Final Rule Summary

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

December 22, 2020

I. Overview

On December 21, 2020, the Centers for Medicare & Medicaid Services (CMS) issued a Final Rule "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." In particular, CMS finalized a proposal released in June 2020 to support value-based purchasing (VBP) arrangements for prescription drugs covered under the Medicaid program. The Final Rule also makes significant changes to the Medicaid Prescription Drug Rebate Program (MDRP) as well, including changing the treatment of patient assistance programs for purposes of government price reporting, establishing a definition of line extensions for purposes of calculating alternative rebate amounts, and implementing statutory changes to how a manufacturer should calculate the average manufacturer price (AMP) of a drug when there is also an authorized generic.

The Final Rule generally aligns closely with the Proposed Rule on these three policy areas. CMS largely finalized its proposal to allow manufacturers to report multiple best prices for drugs provided under a VBP, along with additional guidance on how the policy would operate in practice. Notably, CMS delayed the effective date until January 1, 2022 to allow stakeholders time for implementation. In the accompanying press release, CMS Administrator Seema Verma stated that "drug rebates and related reporting requirements have not been updated in thirty years, and are thwarting innovative payment models in the private sector." She described the VBP-based proposal in this Final Rule as allowing the "market the room to adapt" to innovative payment models while "ensuring that public programs like Medicaid remain sustainable and continue to receive their statutorily required discounts." As part of its VBP-based efforts, CMS also finalized its proposal to allow VBPs to qualify as bundled sales for the purposes of calculating best price.

With respect to accumulators, CMS is finalizing its limitation on the exclusion of manufacturer patient assistance from best price and AMP to the extent the manufacturer ensures that the assistance is passed on to the consumer, consistent with the agency's position in the Proposed Rule. Acknowledging stakeholder concerns about the difficulty of implementing the requirement to ensure that the full value of the assistance is passed on, CMS is also delaying the implementation of the policy until January 1, 2023.

Finally, CMS is finalizing its proposal to expand the definition of "line extension" for the purposes of MDRP rebate liability, although the agency is walking back the broad applicability of its definition in key areas, including but not limited to combination products and changes in indication. The effective date of these regulatory definitions is January 1, 2022.

These changes are summarized in more detail below.

II. Value-based Purchasing (VBP) Arrangements and Best Price

CMS is finalizing its proposals to allow manufacturers to report multiple best prices as part of qualifying VBP arrangements, as defined in the Final Rule. In the Final Rule, CMS provides several clarifications on how best price would be calculated under this approach, including clarifying that manufacturers must offer the VBP arrangement to all state Medicaid programs; however, only states that elect to participate in VBP arrangements would be able to take advantage of the multiple best price reporting approach. CMS is also finalizing its proposal to allow VBP arrangements to qualify as bundled sales, providing manufacturers with an alternate approach to calculating Medicaid best price.

A. CMS Finalizing Proposal To Allow Manufacturers To Report Multiple Best Prices Best Price For Qualifying VBP Arrangements.

CMS is finalizing its proposal to, beginning January 1, 2022, allow manufacturers with qualifying VBP arrangements to report multiple best price points for a single dosage form and strength of a covered outpatient drug, beginning January 1, 2022. To qualify under this multiple best price approach, the manufacturer must offer the VBP arrangement to all state Medicaid programs. States, however, are not required to participate in the VBP arrangements.

A VBP arrangement as defined in regulation must be "***intended to align pricing and/or payments to an observed or expected therapeutic or clinical value in a select population.***" Additionally, the definition requires either (or both) evidence-based measures, which substantially link the cost of a covered outpatient drug to existing evidence of effectiveness and potential value for specific uses of that product, and/or outcomes-based measures, which substantially link payment for the covered outpatient drug to that of the drug's actual performance in a patient or a population, or reduction in other expenses.

Evidence-based measures must be based on clinical data sets and documented evidence. For instance, documented evidence for an oncology product may show complete remission in 80 percent of a population. Thus, the manufacturer negotiates with the payer and agrees to offer a certain rebate if 80% of the payer's patients do not achieve complete remission. Outcomes-based measures need not be based on documented evidence. For example, in the above example, a manufacturer can agree to rebates depending on whether a particular patient actually responds or not. Importantly, CMS declined to define what it means for the cost of the drug to be "substantially" tied to evidence-based and/or outcomes-based measures. In response to comments, the agency stated that manufacturers should document and keep records of how they determined that the cost of a drug is "substantially" tied to selected outcomes, similar to how they make and document "reasonable assumptions" for price reporting.

