ISSUE BRIEF
SEPTEMBER 2017

Outcomes-Based Pharmaceutical Contracts: An Answer to High U.S. Drug Spending?

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ABSTRACT

ISSUE: Brand-name prescription drug prices are increasing in the United States, putting pressure on payers and patients. Some manufacturers have responded by offering outcomes-based contracts, in which rebate levels are tied to a specified outcome in the target population.

GOAL: To assess the expected benefits and limitations of outcomes-based pharmaceutical contracts in the U.S., including their potential impact on prescription drug spending.

METHODS: Semistructured interviews with payers, manufacturers, and policy experts.

KEY FINDINGS: Pharmaceutical manufacturers and some private payers are increasingly interested in outcomes-based contracts for high-cost brand-name drugs. But the power of these contracts to curb spending is questionable, largely because their applicability is restricted to a small subset of drugs and meaningful metrics to evaluate their impact are limited. There is no evidence that these contracts have resulted in less spending or better quality.

CONCLUSIONS: Outcomes-based contracts are intended to shift pharmaceutical spending toward more effective drugs, but their impact is unclear. Voluntary testing and rigorous evaluation of such contracts in the Medicare and Medicaid programs could increase understanding of this new model.

KEY TAKEAWAYS

- In response to high U.S. prescription drug prices, some pharmaceutical manufacturers and private payers have shown interest in outcomes-based contracts, which tie rebates and discounts for expensive drugs to their effectiveness in treating the patients who receive them.
- Outcomes-based contracting can potentially prevent payers from wasting resources on medications that are not as effective outside clinical trials.
- The impact of these contracts on quality of care or costs is unclear, as current applicability is restricted to a small subset of drugs.

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BACKGROUND

In an era of rising health care spending and constrained budgets, U.S. policymakers and payers have tried to shift providers' financial incentives from those that pay for greater volume of care to those that pay for high-value care. This move from volume to value is in its early stages, with most payment still based on old fee-for-service models.[1]

However, fueling the move to value-based purchasing are provisions in legislation such as the Affordable Care Act of 2010 and the Medicare Access and CHIP Reauthorization Act of 2015. These provisions encourage providers to participate in risk-bearing arrangements and institute programs that base Medicare reimbursement on patient clinical outcomes measures, such as hospital readmission rates. Private payers have initiated similar performance-based incentive programs and risk-sharing arrangements for hospitals and physicians.

In this vein, some pharmaceutical manufacturers and private payers are seeking to apply an outcomes-based pricing model to the prescription drug market. Prices for brand-name drugs have risen far above increases in the consumer price index.[2] According to a Kaiser Health Tracking Poll, 77 percent of Americans consider prescription drug costs to be unreasonable.[3] Prescription drug spending is mostly driven by high-price, brand-name drugs, which account for about 12 percent of prescriptions but 72 percent of total drug spending.[4]

One response to these high prices has been increased interest in outcomes-based contracts, which tie rebates and discounts for expensive pharmaceutical products to the outcomes observed in the patients who receive them. Outcomes-based contracts are touted as possible ways for purchasers — such as insurers and health care systems — to improve value. That is because under such contracts, purchasers pay more for a drug when it works and less when it does not. However, whether such arrangements can achieve this goal remains controversial.

To gain insight into the benefits and limitations of outcomes-based pharmaceutical contracts, we interviewed pharmaceutical economics experts and individuals involved in developing these contracts, including those affiliated with pharmaceutical benefits managers and health plans. In this issue brief, we review the main themes that emerged from these data and evaluate whether these arrangements can help improve the value of pharmaceutical spending.

OUTCOMES-BASED PHARMACEUTICAL CONTRACTS

Under an outcomes-based contract between a pharmaceutical manufacturer and a payer, reimbursement for a drug is based in part on observed outcomes of the drug's use in a patient population. This model creates the functional equivalent of a tiered pricing or rebate structure; that is, instead of the payer covering all prescriptions at a single price, the initial price remains in place if a specified percentage of patients achieves the agreed-upon outcome. But if the outcome threshold is not met, the manufacturer refunds some of the original price to the payer. Under this arrangement, the purchaser is typically responsible for analyzing the data and determining if a performance outcome has been triggered.

