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Themed Section: Value-Based Contracting

Pharmaceutical Products and Their Value: Lessons Learned and the Path Ahead



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ABSTRACT

Steep increases in prices and spending on prescription drugs in the United States have triggered public outrage and questions over their value. Value-based pricing has emerged as a preferred alternative to prices determined by what the market will bear. In response, manufacturers and health plans have begun to publicize their efforts to engage in outcomes-based contracts and long-term financing agreements, which they describe as value-based. Nevertheless, both contracting approaches perpetuate existing distortions in the financial incentives of supply chain and prescribing intermediaries, and fail to realign the prices of drugs to their value to patients, the healthcare system, or society. This commentary describes the challenges of managing drugs according to their value, and describes several alternatives that promise greater impact than contracting strategies.

Keywords: innovative contracting, long-term financing, outcomes-based contracting, pharmaceutical products, prescription drugs, value-based contracting, value-based pricing.

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Overview: High Prices as Incentives for Innovation

Spending on prescription drugs has increased at rates that exceed nearly all other areas of healthcare over the past 3 decades. After adjusting for inflation, prescription drug spending per capita has risen by an average of 6% annually since 1982[1]; overall healthcare spending over the same time frame grew at a more moderate rate of 3.5% per year.[2] Responding to public outcry, lawmakers, policymakers, and the industry have put forward numerous proposals to address a growing lack of affordability.[3] Among them is to pay for drugs based on their value, in much the same way that performance-based contracting is being used for medical services. Branded specialty drugs make for a particularly appealing target because they account for 49.5% of prescription drug spending while constituting only 2.2% of prescriptions.[4]

Nevertheless, value-based management may be particularly challenging for pharmaceutical products. Under current law, manufacturers are awarded a period of exclusive rights to market new products on the premise that this stimulates investment in the commercial development of new therapies. The market for these treatments is characterized by the large number of stakeholders who benefit financially when prices are higher, regardless of a drug's value.[5] For example, outpatient offices and clinics are typically paid a percentage markup over the price of a drug, making higher-priced treatments more financially attractive than lower-priced alternatives. Similar distortions affect prescription drugs dispensed by pharmacies. Health plans and pharmacy benefits managers, which determine how easily a patient can obtain one drug over its competitors, offer patient access in exchange for rebates and discounts. Rebates for insulin, for example, routinely exceed 40% of gross sales.[6] Higher list prices allow manufacturers to offer more generous net price concessions while still maintaining their revenues. Nevertheless, this comes at the expense of patients, whose out-of-pocket payments are indexed on list prices.

The undesirable consequences of manufacturer pricing practices extend to drugs with limited competition, in particular when payers are required to cover certain types of treatment.[7] For example, some drugs are under statutory or regulatory protections that restrict the ability of Medicare and other health plans to limit coverage. Such is the case for oncology drugs, which, as a protected class under Centers for Medicare & Medicaid Services rules, enjoy required coverage and reportedly offer minimal net price reductions to payers.[8] This leaves health plans with few tools other than prior authorization, step therapy, and quantity limits to contain costs, which restrict access to these treatments.

Because manufacturers optimize their revenues by choosing higher rather than lower prices, they often fail to correspond to

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the benefits of treatment as they would in a market with clear signals of consumer preferences. In this pricing environment, research and development has become increasingly focused on drugs that are less likely to be subject to payer negotiations. For example, although cardiovascular disease remains the leading cause of death in the United States,[9] approximately 5% of clinical development projects focus on this therapeutic area. Cancers, the second leading cause of mortality and widely protected by federal and state mandates, garner 33%.[10] Even as treatments for rare diseases and cancers lead in recent US Food and Drug Administration approvals,[11] their value has been called into doubt. For example, after adjusting for inflation and survival improvements, the cost of an additional year of life gained from newly approved oncology treatments increased at a rate of $8500 per year between 1995 and 2013.[12]

Value-Based Pricing: Theory Versus Practice

Value-based pricing was proposed as a response to the market's failure to establish prices that reflect the benefits of treatment.[13] The central premise of value-based pricing is that the monetary value of a treatment's benefits can be characterized by using rigorous analytic methods, such as those employed by the Institute for Clinical and Economic Review and by health technology agencies in other countries. Payers can use this information to negotiate to or below the ceiling established by this estimate, in exchange for removing financially motivated restrictions and encouraging use of high-value treatments. This trade-off would allow more patients to benefit from treatment, generating sales that would otherwise have been foregone and creating a tighter link between manufacturers' economic opportunities and unmet needs.