Manufacturers that offer VBP arrangements to all states may then report multiple best prices that

reflect the lowest price available under the VBP arrangement. Manufacturers who entered into a VBP arrangement with commercial payers will report a distinct set of multiple best prices if there is more than one in the marketplace. CMS rejected comments that permitting manufacturers to report multiple best prices for VBP arrangements would weaken the best price requirement and exceeds the agency's statutory authority. State Medicaid programs that participate in the VBP will receive a unit rebate amount for each patient's particular outcome that is reflective of the VBP arrangement best price. CMS provides an example of how the best price would be calculated under this new VBP approach. The manufacturer would provide a best price rebate to the state in the quarter in which the drug is administered, and then could offer varying additional rebates based on a patient's response after the drug is administered. The calculated additional MDRP rebate due to the state using the VBP best price 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. The additional rebate paid for that patient would only represent the amount of rebate due to the state from the manufacturer for that patient, not all patients. As a result, "***the rebate would be specific to that patient's outcome and that price actually realized by the manufacturer, as that price is the lowest price available from the manufacturer based on that patient's outcomes***."

In addition to reporting the multiple best prices, however, manufacturers would also continue to report a non-VBP best price using the existing methodology for reporting best price. As a result, states that opt not to participate in the VBP will continue to receive rebates based on the manufacturer's *non*-VBP best price (i.e. the standard methodology to calculate best price, but not including VBP-derived prices).[1]Thus, states will always be guaranteed *at least* 23.1% of Average Manufacturer Price (AMP), and potentially more depending on whether or not the state enters into the VBP arrangement with the manufacturer and the particular beneficiary meets the specified outcomes. Furthermore, the state Medicaid program may also receive higher rebates that do not trigger best price through supplemental rebate agreements.

Manufacturers will be able to request a change to the 12-quarter reporting timeframe if the terms of a VBP arrangement require the outcome to be evaluated outside of the 12-quarter period. Furthermore, CMS clarifies that manufacturers must report AMP as the full price of the drug at the time it is administered, even if the drug is subject to an installment payment plan that would extend to subsequent quarters. However, CMS clarifies that installment payments will generally not be viewed as VBP arrangements if they do not contain some "substantial" link to evidence-based or outcomes-based measures. CMS also clarifies that that it is appropriate that an installment payment NOT made because of a VBP arrangement outcome (i.e. a "forgiven" installment payment) be treated as lagged price concessions for purposes of determining AMP.

B. CMS Finalizing Its Proposal To Allow VBP Arrangements To Qualify As A Bundled Sale.

CMS is finalizing its proposal to allow VBP arrangements to qualify as a bundled sale for the purposes of calculating best price. CMS notes that it has previously reviewed manufacturer reasonable assumptions outlining these types of approaches to calculating Medicaid best price and the agency did not view them as concerning. CMS' proposed codification into regulations

[1] The 340B ceiling price for a drug under the multiple best price approach would continue to reflect a Medicaid drug rebate based upon the non-VBP best price.

that VBP arrangements involving a performance requirement may qualify as bundled sales provides manufacturers with another methodology to calculate best price. CMS notes in multiple instances that manufacturers may mitigate the challenges of value-based contracting either by taking advantage of the multiple best price approach finalized in the Final Rule, or by leveraging the bundled sales provision in the context of a VBP arrangement.

Importantly, however, CMS clarifies in the Final Rule that manufacturers *cannot* aggregate sales and discounts across purchasers under a VBP arrangement to protect against volatile swings in a best price, regardless of the size of the patient population. CMS states that it does "not believe that the statute supports the inclusion of all VBP prices offered by a manufacturer into the calculation of a single best price under a bundled sales methodology, as the determination of a best price is based on a lowest price available to a specific best price eligible entity, not a price that is an aggregation of sales/discounts/rebates across multiple entities…." Thus, the bundled sales approach to calculating best price may be of limited use for rare and ultra-rare products.

Unlike the other VBP price reporting provision, the bundled sales definition is effective beginning 60 days after publication of the Final Rule.

III. Definition of Line Extension, New Formulation, and Oral Solid Dosage Form for Alternative URA

CMS is finalizing its proposal to define, for the first time in regulation, the terms "line extension", "new formulation", and "oral solid dosage form" at 42 C.F.R. § 447.502 for purposes of calculating the additional, inflation-based rebates under the Medicaid Drug Rebate program. The final rule takes a noticeably more narrow approach to defining line extensions, including by excluding from the definition of a "new formulation" combination products and new indications, but leaves open the possibility of a more expansive definition in the future. While the line extension provision has been in effect since January 1, 2010, until now CMS has only provided guidance to manufacturers to use "reasonable assumptions" in their determination of whether a drug qualifies as a line extension, rather than a regulatory interpretation.

