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January 4, 2021

MEMORANDUM

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SUBJECT: Summary of Final CMS Amendments to Medicaid Drug Rebate Program Regulations

In the Federal Register of December 31, 2020,[1] CMS finalized a rule to implement recent statutory amendments to the Medicaid Drug Rebate Program (MDRP) statute and a not-so-recent issue pending since the 2010 Affordable Care Act. The final rule also goes beyond statutory implementation to add CMS's own policy proposals to encourage value-based purchasing arrangements and discourage patient savings programs. The regulation is effective March 1, 2021, except that the effective date has been extended for certain provisions noted below.

The topics covered in this wide-ranging rule include:

- Best price changes and other measures to encourage value-based purchasing arrangements in Medicaid
- Additional regulations to implement the alternative rebate for line extensions, including a broad definition of "new formulation" and a definition of "oral solid dosage form"
- Introduction of a new, problematic hurdle for claiming the best price exceptions for manufacturer coupon and other patient savings programs
- Clarification of the average manufacturer price (AMP) and best price treatment of rebates to Medicaid Managed Care plans that are not paid pursuant to a CMS-approved supplemental rebate program

[1] 85 Fed. Reg. 87,000 (Dec. 31, 2020).

- Implementation of statutory amendments to exclude sales of authorized generics from the brand AMP, redefine single source and innovator multiple source drugs to remove references to "original NDAs," and redefine multiple source drugs to include OTC drugs that are covered outpatient drugs.

The above topics are addressed in more detail below. Note that the focus of this summary is on provisions of the rule that directly affect drug manufacturer price reporting under the MDRP. The regulation also includes requirements for state Medicaid programs relating to opioid drug utilization review, drug utilization data reports to CMS, and payment of claims, but these provisions are outside of our scope here.

I. VALUE BASED PURCHASING ARRANGEMENTS

CMS has revised the best price regulation to remove impediments to value-based purchasing (VBP) arrangements in Medicaid. The new definition of VBP is "an arrangement or agreement intended to align pricing and/or payments to an observed or expected therapeutic or clinical value in a select population . . . ."[2] The definition includes, but is not limited to, (1) evidence-based measures, which substantially link cost to existing evidence of effectiveness and value for a specific use, and (2) outcomes-based measures, which substantially link a drug's cost to its actual performance or a reduction in medical expenses. An example of an evidence-based measure provided in the preamble is a situation where a manufacturer has documented evidence that its cancer drug achieves an 80% complete remission rate, and offers a payor a rebate for a portion of the covered population if the drug does not achieve that result. The preamble's illustration of an outcomes-based measure is where a manufacturer and payor agree to a payment based on whether or not a patient reaches an agreed upon clinical outcome. Based on these examples, many value-based arrangements might fit into both categories, one important difference being that, for outcomes-based measures, "[t[he outcome may include a reliance upon documented evidence or not."[3] The preamble describes a number of measures of value suggested by commenters, all of which CMS agrees could be used in VBP arrangements: work productivity, patient satisfaction, medical spending reduction, reduction in hospitalization rates or emergency room visits, laboratory tests values, and patient-reported quality of life.[4] Other measures suggested by commenters were clinical endpoints, survival, recovery, adverse event rates, and medication adherence.[5]

[2] 42 C.F.R. § 447.502.
[3] 85 Fed. Reg. 87,013.
[4] *Id.* at 87,015.
[5] *Id.* at 87,013.

Manufacturers have long complained that best price restrictions discourage them from offering creative VBP discounts to commercial payors and Medicaid Managed Care Plans, both of which could affect best price. For example, in the outcomes based VBP arrangement described above, the refund of the drug for even one patient would arguably establish a best price of zero, increasing the unit rebate amount (URA) to an amount equal to the average price paid to the manufacturer by retail community pharmacies and wholesalers. The rule offers two solutions to this best price dilemma.

