



Biotechnology Innovation Organization

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April 8, 2024

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

The Honorable Shalanda Young
Director
Office of Management & Budget
725 17th Street, N.W.
Washington, D.C. 20503

Dear Administrator Brooks-LaSure & Director Young:

We, the undersigned, are writing to express our strong concern with a proposed update to the Medicaid Drug Rebate Program (MDRP). If finalized in its current form, the proposed rule (CMS-2434-P) would impose significant costs to the government and industry and create extensive barriers to patient access to existing innovative therapies and to developing new cures for Medicaid enrollees.¹ As a result, it will deny some of the nation's most vulnerable patients access to the state-of-the-art medical care they deserve, both now and in the future.

We are deeply committed to advancing medical science in ways that prevent suffering and save lives -- particularly for the less advantaged populations Medicaid was created to serve. But the proposed rule would make our continued participation more difficult, while leaving patients who rely on Medicaid profoundly worse off.

Small- and medium-sized biopharmaceutical companies like ours already face severe headwinds in bringing new therapies and cures to patients. Nearly every aspect of the drug development process -- from identifying potential new therapies to securing venture capital and organizing clinical trials -- is fraught with uncertainty, the risk of which translates directly into higher costs. And the overwhelming majority of candidate drugs we pursue ultimately fail, some only after years (or decades) of research and tens of millions of dollars.²

What drives us to overcome these challenges and endure repeated failures is the knowledge that, when we do succeed, our work can save lives and improve health on a massive scale. Our desire to serve our nation's less advantaged populations is central to this mission.

The Medicaid Drug Rebate Program has helped bring hundreds of revolutionary therapies to underserved and marginalized patients while maintaining incentives for continued research into new treatments and cures. Unfortunately, the proposed rule would put this carefully struck balance in jeopardy.

¹ <https://www.cms.gov/newsroom/fact-sheets/misclassification-drugs-program-administration-and-program-integrity-updates-under-medicaid-drug>

² <https://www.nature.com/articles/nrd.2016.136>

Of particular concern is the proposed rule's new definition of "best price." Current law defines this as the lowest or "best" price available to any entity in the drug supply chain, be it a wholesaler, insurer, nonprofit, or government entity.³ The proposed rule would fundamentally change how this best price is determined -- and in a way that makes it vastly more difficult for small- and medium-sized firms like ours to serve Medicaid patients.

Specifically, the proposed rule mandates that companies aggregate or "stack" any discounts or rebates provided to various entities who encounter the drug unit in the drug supply chain in order to calculate the best price.⁴ This task is not only daunting. It is, at present, impossible to implement.

No system exists today that is capable of tracking price concessions given to all entities that purchase or cover a given drug across the supply chain. Such a system would require companies to collect, analyze, and publicize data from potentially hundreds of different stakeholders, which could strain the resources of even the largest pharmaceutical firms, let alone biotech start-ups with less than a dozen employees. Aggregated discount calculations off by a single cent could mean that firms are technically noncompliant with federal policy.

In addition to operational impediments, the rule's overall cost to our companies would be very significant and could make ongoing participation untenable. It could thus dramatically reduce the number of drugs available to vulnerable patients and seniors. In so doing, it further could create perverse incentives, decreasing the potential that companies would offer rebates beyond the statutory minimum Medicaid Drug Rebate for fear of not being able to track such discounts and report them accurately under the new rule. This could lead to further market consolidation and higher ultimate costs for entities like providers and hospitals.

By increasing both the costs and risks involved in serving underprivileged patients through the Medicaid Rebate Program, the rule would discourage investment in medicines from which these vulnerable populations are most likely to benefit. The result would be less innovation, fewer new cures, and worse health outcomes for disadvantaged groups over the long term.

Those of us who are pursuing innovative cell and gene therapies are also concerned with CMS' new definition of covered outpatient drugs.⁵ Many of our companies have worked with states under their existing regulatory authority to establish a reimbursement system for cell and gene therapies that is "site neutral" (i.e., ensuring reimbursement policy does not effectively dictate clinical decision making regarding the best setting of care for an individual patient). This solution both provides for adequate hospital reimbursement and ensures States recoup the mandatory Medicaid Drug Rebate on innovative therapies.

The proposed definitional change would upend this existing solution that is working well. Specifically, it would remove the requirement that inpatient-administered therapies be paid for

³ <https://www.kff.org/medicaid/issue-brief/understanding-the-medicaid-prescription-drug-rebate-program/view/footnotes/#footnote-438418-7>

⁴ <https://www.cms.gov/newsroom/fact-sheets/misclassification-drugs-program-administration-and-program-integrity-updates-under-medicaid-drug>

⁵ <https://www.cms.gov/newsroom/fact-sheets/misclassification-drugs-program-administration-and-program-integrity-updates-under-medicaid-drug>

separately from the reimbursement for the underlying hospital stay in order to qualify for the Medicaid Drug Rebate.⁶ This would be a significant departure from many years of precedent and would endanger hospitals' ability to provide these potentially curative medicines.

As a result of this proposed change, many states may no longer pursue separate reimbursement for certain inpatient therapies, which could, in turn, lead to significant financial losses for hospitals serving low-income populations and threaten patient access to cutting-edge cell and gene therapies. The redefinition could also discourage ongoing investment into high-potential research and development.

Our concerns about negative impacts on patient access extend to another element of the proposed rule: CMS' proposal to implement a new "price verification" survey for certain "high cost" drugs. The proposed survey – which is without any legal grounding – has a troubling, narrow focus on cost-based inputs. It ignores critical areas such as patient outcomes, patient experience, and caregiver impact. CMS should not move forward with implementing this survey and instead, the Agency should shift its mindset to one of identifying innovative payment and contracting approaches that will promote access for vulnerable patients in Medicaid including cell and gene therapies.

Finally, it is imperative that CMS not view proposed changes to Medicaid in a vacuum, but in the broader context of the many other recent changes to federal healthcare programs. The Inflation Reduction Act's recent reforms to Medicare Part D and ongoing negotiations with Part D plan sponsors and pharmacy benefit managers, for instance, have added to the cost of providing Medicare beneficiaries with access to the latest therapies from companies like ours.⁷ As a result, many of our firms may soon lack the resources to offer supplemental rebates to states through Medicaid, thus putting patient access at risk.

We urge you to reconsider these proposals and allow us to continue in our shared mission of promoting health equity and safeguarding the health of vulnerable patients across the country.

Sincerely,

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⁶ <https://www.cms.gov/newsroom/fact-sheets/misclassification-drugs-program-administration-and-program-integrity-updates-under-medicaid-drug#:~:text=Enhancing%20States%E2%80%99%20Abilities%20to%20Manage%20Medicaid%20Drug%20Spending>

⁷ <https://www.milliman.com/en/insight/part-d-redesign-under-ira-potential-financial-ramifications>

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