To provide sufficient time to come into compliance, these definitional changes are effective beginning on January 1, 2022. For period prior, manufacturers may continue to use reasonable assumptions.

A. Line Extension Need Not Be an Oral Solid Dosage Form

In its 2012 MDRP proposed rule, CMS had proposed that, to be a line extension, both the initial brand name drug *and* the line extension drug had to be an oral solid dosage form, but CMS never finalized the line extension provisions of the 2012 proposed rule. In this Final Rule, however, CMS is finalizing a new policy, first proposed in the June, 2020 proposed rule, under which *only* the *initial* single source drug or innovator multiple source drug must be an oral solid dosage form, expanding the universe of drugs that can be line extensions.

In response to its 2020 proposed rule, many commenters had argued against this reinterpretation on the basis that Congress had intended to limit the definition of line extension to only those drugs for which a "slight alteration" had been made, and that a change from an oral solid dosage

form to a different dosage form is a "significant alteration." Setting aside whether or not a change from one dosage form to another is significant, CMS notably rejected this interpretation of Congress' intent in enacting the line extension provision, noting in part: "Had Congress intended to limit the line extension provisions to drugs that were only slight alterations, we believe they would have provided an example of a less significant change than "an extended release formulation."

B. Definition of Line Extension

In the final rule, CMS is adopting a definition of "line extension" at 42 C.F.R. § 447.502 to mean "for a drug, a new formulation of the drug, but does not include an abuse-deterrent formulation of the drug (as determined by the Secretary)." To effectuate this language, CMS is also adopting a new definition for "New Formulation" to mean:

> "… for a drug, a change to the drug, including, but not limited to: an extended release formulation or other change in release mechanism, a change in dosage form, strength, route of administration, or ingredients."

In response to feedback from commenters, this definition of "new formulation" is noticeably more narrow than the definition previously proposed, excluding: changes in pharmacodynamics, or pharmacokinetic properties, changes in indication, and new combination drugs from the definition of line extension (all of which had been included in CMS' proposed rule), as well as removing the requirement that the new formulation contain at least one active ingredient in common with the initial brand name drug. However, in the final rule CMS did not back away from its overarching belief that Congress granted the agency broad discretion to define line extensions broadly. Rejecting statutory arguments from commenters that Congress only intended to target changes in formulation similar to extended release formulations, CMS pointed to Congress' uses of the word "such as" as evidence of Congress' desire to capture a broader range of drug, as well as Congress' selection of "extended release formulations" as an example. According to CMS, an extended release formulation is a general change to a drug for which FDA required additional studies and which may be considered a significant change to an original drug.

Rather than pointing to any statutory limitations, in the final rule CMS acknowledged possible patient harm issues with its broader, proposed definition. In the final rule CMS notes: "We believe that with the exclusion of these proposed changes from the final definition of line extension, that we have maintained incentives for manufacturers to bring such advances to market." CMS specifically points to its exclusion of combination drugs from the final rule as evidence of this policy, although it explicitly reserves the right to reconsider that policy in the future. With respect to combination drugs, CMS also further clarified that a new formulation of an existing combination drug could be a line extension.

C. Defining Oral Solid Dosage Form

Under CMS' current definition of an oral solid dosage form at 42 C.F.R. § 447.502, the term is defined to mean "capsules, tablets, or similar drugs products intended for oral use as defined in

accordance with FDA regulation at 21 CFR 206.3 that defines solid oral dosage form." CMS is now finalizing its proposal to expand this definition to mean:

> "an orally administered dosage form that is not a liquid or gas at the time the drug enters the oral cavity."

This definition is notably broader than FDA's definition, such that it would include, for example, a sublingual film/tablet or a powdered drug administered by oral inhalation. CMS also notes that an oral solid dosage form that incorporates a medical device would not be exempt from this definition solely due to the addition of a device to the oral solid dosage form.

IV. PBM Accumulator Programs and Best Price

CMS is finalizing its proposal to limit 42 CFR §§ 447.504 and 447.505's exclusion of manufacturer patient assistance from best price and AMP to the extent the manufacturer ensures that the assistance is passed on to the consumer. Acknowledging stakeholder concerns about the difficulty of implementing the requirement to ensure that the full value of the assistance is passed on, CMS is delaying the implementation of the policy until January 1, 2023.