As calls for value-based pricing have increased in step with drug spending, manufacturers and health plans have begun to tout contracts they claim to be value based. These assertions typically refer to agreements between payers and manufacturers that modify traditional rebate payments to share or smooth out the financial risk associated with uncertain outcomes and high prices of branded specialty drugs. Most such contracts are outcomes based, promising rebates to payers when patients do not experience the measures of success outlined in the agreement,[14] which are typically some form of treatment outcome, disease milestone, or adherence to treatment. A commonly cited example of this is a contract between Harvard Pilgrim and Amgen that promised a full refund for costs of treatment with evolocumab (Repatha) for patients who initiate the drug and subsequently suffer a heart attack or stroke.[15]

Another approach often is long-term financing or *mortgage pricing* agreements to pay for high-priced 1-time treatments in installments over a predetermined period. This approach is frequently proposed as a solution for the high prices of the first generation of cell and gene therapies. For example, Spark Therapeutics announced that it was seeking agreement from the Centers for Medicare & Medicaid Services for a proposal that would allow the company to offer a 30-month contract installment plan for the $850 000 price of its novel gene therapy for an inherited form of retinal dystrophy, voretigene neparvovec-rzyl (Luxturna).[16] More recently, Novartis created a payment program that would allow health plans to pay $425 000 per year over 5 years for onasemnogene abeparvovec-xioi (Zolgensma), which otherwise commands a lump sum of $2.125 million.[17] Both examples also combine long-term financing with outcomes-based contracting, making payment of later installments contingent on achieving outcomes over longer time frames than demonstrated in clinical trials.

Despite frequent media attention, the total number of publicly known outcomes-based or long-term financing contracts remains small relative to more traditional agreements. One report identified only 49 publicly known instances of nontraditional contracts in the United States between 1990 and 2017,[18] although others have most likely remained confidential, and the total number has increased after a recent spate of announcements. The Pharmaceutical Research and Manufacturers of America, an industry trade group, documents 34 publicly announced contracts since 2017.[19] Representatives of the pharmaceutical and health insurance industries argue that several federal policies designed to mitigate the financial exposure of taxpayers to expanding spending and increased use of high-cost drugs prevent more impactful execution of value-based contracting strategies. Prominent among them is the provision of best price to Medicaid programs, which ensures that they benefit from the lowest price in the US commercial market. Proponents of waiving the best price policy contend that it prevents manufacturers from taking on more risk because offering rebates for the full price of treatment would require them to provide them to Medicaid programs at no cost. Many also push for explicit guidance allowing off-label terms in outcomes-based contracts, on the argument that relying only on endpoints included in a drug's label limits their ability to share risk over the long term, and for outcomes not evaluated during clinical trials.

Nevertheless, legal analysis and real-world experience suggest that these barriers are overstated and can be overcome with practical solutions.[20,21] Best price can be reported as a blended net price over the entirety of a contract rather than simply the floor that would occur in the case of a full refund, and several known examples exist of contracts using off-label terms, including the contract between Amgen and Harvard Pilgrim for evolocumab. Without more compelling reasons, it is difficult to justify waivers of best price and off-label marketing restrictions, especially in light of the raised risk of inappropriate and off-label use and higher Medicaid expenditures.

