Seema Verma
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Hubert H. Humphrey Building, Room 445-G
200 Independence Avenue, SW
Washington, DC 20201

RE: 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 (CMS-2482-P)

Dear Administrator Verma,

The National Organization for Rare Disorders (NORD) appreciates the opportunity to submit comments on the proposed rule captioned above.[1] NORD is a unique federation of voluntary health organizations dedicated to helping the 25-30 million Americans living with a rare disease. We believe that all patients should have access to quality, accessible and affordable health coverage that is best suited to their medical needs.

Both commercial health insurance and Medicaid coverage are extremely important for patients living with rare diseases. Medicaid coverage in particular serves as an invaluable safety net for rare disease patients who often find their financial lives upended by the debilitating nature of their diseases. Therefore, NORD is committed to ensuring the sustainability of state Medicaid programs, including within the context of an ever-increasing shift towards value-based payment reform.

Our comments below focus on the Centers for Medicare & Medicaid Services' (CMS) proposed changes to government price reporting requirements under the Medicaid Drug Rebate Program (MDRP) and are summarized as follows:

- We are concerned that the rule, as currently written, would present operational challenges for state Medicaid programs that may ultimately impact the ability of beneficiaries to access care. We believe that any changes to Medicaid Best Price (BP) for the purposes of value-based purchasing arrangements should be carefully reviewed and audited to ensure that the Medicaid program's entitlement to rebates is not undermined.

[1] "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," 85 Fed. Reg. 37286 (June 19, 2020).

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- NORD is concerned CMS' proposed changes to price reporting in the case of payer-implemented accumulator programs could increase patient cost-sharing and run contrary to the Trump Administration's stated policy priority of reducing patient out-of-pocket (OOP) costs.

- NORD believes that any change to the agency's line extension policy should not come at the expense of innovation, particularly in the rare disease space where patients already experience a lack of targeted treatments for their diseases.

I. CMS should proceed cautiously in revising government price reporting requirements under the Medicaid Drug Rebate Program (MDRP) to support the development and adoption of value-based purchasing (VBP) arrangements.

NORD believes that VBP arrangements have the potential to better align the incentives in drug and biologic reimbursement with the value that a product produces for the patient, both in terms of affordability and access. The current system in which payment arrangements are typically tied solely to the volume of a drug or biologic sold – as opposed to patient outcomes or another measure of value – are generally not responsive to the patient perspective, particularly in the space of rare diseases where the anticipation of a low volume of sales often results in the establishment of high prices, which, in turn, may limit patient access to a therapy.

However, we encourage CMS to proceed cautiously in revising government price reporting requirements under the MDRP to ensure that the proposed policy does not undermine state Medicaid program's budgets, particularly where such policies may encourage only marginally beneficial VBP arrangements. Our comments focus on conveying NORD's key concerns as they relate to CMS' proposed revisions to the Average Manufacturer Price (AMP) and BP reporting requirements for arrangements that fall within CMS' proposed definition for a VBP arrangement.

A. CMS' proposed definition for a VBP agreement requires more specificity to protect against contracting intended to circumvent Medicaid BP.

CMS proposes to address the "regulatory challenges manufacturers encounter when structuring and implementing VBP" arrangements by giving manufacturers the "ability to offer these programs to commercial payers or Medicaid without the negative impact on best price or the potential for MDRP regulatory compliance."[2] Because section 1927 of the Social Security Act does not define a VBP, CMS proposes a definition of a VBP arrangement that would require that, as part of the arrangement, the final cost of the drug be "substantially linked" to an evidence-based or outcomes-based measure.

NORD supports a robust definition of "substantially linked" in order to prevent manufacturers from entering into specious VBP arrangements intended to reduce the manufacturer's BP more so than representing an innovative payment arrangement predicated on the therapy's value proposition, and we would support a significantly high threshold for qualifying a contractual arrangement as a VBP payment arrangement.

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[2] *Id.* at 37291.

Furthermore, NORD requests that CMS provide greater clarity regarding the distinction between "evidence-based" and "outcomes-based" measures referenced in its proposed definition of a VBP arrangement. Generally speaking, outcomes-based VBP arrangements lend themselves to a "substantially linked" threshold because there is a clear variable in the product's price that represents a portion of the product's final price: the outcomes-based rebate. However, it is unclear how "substantially linked" would be calculated in the context of evidence-based VBP arrangements, and how those "evidence-based" VBP arrangements differ from the payment arrangements that already exist under traditional payment structures. NORD is concerned that without greater clarity, evidence-based discounts that currently set BP would no longer be included, and could result in reduced rebates for Medicaid programs.