A. CMS Finalizing Proposal to Include Manufacturer Cost Sharing Assistance in Best Price if Accumulator in Place

Regulations at 42 CFR §§ 447.504(c)(25)-(29) and (e)(13)-(17), and 447.505(c)(8)-(12), exclude certain manufacturer patient assistance to patients from the definition of best price and AMP for the purposes of the Medicaid drug rebate program. These regulatory exclusions apply "only to the extent that the full value of the coupon is passed on to the consumer," or similar qualifications to ensure that only assistance to patients is excluded from best price. CMS is now finalizing its proposed revisions to these regulations "to provide expressly that the exclusions [from best price or AMP for manufacturer patient assistance] apply ***only to the extent the manufacturer ensures the full value of the assistance or benefit is passed on to the consumer or patient."***

In the Final Rule, the agency continues to take a negative tone toward accumulators, and to position its regulatory changes as an anti-accumulator measure. The agency states specifically that "[w]e agree with the many comments that we received expressing concern about the impact of [accumulator] programs on patients, including the sudden impact that such programs can have on patient out-of-pocket spending for their drug." CMS notes, in response to comments, that "[b]anning PBM accumulator programs is outside the scope of this rule." The agency also responds to the view that its regulatory revisions are inconsistent with the 2021 NBPP Final Rule's policy on counting manufacturer cost sharing assistance toward the ACA's annual limitation on cost sharing. This response, however, is broadly a reiteration of the two policies, and does not address the conflict raised by stakeholders. CMS also reiterates its belief that the Final Rule "will encourage manufacturers to ensure the full value of manufacturer-sponsored assistance is extended to the patient."

CMS disagrees with stakeholder comments that its proposed regulatory changes are foreclosed by Section 1927 of the Social Security Act and other provisions. The agency elides many of stakeholders' statutory arguments, mentioning but not refuting, for instance, stakeholders' contention that manufacturer-sponsored assistance designed solely to benefit patients and reduce their out-of-pocket costs cannot constitute a "price available from the manufacturer" because the manufacturer did not intend to offer the price to an eligible third party such as the health plan.

CMS also pushes back on the contention that finalizing these revisions may cause manufacturers to stop providing patient assistance, and takes the position that it does not believe the Final Rule will have a significant impact on Medicare Part B drug payments. The agency notes there could be some impact with respect to the AMP calculation (and thus the Part B reimbursement) for drugs that are classified as "5i" drugs under the MDRP. CMS states that the impact on 340B ceiling prices would depend on the inclusion of the manufacturer-sponsored assistance in the best price, and in some cases the AMP, for the drug for that quarter.

B. Delayed Implementation Until 2023

The agency is, however, seeking to address manufacturer concerns about its regulatory changes to §§ 447.504 and 447.505 by ***delaying the effective date to January 1, 2023***. The purpose of this delay is to "give manufacturers time to implement a system that helps them track their programs to ensure the manufacturer assistance is being passed through to the patient in full, and no other entity is receiving any price concessions." The agency states that it is taking this step to respond to "concern with the impact of this policy on manufacturer's ability to provide assistance during the COVID-19 crisis, and manufacturer[] concern[s] that they may not be able to ensure their manufacturer assistance is going to the patient and not being passed through to the health plan via an electronic means right away."

CMS disagrees "that this regulation creates an insurmountable burden for manufacturers to comply with this new regulatory requirement," noting the lack of specific data provision and verification standards. The agency states that "one of the approaches that manufacturers may be able to use to capture information regarding how their manufacturer-sponsored assistance is used is through an electronic feedback mechanism at the point-of-sale, which appears to be in place at the present time." CMS states that "[w]e believe and have the expectation that PBMs will work with manufacturers to provide [] information to the manufacturers to help them ensure that their assistance is passed through." The agency does not provide any detail on how manufacturers can enforce PBMs' cooperation.

CMS does provide some suggestions on how a manufacturer might be able to work to ensure that the full value of their patient assistance reaches the patient. These include contracting with a third party vendor to track their assistance when provided at the point of sale. CMS also suggests altering the structure of manufacturer-sponsored assistance programs to require patients pay for the drug first and then have the patient collect the rebate directly from the manufacturer (outside of the electronic claims process), in order to "allow a patient's cost sharing at the point of sale to apply to the patient's deductible because the pharmacy and PBM will be unable to identify that the patient used manufacturer-sponsored assistance."

Of note, CMS also suggests that its regulatory changes are not actually altering the requirements imposed on manufacturers. The agency states that "[i]f manufacturers are certifying their AMP and best price data at this time, which they are required to do each quarter, they should be doing so only with the knowledge that such their manufacturer-sponsored assistance is being passed through to the patient in compliance with applicable statutes and regulations." Given the explicit delay of the regulatory revisions until 2023, however, this statement from CMS likely should not be read to impose any requirements on manufacturers with respect to the impact of accumulators on best price and AMP reporting until that time.