A recent well-publicized example involved sacubitril/valsartan (Entresto), a drug that was introduced in 2015 to treat congestive heart failure. One key clinical trial showed a 20 percent relative risk reduction in death or hospitalization (21.8% vs. 26.5% in the comparison group). In 2016, Novartis disclosed that it had established separate deals with multiple private insurers — such as Harvard Pilgrim, Cigna, and Aetna — to provide additional rebates if a higher level of hospitalizations occurred.[5] In return, sacubitril/valsartan was given preferred formulary status, meaning that patients were responsible for lower copayments and overall prescribing of the drug would be expected to rise.

Another example concerns evolocumab (Repatha), a low-density lipoprotein (LDL) cholesterol-lowering drug with a list price of over $14,000 per year. The manufacturer, Amgen, reported contracts in 2016 and 2017 with Harvard Pilgrim, Cigna, and other payers, in which up-front discounts were given with the promise of additional rebates if patients did not experience reductions in LDL

cholesterol comparable to those observed in the drug's preapproval clinical trials. These contracts helped ensure that the insurer would cover the drug and, in some cases, give it preferential formulary status, despite the existence of many lower-cost cholesterol-lowering products (such as generic statins and ezetimibe (Zetia)).

One such contract also included a total spending threshold to protect the payer and a provision that conditioned additional rebates on patient adherence rates. Another contract with Harvard Pilgrim provided a full refund if the patients had a heart attack or stroke, although clinical studies suggest that less than 5 percent of patients would be expected to experience this outcome.[6]

Table 1 shows four examples of outcomes-based contracts in the United States, including information on the outcome metric chosen in each case. Manufacturers and payers have engaged in outcomes-based pharmaceutical contracts for numerous other high-cost, brand-name drugs that treat heart disease, diabetes, osteoporosis, multiple sclerosis, rheumatoid arthritis, hepatitis C, and cancer; in total, we identified more than 25 publicly announced outcomes-based contracts in the U.S. Other contracts have been kept confidential, to be used strategically by manufacturers to gain preferential formulary placement over competitors.

Table 1. Examples of Publicly Disclosed Outcomes-Based Pharmaceutical Contracts in the U.S.

Drug	Manufacturer	Payer(s)	Disease area	Outcome metric and terms	Date
Entresto/sacubitril, valsartan	Novartis	Aetna	Congestive heart failure	Additional rebate given if the drug does not achieve the heart failure admissions reductions it achieved in clinical trials.	Spring 2016
Repatha/evolocumab	Amgen	Harvard Pilgrim	Hypercholesterolemia	Upfront discounts and future rebates given based on meeting specific cholesterol targets, total spending threshold, and adherence in exchange for preferred formulary placement. Full refund if patient has a heart attack or stroke.	Spring 2016 Spring 2017
Rebif/interferon beta-1a	Merck KGaA	Prime Therapeutics	Multiple sclerosis	Rebates given if patients on the drug have total costs to their plans higher than patients on a different MS drug, or if the medication adherence rate reaches a specified level.	March 2011
Januvia and Janumet/ sitagliptin/metformin	Merck & Co.	Cigna	Diabetes	Rebates given if a specified A1c blood sugar level is not met in the patient population. The agreement is also contingent on good adherence.	April 2009

Data: Authors' analysis.

Benefits of Outcomes-Based Contracts

A benefit of outcomes-based contracting is the potential to prevent payers from wasting resources on expensive drugs that are not as effective outside of clinical trials (Table 2). The Food and Drug Administration can approve expensive new drugs based on clinical trials in highly selected populations of participants or without active comparators. These results may not translate to the "real world" population. Many such drugs have been widely prescribed without clear evidence of effectiveness because of their heavy promotion by pharmaceutical manufacturers to physicians and patients.