In addition, it is unclear how initial and final prices would be determined under an evidence-based VBP arrangement. For example, if indications-based pricing is considered an evidence-based outcome, what would it mean for the final price of the product to be "substantially linked" to the evidence-based measure? Should a manufacturer use its pricing for other indications as the comparator price? We request that CMS clearly delineate the parameters of evidence-based and outcomes-based measures for the purposes of qualifying as a VBP arrangement and take measures to ensure that Medicaid rebates owed to states under VBP arrangements are not less than what would be owed under traditional payment arrangements

B. CMS should implement VBP-related revisions to MDRP price reporting requirements in a way that takes into account the impact on patient affordability and access.

As the pace of innovation in the biopharmaceutical industry quickens, the prices for these innovations are increasing significantly and in many cases are beyond the reach of patients. Furthermore, some Medicaid programs are putting significant utilization management measures in place to control cost and preserve overall program financial stability, which can hinder access for the state's most vulnerable populations. VBP arrangements are typically touted as a potential solution to solve these access concerns while promoting innovation. However, these VBP arrangements are often discussed in the context of a value framework that focuses on the manufacturer and payer, with the patient being an ancillary beneficiary of the arrangement. Stated differently, the focus of current VBP arrangements tend to weigh the price of the relevant drug or biologic that a manufacturer can charge against the benefit that the payer is expected to receive, typically in the form of reduced costs (e.g., reduced hospitalizations) as a result of demonstrated clinical efficacy. Although the patient benefits from the clinical efficacy of the underlying medicine, the patient would have benefited from that clinical efficacy regardless of whether the medicine was subject to a VBP arrangement.

We urge CMS to implement VBP-related revisions to the MDRP in a way that puts patients at the forefront. CMS has the opportunity to drive the focal point of VBP arrangements from focusing primarily on the manufacturer and payer to also including a central focus on the patient. For instance, CMS could define VBP arrangements to explicitly require an outcomes-based metric that centers on whether the VBP arrangement reduces the cost-sharing obligations of the patient or enables timely access to services without heavy reliance on restrictive utilization management tools. Patients with rare diseases often face high out-of-pocket cost-sharing obligations, stringent prior authorization requirements and

excessive step therapy protocols due to the high cost of the therapies they need. As proposed, an approved VBP arrangement may benefit from new price reporting treatment, yet provide no new net benefit to patients.

An aspirational objective of CMS' implementation of VBP arrangements reforms should be to explore how to encourage the incorporation of a therapy's intangible improvements to patients' lives into VBP arrangements. Rare diseases often debilitate patients, limiting their ability to care for themselves and increasing their dependence on caregivers, such as family members, for daily living. A drug or biologic that significantly improves a patient's life and empowers them to be self-sufficient, such as by reducing caretaker burden and/or enabling the patient to go back to work, represents a significant benefit to the patient that is not solely a function of a health care-related metric. By taking a more holistic approach to reimbursing therapies, VBP arrangements in the future could potentially serve as an important tool to explore how payment for a therapy can reflect the indirect benefits experienced by patients from taking a particular drug or biologic.

C. NORD is concerned that allowing manufacturers to report multiple BPs could produce a two-tiered pricing system that could ultimately provide limited value, and CMS should accordingly commit to careful pre-authorization requirements and regular OIG audits to ensure Medicaid continues to receive appropriate rebates.

Although NORD is supportive of VBP arrangements, NORD is concerned that manufacturers might use VBP arrangements only when convenient or make them available only to some commercial plans, while continuing to charge high prices for therapies outside of VBP arrangements. Specifically, as therapies become more personalized, we are concerned that manufacturers will bifurcate the contracting for their drug portfolios based on the confidence that they have of predicting an acceptable rate of negative outcomes.

