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POLICY INSIGHT

# Paying For Value From Costly Medical Technologies: A Framework For Applying Value-Based Payment Reforms

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**ABSTRACT** **Innovative medical products offer significant and potentially transformative impacts on health, but they create concerns about rising spending and whether this rise is translating into higher value. The result is increasing pressure to pay for therapies in a way that is tied to their value to stakeholders through improving outcomes, reducing disease complications, and addressing concerns about affordability. Policy responses include the growing application of health technology assessments based on available evidence to determine unit prices, as well as alternatives to volume-based payment that adjust product payments based on predictors or measures of value. Building on existing frameworks for value-based payment for health care providers, we developed an analogous framework for medical products, including drugs, devices, and diagnostic tools. We illustrate each of these types of alternative payment mechanisms and describe the conditions under which each may be useful. We discuss how the use of this framework can help track reforms, improve evidence, and advance policy analysis involving medical product payment.**

With more biomedical advances and accelerated regulatory processes, new medical products are poised to substantially disrupt current treatment models,[1] with the potential to improve the lives of millions of patients. These new products include pharmaceuticals that delay or halt major disease complications, transformational therapies that correct cellular and genetic defects, medical devices that can enable patients' daily functioning and experience, and advanced diagnostic tests and tools that support more personalized and effective care decisions. At the same time, out-of-pocket[2] and total spending on pharmaceuticals and other products continue to rise, and their growth may intensify in the years ahead.[3–6] The result is ongoing, intense public debate about how to improve access to these products and health care affordability, while also supporting continued innovation.

A wide range of value-based payment initiatives are being implemented to address these challenges.[7] A major area of activity is summarizing existing evidence on the value of a drug—generally defined as its impact on outcomes, patient experience, and the total cost of care—through health technology assessment. Payment models based on these assessments set the per unit price based on the available evidence. Specific methods such as those employed by the Institute for Clinical and Economic Review, a nonprofit cost-effectiveness evaluator in the United States,[8] are increasingly cited in payment

negotiations between payers and manufacturers, based on the research and frameworks for describing important aspects of value developed by expert groups such as the International Society for Pharmacoeconomics and Outcomes Research.[9]

These reforms have generally been applied to negotiations about the unit prices of medical products,[10] with discounts or rebates driven by volume and not value. At the same time, value-based reforms for health care providers increasingly aim to generate more value for covered populations by shifting away from payments based only or mainly on the volume of services. These provider payment reforms attempt to encourage the use of appropriate high-value combinations of products and services, such as clinical pathway models (which tie payments to following practice guidelines) and Medicare's Oncology Care Model (which ties payment to reducing overall costs, including physician-administered drug spending, while improving performance on measures of quality or outcomes).[11–13] The provider value-based payment models aim to influence the use of drugs and devices, but they generally do not shift payment for medical products away from fee-for-service.

Alternatives to strictly volume-based payment for drugs and devices have been implemented on a relatively modest basis (such as rebates with some links to quality or utilization measures), with limited consequences for actual payment for the product.[14,15] However, there is increasing activity among payers and manufacturers in designing and implementing such models. As additional costly medical technologies reach the market with potentially large, long-term benefits in preventing disease complications or the diseases themselves; as data systems improve; and as provider payments continue to shift toward value, more opportunities are emerging for manufacturers to participate in value-based payment arrangements for potentially valuable new medical treatments that increase patient access and improve outcomes and evidence, while sustaining transformative innovation.[16–19]

For example, indication-specific pricing methods set per unit prices by clinical indication, if there is evidence that certain clinical subpopulations differ substantially in expected value. Outcomes-based payment methods adjust payment for a treatment based on observed outcomes over time. Subscription payments provide access for a covered population based on a per member per month subscription fee, which may also be adjusted based on the outcomes observed in the population.

While supporters of these reforms are motivated by the same goal of getting more "bang for the buck" for medical technology spending, simply lumping all reform models into the category of value-based payment can create misunderstandings about exactly what reforms are being considered, why they may or may not be helpful, and whether they are achieving their desired effects. The absence of a clear framework for value-based payment reforms for medical products leads to missed opportunities to clearly define these models; increase their impact; and foster consistent analysis and assessment of how they can be used most effectively, either alone or in combination.

