September 9, 2024

Chiquita Brooks-LaSure Administrator
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
Attention: CMS-1809-P
P.O. Box 8010
Baltimore, MD 21244–8010

Submitted via http://www.regulations.gov

Re: Medicare and Medicaid Programs: Hospital Outpatient Prospective Payment and Ambulatory Surgical Center Payment Systems; Quality Reporting Programs, Including the Hospital Inpatient Quality Reporting Program; Health and Safety Standards for Obstetrical Services in Hospitals and Critical Access Hospitals; Prior Authorization; Requests for Information; Medicaid and CHIP Continuous Eligibility; Medicaid Clinic Services Four Walls Exceptions; Individuals Currently or Formerly in Custody of Penal Authorities; Revision to Medicare Special Enrollment Period for Formerly Incarcerated Individuals; and All-Inclusive Rate Add-On Payment for High-Cost Drugs Provided by Indian Health Service and Tribal Facilities [CMS-1809-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 payment updates to the calendar year 2025 Hospital Outpatient Prospective Payment and Ambulatory Surgical Center Payment Systems; Quality Reporting Programs, including the Hospital Inpatient Quality Reporting Program; Health and Safety Standards for Obstetrical Services in Hospitals and Critical Access Hospitals; Prior Authorization; Requests for Information; etc. Proposed Rule (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 400 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions, and patient

[1] 89 Fed. Reg. 59186 (July 22, 2024).

organizations.

As of August 2024, there were 2,919 engineered cell therapy and genetic medicine developers worldwide sponsoring 1,851 clinical trials across dozens of indications, including rare monogenetic diseases, oncology, cardiovascular, central nervous system, musculoskeletal, metabolic disorders, ophthalmological disorders, and more.

To the benefit of Medicare beneficiaries, for the past decade managing the severity of hematologic malignancies has been and remains one of the main indications targeted by engineered cell therapies. Leading the charge are chimeric antigen receptor T-cell ("CAR-T") therapies, which have engineered receptors that target cancer cells. Currently, six CAR-T cell therapies for blood cancers are approved in the United States (US). However, many more cell therapies for blood cancers, including non-CAR-T approaches, are in the clinical pipeline. Of the ten most explored indications in cell therapy, nine fall within the scope of blood cancer. Also, in early December, the Food and Drug Administration (FDA) approved two gene therapies for SCD, one of which is the first approved medication that uses the gene-editing tool CRISPR. These two gene therapies use a novel technique to modify the expression of an individual's genes and result in an individual making more fetal hemoglobin, a type of oxygen-carrying blood protein present at birth. These one-time treatments have created the potential to cure this hereditary condition.

Scientific advancement is driving these therapies to new heights for safety and effectiveness. A handful of the authorized therapies were approved as a last line of treatment, meaning a patient must endure several treatments before being eligible. Since 2022, the U.S. Food and Drug Administration (FDA) has approved some of these cell therapies for earlier lines of treatment based on robust safety and efficacy data. In early 2024, a Tumor-Infiltrating Lymphocytes (TIL) therapy used to treat metastatic melanoma became the first adoptive cell therapy for a solid tumor to be approved by the FDA. Another landmark FDA approval is set for this year with an engineered T-cell receptor (TCR) therapy design to treat advanced synovial sarcoma, a cancer found in soft tissue. And this is just the beginning, the clinical pipeline for cell therapies demonstrates that treatments for solid tumors maintain a sizeable presence in early-stage trials, showing the potential for more therapies to reach the market in the years ahead.

Overall, the cell and gene therapy (CGT) pipeline most relevant to Medicare beneficiaries is set to extend beyond oncology care. It is a potentially unprecedented year for US approvals with eight more expected in 2024. These include therapies to treat steroid-refractory acute graft versus host disease and Hemophilia A and B. The current pipeline of CGTs is expected to result in 66 product-indication approvals (estimated range 54-74) by 2032. Where there are currently 16 product indication approvals and an average of five new approvals annually for the next four years, an estimated 41 conditions will be treated with cell and gene therapies by 2027. The modality of treatments is set expand on the evolution of the sector as well. There are over 225 Phase I clinical trials underway utilizing allogeneic biological sources to bring a therapy to market.