First, CMS gives its imprimatur to a methodology that some manufacturers have already adopted on their own: treating a VBP arrangement as a bundled sale. Under CMS regulations, a bundled sale is, in essence, a sale in which a discount is conditioned on the purchase of the same drug or another product, or another performance requirement (e.g., placement on a formulary tier).[6] While a VBP arrangement might fall within the current definition if it is conditioned on a minimum purchase volume or a formulary tier placement, CMS has explicitly clarified that a VBP arrangement may qualify as a bundled sale, regardless whether it is conditioned on a minimum purchase volume or formulary placement.[7] The advantage of a bundled sale is that the total discount is allocated among all of the items in the bundle in proportion to each item's undiscounted cost.[8] Thus, a full refund on one unit of drug, instead of resulting in a zero best price, may be allocated among all the units sold under the bundled arrangement, reducing the cost of each one by a small amount. The preamble offers an example of an arrangement requiring the purchase of 1,000 units of a drug at $200 per unit, with a $100 refund for each patient who does not meet the clinical outcome measure. If one patient failed to meet the outcome measure, the $100 discount would be allocated across all 1,000 units, resulting in a 10 cent price reduction for each unit – a reduction that is unlikely to set a best price.[9] As CMS acknowledges, the bundled sale approach may not be practical for drugs that treat small populations.[10] The volume of an orphan drug sold during a period may be so small that averaging will not reduce best price appreciably.

As an alternative to the bundled sale approach, the new regulation permits manufacturers offering a VBP arrangement to report multiple best prices for a single dosage form and strength of a drug, as long as the manufacturer makes the VBP arrangement available to all states.[11] For example, one best price could reflect the price under a VBP agreement and another could reflect the price under a non-VBP purchase

[6] 42 C.F.R. § 447.502.
[7] 42 C.F.R. § 447.502.
[8] *See* 42 C.F.R. § 447.502.
[9] *See* 85 Fed. Reg. at 87,022.
[10] *Id.* at 87,024.
[11] 42 C.F.R. § 447.505(a)

agreement. This would necessarily result in two different URAs. CMS explains that one URA would apply to units dispensed to a Medicaid beneficiary under a VBP arrangement where the patient qualified for the VBP discount. The other URA would apply to units dispensed to all other Medicaid beneficiaries.[12]

Currently, manufacturers may exclude from best price any rebates paid under a VPB arrangement that is incorporated into a supplemental rebate agreement approved by CMS pursuant to a Medicaid state plan amendment.[13] (Several states have entered into CMS-approved VBP arrangements with manufacturers.) However, under the new regulation, states need not obtain CMS approval in order to enter into a VBP arrangement, or in order for a manufacturer to be permitted to report multiple best prices pursuant to that arrangement.[14] States may still submit state plan amendments to establish VBP supplemental rebate programs under which the state enters into VBP agreements with manufacturers. States with such programs must submit annual reports to CMS, including data on administrative costs and total savings.[15]

The multiple best price approach has important limitations. First, it presumes that a manufacturer offers the VBP arrangement, not just to commercial payors, but to state Medicaid programs, and that at least one state elects to participate in the arrangement. A manufacturer that offers a VBP arrangement to commercial payors but not Medicaid may not report multiple best prices, but must follow the usual rule of determining best price based on the lowest single price to a best price-eligible entity (including under the VBP arrangement). Second, in order for the scheme to work, state Medicaid programs and Medicaid Managed Care plan sponsors will have to develop systems to track health outcomes, to distinguish units subject to one best price from units subject to another (since each would have a different URA), and to reflect these differences in their invoices and utilization reports to manufacturers and CMS. In recognition of these administrative challenges, the effective date of the revised definition of best price to permit reporting of multiple best prices will not take effect until January 1, 2022.

To avoid discouraging VBP arrangements that involve evidence based or outcomes based measures that are measured in periods greater than three years, or installment payments over such longer periods, the rule permits manufacturers to restate AMP and best price beyond the otherwise applicable three-year limit if a VBP outcome must be measured outside of that period.[16]

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[12] *Id.* at 87,025.
[13] *See* Manufacturer Release 99, July 14, 2016.
[14] 85 Fed. Reg. at 87,028.
[15] 42 C.F.R. § 447.518(d).
[16] 42 C.F.R. § 447.510(b)(1)(vi).