Consequently, we present a framework for describing value-based payment for medical products and illustrate its application using recent examples. It builds on the Health Care Payment Learning and Action Network's framework for categorizing alternatives to fee-for-service payment for providers.[20] As is the case with the Learning and Action Network categories, the intent is to describe how different kinds of value-based payment could increase the value of care involving medical products. Our framework accommodates a variety of definitions and measures of value in particular contexts, including the definitions of *value* used in health technology assessments like those produced by the Institute for Clinical and Economic Review and other health technology assessments. Like the Learning and Action Network value-based payment framework for providers, our framework for medical products describes the extent to which payment shifts away from volume, and it accommodates performance measures and supports varying levels of sophistication.

To facilitate the use of the framework, we describe when payment models in each category are likely to be effective in creating value, reflecting prior reviews and recommendations for many kinds of value-based payment arrangements.[21] Value-based payment models for medical products can create more value not only through prices that better reflect value, but also through supporting better evidence, more appropriate use, more valuable product refinements, and better mechanisms for promoting patient access—all of which lead to more value through better patient outcomes and fewer costly disease complications. The framework can facilitate analyses of how much additional value is created and how the risks and rewards related to high-value performance can be shared among payers, patients, providers, and purchasers. In addition, the framework describes how value-based payment models can support the development of better evidence that will affect future negotiations about both price levels and payment methods. The examples used to illustrate

the framework underscore the urgent need for a consistent value-based payment framework for medical products and the best opportunities for adding value from alternatives to fee-for-service payment.

## The Spectrum Of Value-Based Payment Arrangements For Medical Products

Exhibit 1 summarizes our framework of value-based payment arrangements for medical products. As described more fully below, category 1 refers to the traditional fee-for-service approach to medical product reimbursement: Product revenue is based on sales volume and volume-based rebates or discounts, and health technology assessments may be used to determine if the average price is value based. Category 2 is also fee-for-service payment, but with value-based price adjustments that are set prospectively, based on factors besides volume that are expected to influence how well the product is expected to work. Category 2 includes indication-specific pricing, in which unit prices vary based on existing evidence of significant differences in expected value across subpopulations. It also includes "coverage with evidence" models, in which payment occurs along with the collection and reporting of outcomes or other clinical data—which are viewed as valuable for resolving important evidence questions to improve future use of the products. In "adaptive" payment models, such evidence could lead to future price increases or reductions.

Payments in categories 1 and 2 are clearly not independent of value. Rather, when health technology assessments such as those performed by the Institute for Clinical and Economic Review or the National Institute for Health and Care Excellence in the UK are used to guide negotiations or set prices, the volume-based payment is intended to reflect the existing evidence on value. The price per unit is determined by expected performance based on prior evidence about the value of the product, regardless of actual performance in the populations using it.

Similar to Alternative Payment Models for providers, categories 3 and 4 represent payments that are at least partially delinked from volume and tied to *observed* performance associated with the use of the medical product in a covered population. Payment models in these categories are based, at least partially, on performance measured against specific benchmarks of effectiveness (such as length of patient adherence to a drug regimen or rates of hospitalization for a disease complication). Such benchmarks are intended to serve as a proxy for increased value realized in practice. Category 3 remains largely

**EXHIBIT 1**

**Framework of value-based payment arrangements for medical products**

| Volume-based price | | Alternative Payment Models | |
|---|---|---|---|
| **Category 1: volume based** | **Category 2: volume based with value adjustment** | **Category 3: volume based with link to outcomes** | **Category 4: population based** |
| Payment is based on sales volume less any volume-based rebates or discounts. Health technology assessments may be used to negotiate a base or average price that reflects expected value. | Payment is based on sales volume, with ex ante adjustment related to expected value. Category 2 includes indication-specific pricing, payment linked to data or evidence development, and adaptive licensing. There is no shared risk or tie to observed patient or population outcomes. | Payment is based on sales volume with an adjustment based on outcomes in treated patients or population.<br>**3A, Limited:** Outcome adjustment is based on a limited set of performance measures and affects a relatively small share of payment.<br>**3B, Substantial:** Outcome adjustment involves a substantial share of payments linked to meaningful performance measures. | Payment is partially or fully delinked from sales volume and linked to outcomes in the covered population.<br>**4A, Partially population based:** A significant share of the payment is population based (for example, per member per month plus volume-based payment) and linked to performance measures in the covered population.<br>**4B, Fully population based:** Population-based payment (for example, per member per month) is linked to performance measures in the covered population.<br>**4N, Population based, no linkage to outcomes:** The population-based payment has no adjustment for performance. |

**SOURCE** Authors' analysis. **NOTE** In all models, negotiated payments may be linked to prior evidence on value.

based on fee-for-service, but it has some share of its payment tied to results. Category 4 uses payments that are largely or fully per person or "population based," including a partially capitated payment or subscription payment for access to a product for a population.