Outcomes-based contracts have obvious appeal to pharmaceutical manufacturers and purchasers. In response to cost pressures, a growing number of private payers have tried to reduce drug prices by adopting more restrictive formularies that exclude products. Outcomes-based contracts can provide an alternative option to closed formularies, in which drugs can remain on the formularies in exchange for outcomes guarantees (Table 2). In these cases, manufacturers retain sales volume and purchasers share the risk of low-value spending with manufacturers. This arrangement also can result in increased coverage of medicines for patients as well as excellent public relations for manufacturers and purchasers, showing their willingness to adopt value-based payment for prescription drugs.

Limitations of Outcomes-Based Contracts

Our interviews suggest outcomes-based contracts will not apply to most drugs in the near term because of outcomes measurement limitations (Table 2). The outcomes available for these contracts generally can include only those that can be measured using claims data. This excludes many clinical outcomes, such as changes in symptom control. In most cases, electronic health records remain difficult to access and convert into an analyzable form for this purpose. Even laboratory values may not be available in many claims databases.

In the case of evolocumab, for example, using LDL cholesterol as an outcome metric required that payers retrieve lab data streams from electronic health records, a potentially costly and labor-intensive administrative requirement. Data limitations not only limit the scope of drugs to which outcomes-based contracts would apply but also the meaningfulness of the outcomes that could be the basis for the rebate.

In addition, these contracts may not deliver optimal value, because the potentially actionable outcomes are restricted to those that can be measured in the short term. Instead, the metrics used in these contracts are typically so-called surrogate measures, such as changes in laboratory values or other easy-to-obtain results that may not closely or directly correlate with actual clinical outcomes that are more central to patient health.

For example, a low hemoglobin A1c level is a common measure of diabetes control, but many drugs that slightly reduce this number lack evidence of association with improvements in adverse outcomes from diabetes (like kidney damage) or reductions in diabetes-related cardiovascular death. However, since hemoglobin A1c can be easily measured and may change over a few months, it could be chosen as the basis for a contract. In the case of oncology, clinical trials often use surrogate measures such as tumor shrinkage, but this is not necessarily indicative of more meaningful outcomes, such as cancer survival rates.

Even when outcomes-based contracts are executed, they ultimately may not control pharmaceutical spending. Some payers assert that they have been able to negotiate meaningful rebate differences in outcomes-based contracts, thus offering the potential for real savings. Other payers, however, point out that the rebates they would receive for unmet outcomes would not be enough to offset the increase in costs associated with collecting and analyzing the data (as well as the drug's predicted broader use) (Table 2). Since brand-name pharmaceutical manufacturers set the price of their drugs under monopolistic market conditions, they also can raise a drug's initial price to account for the possibility of an outcomes-based rebate.

Table 2. Benefits and Drawbacks of Outcomes-Based Contracts

Benefits	Drawbacks
Contracts could prevent payers from paying for expensive drugs that are not as effective outside of clinical trials.	Contracts would not apply to most drugs because of outcomes measurement limitations: • Measurement limited to outcomes reported in claims data, excluding such clinical outcomes as changes in symptom control, among others. • Electronic health records remain difficult to access and convert into an analyzable form for this purpose. • Metrics used in these contracts are typically surrogate measures that may not correlate with actual clinical outcomes more central to patients' health.
Contracts could provide an alternative to closed formularies, allowing a drug to remain on a formulary in exchange for outcomes guarantees: • Manufacturer retains sales volume. • Payer and manufacturer share financial risk if drug does not meet target outcome.	Rebates payers would receive for unmet outcomes may not be enough to offset costs associated with data collection and analysis.
Contracts could provide good public relations for manufacturer and payer seen to make decisions based on clinical evidence.	Because manufacturers' rebates are calculated after the prescription is filled, contracts do not affect patient out-of-pocket costs, which occur at point of sale.

Regardless of rebate levels, the contracts do not affect patient out-of-pocket costs, and it is difficult to determine whether they ultimately result in lower premiums. Patient costs occur at the point of sale, while the contracts provide retrospective rebates from manufacturers to the payers that might be calculated months (or years) after the prescription is filled. In addition, the contracts would not be able to help patients who decided not to fill needed medications because they could not afford out-of-pocket costs. As outcomes-based contracts evolve, it will be important to consider potential ways to allow patients to share in up-front savings.

