PERSONALIZED MEDICINE COALITION

May 14, 2024

Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244-1850

BY ELECTRONIC DELIVERY

Re: *Medicaid Program; Misclassification of Drugs, Program Administration and Program Integrity Updates under the Medicaid Drug Rebate Program* final rule

Dear Administrator Brooks-LaSure:

On behalf of the Personalized Medicine Coalition (PMC), a multi-stakeholder group established 20 years ago and comprising more than 200 institutions to promote the understanding and adoption of personalized medicine concepts, services, and products for the benefit of patients and the health care system, I am writing to urge the Centers for Medicare & Medicaid Services (CMS) to withdraw its proposed rule titled *Misclassification of Drugs, Program Administration and Program Integrity Updates Under the Medicaid Drug Rebate Program*.[i] The Medicaid Drug Rebate Program (MDRP) requires an agreement between participating manufacturers and the Secretary of the Department of Health and Human Services for manufacturers to offer their drugs within the Medicaid program. The program helps offset the federal and state costs of outpatient prescription drugs dispensed to Medicaid patients. PMC is concerned that certain changes proposed in this rule may negatively impact access to cell and gene therapies by disincentivizing investment in this promising space and discouraging the adoption of value-based agreements that can help facilitate patient access to these transformative treatments. We believe the impacts of these proposed changes are misaligned with other agency efforts to facilitate Medicaid beneficiaries' access to cell and gene therapies.

Personalized medicine is a rapidly evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By making long-lasting changes to the genetic make-up of patients' cells, cell and gene therapies can reverse the root causes of certain cancers and rare diseases with just one or a few treatments.

Personalized medicine is helping to shift the patient and provider experiences away from trial-and-error toward a more streamlined process for making clinical decisions, which will lead to improved patient outcomes, a reduction in unnecessary treatment costs, and better patient and provider satisfaction. PMC and its members are leading

the way in personalized medicine and in developing evidence showing how patients and the health care system can benefit from appropriate testing and tailored treatment as soon as possible during their clinical experiences. There are over 700 cell and gene therapies in development,[ii] and these therapies present tremendous opportunities for patients with unmet medical needs.

Statement of Neutrality

PMC's members may have already presented their own responses to CMS' *Misclassification of Drugs, Program Administration and Program Integrity Updates Under the Medicaid Drug Rebate Program* (CMS-2434-P) proposed rule during or since the public comment period and may actively advocate for those positions. PMC's response is designed to provide feedback so that the general concept of personalized medicine can advance, and is not intended to impact adversely the ability of individual PMC members, alone or in combination, to communicate separately with CMS on this proposed rule or related issues.

Creating disincentives for cell and gene therapies

PMC's annual analysis of new personalized medicines identified six new gene or cell therapies approved by the FDA in 2023 that can improve the lives of individuals living with sickle cell disease, blood cancers, or rare genetic diseases, including children with Duchenne muscular dystrophy.[iii] By providing health care coverage for lower-income Americans, including children and their parents, pregnant women, the elderly, and people living with disabilities, Medicaid is an important payer for cell and gene therapies, including recently approved gene therapies for sickle cell disease. As a condition of making drugs available to Medicaid beneficiaries, CMS proposed a new "drug price verification survey" in the rule that would require drug manufacturers meeting certain criteria to report unprecedented amounts of data to CMS, including pricing, utilization, and manufacturing cost data, as well as other proprietary and clinical information.

CMS indicates the purpose of the survey is to allow states to better understand how prices are derived under the MDRP. Unfortunately, the survey targets drugs with the highest Medicaid spending, which would disproportionately burden manufacturers of cell and gene therapies because they typically come to market with higher up-front costs. (The tremendous clinical and economic value of these therapies becomes clear over longer time horizons.) In addition to the increased compliance burden, PMC is concerned that the survey could have broader negative impacts on investment decisions and patient communities' access to potentially transformative therapies if the information CMS collects and publishes from these surveys is used to gather additional rebates from manufacturers and if manufacturers are disincentivized to continue to offer some of their medicines in the Medicaid program.

Discouraging value-based agreements for cell and gene therapies

Value-based agreements allow drug manufacturers and payers to collaborate to ensure that patients have access to new, potentially curative personalized treatments. Through listening sessions with the patient community, PMC has heard directly from patients that they believe value-based agreements can play a pivotal role in advancing their access to new personalized medicines.[iv] Value-based agreements can help states reap the clinical and economic benefits of cell and gene therapies by establishing outcomes-based arrangements or by smoothing states' high up-front costs for these therapies over time.