For instance, because manufacturers may have a high rate of confidence that they can accurately predict expected revenue under an outcomes-based regime for a particular drug in their portfolio, they may be incentivized to offer VBP arrangements with high starting prices, but the potential for significant discounts in the *limited* instances where the expected outcomes are not met. To the extent such limited instances are focused on the healthiest patients mostly likely to achieve certain therapeutic outcomes, rare disease patients may be less likely than others to receive the benefits of a VBP arrangement. Such a pricing approach does not solve an access problem insomuch as it gives manufacturers a reason to justify high prices and provides them with a public relations benefit of promising significant rebates in certain situations.

On the other hand, in cases where the manufacturer cannot accurately predict the patient's response and outcomes, the manufacturer will have less of an incentive to offer VBP arrangements with significant discounts, and instead will have an incentive to price their product according to whatever they believe the market could bear under traditional payment arrangements. In the U.S. market, we are seeing prices regularly reach the middle-to-high six-digit figures for rare disease drugs and biologics.

The same concern applies for evidence-based measures. In the case of indications-based pricing, manufacturers could segregate the clinical evidence of their products by payer mix, which may serve as a proxy for patient characteristics that could adversely affect the efficacy of their therapy. For example, manufacturers could extend indications-based pricing only to certain commercial payers which they have determined contain patients with characteristics that support the improved efficacy of their product, and "evidence-based" pricing could otherwise justify highly segmented pricing practices by manufacturers that exclude Medicaid beneficiaries.

In short, we are concerned that the ability to report multiple best prices will create a two-tiered system where VBP arrangements are offered by manufacturers only when they can accurately predict limited financial liability. This will limit the impact of VBP arrangements on increasing access, while allowing manufacturers to continue to price their products according to what they believe the market will bear for products which they cannot accurately predict outcomes for.

Moreover, even if manufacturers are *required* to extend the option of participating in a given VBP arrangement to Medicaid programs (which we assume CMS would require as part of its proposal and should clarify in the final rule), this would not necessarily eliminate the concern of a two-tiered system we describe above. This is because many Medicaid programs may decline VBP arrangements solely based on operational capabilities and costs. The data tracking and analytics that may be necessary to properly implement a VBP will likely be significant and outside of Medicaid's current operational capabilities. If Medicaid is unable to operationalize a VBP, and thereby declines participating in a VBP, CMS' multiple best price proposal effectively amounts to a "carve out" for manufacturer price reporting where they can avoid triggering BP by offering VBP arrangements that state Medicaid programs must decline because of operational limitations.

NORD recommends that if CMS proceeds with its proposal, the Department of Health and Human Services (HHS) should closely study and evaluate the impact that its proposal has on manufacturer pricing patterns. The Office of Inspector General (OIG) should be able to audit VBP arrangements and identify trends produced by allowing the reporting of multiple BPs, which in turn could inform CMS' future policy decision-making in this area. Moreover, to *substantively* ensure Medicaid access to VBP arrangements and the value they represent, states must be provided with sufficient technical and financial resources, either by CMS or manufacturers. If such assistance comes from manufacturers, the OIG may need to adopt targeted revisions to the anti-kickback statute and other fraud and abuse laws to facilitate such assistance. Medicaid's entitlement to BP is a cornerstone of the MDRP, and it is important that Medicaid programs be given both the option and necessary resources to participate in commercially established VBPs to prevent market bifurcation where Medicaid is effectively excluded from VBP arrangements that are proliferating in the commercial market.

D. NORD is concerned about the lack of detailed guidance on how CMS plans to implement its VBP-related revisions to MDRP price reporting requirements.

NORD is concerned about the lack of operational details surrounding Medicaid's entitlement to rebates given the lengthy time horizons and detailed level of health tracking that would be required under VBPs.

CMS indicates that the calculated rebate due to the state using a VBP BP would be a "function of whether or not the Medicaid rebate is being paid on a unit of a drug dispensed to a Medicaid patient that participated in a VBP, and the level of rebate associated with that patient's outcome."[3] In other words, the rebate would "be specific to the patient's outcome," otherwise the price used in the Medicaid rebate formula would "mirror the lowest price available absent a VBP arrangement."[4]

First, it is unclear how CMS intends to ensure that Medicaid programs are not financially cash-strapped as a result of lengthy time horizons for VBP arrangements. Many VBP arrangements may span for several years and a complete and accurate outcomes-based rebate on a particular Medicaid beneficiary may not be owed until several years after the Medicaid program has paid the full selling price. Because Medicaid programs are budget constrained, tied to state fiscal years, and require predictability in expenditures to operate successfully, the reporting of multiple BPs based on the applicability of a VBP arrangement could prove unworkable for Medicaid programs from a budgetary perspective.