DISCUSSION: ARE OUTCOMES-BASED CONTRACTS A SOLUTION TO HIGH DRUG PRICES?

Outcomes-based pharmaceutical contracts have the potential to steer spending toward more effective treatments, but they are not likely to lower spending on a broad scale, if at all, because of practical limits on their applicability and meaningful metrics. The contracts apply only to a limited subset of drugs and, in many cases, are tied to surrogate measures that do not directly reflect patient health outcomes. Even if these logistics are addressed, there is no evidence to date that the rebates will result in lower drug prices, in part because the prospect of rebates may simply be factored into the prerebate price. Little information is publicly available on which to assess the model's impact, in part because these contracts have been primarily limited to the private insurance market.

A current point of debate is whether Medicare or Medicaid should consider outcomes-based pharmaceutical contracts. The best way to determine whether the approach can improve value is to test these arrangements through the Center for Medicare and Medicaid Innovation. However, recently proposed regulations to test new pricing mechanisms under Medicare Part B, which could have included outcomes-based contracts, resulted in political backlash led by the pharmaceutical industry and were ultimately scrapped. It is therefore important to consider the political challenges that will inevitably arise from experiments with new forms of pharmaceutical reimbursement.

The decision to test outcomes-based contracts under Medicare Part D needs to be made independent of other potential changes in pharmaceutical reimbursement, such as direct pricing reform. Contracts between manufacturers and health plans could include private plans administering Medicaid and Medicare. Price and health outcomes data would need to be kept confidential but rigorously and independently analyzed. Most important, any outcomes-based contracting pilot would need to be voluntary until more public information on its implications is available.

The high costs of new brand-name drugs are squeezing the budgets of public and private payers as well as patients, often without clear additional health benefits. Outcomes-based pharmaceutical contracts have been a source of increased interest, but many questions remain regarding their power to improve quality or produce real savings. Testing outcomes-based contracts in Medicare and Medicaid populations could act as an important first step in determining whether the contracts primarily function as public relations strategies or represent true improvements in the value of pharmaceutical care.

HOW THIS STUDY WAS CONDUCTED

We interviewed six pharmaceutical economics experts and individuals involved in developing these contracts at nine pharmaceutical manufacturers and eight payers, including both pharmaceutical benefits managers and health plans. The interviews covered a range of topics, including motivation, administration, and experiences with existing contracts. We also conducted a literature review, including journal articles, pharmaceutical news sources, and companies' press releases.

NOTES

1 S. H. Zuvekas and J. W. Cohen, "Fee-for-Service, While Much Maligned, Remains the Dominant Payment Method for Physician Visits," *Health Affairs,* March 2016 35(3):411–14.

2 Quintiles IMS, "IMS Health Study: U.S. Drug Spending Growth Reaches 8.5 Percent in 2015," Press release (Quintiles IMS, April 14, 2016).

3 A. Kirzinger, B. Wu, and M. Brodie, *Kaiser Health Tracking Poll: September 2016* (Henry J. Kaiser Family Foundation, Sept. 2016).

4 A. S. Kesselheim, J. Avorn, and A. Sarpatwari, "The High Cost of Prescription Drugs in the United States: Origins and Prospects for Reform," *Journal of the American Medical Association,* Aug. 23–30, 2016 316(8):858–71.

5 T. Walker, "Novartis Signs on to Value-Based Pricing for Entresto," *Managed Healthcare Executive,* May 4, 2016.

6 E. Sagonowsky, "Amgen Puts Repatha Outcomes Data to Work in Refund Deal with Harvard Pilgrim," *FiercePharma,* May 3, 2017; and A. Kaltenboeck and P. B. Bach, "Outcomes-Based Drug Contracts Do Not Move Us Closer to Value," *Morning Consult,* June 21, 2017.

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Editorial support was provided by Hannah Fein.

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