The proposals outlined in the Proposed Rule address forward thinking approaches that enhance Medicare beneficiaries' access to advanced therapies. ARM thanks CMS for its noticeable efforts to promote Medicare beneficiary access to cell and gene therapies, and ARM recognizes that many of

CMS's recent proposals – both in the Proposed Rule and in other areas—are intended to foster such access. To further promote appropriate access to cell and gene therapies, ARM urges CMS to:

- Ensure that cell and gene therapies are not packaged under comprehensive ambulatory payment classifications ("C-APCs") by making the agency's proposed separate payment approach permanent.
- Acknowledge separate payments for Category I CAR-T CPT Codes
- Adopt its proposal to pay separately for high-cost drugs administered in Indian Health Services ("IHS") and tribal facilities with a modification to pay at Average Sales Price ("ASP") plus 6 percent.

The remainder of our letter addresses these issues in more detail.

CMS Should Ensure That All Cell and Gene Therapies Are Always Exempt from Packaging Under C-APCs

ARM thanks CMS for its proposal to exempt cell and gene therapies from C-APC packaging for 2025. As CMS correctly recognizes, "[w]hen these products are administered, they are the primary treatment being administered to a patient and thus, are not integral, ancillary, supportive, dependent, or adjunctive to any primary C–APC services."[2] Therefore, and as stated in previous comment letters, ARM concurs with CMS's conclusion that these cell and gene therapies should be separately payable even in cases where they are included on a C-APC claim. However, the current proposal is limited to cell and gene therapies with status indicator "K". Because cell and gene therapies with status indicator "G" have similar clinical considerations, it would be appropriate for the Agency to include them in this proposal and ensure they are eligible for separate payment at average sales price (ASP) + 6%. Broadly ensuring adequate reimbursement, appropriately incentivizes providers to make treatment choices based on clinical eligibility, rather than reimbursement, reducing the potential for unintended consequences for patient access.

The purpose of C-APC packaging is to bundle payment for items and services that are typically integral, ancillary, supportive, dependent, or adjunctive to a primary service into the payment for that primary service. As CMS notes, these bundled services typically include diagnostic procedures and laboratory tests bundled into a primary service such as lymphatic surgery and musculoskeletal procedures. Cell and gene therapies clearly are not ancillary services designed to support these types of surgeries and procedures; in fact, they themselves will always serve as the primary service. The administration of cell and gene therapies often calls for their own supportive ancillary procedures, such as post-administration laboratory tests. Cell and gene therapies are very different than the categories of drugs that do support primary procedures, such as pain medications and blood pressure medications, which are intended to improve the outcomes of primary procedures. As CMS notes, the cell and gene therapies listed on Table 1 of the Proposed Rule "would not be used to support the outcome of any primary C-APC procedure" as they do not "promote a beneficial outcome" or "prevent possible complications" of any of the primary C-APC services. This

[2] 89 Fed. Reg. 59186, 59202 (July 22, 2024).

is certainly true in 2025, but it will remain true in 2026, 2027, and beyond.

However, rather than limiting it to 2025, CMS should make this proposal permanent. CMS suggests a one-year time horizon for the policy so that the agency may "gather more information from interested parties as to whether this proposed policy appropriately captures all of the unique therapies, such as the cell and gene therapies listed in the aforementioned table, that function as primary treatments and do not support C–APC primary services." ARM agrees that additional therapies will need to be added to Table 1 in the future, given the robust pipeline of cell and gene therapies. ARM also agrees that other drug classes besides cell and gene therapies may be included in future years as well. The concern that CMS may need to expand the list in the future is not a justification for declining to commit to separate payment for C-APCs beyond 2025. We instead recommend an approach that CMS adopt a permanent policy of excluding cell and gene therapies and other drug classes that are not used to support the outcome of any primary C-APC procedure.