**CATEGORY 1: VOLUME BASED** The price set for medical products may be influenced by evidence of value based on health technology assessments or other approaches, but revenue for particular uses of the product is determined by volume and volume discounts. In this typical approach, payers use formulary tiers that include preferred placement of brand-name treatments in conjunction with manufacturer discounts that are based on sales volume. Preferred placement on the formulary can be accompanied by prior authorization requirements and utilization reviews. The wide use of such fee-for-service payment models reflects their utility in many cases.

**CATEGORY 2: VOLUME BASED WITH VALUE ADJUSTMENT** Category 2 payments are also determined by volume. However, medical product prices are adjusted to reflect differences in expected value across patient groups or settings. Indication-specific pricing refers to the establishment of prices that differ across subpopulations of patients based on certain measurable characteristics (for example, people with a specific biomarker or form of a disease), where evidence shows that the group benefits more or less than does the average patient in the population. Both CVS and Express Scripts have explored indication-specific pricing tools for oncology.[22] Another type of ex ante payment refinement intended to improve value is an adjustment (up or down) for submitting additional data related to additional patient characteristics, quality, or outcomes not captured in claims. Such "quality reporting" adjustments are now used in many provider payment systems, including more than 90 percent of Medicare payments.[11] Similarly, in coverage with evidence development,[23] payment occurs in conjunction with participation in a postmarket study or registry of patients using the product. In an adaptive licensing model, so far used only outside the US,[24] the manufacturer accepts relatively low initial payment rates for new indications or a new technology for initial coverage, with the potential for a significantly higher payment rate when more robust evidence of value is developed.

**CATEGORY 3: VOLUME BASED WITH LINK TO OUTCOMES** Category 3 contracts are analogous to provider Alternative Payment Models with shared savings or shared risk, with most payments still determined by volume. Unlike payments in category 1 or 2, these fee-for-service payments include a component tied to actual results in the treated populations. Most outcomes-based contracts for drugs and devices implemented in the US are in category 3, with most of the payment still tied to volume-based rebates or discounts. Our recent survey of outcomes-based contracts found that volume rebates remained the main payment mechanism for approximately 80 percent of the reported manufacturer contracts and around 70 percent of reported health plan and pharmacy benefit management contracts.[14]

▶ **CATEGORY 3A, LIMITED:** In category 3A, payment is tied to a single performance measure or a few performance measures that are proxies for improved health status or cost outcomes, and only a small share of reimbursement is linked to these outcome measures. For example, patient adherence to a medication, as measured through prescription refills, could be used as a proxy for the medication's effectiveness. If the patient discontinued use before a benchmark time, the manufacturer would give the payer as a rebate a prespecified, limited portion of the total payment for the population. AstraZeneca's agreement with Express Scripts[25] allows a refund of the amount spent on Iressa if a patient stops treatment before the third prescription refill (a proxy for patient response). As another example, Amgen's agreement with Harvard Pilgrim ties rebate payments to Repatha's[26] ability to lower cholesterol (a surrogate for reducing the number of significant cardiovascular events) to the level observed in clinical trials. In addition, Novartis has a risk-sharing arrangement for payment for Entresto in some contracts,[27] based on whether the heart failure hospitalization rate of patients taking the medication exceeds or falls below a prespecified threshold derived from the drug's performance in clinical trials. These outcomes-based contracts affect a minority of the total payment for the treatment.

