American Society of Gene + Cell Therapy

July 25, 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-2434-P, which includes proposed regulatory updates to the Medicaid Drug Rebate Program (MDRP).

ASGCT appreciates CMS' commitment to program integrity in the MDRP. Accuracy in payment and protecting taxpayer resources are laudable goals for any government agency. However, the Society is concerned with multiple proposals CMS has put forth in this regulation. We believe these policies could negatively impact beneficiary access to the life-changing cell and gene therapies.

Specifically, we will offer comments on three policy areas:

- Surveys of drug manufacturers
- Changes in the definition of a covered outpatient drug
- Best price and "stacking" of discounts

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; in fact, over 77% of our membership are researchers.

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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 payment policies that foster the adoption of, and patient access to, new therapies, which thereby encourage continued development of these innovative treatments.

ASGCT supports the development of value-based arrangements between state Medicaid programs and the manufacturers of gene and cell therapies that support greater patient access for these durable, and potentially curative treatments. The accessibility of therapies to patients is of paramount importance to the Society's membership. If implemented properly, value-based payment arrangements could help Medicaid beneficiaries gain access to potentially life-changing therapies. Outcomes-based arrangements negotiated between state Medicaid programs and manufacturers represent an opportunity to develop methods of payment that minimize the importance of one-time transactions and create greater focus on beneficiary access and quality of care.

Proposals

CMS proposes to establish a drug price verification survey process of certain reported Covered Outpatient Drugs (CODs), collecting new information from manufacturers including production costs. Under the proposal, CMS would identify CODs for surveys based on metrics around Medicaid spending and price, excluding those offering supplemental rebates and those negotiating price with CMS, and other factors. Subsequently, CMS would survey manufacturers to collect several pieces of information, including costs of production and research. CMS also indicates that some amount of data would be made public. In describing the rationale, CMS references gene and cell therapies multiple times. CMS notes these therapies are "transformative in terms of therapeutic benefits," while noting concerns about price.

ASGCT is greatly concerned about CMS' proposed drug price verification system. Specifically, the Society is concerned about the administrative burden and the precedent set by this expansion of CMS' survey authority.

First, the issue of administrative burden on manufacturers is significant. Many manufacturers of gene and cell therapies are attempting to bring new products to market that, as CMS acknowledges, hold the potential to be "transformative in terms of therapeutic benefits". Some of these manufacturers are smaller, innovator companies with more limited resources, and narrow product lines focused on the new product coming to market. These companies are working to bring these products to market in a safe and efficient manner while addressing challenges in entering a market with a multitude of government and commercial payers. Imposing additional survey requirements on these

manufacturers represents yet another additional layer of government regulation, which could lead to unnecessary delays in new therapies reaching patients.

In addition, CMS is requesting data it does not need to accurately execute the MDRP. The scope of data requested by the survey includes cost information that is not relevant to CMS' identification of Medicaid best price and calculation of rebates, including research and manufacturing costs. Collection of this information represents a sizeable – and unnecessary – burden on manufacturers, leaving CMS with proprietary information that is not essential to execution of the nation's laws and regulations.

CMS' proposal to exclude certain drugs may also steer manufacturers away from negotiating novel payment arrangements with states. CMS proposes to exclude from the list drugs subject to verification any drug whose price has been negotiated with CMS (under the Inflation Reduction Act's negotiation authority, or other initiatives) or any drugs for which CMS has authorized a supplemental rebate with at least 50% of states. That approach potentially incents manufacturers to follow more narrowly tailored approaches in order to meet these exceptions, as opposed to agreements tailored specifically to the therapy, both in terms of clinical outcomes and the unique nature of the eligible patient population.

Finally, it is not clear to ASGCT that CMS has the authority to embark on the drug verification process it describes in the proposed rule. As CMS notes, the Social Security Act provides the Secretary with the authority to survey wholesalers and manufacturers – but only "to verify manufacturer prices" that are reported to CMS, whereas the proposed rule seeks to exceed the verification process and place additional burdens upon certain manufacturers.

ASGCT does appreciate CMS' interest to better understand cell and gene therapies. These transformative therapies represent astounding gains in the field of medical science, and a triumph of modern medicine. Products already in the market offer durable and potentially curative treatments for patients with conditions spanning advanced blood cancers, rare diseases and more. Products in the pipeline hold the potential to cure sickle cell disease, among other significant advances, and timely patient access to these long-awaited innovative gene and cell therapies is critical. The development, manufacturing, and administration of these products is novel. To that end, ASGCT encourages CMS to more fully engage with stakeholders to further CMS' understanding of the field. ASGCT members are prepared to offer site visits; facilitate dialogue with scientists, clinicians, and other members involved in the development and administration of these therapies; and offer any educational resources that might be of assistance to CMS. Again, ASGCT urges CMS to reconsider the drug price verification system proposed in the rule.

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CMS proposes to modify the definition of "covered outpatient drug" to include instances in which a drug is not only separately payable, but when a claim identifies the drug plus the itemized cost of the drug.

ASGCT is concerned that CMS' proposed expansion of the term "covered outpatient drug" may inadvertently create new access challenges for Medicaid beneficiaries seeking new gene and cell therapies.

As noted earlier, ASGCT believes that outcomes-based arrangements hold tremendous potential for improving beneficiary access to gene and cell therapies. Gene and cell therapies represent a new paradigm for the health care system, trading the upfront cost of a single or limited-time application of a therapy for (often) a lifetime of disease management and worse health outcomes. CMS' proposal may create new instances in which a therapy administered in the inpatient or outpatient setting would be subject to a bevy of new rebate requirements and other regulations associated with the MDRP. These constraints may drive manufacturers away from innovative, outcomes-based arrangements with states, forcing a one-size fits all approach on a uniquely dynamic industry. ASGCT encourages CMS to rescind the proposed expansion of covered outpatient drugs.

CMS proposes to apply price concessions applied to multiple parts of the supply chain when calculating Medicaid best price, often referred to as stacking.

ASGCT is concerned with the proposal to apply stacking to the assessment of Medicaid best price. The Society believes that incorporating stacking is not an accurate representation of the best price by which a manufacturer makes a product available. Rather than assessing the best price by which a drug manufacturer makes a product available to an individual actor, CMS' proposal would apply unrelated concessions in the market to the best price.

Incorporating the stacking of multiple discounts is not a determination of best price, but a commentary on a complex system that requires a multitude of transactions. Particularly for smaller innovator companies with limited product lines, this complex marketplace can be challenging to navigate. Incorporating aspects of not one but all transactions into the calculation of the best price can make bringing a potentially life-changing therapy to market even more challenging. As with earlier proposals, ASGCT encourages CMS to rescind the proposal to incorporate stacked price concessions into the calculation of Medicaid best price.

Thank you for the opportunity to submit comments. Please contact Margarita Valdez Martínez, Director of Policy and Advocacy, at mvaldez@asgct.org, with any questions.

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Sincerely,

David Barrett, JD
Chief Executive Officer