American Society of Gene + Cell Therapy

June 10, 2024

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
U. S. Department of Health and Human Services
200 Independence Avenue, SW
Washington, DC 20201

Dear Administrator Brooks-LaSure:

The American Society of Gene and Cell Therapy appreciates the opportunity to comment on CMS-1808-P, the proposed rule for Medicare's Hospital Inpatient Prospective Payment System (IPPS) for 2025.

About ASGCT

The American Society of Gene and Cell Therapy (ASGCT) is a nonprofit professional membership organization comprised of more than 6,200 scientists, physicians, patient advocates, and other professionals. Our members work in a wide range of settings including universities, hospitals, government agencies, foundations, and biotechnology and pharmaceutical companies. Many of our members have spent their careers in this field performing the underlying research that has led to today's robust pipeline of transformative therapies. A core portion of ASGCT's mission is to advance the discovery and clinical application of genetic and cellular therapies to alleviate human disease. To that end, ASGCT supports Medicare payment policies that foster the adoption of, and patient access to, new therapies, which thereby encourage continued development of these innovative treatments. The Society's support of sufficient and appropriate reimbursement levels to providers to facilitate patient access does not imply endorsement of any individual pricing decisions.

2025 Proposals

New Technology Add-On Payment

The New Technology Add-On Payment (NTAP) is a critical tool for CMS to support patient access to new gene and cell therapies coming to market. These therapies are re-shaping the landscape of treatment for rare diseases, offering unprecedented opportunities to impact the lives of patients who suffer from them. However, cell and gene therapies also represent a paradigm shift; rather than treating a disease with a lifetime

of medications, these therapies typically involve a limited number of treatments. The limited number of treatments result in a pricing structure that differs significantly from traditional medicines and therapies.

In recent years, CMS has taken steps to acknowledge the unique nature of gene and cell therapies, following the approval of Chimeric Antigen Receptor (CAR) T-Cell Therapy. CMS took the step of establishing a new MS-DRG specifically for CAR T-cell therapy, despite the relatively low volume of cases applicable to the DRG. However, before CMS established the DRG – CMS awarded the NTAP for two CAR T-cell therapy products. This decision provided a critical access bridge for these products, ensuring that providers could continue to make the products available to patients. This case study is illustrative of the importance of an effective NTAP policy in supporting patient access to new gene and cell therapies coming to market.

CMS proposes to increase the New Technology Add-On Payment (NTAP) percentage from 65% to 75% for gene therapies used to treat sickle cell disease.

ASGCT supports CMS' proposal to increase the NTAP percentage for gene therapies used to treat sickle cell disease. Last year, the Food and Drug Administration (FDA) approved two new sickle cell therapies for use in patients. We are grateful that, moving forward, sickle cell disease patients will finally be able to experience some relief from pain crises and other serious complications that come with the disease.

ASGCT supports policies that enable patient access to these innovative new therapies, including changes to the NTAP. As stated earlier, the NTAP can provide a critical access bridge for new gene and cell therapies coming to market, supporting beneficiary access to new products. Increasing the percentage of the therapy cost included in the NTAP can potentially help make these innovative new therapies available to Medicare beneficiaries living with sickle cell disease.

CMS proposes to modify the effective date of a product's newness period, tying it to the start of the Fiscal Year rather than April 1.

ASGCT supports policies that ensure the NTAP provides the largest possible impact on Medicare beneficiary access to new products.

ASGCT remains concerned with policy changes that CMS made in the FY 2024 IPPS rule that could impact which products can gain NTAP status. Specifically, CMS finalized a policy that required products to have FDA approval by May 1 rather than July 1 in order to be considered for NTAP status in a fiscal year. Restricting access to the NTAP for future gene and cell therapies could have significant implications for patients' access to new, life-changing therapies arriving on the market. Modifying the deadline for FDA marketing authorization could jeopardize the ability of manufacturers to submit applications in a time that maintains their eligibility for the NTAP.

