August 14, 2023

The Honorable Jason Smith
Chair
Committee on Ways & Means
U.S. House of Representatives
Washington, DC 20515

The Honorable Richard Neal
Ranking Member
Committee on Ways & Means
U.S. House of Representatives
Washington, DC 20515

Dear Chair Smith and Ranking Member Neal:

Given your strong interest in coverage of innovative technologies for Medicare beneficiaries and addressing policies limiting patient access to treatments and cures, the Alliance for Regenerative Medicine (ARM) offers the following comments detailing the challenges of the New Technology Add-on Program (NTAP). 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 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.

Signed into law in 2001, the NTAP program was created by Congress to help provide timely access to new, clinically significant technologies for Medicare beneficiaries. The Centers for Medicare & Medicaid Services (CMS) uses its discretionary authority to issue NTAP regulations to determine a process for issuing add-on payments for new medical services and technology.

In recent years, numerous transformative – and sometimes life-saving -- cell and gene therapies (CGTs) have been approved by the Food and Drug Administration (FDA) for some of the most difficult-to-treat conditions, 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, including for therapies that could provide durable treatments and possibly cures for diseases affecting Medicare beneficiaries. As many CGTs are administered in the inpatient site of care, the NTAP program is a crucial factor in ensuring beneficiaries have timely access to these therapies.

Unfortunately, on August 1, 2023, CMS finalized a new policy that will make it even more challenging for new therapies, including CGTs, to receive the NTAP payments following approval by the FDA.[1] Specifically, beginning with NTAP applications for FY 2025, to be eligible for an NTAP a product must receive FDA approval by May 1 of the previous fiscal year. Therefore, if a therapy receives FDA approval on May 2, 2024, the NTAP could not take effect until October 1, 2025, 17 months after FDA approval. ARM believes the requirement that technologies receive FDA approval by May 1 unnecessarily delays access to innovative and often lifesaving therapies for Medicare beneficiaries, especially those approved just after the new May 1 deadline. CMS has implemented this policy to lessen the agency's burden of

[1] https://www.cms.gov/newsroom/fact-sheets/fy-2024-hospital-inpatient-prospective-payment-system-ipps-and-long-term-care-hospital-prospective-0

reviewing NTAP applications. However, Medicare beneficiaries cannot afford to wait 17 months to access a life-saving CGT and ARM believes there is a better solution that both reduces CMS' burden and expedites access.

Specifically, ARM submitted comments to recommend that CMS implement a biannual NTAP application process for CGTs. A biennial process would both expedite access and minimize CMS's burden by allowing manufacturers the opportunity to submit NTAP applications closer to (or even after) FDA approval. CMS has indicated that it is infeasible to adopt this approach under existing law. **We therefore encourage Congress to legislate a biannual NTAP application process for CGTs to ensure timely access to these life-saving therapies.**

ARM also believes that an increased NTAP reimbursement rate will help expedite access to CGTs. The current NTAP rate equals the lessor of the costs of the new medical service or technology or 65 percent of the amount by which the costs of the case exceed the standard DRG payment. This NTAP reimbursement formula, however, deflates the overall amount because it focuses on an amount that is the "lesser of" two calculations. Congress instructed CMS to reimburse hospitals an amount that reflects the estimated average cost of the technology. ARM respectfully disagrees that the 65 percent payment rate within the current "lessor of" formula satisfies Congressional intent.

ARM believes that the reimbursement formula does not adequately reimburse hospitals for providing new technology. Even after reimbursement approval, a gap between pricing and reimbursement often exists, meaning that many early hospital adopters of new cell and gene therapies could lose money on each procedure performed. Accordingly, inadequate reimbursement can decrease hospital adoption of CGTs. **ARM urges Congress to require CMS to set the NTAP rate at 80 percent of the amount by which the costs of the case exceed the standard DRG payment, aligning it with the Medicare outlier payment rate[2] and alleviating strain on hospitals providing new and innovative CGTs to Medicare beneficiaries.**

We look forward to working with you and other stakeholders to advance policies that ensure Medicare patients have timely access to transformative therapies, including CGTs. If you have any questions, please contact me at ecischke@alliancerm.org.

Sincerely,

Erica Cischke, MPH

Vice President, Government Affairs

Alliance for Regenerative Medicine

[2] https://www.cms.gov/medicare/medicare-fee-for-service-payment/acuteinpatientpps/outlier