Experiences to date also suggest that outcomes-based contracts and long-term financing deals hold insufficient promise of achieving meaningful risk-sharing or value-based prices to revisit these protections. Although the confidential nature of contracts between payers and manufacturers limits insight on net prices, in instances when information is publicly available to estimate them, they do not align with value. For example, the contract that committed Amgen to a full refund for the price of treatment with Repatha to Harvard Pilgrim was contingent on patients having a heart attack or stroke. Results from the Further Cardiovascular Outcomes Research With PCSK9 Inhibition in Subjects With Elevated Risk clinical trial,[22] which were announced the same month, showed that the risk of heart attack or stroke in the indicated population was less than 4%, leaving the net price far short of the 85% to 88% discount that would have been necessary to achieve the value-based price identified by the Institute for Clinical and Economic Review in its review of PCSK9 inhibitors.[23] Lessons from other countries also call into question whether this approach meets performance goals under even the most favorable conditions. Italy, which implemented outcomes-based contracts in a systematic and deliberate effort over several years, achieved estimated savings of approximately 3% of the total costs of the pharmaceutical products used. In response, Italy's national health system has begun to issue payment for certain drugs only after treatment response has been confirmed, rather than paying at the outset and receiving clawbacks later.[24]

In addition to their limited prospects for arriving at a value-based price, outcomes-based and long-term financing contracts require resources to implement and can be difficult to scale across a health plan's various books of business. Monitoring patients'

outcomes is resource-intensive, requiring verification and adjudication of outcomes and movement of beneficiaries between different health plans. The terms of each program must also be tailored to payer type. For example, Medicaid programs are prevented from excluding the drugs of manufacturers participating in the Medicaid Drug Rebate Program. A health plan working to exclude lower-value competitors in favor of a high-value product for is commercial customers could not easily adapt this initiative for its Medicaid contracts.

A Path Toward Value

In practice, outcomes-based and long-term financing contracts appear similar to their conventional predecessors, which trade preferred terms of access for net price concessions and reduced spending variation. Neither solves the problem of financial incentives that travel with price rather than value. Outcomes-based contracts hide the net price of the treatment, obscuring whether the drug performed to any available value-based price benchmark, and fail to make drugs more affordable to patients. Long-term financing presents another concern in that it establishes a pathway for health plans to offload financial liability after the first payment, passing subsequent payments through to beneficiaries in the form of rising premiums.

More direct approaches exist to promote value while balancing access, affordability, and innovation. One example is a competitive acquisition program (CAP).[25] Under this approach, a third-party vendor contracts with a payer to negotiate with manufacturers for discounts on the price of their drugs and arranges for the discounted price to be extended directly to the payer, without markup through the healthcare providers. Medicare used this approach for 2 years before ending the program in 2008 after it failed to produce savings and enroll enough providers. Nevertheless, the idea has its merits in that it reduces inflationary financial incentives and has the potential to reinvigorate price competition among drugs. Coupled with modifications to address its initial deficiencies and tailored toward value-based management, CAP offers policymakers tools to reduce inflationary pressure on prices, which contracting strategies do not. In 2017, MedPac proposed a restructured version of CAP in which third-party providers would order the product at lower prices negotiated through third-party vendors acting on Medicare's behalf, which they would pass through to Medicare without markup. In exchange for their participation, Medicare would share savings with participating providers while phasing out the inflationary markup on reimbursement.[26]

To some extent, payers can also act unilaterally. Value-based insurance design, in which copayments are reduced or eliminated for high-value treatments, can be used to encourage patients to make choices based on value.[27] Health plans can also reward prescription drugs priced at value by placing them on lower tiers and reducing copay requirements or decline coverage for drugs priced above their value. Although Medicare and Medicaid programs are in most cases legally bound to cover such drugs,[7] health plans can and have taken steps to restrict access or exclude them from their formularies. CVS took this approach in 2018 when it announced that drugs launched at more than $100 000 per quality-adjusted life year would not be covered, with exceptions for drugs with breakthrough designation.[28] New York State's Medicaid program, although unable to refuse coverage outright, has established a review board that evaluates high-cost treatments against their value and can recommend supplemental rebates or coverage restrictions.[29]

These solutions share in common that they break with existing relationships and transactions that perpetuate price distortions. The experience with outcomes-based contracts and long-term financing suggests the opposite, in large part because they rely on well-established intermediaries whose financial well-being improves with higher drug prices. Value-based management and pricing of specialty drugs require that the supply chain and prescriber incentives be realigned to travel with value rather than price.

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