February 28, 2024

The Honorable Cathy McMorris Rodgers
Chair, Committee on Energy and Commerce
United States House of Representatives
Washington, DC 20510

The Honorable Frank Pallone
Ranking Member, Committee on Energy and Commerce
United States House of Representatives
Washington, DC 20510

The Honorable Brett Guthrie
Chair, Committee on Energy and Commerce Health Subcommittee
United States House of Representatives
Washington, DC 20510

The Honorable Anna G. Eshoo
Ranking Member, Committee on Energy and Commerce Health Subcommittee
United States House of Representatives
Washington, DC 20510

Dear Chairs Rodgers and Guthrie and Ranking Members Pallone and Eshoo:

On behalf of the Alliance for Regenerative Medicine (ARM), which represents more than 400 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions, and patient organizations, I commend the Energy and Commerce Health Subcommittee for holding a hearing to discuss "Legislative Proposals to Support Patients with Rare Diseases".

ARM is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. Because over 70% of rare disorders have genetic causes[i], cell and gene therapies (CGTs) are critical in targeting the root causes of these diseases rather than treating symptoms and have the potential to transform the lives of afflicted patients.

It is becoming increasingly clear that the promise of CGTs is bearing fruit for rare disease patients. Gene therapy seeks to modify or introduce genes into a patient's body with the goal of durably treating, preventing or potentially curing a disease. There are currently ten gene therapies approved for rare genetic diseases for conditions such as Duchenne muscular dystrophy, sickle cell disease and two forms of hemophilia. In 2024, three gene therapies for rare genetic diseases already have FDA decision dates while regulatory submissions are possible for an additional three. Cell therapy is the administration of viable, often purified cells into a patient's body to grow, replace, or repair damaged tissue. In 2024, the Food and Drug Administration (FDA) approved the first-ever adoptive cell therapy – for metastatic melanoma. There are also several pending FDA approval decisions on new cell therapies such as those for advanced synovial sarcoma, a rare type of cancer that attacks large joints, and for dystrophic epidermolysis bullosa, a rare skin condition that causes widespread blistering that can lead to vision loss or permanent scarring. However, despite these advances, more than 90 percent of the estimated 10,000+ rare diseases still have no FDA-approved products, and about half of those diseases affect children. Given the hope CGTs bring to patients, it is particularly important that these innovative

breakthroughs are met with a proactive and nimble legislative framework. ARM supports several of the legislative proposals noticed by the Subcommittee on Health and hopes to see timely action on the following:

Creating Hope Reauthorization Act of 2024 (H.R. 7384)
Because of the challenges in reaching a timely diagnosis and the corresponding clinical prognosis of rare diseases in the pediatric population, it is particularly important that biotechnology companies have the appropriate incentives and regulatory mechanisms to facilitate expeditious development of these products. The Rare Pediatric Disease Priority Review Voucher (PRV) program has stood as a key component in propelling product development for these populations.

To date, several CGT sponsors have received PRVs for therapeutics that treat a range of devastating pediatric diseases with high mortality and morbidity. The PRV program notably does not cost US taxpayers – but rather leverages market forces and regulatory flexibility to achieve the goal of accelerating the provision of medical advances to children who often have no other treatment options. Similarly, this program does not negatively affect the FDA's budgetary capabilities as companies that use vouchers must still pay the Agency's user fees. While the statute allows products late in the pipeline to be "grandfathered" into vouchers through 2026, any disruption to this program may have a significant negative impact on companies making decisions regarding their early-stage pipelines, which ultimately impacts access to future treatments for rare diseases. ARM has endorsed H.R. 7384 and urges Congress to swiftly reauthorize the Rare Pediatric Disease PRV Program.

Accelerating Kids Access to Care Act (H.R. 4758)
Medicaid beneficiaries face numerous challenges accessing CGTs, in part, because of the geographic limitation of highly specialized providers required to administer these innovative therapies. As an emerging field, the unique specialization necessary for the administration of CGTs requires biotechnology companies to contract directly with providers in a growing, but limited, number of states. Patients seeking CGT treatments, who in many cases tend to be critically ill with medically complex conditions, must travel beyond their home states to obtain these treatments and to receive necessary pre- and post-administration care.

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 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, patients can face weeks- or months-long delays in receiving treatment while these issues are resolved.

STAT News recently reported on the experience of an infant, Sufyan, that has an ultra-rate genetic disorder residing in Texas who needed to receive CGT treatment in Minnesota and illustrates this problem: "At one point, it seemed as though every provider in Minnesota that might care for the child — and ultimately bill for the care provided — would have to be credentialed as a Texas Medicaid provider, including surgeons, brain specialists, ICU staff, and possibly dozens of other doctors and nurses. That process generally takes months, months that Sufyan may not have."

Because of their complex and burdensome requirements, 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.

ARM has endorsed HR 4758 and believes it is a helpful first step in alleviating administrative delays for Medicaid patients. As drafted, the legislation only applies to patients under the age of eighteen. ARM recommends amending the legislation to include specialized providers who treat Medicaid patients of all ages. We also urge the Committee to implore the Centers for Medicaid and CHIP Services to take immediate action within their existing authority to streamline Medicaid provider credentialling.

