Research

Current Impediments to Value-Based Pricing for Prescription Drugs

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Executive Summary

Certain laws and regulations currently in place are impeding the development of value-based pricing agreements for pharmaceuticals. Three in particular are commonly referenced as the most prohibitive policies in this regard.

- The Medicaid "best price" policy—specifically, the regulation's requirement that manufacturers report drug prices on a "per unit" basis and provide rebates for all Medicaid recipients of that drug based on that best price—disincentives manufacturers from agreeing to offer substantial rebates for any drug that does not prove effect for a particular patient. Clarity from the Centers for Medicare and Medicaid Services (CMS) regarding the calculation of a drug's best price could remove this disincentive.
- Federal anti-fraud policies, such as the Stark Law and Anti-Kickback Statute, complicate the ability of drug manufacturers to enter into value-based contracts with insurers and providers because such agreements may be viewed as inducing providers to prescribe a particular medicine. Congress and CMS should clarify and update these laws' safe harbor provisions to reflect the needs of the changing payment landscape.
- Food and Drug Administration (FDA) regulations limiting the promotion and sharing of information relating to investigational medicines and off-label use of existing approved drugs delay patient access to new therapies and treatment options. The 21st Century Cures Act expands the scope of information and the audience with which manufacturers may share such information prior to a drug's approval or regarding off-label use, but so far the FDA has only published draft guidance on this topic. Additional legislation and final regulations on the topic are needed to provide additional clarity, particularly regarding communications about off-label drug use.

Introduction

The cost of prescription drugs has been a subject of growing concern for several years. However, as shown in this research from the American Action Forum (AAF), and further explained here, the scope of the problem is much narrower than commonly assumed. In particular, the development of advanced new treatments for previously fatal or debilitating diseases, such as cancer and hepatitis C, has played an outsized role in increasing spending on pharmaceuticals.

Scientific discovery and medical breakthroughs come at a high price. With the unprecedented number of specialty medicines and oncology treatments expected over the next few years, the cost of prescription drugs will continue to be a concern for all stakeholders.[1] QuintilesIMS Institute finds that 28 percent of new drugs currently being developed are oncology medicines, and nearly half of all drug spending in the U.S. will be for specialty medicines by 2021.[2] Ensuring our health care dollars are being spent wisely, on high-value goods and services, is more important than ever.

Value-based payment (VBP) reform is moving forward—slowly but surely—when it comes to payment for services, driven largely by changes in Medicare as a result of the Medicare Access and CHIP Reauthorization Act (MACRA). However, value-based payment models for prescription drugs are moving at a slower pace and in a more piecemeal fashion. While a handful of drug manufacturers have recently announced VBP arrangements with insurers or provider entities, they are the exception. There are several current laws and regulations, as well as other industry factors, creating impediments to expanded use of value-based contracts for medicines.

Value-Based Contract Agreements

Value-based payment models may include value-based purchasing, value-based pricing, and value-based insurance design, all of which take cost, quality, and efficacy into account in some manner to establish payment amounts. Value-based insurance design refers to the methodical structuring of insurance benefits and costsharing requirements in a way that encourages patients to seek high-value goods and services. Value-based purchasing refers to reimbursement strategies of payers of health care goods and services based on the payer's perceived value provided, while value-based pricing refers to the setting of prices by suppliers of health care goods and services based on the value they believe is provided by their product. Most people may not distinguish between these latter two types of payment models because ultimately a contract agreement is dependent upon the payer and supplier agreeing to a final price, but the initial price set by the supplier is much more determinative of the final price than the amount of the discount, rebate, or price adjustment that may be agreed to after the fact. For example, the total amount of discounts, rebates, and other forms of direct and indirect remuneration in Medicare Part D was less than 18 percent of total drug costs in 2015.[3] That being said, insurers are gaining leverage and increasingly able to affect prices to a larger degree through these value-based contracts.

While some value-based contracts are primarily focused on cost and financial risk-sharing, more comprehensive value-based contract agreements are ultimately also tied to patient outcomes. An outcomes-based contract regarding payment for medicines typically stipulates that the insurer will pay less for a drug that proves ineffective for a particular patient or group of patients. In other words, the insurer will not pay the full cost of a drug that does not work as intended. But there must first be agreement on which outcomes will be used to determine the drug's effectiveness, how and when those outcomes will be measured, and what threshold must be met.