II. ALTERNATIVE REBATE FOR LINE EXTENSIONS

The alternative rebate for line extensions was enacted as part of the Affordable Care Act. The alternative rebate applies to "a line extension of a single source drug or an innovator multiple source drug that is an oral solid dosage form."[17] The statute defines a line extension as a change to the drug, including, but not limited to, a "new formulation of the drug, such as an extended release formulation, but does not include an abuse-deterrent formulation of the drug (as determined by the Secretary), regardless of whether such abuse-deterrent formulation is an extended release formulation."[18] While CMS has promulgated regulations regarding the calculation of the alternative rebate, CMS has not, until now, provided a regulatory definition of a line extension.[19] Without a regulatory definition, CMS has directed manufacturers to rely on the statutory definition of line extension and use reasonable assumptions in determining whether a drug qualifies.

The new definition of a line extension is consistent with that of the statute.[20] The final definition of a new formulation, however, is considerably broader than the extended release formulation example contained in the statute, though somewhat narrower than the definition that CMS originally proposed. CMS defines a new formulation as "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."[21]

As an initial matter, CMS interprets the statute to provide that only the initial brand name listed drug need be an oral solid dosage form.[22] Accordingly, new dosage forms and routes of administration – even non-oral ones – may be considered new formulations.[23] This represents a reversal of CMS's former proposal that both the initial drug and the line extension must be oral solid dosage forms in order for the alternative rebate to apply.[24]

CMS has included a new strength in the final definition of a new formulation, reversing its 2016 guidance.[25] However, in the face of considerable opposition to its extremely broad proposed definition, CMS has removed combination drugs, new

[17] 42 U.S.C. § 1396r-8(c)(2)(C)(i).
[18] *Id.* § 1396r-8(c)(2)(C).
[19] CMS proposed a definition in 2012, which it did not finalize. CMS requested further comments on the topic.
[20] 85 Fed. Reg. 87,000 (Dec. 31, 2020). .
[21] *Id.* at 87,044.
[22] *Id.* at 87,034.
[23] *Id*. at 87,043.
[24] 81 Fed. Reg. 5318, 5338 (Feb. 2, 2012).
[25] 81 Fed. Reg. 5170, 5267 (Feb. 1, 2016).

indications, and changes in pharmacodynamics or pharmacokinetic properties from the definition of a new formulation.[26]

CMS has finalized its proposed definition of oral solid dosage form: "an orally administered dosage form that is not a liquid or gas at the time the drug enters the oral cavity."[27] This would include, but not be limited to, a tablet or film administered sublingually and a drug that is orally inhaled.[28]

III. OTHER BEST PRICE ISSUES

A. Patient Assistance Exclusions Narrowed

Best Price and AMP currently exclude patient savings programs in the form of manufacturer discount cards, coupons, copayment assistance, patient rebates, and free-product vouchers (collectively, "patient savings programs"), provided that the full value of the benefit provided is received by the consumer.[29] Historically, manufacturers have reasonably assumed that their programs providing a certain dollar amount of savings to the patient at the point of sale (or rebates sent to the patient afterward) necessarily meet the requirement that the program benefits are provided entirely to the patient. The final rule throws a wrench into that assumption.

In recent years, PBMs, commercial payors, and Medicare Part D plan sponsors have implemented so-called "accumulator programs" to discourage manufacturer copay assistance programs. Payors complain that copay assistance programs defeat the payor's formulary by causing patients to use more expensive non-preferred drugs instead of lower cost preferred or generic drugs. Under an accumulator program, manufacturer subsidies for patient copays and deductibles are not counted toward the patient's deductible or out-of-pocket limits. CMS recently issued a final rule expressly permitting Affordable Care Act exchange plans and individual and group health plans to implement accumulator programs.[30]

In the final rule, CMS has taken the view that, where a plan has a copay accumulator, a manufacturer patient copay assistance program does not benefit the patient, who still has to pay the same amount out of pocket to meet his/her deductibles and out-of-pocket limits. Instead, it benefits the plan, which gets to delay payment for drugs until the patient works his/her way through the deductible or out-of-pocket maximum without the help of the manufacturer subsidy. From this, CMS concludes that

[26] 85 Fed. Reg. at 87,039, 87,040, 87,042.
[27] 42 C.F.R. § 447.502.
[28] 85 Fed. Reg. at 87,044.
[29] 42 C.F.R. §§ 447.504(c)(25) through (29) and 447.505(c)(8) through (12).
[30] *See* 45 C.F.R. § 156.130(h), 85 Fed. Reg. 29164, 29261 (May 14, 2020).

a manufacturer cannot reasonably assume that its copay subsidy program meets the best price exclusions' requirement that the patient must receive the full benefit of the patient savings program.[31] CMS's solution is to permit a manufacturer patient savings subsidy to be excluded from best price (and AMP) only "to the extent that the manufacturer *ensures* that" the full value of the benefit is received by the patient.[32] In other words, a manufacturer may no longer reasonably assume that the benefit is received by the patient, but now must "ensure" it.