In the FY 2025 proposed rule, ASGCT appreciates that CMS has proposed changes intended to mitigate the impact of the FY 2024 change. Specifically, CMS has proposed to start the clock on a product's newness status on October 1 rather than April 1, helping a product to receive three complete years of NTAP status. Changes that help products keep an NTAP status for a longer duration can help to ease risks of patient access associated with the end of the NTAP and transition to payment through the normal Diagnostic Related Group (DRG) payment system.

Further Recommendations for the NTAP

Beyond the proposals included in the FY 2025 IPPS rule, ASGCT reiterates the following additional recommendations:

CMS should establish multiple review periods for NTAP approval during the year.

Establishing multiple periods for NTAP review and approval during the year, as well as beginning NTAP payments outside of the strict fiscal year cycle, would relieve much of the pressure associated with deadlines for the Fiscal Year rule cycle. Specifically, ASGCT recommends that CMS establish a quarterly review process for NTAP-qualifying products approved by the FDA, regardless of the approval pathway. The NTAP should be immediately accessible for new technologies coming to market and not be tied to an annual rulemaking cycle.

Other recommendations

- The ability for manufacturers to apply for NTAP when they have data to complete an NTAP application and CMS to "pend" those applications deemed to meet the applicable NTAP criterion until the product is marketed.
- An increase in the cap for NTAP amounts from 65 percent to 100 percent or a uniform NTAP equal to the product acquisition cost for gene and cell therapies. We appreciate the recent actions of CMS to increase the NTAP cap in FY 2020 from 50 percent to 65 percent, as well as the proposed changes specific to sickle cell therapies included in the FY 2025 proposed rule. However, even the 65 percent level would not be expected to sufficiently fill the gap in reimbursement to providers.
- Continue to recognize the limited patient populations (especially for products indicated for rare diseases) when considering the number of cases (excluding clinical trials cases) sufficient to establish a new DRG. Because the process for establishing new MS-DRGs is dependent upon CMS having sufficient data on charges for therapy, the creation of DRGs for gene and cell therapies for rare diseases with small populations can be delayed well past the NTAP period. If CMS intends to pay for future gene and cell therapies in a similar fashion to CAR T cell therapy through NTAP assignment as applicable, followed by the establishment of new DRGs, CMS must have flexibility in its metrics for such establishment.

American Society of Gene + Cell Therapy

Predictability for Gene and Cell Therapies Coming to Market

As ASGCT has shared with CMS in prior comment letters, we remain concerned about the uncertainty for new gene and cell therapies coming to market. To that end, we encourage CMS to be transparent and forthcoming in potential approaches to paying for new gene and cell therapies.

ASGCT supported CMS' decision to establish a new DRG 018 for CAR T-cell therapy. The Society believes it was an appropriate step to ensure CMS would develop accurate coverage for this therapy. However, CMS has broadened the title of MS-DRG 018 to apply not just to CAR T-cell therapy but to other immunotherapies. Adding other therapies to MS-DRG 018 could have significant consequences on the accuracy of payments for CAR T-cell therapies and other gene or cell therapies included. If CMS were to assign higher volume, lower cost technologies to MS-DRG 018, it likely would distort the relative weight of the MS-DRG, potentially under-reimbursing autologous CAR-Ts.

ASGCT also remains interested in whether CMS intends to explore further changes to Medicare payment for gene and cell therapies through the Center for Medicare and Medicaid Innovation (CMMI). ASGCT was interested to see Medicare payment for gene and cell therapies discussed in the Secretary's report responding to the Executive Order "Lowering Prescription Drug Costs for Americans." The report indicates that CMMI will consider "potential Medicare fee-for-service options to support [Cell and Gene Therapy] access and affordability," identifying models such as bundled payments to replace traditional fee-for-service billing. To date, it appears that CMS has not released any additional information on this potential approach, though ASGCT is following CMS' implementation of the Cell and Gene Therapy Access Model focused on Medicaid beneficiaries.

Thank you for the opportunity to submit comments on Medicare's proposed update to inpatient payments in FY 2024. Please contact Margarita Valdez Martínez, Chief Advocacy Officer, at mvaldez@asgct.org, with any questions.

Sincerely,

David Barrett, JD,
Chief Executive Officer