American Society of Gene + Cell Therapy

June 7, 2023

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-1785-P, the proposed rule for Medicare's Hospital Inpatient Prospective Payment System (IPPS) for 2024.

About ASGCT

The American Society of Gene and Cell Therapy (ASGCT) is a nonprofit professional membership organization comprised of more than 6,000 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.

2024 Proposals

<u>New Technology Add-On Payment</u>

CMS proposes to modify new technology add-on payment (NTAP) application eligibility requirements related to FDA application status to

move FDA marketing authorization deadline from July 1 to May 1 for technologies that are not already FDA market authorized.

ASGCT has significant concerns with CMS' proposal to establish an earlier marketing authorization deadline for products as a part of eligibility for the NTAP. The Society appreciates the administrative challenges to CMS presented by the current NTAP process. CMS cites a higher volume of NTAP applications, which has placed an additional burden on agency reviewers. However, restricting access to the NTAP could have catastrophic implications for patient access – particularly with respect to new gene and cell therapies coming to market.

Cell and gene 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, cell and gene 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.

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. While ASGCT appreciates the administrative challenges presented by the higher volume of applications, we urge CMS to find an alternative means by which to address the difficulties without jeopardizing patient access.

CMS proposes to require that an applicant must have already submitted an FDA market authorization request before submitting an application for the NTAP, effective FY 2025.

As with the previous proposal, ASGCT has concerns with any action that might limit eligibility for the NTAP based strictly on timing of interactions with the FDA.

CMS raises concerns about the totality of data available to reviewers of NTAP applications in cases where NTAP applications are submitted prior to FDA applications. Specifically, CMS argues that this change "would further increase transparency and improve the evaluation process." As described earlier, the NTAP played a critical role in supporting patients' access to CAR T-cell therapies when they arrived to market. ASGCT encourages CMS to find alternative methods for collecting the needed information for NTAP reviewers that does not disqualify products based solely on the timing of submission to FDA.

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

An alternative approach to the restrictions CMS described would be establishing multiple periods for NTAP review and approval during the year, as well as beginning NTAP payments outside of the strict fiscal year cycle. If CMS were to allow manufacturers to apply for and receive NTAP status at multiple points during the year, the stakes of meeting deadlines for the annual rulemaking cycle would be significantly dissipated. 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.

ASGCT also offers the following additional recommendations for the NTAP.

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; however, even the 65 percent level would not be expected to sufficiently fill the gap in reimbursement to providers.
- NTAP eligibility for three full years to allow the increased collection of cost data for the small populations often treated by gene and cell therapies, prior to rate-setting, or establishing new MS-DRGs prior to NTAP expiration.
- 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.

Changes to MS DRG-018

CMS proposes multiple changes to the calculation used to determine the relative weight of MS-DRG 018. CMS proposes to exclude claims with the presence of condition code "90" or "ZB," and claims that contain ICD-19-CM diagnosis code Z00.5 with payer-only code "ZC" that group. CMS also proposes to stop using a proxy of charges less than $373,000 for identifying clinical trials.

ASGCT encourages CMS to ensure that any policy changes with respect to calculation of the relative weight of MS-DRG 018 maintain CMS' stated policy of excluding clinical trial cases and expanded use immunotherapy cases.

In developing DRG 018, CMS made the deliberate decision to exclude certain cases from the calculation of the relative weight. Namely, CMS opted to exclude cases involving clinical trials, which may not reflect the full cost of the therapy, and cases involving expanded use of immunotherapy. CMS made these decisions recognizing that inclusion of these cases would inappropriately skew CMS' calculation of the relative weight, a particular challenge in a DRG with a limited number of cases.

CMS has thus far implemented the changes by excluding claims with certain indicators. However, CMS has also used a proxy amount of $373,000 to identify clinical trial cases, recognizing that cases below that threshold are unlikely to represent the cost of a full case in which the provider purchased the therapy through normal means – a backstop, recognizing that cases may not always be properly coded.

CMS is proposing to eliminate the $373,000 proxy used to identify clinical trial claims. ASGCT is concerned that this change could lead to CMS inadvertently including inappropriate clinical trial cases in the calculation of the relative weight of MS-DRG 018. While coding accuracy has no doubt improved, the proxy amount remains a valuable way to ensure coding mistakes do not inadvertently lead to calculations that do not reflect CMS policy.

Predictability for Gene and Cell Therapies Coming to Market

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 payment amounts for payment of the therapy.

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However, CMS' process maintains significant uncertainty for new gene and cell therapies coming to market, impacting their manufacturers and providers looking to make these innovative new treatments available. CMS has broadened the title of MS-DRG 018 to apply not just to CAR T-cell therapy but to other immunotherapies. However, it remains unclear how CMS may treat the administration of gene and cell therapies outside of that bucket when they come to market. Simply adding all new gene and cell therapies to MS-DRG 018 could have significant consequences to the accuracy of payments. 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.

Additionally, the Society 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.

ASGCT has previously recommended that CMS invoke the authority of CMMI to establish new, alternative payment models for gene and cell therapies, outside of the constraints of the IPPS. By establishing a new, value-based payment model, CMMI could establish a clearer path to coverage and payment policy that can improve patient access. If implemented properly, the Cell and Gene Therapy Access Model could help beneficiaries gain access to potentially life-changing therapies. The Society encourages CMS to work with stakeholders to construct successful models that will ensure patient access to innovative therapies.

ASGCT applauds CMS for committing to a continued dialogue with stakeholders related to Medicare payment for cell and gene therapies. We urge CMS to continue engaging with stakeholders on this matter in an open and transparent fashion. Thank you for the opportunity to submit comments on Medicare's proposed update to inpatient payments in FY 2024. Please contact Margarita Valdez Martínez, Director of Policy and Advocacy, at mvaldez@asgct.org, with any questions.

Sincerely,

David Barrett, JD
Chief Executive Officer