In order for a manufacturer to exclude patients whose plans have accumulator programs from eligibility, the manufacturer must find out whether a particular patient's plan has an accumulator program, which typically is not publicly available information and may not be known to the patient. As to how manufacturers are supposed to "ensure" that the benefit is received by the patient, CMS offers three suggestions, two of which unfortunately are not currently practicable, while the third would revert to the mail-in rebate coupons of the 1980s and 1990s. CMS' first "solution" is that manufacturers rely on switches to obtain necessary patient information. CMS notes that, for processing copay subsidies at the point of service, manufacturers or their vendors typically rely on "switches" (electronic claim adjudication systems) that collect insurance information about the patient and a BIN/PCN number for processing the copay subsidy.[33] CMS does not mention that this information currently does not include whether a patient's plan operates an accumulator program, but CMS apparently believes that manufacturers and switch operators will get together and come up with a way to collect this information electronically and act upon it accordingly.

CMS's second "solution" is for manufacturers to use PBM rebate agreements to require PBMs to implement mechanisms to identify patients with plans that have accumulator programs. Ignoring the fact that these rebate agreements typically have nothing whatever to do with manufacturer copay coupons or subsidies, CMS expresses unwarranted optimism that "that PBMs will work with manufacturers to provide this information to the manufacturers to help them ensure that their assistance is passed through."[34]

As the third solution, CMS offers the "manual approach" – i.e., requiring patients to pay for the drug first, then having the patient collect the rebate directly from the manufacturer. This would require manufacturer to transform their copay assistance

[31] 85 Fed. Reg. at 87,048-87,049.
[32] 42 C.F.R. § 447.505(c)(8)-(12) (emphasis added).
[33] 85 Fed. Reg. at 87,053.

[34] *Id.*

programs into the kind of mail-in rebates that became largely extinct earlier in this century.

CMS displayed some recognition of the difficult barriers it is erecting against copay assistance to the extent that it is delaying the effective date of these amendments to the best price exclusions until January 1, 2023. It is possible that manufacturers will be able to overcome these barriers sufficiently to operate copay assistance programs, but it is by no means a foregone conclusion that these programs will be as prevalent after 2022 as they are today.

B. AMP/Best Price Treatment of Supplemental Rebates to Medicaid Managed Care

Under the Medicaid Rebate statute, states are permitted to enter into separate supplemental drug rebate agreements with manufacturers, subject to CMS approval of a state plan amendment.[35] Such agreements often offer manufacturers eligibility for placement of their drugs on the state's preferred drug list in exchange for the supplemental rebates. Supplemental rebate agreements may require rebates to be paid, not only on Medicaid fee-for-service utilization, but also units dispensed to Medicaid Managed Care Organization (MMCO) enrollees. With regard to the latter, some states directly collect supplement rebates for units dispensed to MMCO enrollees, while others require MMCOs to collect and share the rebates with the state Medicaid agency. Still other states permit MMCOs to negotiate their own rebates with manufacturers outside of any CMS-authorized supplemental rebate agreements, which allows the MMCO to keep the savings generated by the supplemental rebates.

Under current CMS regulations, rebates paid under "CMS-authorized State supplemental rebate agreements" are excluded from both AMP and best price.[36] However, CMS explains that some manufacturers have mistakenly assumed that all rebates paid to MMCOs are rebates paid under CMS-authorized agreements. To clarify this point, CMS is finalizing a new definition of a "CMS-authorized supplemental rebate agreement," which would specify that such agreements must be approved by CMS through a state plan amendment, and the revenues therefrom must be passed through to the state.[37] Accordingly, rebates paid to MMCOs that are not under a CMS-authorized supplemental rebate agreement may not be excluded from AMP or best price.[38]

IV. IMPLEMENTATION OF OTHER STATUTORY CHANGES

[35] 42 U.S.C. § 1396r–8(a)(1).
[36] 42 C.F.R. §§ 447.504(c)(19) and (e)(9) (AMP) and 447.505(c)(7) (best price).
[37] 42 C.F.R. § 447.502.
[38] 85 Fed. Reg. at 87,047.