Appropriate outcomes are those for which a significant correlation has been shown to exist with product use. That correlation should be expected to appear in a relatively short time-frame (ideally, in less than a year—the standard length of an insurance plan), and there should be a simple method for reliably and objectively measuring the effect. Outcomes that are dependent upon patient behavior, and those which will take years to observe will be difficult to use with this type of payment arrangement. Also critical to the success of these types of agreements is appropriateness of care—using the right drugs for the right patients at the right time, in order to avoid patients who are not likely to respond well to the medication. For instance, patients with multiple chronic conditions may have other confounding factors making it difficult to determine the exact reason a particular

drug is ineffective for that patient, or if something else is inhibiting a patient's health from improving.

Examples of Current Value-based Contracts

Merck has signed several VBP agreements over the years, including one of the first of these types of agreements with Cigna in 2009 for two of its diabetes drugs, Januvia and Janumet, which was quite unusual.[4] Under this agreement, Merck offered rebates if patients taking *any* oral antidiabetic drug (including those from other manufacturers) achieved certain medication adherence benchmarks and had improved blood sugar levels. An additional rebate was provided if adherence rates for patients taking either of Merck's drugs improved even further.[5]

According to results released by Cigna a year later, medication adherence improved dramatically and patients' blood sugar levels improved by more than 5 percent on average.[6] This agreement, conceptually, turns the traditional model of value-based purchasing on its head; typically, manufacturers would provide a greater discount or be reimbursed less for a product or service that leads to *worse* outcomes. Paying greater discounts for better outcomes seems to undermine the concept of paying more for greater value. One can only assume the increased sales volume resulting from preferred formulary placement and increased medication adherence is enough to offset the discounts.

Merck recently entered another VBP deal with Aetna,[7] and announced another contract under development with Optum. Merck and Optum will collaborate on a multi-year project, sharing and analyzing patient claims data and medical outcomes. The findings will be publicly released with the hope of informing the industry and facilitating expanded development of what Optum is calling "outcomes-based risk sharing agreements."[8]

Novartis entered into VBP agreements with Aetna and Cigna in February 2016 for its new drug to treat chronic heart failure, Entresto, which has an average treatment cost of roughly $9,000.[9] These agreements are based on the drug's clinical trial results showing use of the drug was associated with a 21 percent reduction in heart failure hospitalizations, a 20 percent reduction in the risk of death from cardiovascular causes, and a 16 percent reduction in mortality for any reason, compared with other drugs.[10] Specifically, Cigna's payments will depend on the percentage of Entresto patients admitted to the hospital for heart failure; Aetna's reimbursement is based on the drug's ability to replicate the results from the clinical trials, and on the rate of heart failure-related deaths among patients taking Entresto.[11] Under both agreements, the drug will be on the preferred formulary, reducing patient's out-of-pocket costs. However, many doctors typically prescribe a cheaper generic for heart failure and may be reluctant to prescribe a more expensive drug without encouragement.[12]

ExpressScripts and CVS announced in April 2016 that they would set prices for some cancer drugs based on specific indication-based values.[13] Under this type of agreement, drugs are paid more or less depending on how cost-effective and valuable they are for the specific indication for which they are prescribed.

Most recently, Amgen signed what is being described as essentially a money-back guarantee by agreeing to provide full reimbursement for the cost of Repatha (approximately $14,000[14]) for patients of Harvard Pilgrim who have a heart attack or stroke while using the drug;[15] the way such an agreement will work in practice, though, remains to be seen.[16] Amgen has decided to take this risk based on its clinical trial results which showed patients had a 27 percent reduced risk of a heart attack, 21 percent reduced risk for stroke, and 22 percent reduced risk for coronary revascularization. The Institute for Clinical and Economic Research (ICER) originally claimed that available evidence regarding PCKS9 inhibitors, such as Repatha, was insufficient to determine the drugs would "[provide] a net health benefit for patients with cardiovascular disease who cannot

take statins, or who take statins but have not reached a suitable cholesterol level," and as such, stated the drugs were substantially overpriced.[17] However, in light of Amgen's recent release of these additional findings, ICER is updating its evaluation, which should be published soon. This brings up an important point, though—assessing the value of a new drug may take years and the basis of that assessment may not be viewed equally by all.

Assessing Value

As discussed above, most value-based contract agreements rely on some mix of objective, measurable patient outcomes and a comparative cost of treatment, but value in any context is, of course, subjective to at least some degree and may vary from one person's perspective to the next. In the context of health care, it is important to consider what each patient values to truly assess a product's value on an individual basis. How does the patient value a longer life relative to the quality of life they will endure during those years? What is the patient's tolerance to pain? How much disruption to their daily life will the patient accept? What is the strength of the patient's support system? The fact that these answers will not be uniform across a patient population certainly complicates the establishment of such payment models, and limits the ability to develop payment models based solely on value.

