August 14, 2023

The Honorable Cathy McMorris Rodgers
Chair
Committee on Energy & Commerce
U.S. House of Representatives
Washington, DC 20515

The Honorable Frank Pallone
Ranking Member
Committee on Energy & Commerce
U.S. House of Representatives
Washington, DC 20515

Dear Chair Rodgers and Ranking Member Pallone:

The Alliance for Regenerative Medicine (ARM) writes to ask for your support of policies ensuring that patients with healthcare coverage through state Medicaid programs can access emerging cell and gene therapies (CGTs).

ARM is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. 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.

In recent years, numerous transformative – and sometimes life-saving -- CGTs have been approved by the Food and Drug Administration (FDA) for some of the most difficult-to-treat conditions affecting both children and adults, including cerebral adrenoleukodystrophy, beta-thalassemia, spinal muscular atrophy, hemophilia A and B, Duchenne muscular dystrophy, and various forms of cancer. There are nearly 1,000 CGT clinical trials ongoing in the US to test the next generation of therapies. Many patients who can benefit from CGTs are children or individuals living with disability who rely on Medicaid for their health coverage. Notably, two gene therapies for sickle cell disease patients – many of whom are enrolled in Medicaid – could be approved by the FDA in December 2023.

CGTs hold tremendous promise for patients and our health care system. However, Medicaid programs face numerous challenges in ensuring access to these novel products because current payment methodologies – originally designed to pay for the treatment of ongoing, chronic conditions—were adopted well before these durable, potentially curative, therapies were conceived[i].

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To that end, we ask that you consider the following regulatory and legislative opportunities to address these challenges:

- **Achieve shorter, streamlined drug review processes.** Each state Medicaid program follows its own processes and timelines to review products newly approved by FDA to determine the program's coverage policy. This process is generally administered by state Pharmacy and Therapeutics Committees or Drug Utilization Review Boards (DURB). Many states permit or even mandate extended review timelines that can stretch months or even years. Such delays can result in unreasonable access restrictions on new drugs. Congress should work with CMS to require that states meet their legal obligations to provide access to FDA-approved drugs *upon availability*. Congress may suggest that CMS offer proposed timelines for new drug review procedures that are more closely aligned to the Medicare Part D review timelines. This approach may drive states to enact necessary legislative amendments or other policy changes that can reduce or eliminate barriers to access caused by delayed review periods. Congress should also consider offering incentives for states to conduct expedient coverage reviews of newly available CGT's (for example, by the next scheduled DURB meeting after availability, and in no case later than 90 days).

- **Create universal credentialing standards for CGT providers treating out-of-state patients.** The intricacies of CGTs may limit the number of centers of excellence (COEs) offering these complex therapies. As a result, some Medicaid beneficiaries may be required to travel out of their home states for treatment. A provider willing to treat out-of-state Medicaid patients is required to bill and accept payment from the patient's home state Medicaid program. However, the provider is only authorized to bill for such services if it is credentialed by and enrolled in that state's Medicaid program. Each state generally develops and administers its own credentialing rules, and the credentialing process can be complex, time-consuming, and expensive for providers to complete. We urge Congress to work with CMS to develop a minimum national credentialing standard for providers seeking to administer CGTs to out-of-state beneficiaries of all ages. ARM supports efforts to streamline the Medicaid credentialling process, like the *Accelerating Kids' Access to Care Act* (H.R. 4758), to facilitate the treatment of CGT patients by out-of-state providers and reduce unnecessary delays and other barriers to care for these patients.

- **Ensure Adequate Medicaid reimbursement for CGTs regardless of setting of administration (inpatient or outpatient).** Many CGTs are administered in the inpatient setting, for which providers are generally paid a bundled rate (typically a DRG or per diem) that in many cases can be inadequate to cover the hospital's cost of purchasing

innovative new therapies. As a result, hospitals can be disincentivized to purchase and administer newly available CGTs products to Medicaid patients because of the risk of significant financial losses. Separate payment that reimburses the hospital directly for its actual cost of purchasing CGTs administered inpatient can make hospitals whole for such costs and thus encourage the acquisition and administration of these therapies. Separate payment methodologies for CGTs have already been adopted in several states. To ensure adequate reimbursement for CGTs across sites of care:

  - Congress should increase the Federal Medical Assistance Percentage (FMAP) to states for CGTs to support the reimbursement levels necessary to ensure that hospitals are sufficiently compensated for treating Medicaid patients with these innovative therapies without risking significant financial losses.
  - Congress should oppose the proposal by CMS to change the definition of "covered outpatient drug" in the Misclassification of Drugs, Program Administration and Program Integrity Updates under the Medicaid Drug Rebate Program ("MRDP proposed rule") released May 23, 2023. We are deeply concerned that CMS is currently considering changing the interpretation of this term to include not only inpatient-administered products paid separately as described above, but also products that are simply *listed* separately on the claim form without any form of separate payment. Not only is this interpretation inconsistent with the governing statute, allowing states to seek rebates on inpatient-administered drugs merely by identifying the product on the claim form, and without actual, separate payment to the provider for the cost of the drug, would undermine the mutually beneficial balance that separate payment arrangements for inpatient CGTs strike – that is, ensuring adequate payment to hospitals for CGTs while authorizing MDRP rebates for states on those products.

- **Establish a new Anti-Kickback Statute (AKS) safe harbor for manufacturer assistance for travel and lodging for CGT patients and caregivers**. CGT treatments are generally available through only a limited number of centers of excellence (COEs) nationwide. Travel, lodging and related expenses, particularly those incurred for out-of-state travel, can be particularly burdensome for Medicaid patients in need of CGT treatments. However, state Medicaid programs offer limited benefits in this area. While HHS-OIG has issued advisory opinions finding that manufacturer assistance programs for patient travel can pose limited risk under the AKS, to provide greater clarity and certainty regarding these important patient assistance programs, Congress should create a statutory AKS safe harbor applicable to patient assistance programs for travel and lodging for CGT administration and related care.

- **Enhance Medicaid Funding for Interstate Travel, Lodging, and Related Expenses**. State benefits for reimbursement of travel and lodging expenses can be insufficient to adequately cover the costs incurred by patients and their caregivers who may be required travel extensive distances to reach qualified COEs. Congress should consider increased funding to states to enhance their non-emergency travel benefits for CGT patients and their caregivers in need of such support.

- **Encourage CMS to remove the proposed price verification survey from the *Misclassification of Drugs, Program Administration and Program Integrity Updates under the Medicaid Drug Rebate Program ("MRDP proposed rule")* released May 23, 2023** – CMS's recent proposal that manufacturers of certain therapies make extensive disclosures of sensitive data regarding R&D, manufacturing, clinical trials, and marketing, not to mention any other data that the Secretary may deem necessary relies upon unrelated authorities. While our member companies support efforts to verify that all prices reported to CMS are accurate, nothing in the controlling statutes authorizes CMS to require manufacturers to *justify* prices. ARM is most concerned that the agency unfairly and inappropriately focuses on the price of CGT therapies, failing to consider how such therapies differ from traditional pharmaceuticals and can improve outcomes and reduce downstream medical costs. CMS has no expertise or authority to evaluate the relative value or even the price of a given therapy, and none of the information CMS seeks to collect with respect to a targeted drug appears in any way related to its value. Therefore, the proposed transparency requirements and the proposed penalties for non-compliance are unreasonably burdensome and punitive. We urge Congress to send a clear message to CMS opposing their survey proposal and urging the agency to work with manufacturers and states on a voluntary basis to develop alternative methodologies for addressing the short-term cost of high-value CGTs that preserve, rather than threaten, patient access and continued innovation and development of new, groundbreaking treatments.

We appreciate your focus on improving the lives of patients suffering from complex medical conditions, for some of whom CGTs may be the only treatment option. We look forward to working with you, the Administration, and other stakeholders to advance policies that improve and expedite Medicaid patients' access to these transformative therapies. If you have any questions, please contact me at ecischke@alliancerm.org

Sincerely,

Erica Cischke, MPH
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[i] Allen, J., Berry, D., Cook, F., Rouce, R., Srirangam, A., Wellman, J., & McCombs, C. (2023). Medicaid coverage practices for approved gene and cell therapies: Existing barriers and proposed policy solutions. *Molecular Therapy*. https://doi.org/10.1016/j.omtm.2023.05.015