ARM recommends acknowledging separate payments for Category I CAR-T CPT Codes

CAR-T therapy involves various distinct clinical services that can be performed in different settings, including those ordered by treating specialists and provided by hospitals. These services are integral to the comprehensive treatment of a patient's illness, occurring separate and apart from the cell manufacturing process. During these times, the manufacturer does not maintain control of the patient's cells, and hospitals are entirely responsible for the patient's individualized care. As therapeutic approaches evolve, critical elements upstream in the administration process, such as cell collection or dose preparation, have shifted to physician offices or other outpatient locations. In such cases, one entity will be reimbursed for cell collection activities, and a different entity will receive reimbursement for product costs. Medicare coding should facilitate, rather than hinder, arrangements that improve access to care. CMS has previously recognized the importance of enabling beneficiaries to access services in community settings. Given this, it is essential to modify CMS's payment policy for these codes.

Currently, CMS has not clarified why it generally does not pay for each step required to manufacture a drug or biological. However, our members observe that this approach does not align with the processing of ancillary services necessary to prepare a dose for patient administration. To promote consistency and patient access, the Agency should support the use of cell and gene therapies in non-hospital settings, which are often more cost-effective and convenient. ARM recommends that CMS finalize CPT codes 3X018, 3X019, and 3X020 with a status indicator of "S" to appropriately recognize these distinct clinical services in the final rule.

CMS Rightly Calls for Separate Payment for High-Cost Drugs Provided in IHS and Tribal Facilities, But Should Ensure Rates Are Sufficient

ARM agrees with CMS's proposal to provide separate payment for high-cost drugs provided by IHS and tribal facilities. CMS notes that separate reimbursement is necessary in this circumstance to protect access to care as "if providing a drug or service costs IHS and tribal facilities thousands of dollars more than the payment they receive through the AIR, it is likely not financially feasible for these facilities to routinely provide that drug or service." As CMS rightly observes, this is an issue of

equity and access. Medicare beneficiaries relying on IHS and tribal facilities should have the same access to drugs as Medicare beneficiaries who receive care in other settings. Continuing to deny separate payment for these therapies in IHS and tribal facilities – even in cases where the same drugs are paid for separately under the OPPS – would undermine CMS's equity and access goals.

CMS is also correct to expand the definition of high-cost drugs beyond therapeutics for oncology. Cancer treatments are not the only specialty drugs that may be administered in IHS and tribal facilities. While cell and gene therapies are typically not provided in these settings, ARM and its members are dedicated to improving access to such therapies, and we hope those therapies are more routinely administered in IHS and tribal facilities soon. Medicare payment policy should not be a barrier to that potential reality.

As CMS notes, the cost of living is higher in Alaska than the lower 48 states and an adjustment to payment is necessary to consider cost realities and parallel payment under OPPS. However, we recommend that CMS consider paying facilities at ASP+6%, rather than ASP without an add-on. CMS's stated policy for the lower payment rate is that IHS and tribal facilities typically acquire drugs under the Federal Supply Schedule ("FSS"), which typically has a lower acquisition cost than the price at which facilities acquire drugs that operate under the OPPS. However, ARM suggest that CMS pay at ASP+6% until it can determine the frequency at which IHS and tribal acquire drugs at a lower rate than facilities subject to the OPPS.

Conclusion

The field of regenerative medicine has the potential to heal people while lowering long-term costs and improving quality outcomes. CMS should continue to take steps to ensure Medicare beneficiaries can benefit from cell and gene therapies.

We thank CMS for its consideration of our comments. Please feel free to contact Monet Stanford at mstanford@alliancerm.org with questions.

Sincerely,

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