Second, it is unclear how CMS intends to account for high rates of "churn" among Medicaid beneficiaries, which has been as high as 30%.[5] High rates of disruption in Medicaid coverage pose problems for VBP arrangements that involve significant time horizons because it is unclear when the Medicaid program would be owed the BP if the manufacturer makes a sale to the Medicaid program, but the beneficiary enrolls under a commercial plan during the life of the VBP arrangement. Is the manufacturer required to report a BP under the VBP arrangement because the payer could not determine if the outcome metric was met by the enrollee? Or must the manufacturer continue to track that patient's outcome for the purposes of BP reporting? What BP would ultimately be reported for the purposes of Medicaid rebate liability? In the proposed rule, CMS acknowledges that they do not currently have the systems required for the complex tracking that would be required by these arrangements. As mentioned previously, it is highly unlikely that states would be able to develop such systems without significant assistance, and this could prevent Medicaid programs from realizing the full benefit of VBP arrangements.

In summary, we are concerned that the administrative complexity that CMS' proposal involves could divert scarce state Medicaid program resources away from ensuring access to and improving patient care. Unless CMS provides greater clarity on key operational questions, we are concerned that the agency's multiple BP proposal threatens the overall predictability of Medicaid rebates for state Medicaid program budgets and would be administratively burdensome for Medicaid to operationalize and track. In addition to requesting that CMS analyze the anticipated impact on Medicaid prescription drug costs prior to finalizing this proposed rule, we request that CMS consider the following important questions in assessing whether to finalize its proposed reforms to Medicaid BP:

- How is the initial BP under VBP arrangements reported?
- What is the time frame in which manufacturers must report updates to their BPs under a VBP arrangement?

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[3] *Id.* at 37293.
[4] *Id.*
[5] Roberts, Eric T, and Craig Evan Pollack. "Does Churning in Medicaid Affect Health Care Use?." *Medical care* vol. 54,5 (2016): 483-9. doi:10.1097/MLR.0000000000000509.

- What type of financial and technical assistance would CMS provide to state Medicaid programs for the purposes of data collection, monitoring and analysis under VBP arrangements?
- Would manufacturers be allowed to provide state Medicaid programs and participating providers with data collection, monitoring and analysis capabilities without violating the anti-kickback statute?
- Who decides on the outcomes and whether they are met, particularly if a patient moves between payers during the life of the VBP arrangement?
- Would state Medicaid programs have access to a manufacturer's VBP arrangements in other states, or would they be restricted to accessing VBP arrangements offered within their respective states? What about VBP arrangements with national payers?
- How would Medicaid BP be set if manufacturers offer multiple VBP arrangements, based on different outcome-based measures, across multiple commercial payers as well as Medicaid programs?

II. NORD Opposes CMS' Proposed Accumulator Policy, to the Extent it Harms Patient Access

In the proposed rule, CMS proposes regulatory revisions to the definitions of BP and AMP to provide that the exclusion of manufacturer assistance to patients from best price and AMP applies only to the extent that the manufacturer "ensures the full value of the assistance or benefit is passed on to the consumer or patient."[6] Under this proposed policy, manufacturer cost-sharing assistance would not be exempt from best price to the extent such amounts are captured by an accumulator program operated by a health care payor or PBM. While NORD recognizes the vested interest the Federal government and states have in accurate price reporting, and believes in the principles underlying the agency's longstanding BP policy, we are concerned that this policy, as proposed, will largely be unworkable and could have the unintended impact of freezing or significantly reducing currently available dollars for patient assistance. Given the critical role these patient assistance programs play in allowing rare disease patients to access otherwise unaffordable, life-saving therapies, we urge the agency to consider any unintended consequences of the proposed policy on patient access.

NORD has long been a critical advocate for a patient's ability to obtain live-saving or life-sustaining medications they cannot otherwise afford, having operated patient assistance programs to ensure that rare disease patients face minimal barriers to accessing these therapies. These programs provide free medication, financial assistance with insurance premiums and co-pays, diagnostic testing assistance and travel assistance for clinical trials or consultation with disease specialists. While we recognize that CMS' goal in revising the price reporting policies is to ultimately lower the list price of medicines to benefit all patients, we are concerned that CMS' approach appears to be based on an incorrect interpretation of how accumulator programs operate.

