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Financing and Reimbursement of Cures in the US

RESEARCH BRIEF

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Incorporation of Value-Based Payment Agreements into the Calculation of Medicaid Drug Rebates.

Value-Based Payment (VBP) agreements will play an important role in mitigating uncertainty in durable therapies, where it is unclear whether a large upfront payment is justified based on limited clinical data regarding magnitude and durability of benefits. Current Medicaid Drug Rebate regulations create significant uncertainty and financial exposure for manufacturers contemplating these agreements, as the current regulations are not designed for variable payments. The goal of this research brief is to identify alternative calculations of the Medicaid Drug Rebate for VBP agreements that address the needs of both manufacturers and the Medicaid program.

Medicaid drug rebate program background

The Medicaid Drug Rebate Program was created in 1990 to help offset state and federal costs for outpatient prescription drugs dispensed to Medicaid patients. It was last revised in 2010 as part of the Patient Protection and Affordable Care Act, with enabling final rules issued by CMS in 2016. [1] [2] For covered outpatient drugs, Medicaid receives a "Unit Rebate" on every unit purchased for Medicaid patients. These rebates are intended to provide Medicaid with net prices that are comparable to or lower than the best prices received by most other payers.

While the details of the Medicaid Drug Rebate calculation are beyond the scope of this Research Brief, [3] the key aspects are the calculation of Average Manufacturer Price (AMP) and the determination of Unit Rebate based on the greater of a statutory rebate (23.1% of AMP for most branded products) or the difference between the "Best Price" available to certain classes of purchasers and AMP. Adjustments are made based on increases in drug price that are faster than inflation.

KEY TAKEAWAYS

Payers, Manufacturers and ultimately Patients would benefit from updates to the Medicaid Drug Rebate calculation that would enable the use of Value-Based Payment (VBP) agreements

The largest current challenge to the use of VBP agreements is that poor outcomes for one payer could lead to an artificially low Best Price that extends to all Medicaid patients

Alternative calculations of the Medicaid Drug Rebate with differing complexity and accuracy (such as those described herein) could alleviate this issue

AMP is calculated in two different ways. For drugs distributed through retail pharmacies, AMP is the average price paid to the manufacturer by the relevant supply chain purchaser (such as wholesalers for drugs distributed to retail pharmacies), including relevant discounts and rebates to that entity, but NOT including rebates paid to those who reimburse the product (such as insurers).

Drugs that are inhaled, infused, injected, instilled or implanted ("5i drugs") are typically administered by a physician instead of being purchased from a retail pharmacy; AMP for these products is calculated based on the average price paid to the manufacturer net of ALL discounts and rebates paid to any entity (including, for example, rebates to BOTH a specialty pharma distributor and an insurer).

Best price is calculated based on the lowest price made available to any purchaser. It includes all discounts and rebates available both directly to the immediate purchaser and indirectly to the ultimate payer.

The calculation of Medicaid Drug Rebate is tied to pricing for 340B covered entities. The maximum price that can be charged to those entities ("ceiling price") is AMP less the Unit Rebate. Lowering AMP can thus also decrease pricing for 340B entities, which may become more important as durable treatments increase in the 340B setting. Implications for 340B pricing will not be mentioned explicitly below but need to be considered when making any modifications to the calculation of AMP and the Unit Rebate.

DURABLE THERAPIES, VALUE-BASED PAYMENT AGREEMENTS, AND THE MEDICAID DRUG REBATE

In recent years, there has been growing interest in value-based payment (VBP) agreements, where prices for treatments may vary depending on actual outcomes for patients. These approaches are particularly pertinent for durable therapies, where the potential for long-term benefit based on short treatment can cause significant challenges for payers: A large upfront payment might be appropriate if the product actually provides long-term benefits, but there may be limited clinical evidence at launch and thus substantial uncertainty regarding magnitude and durability of benefit. In the absence of a VBP agreement, the manufacturer and buyer would need to agree on a specific price for the product that might differ substantially from the actual value created by the product, creating risk for both parties and potential impact on appropriate utilization.