A. Exclusion of Authorized Generic Sales From Brand AMP

In Section 1603 of Continuing Appropriations Act, 2020, and the Health Extenders Act of 2019,[39] Congress revised the AMP definition so that the AMP of a brand drug that has an authorized generic excludes sales of the authorized generic. This change became effective for rebate periods beginning October 1, 2019.[40] CMS issued guidance to implement these amendments in Manufacturer Releases 111 (Oct. 17, 2019) and 112 (May 18, 2020). In the latter Release, CMS clarified that the exclusion from AMP not only applies to sales of an authorized generic by the NDA holder to an unaffiliated entity, but also applies where the same company or two corporate affiliates market both the brand version and the authorized generic.

In the final rule, CMS has revised its regulations consistent with the statutory amendments. Among other things, CMS has revised its authorized generic regulation to state that the primary manufacturer (i.e., NDA holder) must exclude from its calculation of the brand AMP any sales of authorized generic drugs to wholesalers for drugs distributed to retail community pharmacies. Instead, the brand drug will have an AMP exclusive of any authorized generic sale, and the authorized generic will have its own separate AMP. The preamble also reiterates the guidance in Release 112 – that the exclusion of authorized generic sales from the brand AMP applies even when the brand and the authorized generic are marketed by the same company or by two corporate affiliates.[41] This change will likely result in higher AMPs and increased rebates for the brand drugs.

Manufacturers that have been including authorized generic sales in the AMP of the brand version are expected to revise their AMP calculations for rebate periods beginning with 4Q 2019, but will have a 12 quarter window (i.e., until January 30, 2023) in which to restate their prior period AMPs.

B. Revising the Definitions of Single Source, Innovator Multiple Source, and Multiple Source Drugs

Until April 2019, single source drugs and innovator multiple source drugs, which are subject to a substantially higher per-unit rebate than non-innovator drugs, were defined in the statute as drugs approved under an "original new drug application." This undefined term generated confusion until February 2016, when CMS issued a regulation defining an "original new drug application" as simply an approved NDA, "unless CMS

[39] Pub. L. 116-59 (Sept. 27, 2019).
[40] *See* our blog post on this subject at http://www.fdalawblog.net/2019/10/continuing-appropriations-act-changes-treatment-of-authorized-generics-in-medicaid-rebate-average-manufacturer-price/ .
[41] 85 Fed. Reg. at 87,060.

determines that a narrow exception applies."[42] In the preamble to the 2016 rule, CMS advised that narrow exceptions would only be granted for drugs that were approved under FDA's paper NDA policy prior to 1984 or under literature-based 505(b)(2) applications, and that had no patent protection or statutory exclusivity.[43]

In Section 6(c)(2) of the Medicaid Services Investment and Accountability Act of 2019 ("MSIAA"),[44] Congress codified CMS's interpretation by deleting the word "original" before "new drug application" in the statute and codifying the narrow exception process. Accordingly, the Medicaid rebate statute now defines a single source drug and an innovator multiple source drug, in part, as a drug that is marketed under an NDA approved by the FDA, "unless the Secretary determines that a narrow exception applies (as described in § 447.502 (or any successor regulation))."[45] CMS is now proposing to make conforming changes in the definitions of single source drug and innovator multiple source drug by deleting the term "original" altogether.

Also to conform to amendments in the MSIAA, CMS is proposing to revise the definitions of "single source drug" and "multiple source drug" to include non-prescription drugs that qualify as covered outpatient drugs, and that meet the other conditions of those definitions.[46]

[42] 42 C.F.R. § 447.502.
[43] 81 Fed. Reg. at 5191; *see also* Manufacturer Release 98 (May 2, 2016).
[44] Pub. L. 116-16, April 18, 2019.
[45] 42 U.S.C. § 1396r-8(k)(7).
[46] 42 C.F.R. § 447.502.