Establishment of a safe harbor from the federal Anti-kickback Statute to permit organizations to provide travel and lodging assistance for patients who must travel to receive specialized care
Because of their unique pre-treatment and on-site manufacturing requirements, CGTs must be administered at highly specialized Centers of Excellence and thus, as a therapeutic modality, differ from other types of medical treatments. As a result, travel, lodging and related expenses, particularly those incurred for out-of-state travel, are often required and can be particularly burdensome for Medicaid patients in need of CGTs. This concern warrants a clear legislative framework that enables biotechnology companies to support patients, particularly in underserved populations, to benefit from innovations in regenerative medicine.

For example, the highest concentration of the population affected by sickle cell disease are in states such as Florida, Georgia, Alabama, Mississippi, Louisiana and South Carolina, however, despite the availability of two groundbreaking gene therapies that can offer relief for those who suffer from the most severe form of the disease, none of the aforementioned states have a sickle cell gene therapy treatment center authorized to administer this medication and patients are required to travel multiple times to seek care.[ii,iii]

ARM believes that creating a safe harbor to address travel and lodging assistance for patients eligible for CGT treatments can provide greater certainty to biotechnology companies and other entities seeking to reduce barriers and eliminates the need for the Department of Health and Human Services Office of Inspector General ("OIG") to issue individual advisory opinions. The OIG has already observed that travel, lodging and associated cost assistance provided to patients receiving CGTs would not cause overutilization of healthcare or steer patients to particular providers or therapies.[iv, v] We thank Congressman Guthrie for drafting legislation to establish a new safe harbor for travel and lodging assistance and urge the Committee to consider it's passage. ARM has also called for the establishment of an anti-kickback statute safe harbor to allow CGT companies to provide support for fertility preservation for patients who risk compromised fertility associated with the administration of certain CGTs.

Additionally, ARM is concerned that several of the changes to the Medicaid Drug Rebate Program (MDRP) that the Centers for Medicare and Medicaid Services (CMS) has proposed could undermine patients' access to CGTs and disincentivize the development of new rare disease treatments. We oppose CMS reinterpreting the definition of "covered outpatient drug" which would eliminate the

incentive for states to provide separate payment for CGTs outside of reimbursement for hospital services – a tactic that has helped improve patient access to innovative new therapies by protecting providers from unsustainable financial losses. We also oppose the proposed drug price verification survey which would require drug manufacturers to submit unprecedented amounts of new data and disproportionately targets CGTs given the upfront investment associated with a single administration product. The price verification survey ignores the long-term positive impact that CGTs are likely to have on patients' healthcare utilization and Medicaid spending. As currently proposed, there are no exemptions for products with an orphan drug or rare disease designation. Using the threat of public disclosure to coerce manufacturers into offering additional Medicaid rebates could threaten the commercial viability of CGTs for rare diseases and ultimately cause companies to abandon those programs leaving many rare disease patients with no hope. We urge members of this Committee to continue engaging with CMS to ensure implementation of the MDRP in a way that is consistent with its Congressional intent and protects beneficiaries' access to FDA-approved therapies.

ARM recommends that CMS collaborate with manufacturers and states on a voluntary basis to develop alternative methodologies for addressing the short-term cost of high-value CGTs that support, rather than threaten, patient access. The recently announced Cell and Gene Therapy Access Model which will launch for sickle cell disease patients next year may be one such example.

As evidenced by the many powerful testimonies of patients and their advocates heard throughout Rare Disease Week, rare diseases profoundly impact the quality of life of affected individuals and their families. The CGT sector holds great promise for transforming the landscape of rare disease treatment by offering the innovative, targeted, and potentially curative therapies these patients deserve. We thank you for your continued focus on improving the lives of those living with rare medical conditions, for some of whom CGTs may be the only treatment option.

ARM strives to be a resource for this Committee. We look forward to working with you to advance the aforementioned legislation and to develop additional policy solutions that bring safe and effective regenerative medicines to patients. Should you have any questions or concerns, feel free to contact me at ecischke@alliancerm.org.

Sincerely,

Erica Cischke, MPH
Vice President, Government Affairs
Alliance for Regenerative Medicine

[i] Nguengang Wakap, S., Lambert, D.M., Olry, A. et al. Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. Eur J Hum Genet 28, 165–173 (2020). Available at: https://doi.org/10.1038/s41431-019-0508-0

[ii] Phillips S, Chen Y, Masese R, Noisette L, Jordan K, Jacobs S, et al. (2022) Perspectives Of Individuals With Sickle Cell Disease On Barriers To Care. PLoS ONE 17(3): e0265342. Available at: https://doi.org/ 10.1371/journal.pone.0265342

[iii] Feuerstein, A. (December 2023) In historic decision, FDA Approves A CRISPR-Based Medicine For Treatment Of Sickle Cell Disease. Available at: https://www.statnews.com/2023/12/08/fda-approves-casgevy-crispr-based-medicine-for-treatment-of-sickle-cell-disease/

[iv] Notice of Modification of Advisory Opinion 20-02, OIG (May 26, 2022) Available at: https://oig.hhs.gov/documents/advisory-opinions/1035/Modification-AO-20-02.pdf.

[v] Advisory Opinion No. 20-09, OIG (December 28, 2020) Available at: https://oig.hhs.gov/documents/advisoryopinions/772/AO-20-09.pdf.