Most durable treatments in development are for rare diseases, and payers would be expected to have very few patients who receive such a treatment during a particular quarter. If an agreement specifies that a payer receives a rebate of 50% if a product fails and the product actually does fail in the only patient treated by that payer during a quarter, that payer would be establishing a "Best Price" that is approximately half of typical pricing, even if 95% of patients who receive the product have successful outcomes nationwide. This price would lead to an inappropriately low price for all Medicaid patients.

VBP agreements can be set up in a variety of ways, which can lead to differing issues based on the VBP agreement structure and interpretation of the Medicaid Drug Rebate regulations. Some of the potential problems include the following:

- Individual payments could be used for calculation of AMP or Best Price
- Small sample size could lead to:
  - AMP volatility from period to period
  - Pricing and Medicaid rebate uncertainty
  - Inflationary penalties
  - Artificially low best prices
  - Low AMP in some periods that might limit the Medicaid Drug Rebate

In order to avoid these consequences, manufacturers limit their use of VBP agreements that could facilitate more appropriate drug pricing.

POTENTIAL SOLUTIONS TO MEDICAID PRICE REGULATORY CHALLENGES

The goal of this Research Brief is to suggest means by which Medicaid drug rebate calculations can be extended to allow appropriate VBP agreements to be implemented. While there are many detailed regulatory modifications that may be necessary to accomplish this, the focus for this brief is on the choice of appropriate high-level methodologies for incorporating VBP agreements into Medicaid Drug Rebate calculations. The guiding principles used to develop potential solutions are as follows:

- Enable Value-Based Payment agreements
- Provide Medicaid with a rebate consistent with the spirit of current practice
- Protect manufacturers from artificially low best prices due to poor outcomes in a small sample

As volatility due to small sample size is the major issue, this brief will start by discussing methodologies for the calculation of Best Price, which is inherently more volatile than AMP because it is based on a single price instead of the average of prices. The brief will then return to the calculation of AMP and conclude by noting additional considerations that are out of scope for this research brief.

We suggest three approaches that might be appropriate for managing the Best Price calculation, with further discussion of each and a clarifying example below:

1) Calculate Best Price by applying clinical trial data to the best VBP agreement
2) Calculate Best Price by averaging actual net payments by payers under VBP agreements
3) Calculate Best Price by applying average outcomes across all payers to the best VBP agreement

We will demonstrate these three approaches using a simple example where there are 20 patients in VBP agreements across two payers and a larger number of patients managed through flat pricing:

Example Overview
Clinical Trial Success Rate (VBP outcome of interest): 90%
AMP (assumed level): $1000

Payer 1: $1000 on success, $0 on failure. Actual performance: 6/10 positive
Payer 2: $975 on success, $0 on failure. Actual performance: 8/10 positive

Success rate for all payers (outcome of interest): 70%

APPROACH #1: Calculate Best Price by applying clinical trial data to the best VBP agreement

In the first approach, clinical trial data are initially used as the best estimate of what real-world patient outcomes will look like in the VBP agreement. In the example, the 90% outcome success rate from clinical trials is plugged into actual VBP terms for the two payers to generate expected prices for each of the payers. Rebate is calculated as the greater of the statutory rebate (23.1% * AMP) and the difference between AMP and the best price for any payer (assumed to be the best price of any payer in the VBP agreement).

Approach #1
Payer 1: $1000 * 90% expected success → $900 / patient
Payer 2: $975 * 90% expected success → $877.50 / patient

AMP * 23.1% = $231
AMP – Best Price = $1000 - $877.50 = $122.50
Rebate = Max($231, $122.50) = $231

The advantages of this approach are that it is the simplest to calculate and can be calculated at the time the VBP agreement is initiated. However, it is dependent on clinical trial data, which may have a small sample size that is not representative of real-world outcomes and might in some cases be very dependent on manufacturer choices (such as which patients are eligible for the trial). In addition, outcomes of interest that reflect durable benefit may not be available at launch other than as projections or very small numbers of patients. After launch, expected outcomes may be based on representative real-world outcomes, which may lead to better approximations of actual expected patient outcomes.

