

June 28, 2021

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The Honorable Chiquita Brooks-LaSure  
Centers for Medicare & Medicaid Services  
Department of Health and Human Services  
PO Box 8011  
Baltimore, MD 21244-1850

Dear Administrator Brooks-LaSure:

The American Society of Gene and Cell Therapy (ASGCT) appreciates the opportunity to comment on CMS-2482-P2, the proposed rule to delay for six months the January 1, 2022 effective date for amendatory instruction 10.a., which addresses the reporting by manufacturers of multiple best prices connected to a value based purchasing (VBP) arrangement, of the final rule entitled, "Medicaid Program; Establishing Minimum Standards in Medicaid State Drug Utilization Review (DUR) and Supporting Value-Based Purchasing (VBP) for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third Party Liability (TPL) Requirements."

ASGCT is a nonprofit professional membership organization comprised of more than 4,600 scientists, physicians, and other professionals working in gene and cell therapy in settings such as universities, hospitals, and biotechnology 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.

To that end, ASGCT has been supportive of the development of innovative payment models, including value-based payment arrangements. To the degree these arrangements hold potential to foster greater beneficiary access to innovative, lifesavings therapies, ASGCT encourages the Centers for Medicare & Medicaid Services (CMS) to eliminate barriers to value-based payment arrangements. The Society's support of new payment models does not imply endorsement of any individual pricing decisions.

ASGCT was pleased to see CMS take steps to remove barriers to value-based purchasing (VBP) arrangements for drugs and biologics, including

gene and cell therapies, in the 2020 final rule (CMS-2482-F). ASGCT is supportive of efforts from CMS to address uncertainty in value-based payment arrangements created by Medicaid best price reporting.

While ASGCT is eager for implementation of the rule, the Society understands that this proposed six-month implementation delay to certain provisions of the final rule will allow CMS to more effectively operationalize the complex changes required.

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 chronically delivered therapeutics. 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 lifesaving treatments. Our positions in support of the facilitation of VBP, outlined below, reflect the perspective of our professional membership, including the unique viewpoint of the scientific research community in the gene and cell therapy space.

#### *Durability and Payment Over Time*

ASGCT appreciates CMS' willingness to extend the reporting period beyond 12 quarters, to allow manufacturers to engage in VBP arrangements that include reporting more durable outcomes and allowing for VBP arrangements that include a pay-over-time component. 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. We therefore appreciate that CMS indicates in the final rule that it will consider issuing operational guidance to assist manufacturers in the reporting of average manufacturer price and best price reporting requirements, which will clarify such requirements for value-based pay-over-time arrangements.

#### *Additional Considerations*

ASGCT appreciates that in the final rule, CMS noted it will consider whether additional guidance may be needed at a later date to address other regulatory obstacles to the uptake and operationalization of VBP arrangements, including potential implications of the Anti-Kickback Statute. The Society encourages CMS to issue this additional guidance. 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 enabling VBP arrangements through this regulation. The Society appreciates continued Agency attention to stakeholder comments in determining the details of its operationalization of this rule. If you have any questions, please contact Betsy Foss-Campbell, ASGCT's Director of Policy and Advocacy, at [bfoss@asgct.org](mailto:bfoss@asgct.org).

Sincerely,

A handwritten signature in black ink, appearing to read 'David Barrett', is written over a horizontal line.

David Barrett, JD  
Chief Executive Officer