Policy Impediments to Further Development of Value-Based Contracts

Medicaid's "Best Price" Policy

In 1990, Congress created the Medicaid Drug Rebate Program with the intent of lowering the cost of prescription drugs for both the state and federal governments, as well as the beneficiaries of the program. The program requires that, as a condition of having their drugs covered by Medicaid, drug manufacturers must agree to provide rebates for all their drugs when purchased by Medicaid. The rebate amounts are based on the lower of the drug's Average Manufacturer Price (AMP) or the best price given to any commercial buyer, calculated, pursuant to the regulation, on a "per unit" basis.[18]

Just as Medicaid's "best price" policy caused the unintended consequence of limiting charity care, which led to the creation of the 340B program, this policy is now making it unfavorable for drug manufacturers to enter into value-based contracts for their drugs. If, for an extreme example, a manufacturer has an agreement that stipulates payment for the drug will be zero if it does not work as intended for a given patient, and in fact the drug does not work, then the "best price" that the manufacturer would have to report to Medicaid would be zero. The manufacturer would then have to rebate the full cost of the drug for every Medicaid prescription in that quarter. In other words, the manufacturer would lose all Medicaid revenue for that drug in each quarter that it failed to work for a single patient under such a contract. However, there may be a way to mitigate this extreme consequence of the "best price" policy.

Rather than setting a value-based price on a per patient basis, insurers could evaluate a drug's performance across a patient population on an aggregate basis. Rachel Sachs, et al., suggests in this Health Affairs article, that using this method could make it possible to require a steep discount for an occasionally ineffective drug without necessarily triggering the "best price" rule if the drug is effective more often than it is not. On the other hand, if the drug is not typically effective, it does not deserve to have its profits protected.

Manufacturers would certainly benefit, though, from clarity on calculating the price per drug for the Medicaid Rebate Program, which is currently done on a per unit basis. In the most recent final rule for this program, the

Centers for Medicare and Medicaid Services (CMS) acknowledged the challenge presented by the Medicaid "best price" policy to the establishment of value-based pricing arrangements and promised to provide further guidance on the subject in the future.[19]

Anti-Kickback Statutes

Laws put into place to prevent fraud may also be inadvertently inhibiting the development of value-based pricing arrangements among providers who treat or insurers that cover Medicare and Medicaid patients on behalf of the federal government. The Physician Self-Referral (Stark) Law prohibits physicians from referring patients for items or services reimbursable by the federal government to a health care entity with which the physician has a financial relationship.[20] The Stark Law is a civil statue with a "strict liability" test which means that the offender does not have to be found guilty of *intending* to break the law, but simply breaking it, even unknowingly. Violation can result in a civil monetary penalty of up to $100,000 and exclusion from participation in federal health care programs.

The Anti-Kickback Statute (AKS) prohibits the offering or receipt of anything of value in return for referring a patient for goods or services reimbursed by a federal health care program.[21] The AKS is a criminal statute which can result in up to $25,000 in fines and five years in prison for a single violation; civil penalties may also be applied and immediate exclusion from participation in all federal health care programs.[22] The AKS does require proof of intent, but the precedent of the "one purpose" test, which requires only that one purpose of the payment be to induce referrals, forces them to proceed with caution.[23]

With the increased use of Advanced Payment Models, the likelihood of a potential violation of these laws may also be on the rise. If a physician were to enter into a contract under which their reimbursements were in some way tied to the performance of a drug they prescribe or under which they shared any savings achieved, then prescribing that drug to a Medicare or Medicaid patient could potentially be a violation of these laws. While it may be possible to avoid this potential conflict by keeping the provider's reimbursement completely disassociated from use or outcomes of any particular drug, doing so would likely lead to less than optimal results. APMs rely primarily on well-coordinated care across a patient's entire team of health care professionals. Further, such an arrangement would likely mean that the drug is not prescribed as often which would make it difficult to use the type of payment scheme discussed above where reimbursement is based on the aggregated results of a group of patients rather than the outcomes of each individual patient in order to avoid the harm of the Medicaid "best price" policy. This payment framework works better the more patients covered, and without involving the physician, it may not succeed. Manufacturer payments to patients to encourage increased medication adherence may also violate such laws.

CMS has the authority to grant waivers or safe harbors to these laws as necessary when testing new care delivery models or efforts to reduce expenditures through the Center for Medicare Medicaid Innovation.[24] Under the ACA, MSSP ACOs were given waivers from both laws,[25] and the Anti-Kickback Statute was amended to exclude prescription drug discounts manufacturers provide directly to certain beneficiaries from the CMS definition of remuneration.[26] Congress and CMS should work together to create additional safe harbors which balance the need for expanded use of value-based contracts in the commercial sector with continued safeguarding of taxpayer dollars.