APPROACH #2 Calculate Best Price by averaging actual net payments by payers under VBP agreements

In the second approach, the actual payments by payers are used as the basis for determining best price. Because individual payers may only have a small number of relevant patients in a reporting period, patients are aggregated across payers that are participating in similar VBP agreements. For simplicity in the example, it is assumed that the two payers together provide adequate reduction of variability, but the actual number of patients required depends on the success rate for the outcome and tolerance for error (See Appendix Table 1 below). Aggregating patients across longer periods (such as a full calendar year), may be appropriate to improve accuracy.

Approach #2
Payer 1: $1000 * 6 actual successes → $6000
Payer 2: $975 * 8 actual successes → $7800
Average VBP price = ($6000+$7800)/20 patients →$690/patient

AMP * 23.1% = $231
AMP – Best Price = $1000 - $690 = $310
Rebate = Max($231, $310) = $310

The advantages of this approach are that it provides a much better measure of the true best price for payers, as it is based on what they actually pay. However, it adds complexity to the calculation, particularly because final calculation of best price cannot occur until after the completion of the VBP agreement, which may be several years after the initial patient treatment.

APPROACH #3: Calculate Best Price by applying average outcomes across all payers to the best VBP agreement

In the third approach, average outcomes (based on how many patients on a national basis achieve VBP performance hurdles) are applied to actual contract terms for each individual payer in order to calculate what the expected payments would be for each payer if outcome variability for that payer is removed. The national average for an outcome parameter needs to be calculated by the manufacturer from all available data at the end of the contract, so the manufacturer needs to ensure that the relevant data for a VBP agreement outcome is widely available.

The advantage of this approach is that a reasonable Best Price can be calculated for each payer based on its terms, even if it has very few patients. However, the calculation is more complex, and determination of national outcomes may

Approach #3
Payer 1: $1000 * 70% national success rate → $700/patient
Payer 2: $975 * 70% national success rate → $682.50/patient

AMP * 23.1% = $231
AMP – Best Price = $1000 - $682.50 = $317.50
Rebate = Max($231, $317.50) = $317.50

be subject to some interpretation when actual collected data varies.

Figure 1 provides a rough comparison of the three approaches.

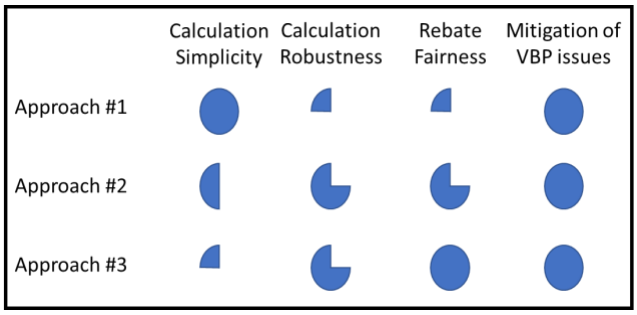

Figure 1. Comparison of the benefits of the different approaches.

CALCULATION OF AMP

As noted above, AMP is calculated differently for retail and 5i drugs, with the calculation for 5i drugs including rebates beyond the direct purchaser. Essentially all durable therapies are likely to be 5i drugs, though VBP agreements could also be used for retail drugs. We will focus on the calculation for 5i drugs; retail drugs could be calculated by the same or a different methodology.

For 5i drugs, it would be possible to exclude VBP agreements completely from AMP calculations, include them in AMP calculations using the net price (after rebates), or include them using the full price (assuming treatment success). As VBP agreements may become the preferred way to manage products with uncertain benefits, completely excluding them from AMP calculations is not recommended, as it could make the calculation of AMP impossible if there are no other prices available.