In particular, we are concerned CMS may hold the incorrect assumption that manufacturers operating financial assistance programs will be able to know when an accumulator program is imposed on their prescription drug products. We believe, in most cases, they will not have this information, nor is there

[6] *Id.* at 37299.

an obvious way to contract for such data.[7] As a result of this information gap, if the agency's proposed regulatory changes obligate manufacturers to count assistance to commercial insurance enrollees in determining BP and AMP in Medicaid, finalizing this proposed policy could result in manufacturers significantly limiting such assistance to commercial enrollees, with potentially serious consequences for patient adherence to their drug regimens. In particular, to comply with applicable price reporting rules, manufacturers may be forced to assume that *all* assistance will be redirected towards plans and treat such amounts as price concessions not exempt from best price.

We believe the (unintended) net result of this policy will be the curtailing of financial assistance manufacturers currently offer to patients, which would have a devastating impact on patient access to lifesaving therapies. This impact will be felt particularly strong by rare disease patients who often rely on the ability to access a single, expensive therapy for which there is no generic medication or treatment available. We urge CMS to consider such adverse consequences prior to finalizing this proposed policy.

III. Any Final Line Extension Definition Should Not Come at the Expense of Innovation that Benefits Rare Disease Patients

NORD is supportive of the Medicaid drug rebate program, which ensures states can fund already cash-strapped Medicaid programs, many of which serve our rare disease patient community. As the agency is well-aware, the last decade has seen a tremendous growth in new, innovative therapies for rare disease patients.[8] While many of these therapies come in the form of a single new molecular entity, others may arguably fall within CMS' overly-broad, proposed definition of a "new formulation."

In CMS' proposed line extension policy, the agency now proposes to treat certain combination products as line extensions, despite the fact that these products may offer a treatment for a novel patient population or even include a new molecular entity. In just the last year, the U.S. Food and Drug Administration (FDA) approved a number of orphan drug therapies that would be treated as combinations products, and thus line extensions, under CMS' proposed rule. For example, the FDA recently approved Tecentriq® (atezolizumab) in combination with Avastin® (bevacizumab) for the treatment of people with unresectable or metastatic hepatocellular carcinoma (HCC) who have not received prior systemic therapy. So, too, the FDA recently approved Epclusa® (a combination of sofosbuvir and velpatasvir) for the treatment of pediatric chronic hepatitis C Virus (HCV) infection.

Our understanding of the legislative intent of the line extension provision in the Affordable Care Act (ACA) is that Congress intended to target new therapies that are simply "slight alterations' of existing products.[9] While NORD believes there are many therapies that very clearly fall within this definition (such as certain extended release formulations), we are concerned that the proposed definition may

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[7] In the proposed rule, CMS notes: "We believe manufacturers have the ability to establish coverage criteria around their manufacturer assistance programs to ensure the benefit goes exclusively to the consumer or patient," yet cites no evidence for such supposition. *See* 85 Fed. Reg. at 37,299.

[8] According to a recent study authored by Tufts Center for the Study of Drug Development, the share of new drug approvals worldwide for rare diseases doubled from 29% of all approvals in 2010 to 58% in 2018.

[9] Senate Finance Committee, "Financing Comprehensive Health Care Reform: Proposed Health System Savings and Revenue Options" at 12 (May 20, 2009).

also encompass many novel products that have the potential to benefit the rare disease community. There are over 7,000 rare diseases and over 90% of them do not have an FDA-approved therapy. Given this landscape, getting an on-label indication provides a great benefit for the rare disease community, whether that be a combination of products previously on the market, a new indication for an existing product or a novel therapy. As CMS balances the "right" definition, we urge a definition that does not come at the expense of innovation that benefits patients.

NORD thanks CMS for the opportunity to comment and looks forward to working with the Agency to ensure that rare disease patients on Medicaid have access to essential, high-quality health care. For questions regarding NORD or these comments, please contact me at rsher@rarediseases.org, or 202-588-5700.

Sincerely,

Rachel Sher
Vice President, Policy and Regulatory Affairs