Pre-approval and Off-label Marketing and Communication Rules

Before a drug has been approved by the Food and Drug Administration (FDA), regulations prohibit drug

manufacturers from sharing all information about the investigational drug with payors or providers or promoting it as safe or effective.[27] The same is true regarding information about off-label use of drugs which have already been approved for one indication but are being prescribed for another yet-to-be-approved indication.

Many drug manufacturers and insurers alike have expressed concerns that these regulations make it difficult to enter into contracts with payors prior to a drug's approval or for indications for which a drug has not yet been approved. Because insurers must design their plan benefits and set premium and cost-sharing amounts months before the plan's coverage takes effect and most plans are set on an annual basis, these communication restrictions can make it very difficult for a manufacturer to get their drug covered as soon as it is approved. As such, patients may not have immediate access to potentially life-saving new medicines as insurers work to evaluate the drug's safety and efficacy once all information becomes available upon approval. This problem is even more pronounced if the insurer and manufacturer wish to establish a value-based contract; these contracts are much more complicated than standard contracts and require significantly more time to establish, meaning patients would have to wait even longer for the drug to be available.

The 21st Century Cures Act, passed in 2016, expands the scope of information and the audience with which manufacturers may share such information prior to a drug's approval or regarding off-label use,[28] but so far the FDA has only published draft guidance on this topic.[29]

During the investigational stage of the drug's approval process, manufacturers are allowed to share scientific information about the drug, including product information such as drug class or device design; and information about the indication sought, such as the endpoint and patient population being studied in the clinical trial. Other permitted information include factual presentations of results from clinical or preclinical studies, (though there should be no conclusions presented or characterizations made about what the results imply); when the company expects to receive approval; pricing information; strategies the company will employ to market the product; and information pertaining to product-related programs or services, such as patient support programs.[30] As a result of this new legislation, this information may now also be shared with payors. Manufacturers must clearly state that the product is still under review and its safety and efficacy has not yet been established.

Regarding communications for off-label use, Section 2101 of the 21st Century Cures Act loosens the language regarding the dissemination of health care economic information (HCEI) such that HCEI communications may merely be "related" to an approved indication, rather than "directly related," but this does not extend as far as unapproved indications.[31] FDA's most recent comprehensive guidance documents on this subject are from 2009[32], 2011[33], and 2014.[34] Congressman Morgan Griffith (R-WV) has introduced legislation, H.R. 1703, to further clarify and expand the ability of manufacturers to disseminate scientific information regarding off-label use of drugs or medical devices.[35] This legislation was being considered for inclusion in the FDA's Reauthorization Act currently being considered by Congress but was ultimately excluded following opposition from Democratic policymakers.

While the guidance issued thus far is helpful to the industry, agency guidance does not carry the same authority or certainty as law or formal regulation, and additional authority from Congress is still needed. The stakes for noncompliance are high, and the industry is concerned that current regulations remain too ambiguous. A violation of the promotional rules can result in a drug's application being terminated; all work up to that point would essentially be lost, and the manufacturer would have to start the clinical trials anew.[36]

Other Challenges

Besides these potential legal impediments, there are other challenges to establishing these types of contracts. Primarily, tracking the outcomes of patients over long periods of time, and ensuring those outcomes are causally related to the drug they were prescribed. Patients needing expensive drugs are very likely dealing with multiple conditions and/or have a condition that is severe enough to warrant a more comprehensive treatment plan than just a prescription drug regimen.

A significant amount of data-sharing between insurers, providers, and drug manufacturers will be required—particularly regarding the tracking and sharing of outcome measures—and the industry continues to struggle with EHR interoperability. Patient churn between insurers and providers further exacerbates this challenge, which is why these contracts appear best suited to medicines likely to produce results in less than a year.

Conclusion

Value-based contracts for pharmaceuticals will be very important in the effort to control health care costs in the years to come. CMS and the FDA can assist in allowing more wide-spread adoption of such contracts by providing further clarity on the Medicaid "best price" policy, the Anti-Kickback Statute and Stark Laws, and regulations surrounding information sharing for investigational drugs. Congress should continue working towards bi-partisan approaches to expand safe harbors for the communication of truthful, non-misleading, scientific information regarding off-label use of medical products. Increased interoperability of electronic medical record systems and further consensus on and development of reliable, scientifically-based outcome metrics will also greatly ease the challenges of developing such contracts. Swift action on these fronts is critical to reining in health care costs and improving patient outcomes.

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