The following considerations influence the choice between including VBP agreement prices with or without the performance rebates:

- Small sample size could make AMP volatile if performance rebates are included (and might trigger inflationary penalties)
- Including VBP agreement prices at full price might lead to an artificially high AMP and a higher Unit Rebate
- Including VBP agreement prices at full price could make AMP volatile if full price varies from agreement to agreement (Example: If the success rate for a product is expected to be 80%, two payers might negotiate agreements wherein one pays $100 on success and $20 on failure and the other pays $85 on success and $80 on failure. Both would expect to pay $84 on average, but the full price would differ substantially)
- Including rebates in the calculation of AMP would lead to a similar impact on AMP for 5i products regardless of whether a product is in a VBP agreement or not (assuming that the expected price after rebates is comparable), but excluding rebates might create inappropriate incentives to avoid VBP agreements, which would lead to higher expected rebates
- If Medicaid pays for a product at a fixed price and commercial payers all utilize VBP agreements and the products perform very poorly, AMP may be pulled down and the rebate may be inappropriately small

While small sample size is the largest issue with durable products, the methodologies discussed above for Best Price could also be used to reduce the volatility of AMP calculations, mitigating the main issue with using price net of rebates. AMP calculations might still be volatile in very low sample size situations, but it is likely that an alternative calculation overall for the Medicaid Drug Rebate might be more appropriate in such a circumstance, as Best Price would be even more volatile. Inclusion of all rebates in the AMP calculation, aligned with how AMP is currently calculated for 5i drug, is likely the better option, though Medicaid may itself choose to use VBP agreements to protect itself from poor performance if VBP agreements are common and treatment benefit is highly uncertain.

CONCLUSION

Detailed enablement is beyond the scope of this Research Brief. Some of the factors that need to be considered include:

- Determination of patient cohorts (e.g. by year to increase sample size), with potential alternative calculations if the number of patients falls below some threshold (ultra-orphan disease, slow uptake, few VBP agreements)
- Maximum amount of time permitted for any agreement, and methods for combining outcomes from different agreement types (e.g. three- and four-year agreements)
- Mechanisms for paying rebates between initiation of contracts and final settlement, such as on an expected outcomes basis or based on assuming maximum possible payments for all patients (given current outcomes status), which ensures that Medicaid need never repay part of a rebate
- Discounting of future year payments (within VBP contract, of Medicaid rebates)
- Incorporation of the inflation adjustment

		Number of Patients					
		5	10	25	50	100	200
Prob(Pay)	25%	19.4%	13.7%	8.7%	6.1%	4.3%	3.1%
	50%	22.4%	15.8%	10.0%	7.1%	5.0%	3.5%
	60%	21.9%	15.5%	9.8%	6.9%	4.9%	3.5%
	70%	20.5%	14.5%	9.2%	6.5%	4.6%	3.2%
	80%	17.9%	12.6%	8.0%	5.7%	4.0%	2.8%
	90%	13.4%	9.5%	6.0%	4.2%	3.0%	2.1%
	95%	9.7%	6.9%	4.4%	3.1%	2.2%	1.5%
	99%	4.4%	3.1%	2.0%	1.4%	1.0%	0.7%

Appendix Table 1. Standard error as a function of true expected outcome probability and number of patients.

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ABOUT FOCUS

The MIT NEWDIGS consortium FoCUS Project (Financing and Reimbursement of Cures in the US) seeks to collaboratively address the need for new, innovative financing and reimbursement models for durable and curable therapies that ensure patient access and sustainability for all stakeholders. Our mission is to deliver an understanding of financial challenges created by these therapies leading to system-wide, implementable precision financing models. This multi-stakeholder effort gathers developers, providers, regulators, patient advocacy groups, payers from all segments of the US healthcare system, and academics working in healthcare policy, financing, and reimbursement in this endeavor.

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