To help facilitate access to new gene therapies and improve the lives of Medicaid beneficiaries, the CMS Innovation Center recently announced a Cell and Gene Therapy Access Model, which aims to make it easier for states to pay for cell and gene therapies by supporting value-based agreements between manufacturers and state Medicaid programs, beginning with new gene therapies for sickle cell disease.[v] However, progress in adopting these agreements could be undercut by significant changes to the MDRP proposed by CMS.

CMS is proposing to aggregate, or "stack," price concessions across sales transactions and entities when determining "best price" under the MDRP, including rebates and discounts made to pharmacies, payers, and providers. By effectively increasing the rebates manufacturers pay to states and the federal government, the stacking proposal would decrease manufacturers' willingness to enter into value-based agreements with state Medicaid programs where manufacturers may voluntarily agree to additional discounts or rebates to enhance patient access. As a result, CMS' proposal could disincentive the development of future value-based agreements for cell and gene therapies.

Lastly, drugs covered under the Medicaid Drug Rebate Program are "Covered Outpatient Drugs" (CODs), which include pharmacy-dispensed drugs and some clinician-administered drugs. Under CMS' amended definition for CODs in the rule, any drug, including cell and gene therapies administered in an inpatient setting, whose costs are separately identified on a claim for payment would now be considered a COD. Many cell and gene therapies are administered in the hospital inpatient setting to ensure patient safety. By broadening the definition of CODs to count cell and gene therapies when they are identified separately on a claim form, CMS expands manufacturers' rebate liability, removes a state's incentive to pay separately for these therapies, and ultimately decreases reimbursement to hospitals. Separate payment, as currently practiced, protects hospitals from financial losses when bundled payment is inadequate for administering cell and gene therapies to patients. Adequate hospital reimbursement is necessary to foster clinical uptake of and patient access to new cell and gene therapies.

Conclusion

By creating uncertainties around the viability of value-based agreements and potential disincentives for the investment in and clinical adoption of cell and gene therapies, CMS' proposed changes to the MDRP could impede the ability of Medicaid beneficiaries with unmet medical needs to benefit from transformative cell and gene therapies, now and in the future. Given the potential for negative impacts of these proposals and their misalignment with the goals of the CMS Innovation Center Cell and Gene Therapy Access Model, we urge CMS to withdraw its proposed rule for *Misclassification of Drugs, Program Administration and Program Integrity Updates Under the Medicaid Drug Rebate Program* (CMS-2434-P). If you have any questions about the content of this letter, please contact me at 202-499-0986 or cbens@personalizedmedicinecoalition.org, or David Davenport, PMC's Manager of Public and Science Policy, at ddavenport@personalizedmedicinecoalition.org or 804-291-8572.

Sincerely,

Cynthia A. Bens
Senior Vice President, Public Policy

cc: Committee on Finance
U.S. Senate

Domestic Policy Council
The White House

Office of Management & Budget
The White House

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[i] Centers for Medicare & Medicaid Services. *Medicaid Program; Misclassification of Drugs, Program Administration and Program Integrity Updates Under the Medicaid Drug Rebate Program*: Proposed Rule (CMS-2434-P). https://www.federalregister.gov/d/2023-10934 (accessed May 3, 2024).

[ii] Lutzmayer, S & Wright, A. "Piping Hot: A Look at the State of Cell, Gene and RNA Therapies in Early 2023." *IQUVIA.* May 2, 2023. https://www.iqvia.com/blogs/2023/04/piping-hot-a-look-at-the-state-of-cell-gene-and-rna-therapies-in-early-2023 (accessed May 3, 2024).

[iii] Personalized Medicine Coalition. *Personalized Medicine at FDA: The Scope and Significance of Progress in 2023*. February 29, 2024. https://www.personalizedmedicinecoalition.org/wp-content/uploads/2024/02/report-3.pdf (accessed May 3, 2024).

[iv] Personalized Medicine Coalition. *Moving Beyond Population Averages: A Patient-Centered Research Agenda Advancing Personalized Medicine.* August 2020. https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_Moving_Beyond_Population_Averages_A_Patient-Centered_Research_Agenda_Advancing_Personalized_Medicine1.pdf (accessed May 3, 2024).

[v] Centers for Medicare & Medicaid Services. *Cell and Gene Therapy (CGT) Access Model*. https://www.cms.gov/priorities/innovation/innovation-models/cgt (accessed May 3, 2024).