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The Honorable Seema Verma, Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
P.O. Box 8011, Baltimore, MD 21244-1850

Dear Administrator Verma:

The American Society of Gene and Cell Therapy (ASGCT) appreciates the opportunity to comment on CMS-2482-P, the proposed rule for Establishing Minimum Standards in Medicaid State Drug Utilization Review and Supporting Value-Based Purchasing for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third Party Liability.

ASGCT is a professional membership organization representing over 4,300 individuals, including scientists, physicians, and other professionals in gene and cell therapy working in settings such as academic institutions, hospitals, 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. ASGCT therefore supports Medicaid payment policies that foster the adoption of, and patient access to, new therapies while avoiding undue financial strain to entities within the healthcare system. The Society's support of new payment models does not imply endorsement of any individual pricing decisions.

ASGCT supports CMS' proposal within this rule to enable value-based purchasing (VBP) models for drugs and biologics, including gene and cell therapies. The Society greatly appreciates the responsiveness of the Agency to stakeholder input to address the unique nature of gene therapies as high value treatments intended to be delivered in a single administration with a durable therapeutic benefit. Gene and cell therapies are accompanied by high upfront costs relative to traditional therapeutics delivered over the course of time. VBP arrangements spread risks between payers and manufacturers and distribute costs more equitably based on individual patient outcomes, which has the potential to improve access to these life-saving treatments. We respectfully offer additional comments on the rule to provide the perspective of ASGCT's professional membership, including the unique viewpoint of the scientific research community in the gene and cell therapy space.

Durability and Payment Over Time

Assessing the value of a product may include a review of its efficacy over a period of time. While short-term improvements are important to evaluate, longer-term impact is often a critical aspect of efficacy for gene therapies. ASGCT therefore appreciates CMS' proposal to extend the reporting period beyond 12 quarters, to allow manufacturers to engage in VBP arrangements that include reporting more durable outcomes and providing rebates for cases that do not meet longer-term predetermined outcomes.

ASGCT respectfully requests that CMS clarify whether it intends to enable VBP arrangements that contain payment-over-time provisions, rather than the provision of one-time rebates. The rule mentions payment-over-time models in section II. G., *Requirements for Manufacturers*, such as in the statement, "Many VBP arrangements or pay-over-time models may be better suited for periods longer than 12 quarters, and manufacturers entering into such arrangements may need to adjust AMPs and best prices beyond the 12 quarters because the evidence-based or outcomes-based measures are being measured beyond a period of 12 quarters or a final installment payment is being made outside of the 12 quarters." However, CMS does not indicate that it would adjust AMP and best price reporting mechanisms to enable value-based installment payments, which ASGCT would support.

Outcome Measures

Within section II, Subpart I, 1a, *Value-Based Purchasing Arrangements*, CMS welcomes suggestions for other measures of value/outcomes. In response, ASGCT suggests that CMS consider noting outcomes such as disease regression or halting of disease progression. For example, in oncology, tumor shrinkage may be significantly associated with overall survival for some treatments.¹ Additionally, gene therapies for rare diseases often result in halting of disease progression, which is quite significant for progressive diseases, some of which have high mortality rates in early childhood.² ASGCT also supports the incorporation of patient input into the selection of functionally relevant measures of efficacy.³

Lastly, aligning outcomes for value-based payment with those used for regulatory approval may be useful in some instances, including surrogate endpoints for trials. For example, clotting factor activity levels, used as a surrogate endpoint for regulatory approval for the treatment of hemophilia, also may be relevant outcomes for demonstration of efficacy for VBP arrangements.⁴

To support providers and manufacturers in obtaining and reporting outcomes, ASGCT encourages CMS and its federal partners to collaborate with state stakeholders on best practices related to collecting outcomes data, such as through patient registries that assist in patient follow-up efforts.

In addition, we encourage CMS to consider the burden on providers and patients alike of requirements for follow-up reporting. When possible, telehealth may be acceptable for collection of patient-reported

¹ Hopkins, A. M., Kichenadasse, G., Karapetis, C. S., Rowland, A. & Sorich, M. J. (2020). Early tumor shrinkage identifies long-term disease control and survival in patients with lung cancer treated with atezolizumab. *Journal for ImmunoTherapy of Cancer*, 8(1). <https://doi.org/10.1136/jitc-2019-000500>

² Park, H. B. et al. (2010). Survival analysis of spinal muscular atrophy type I. *Korean Journal of Pediatrics*, 53(11), 965-970. <https://doi.org/10.3345/kjp.2010.53.11.965>

³ Squitieri, L., Bozic, K. J. & Pusic, A. L. (2017). The role of patient-reported outcome measures in value-based payment reform. *Value Health*, 20(6), 834-836. <https://doi.org/10.1016/j.jval.2017.02.003>

⁴ Konkle, B. A., Skinner, M. & Iorio, A. (2019). Hemophilia trials in the twenty-first century: Defining patient important outcomes. *Research and Practice in Thrombosis and Haemostasis*, 3(2), 184-192. <https://doi.org/10.1002/rth2.12195>

outcomes. ASGCT would gladly participate in any efforts to collaborate and share information with members as appropriate.

Additional Considerations

ASGCT respectfully requests that CMS work with necessary federal partners in the executive and legislative branches to address any other potential barriers to value-based payment arrangements. For example, we encourage CMS to work with federal partners to address any implications of the Anti-Kickback Statute. Even if policy changes are ultimately unnecessary, it may be helpful for CMS to state that an existing safe harbor allows the provision of rebates up to 30 months after treatment and to indicate whether rebates beyond this time frame are allowable under VBP arrangements.

The Society also encourages CMS to continue to address other issues that affect the adoption of, and patient access to, gene and cell therapies, through provision of information to states on coverage requirements for FDA-labelled indications and on best practices for state mechanisms to reimburse providers sufficiently for them to be able to provide these therapies to Medicaid beneficiaries.

To conclude, we reiterate our sincere gratitude to CMS for proposing the enabling of VBP arrangements. ASGCT encourages finalization of this provision of the rule and appreciates Agency attention to stakeholder comments in determining the details of its operationalization. Please let us know if you have any questions.

Sincerely,



Stephen Russell, MD, PhD
President
American Society of Gene & Cell Therapy