiaries with severe SCD, and we are encouraged that, through the model, CMS acknowledges the challenges associated with seeking CGT treatments out-of-state. However, these access challenges are not limited to SCD patients, and we urge CMS to consider broader solutions that help all patients across all states.

ARM notes that a bipartisan group of lawmakers recently introduced legislation that would again address the issue of cross-state provider enrollment for providers treating children. The Accelerating Kids' Access to Care Act (HR 4758/S.2372) would (broadly) direct states to adopt and implement a process to enable out-of-state providers to enroll as a participating provider in a state plan without additional screening requirements. ARM supports this legislation and would like to see it broadened to include specialized providers who treat Medicaid patients of all ages.

While ARM applauds efforts by both Congress and CMS to facilitate access to care for children, ARM would also note the importance of access to care for all. Recently approved CGTs and those in the near-term pipeline for are expected to impact many adults currently enrolled in Medicaid. While many children's hospitals have experience furnishing care to patients from other states, providers qualified to furnish CGTs to adults may be less likely to have familiarity with the process of credentialling with out-of-state Medicaid programs. We also note that ensuring access to CGTs for adult Medicaid patients does not require an act of Congress, but rather can be accomplished under CMS' existing authorities.

**Recommendations for CMS**

In anticipation of these new CGTs coming to market, ARM encourages CMS to identify opportunities to create a more seamless care experience for adult Medicaid beneficiaries seeking care outside their home state.

To that end, **ARM urges CMS to consider establishing a uniform credentialing standard** that could be applied for all providers involved in the administration of CGTs. Consistent with CMS' 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, 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.

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[6] CMS, Biden-Harris Administration Announces Action to Increase Access to Sickle Cell Disease Treatments, Jan. 30, 2024, *available at*: https://www.cms.gov/newsroom/press-releases/biden-harris-administration-announces-action-increase-access-sickle-cell-disease-treatments.

In addition, **ARM encourages CMS to issue information about best practices to states** in advance of the new CGTs coming to market, comparable to what CMS provided to states in the ACE Kids Act. This new guidance could reflect strategies for credentialing out-of-state providers treating both children and adults.

Moreover, as noted above, ARM stresses that, while critical, out-of-state credentialing is but one of many continuing barriers to access for Medicaid beneficiaries. CMS should continue to work with state Medicaid programs to address other issues, including the following:

- Reminding states of their obligation to provide coverage for covered outpatient drugs as soon as those drugs are approved by the FDA and enter the market.[7] The Medicaid Drug Rebate Program generally requires states to cover the products of participating manufacturers upon approval and market entry, but states consistently and improperly impose coverage limitations and utilization management techniques that result in the denial of timely access when prescribed for their medically accepted indication.[8]
- Encouraging states to explore innovative contracting arrangements with willing manufacturers. Although the Cell and Gene Therapy Access Model focuses in part on contracting, value-based contracting nonetheless remains inconsistent at the state level, which CMS can help to address by encouraging states to seek approval for value-based purchasing through a State Plan amendment.
- Encouraging states to exercise their mandatory oversight of Medicaid managed care organizations (MCOs) which "requires [that] services covered under Medicaid managed care contracts … must be furnished in an amount, duration, and scope that is no less than the amount, duration, and scope for the same services for beneficiaries under FFS Medicaid" instructed and to monitor to ensure their MCOs are providing appropriate access.[9,10] Managed care organizations contracting with state Medicaid agencies frequently and improperly impose more restrictive policies than that otherwise used by the state Medicaid program.
- Encouraging states to provide adequate reimbursement for CGTs. Because CGTs are administered in inpatient settings, states will sometimes pay hospitals for CGTs via the bundled payment for the inpatient service, which does not adequately account for the cost of purchasing the therapy itself, leading to inadequate reimbursement for hospitals. To protect such hospitals and to encourage them to continue to administer CGTs to Medicaid patients, a limited number of states have begun paying separately for the cost of acquisition – CMS should encourage this pro-patient approach.

[7] 78 Fed. Reg. 4594, 4631 (Jan. 23, 2013)
[8] 42 U.S.C. §§ 1396r-8(b)-(d)
[9]CMS Medicaid Drug Rebate Program Notice, Release No. 172. Assuring Medicaid Beneficiaries Access to Hepatitis C (HCV) Drugs. November 5, 2015
[10] 42 CFR § 438.3(s)(1)

The arrival of new CGTs offers tremendous potential for transforming the lives of Medicaid beneficiaries living with sickle cell disease, hemophilia, and other conditions. However, this promise will not be realized if access to these therapies is limited by administrative hurdles. We encourage CMS to take steps to ensure that Medicaid beneficiaries are not unfairly impacted by these issues.

We commend CMS for its continued efforts to strengthen access to care, improve care quality and health outcomes, and address health equity issues for Medicaid beneficiaries, and we thank you in advance for considering our recommendations. We look forward to working with you to ensure that all beneficiaries have appropriate and timely access to CGTs and welcome the opportunity for continued discussions. Please feel free to contact me at ecischke@alliancerm.org with any questions.

Sincerely,

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Alliance for Regenerative Medicine