▶ **CATEGORY 3B, SUBSTANTIAL:** In category 3B, payments are still based on the volume of patients treated, but payments are more substantially linked to important measures of value. Such linkages could include improvements relative to benchmarks that reflect key patient-reported outcome measures (for example, functional status or pain), key clinical outcomes (such as hospitalization with disease complications), or a reduction in the total cost of care. A significant proportion of manufacturer payments would depend on these outcome measures. An example of the category 3B payment model is the arrangement for Luxturna between Spark Therapeutics and Harvard Pilgrim Health Care,[28] according to which Harvard Pilgrim makes an up-front payment for the drug but may receive substantial rebates if it does not have

a durable impact based on a patient's prespecified visual acuity measures over time. Another example is bluebird bio's proposed payment model for the company's forthcoming gene therapy for transfusion-dependent beta thalassemia, a genetic hemoglobin disorder. Following an initial charge for the treatment, subsequent payments would occur over time only to the extent that measures of effectiveness of the treatment (for example, avoiding the need for transfusions) are achieved. Substantial payments are at risk over both the short and long terms, relative to benchmarks on meaningful measures of performance.[29,30]

**CATEGORY 4: POPULATION BASED** Category 4 payments represent a more substantial shift away from fee-for-service and are analogous to population-based Alternative Payment Models for health care providers (that is, models that pay providers per member per month). These models aim to delink payment from volume by shifting to a partially or fully population-based payment. As in the Learning and Action Network payment framework for medical service providers, we included a subcategory called 4N for subscription models that pay for access to a treatment at the population level but do not directly tie these payments to outcomes.

▶ **CATEGORY 4A, PARTIALLY POPULATION BASED:** In category 4A, payment for the product is partially based on a capitation or monthly fee per person covered in the population and partially based on actual use. One proposed example[31,32] (not yet implemented substantially) involves payment for antimicrobials for resistant organisms that pose a public health threat. In addition to fee-for-service payments for each use of such an antimicrobial, a manufacturer might receive a market entry reward linked to providing an adequate supply of the treatment over time.

▶ **CATEGORY 4B, FULLY POPULATION BASED:** In category 4B, payment is a fixed amount for a covered population. The payment model would include unlimited access to the drug for covered populations, with accountability for improvements in performance measures, such as lower rates of resistant infection in the population and lower rates of costly disease complications relative to a benchmark level. Population-based access to drugs for hepatitis C could be an example of this model, if payment were adjusted for population-based results. This payment approach aims to achieve higher value through broad access, combined with lower costs associated with adverse clinical and functional outcomes. The model also aligns the incentives of manufacturers and providers that participate in payment models in which they are increasingly accountable for full population health and cost outcomes.

▶ **CATEGORY 4N, POPULATION BASED, NO LINKAGE TO OUTCOMES:** In category 4N, payments are similarly based primarily or entirely on per person or per population prices, with no adjustments for population-level results. Louisiana's approach to providing hepatitis C drugs for its Medicaid beneficiaries and institutionalized populations—a contract with a manufacturer for unlimited drug access along with a cap on total drug expenditures for the population—fits in this category.[33]

## When Are Value-Based Payment Arrangements For Medical Products Likely To Have A Substantial Impact On Value?

Online appendix exhibit 1 summarizes key considerations for when different value-based payment arrangements are likely to be worthwhile.[34]

Fee-for-service payments with volume discounts (category 1) are appropriate when evidence on the product's risks, benefits, and costs is reasonably well understood and does not differ much across covered patients, which leads to relative agreement in a health technology assessment. Such payment methods are well suited to cases in which there is little potential for a manufacturer to influence effective use—for example, through marketing that helps with adherence, patient support resources, or analytics. Category 1 models are also appropriate when the treatment is straightforward (such as a small-molecule drug) versus one that is more complex and may improve significantly with experience (for example, an implantable device or a gene therapy). In category 1, important tools related to value include health technology assessments and other considerations such as volume discounts where competitive alternatives exist, along with patient-appropriate coverage restrictions and utilization management.

Category 2 payments adjust fee-for-service prices directly based on existing evidence, including evidence gaps. Indication-specific pricing may be beneficial when there is clear evidence of differential value across identifiable groups of patients or when the strength of evidence varies across identifiable patient groups—for example, patients with different cancer profiles that lead to differential responses to a particular drug. Differential pricing may enable greater access if the groups for which the product has lower evidence of value would otherwise face a single higher price. However, differential upfront prices may be difficult to implement and could add to concerns about manufacturers' cap-

turing too much of the additional value.[35] Payments linked to additional data reporting and support for studies (for example, on drug cost-effectiveness) can lead to better care decisions and more informed negotiations. Most such programs have been implemented by public payers such as the Centers for Medicare and Medicaid Services, but improving data infrastructure and multipayer collaborations may make such models more attractive.

Category 3 contracts are suited for medical products for which there is significant uncertainty about benefits and risks coupled with high up-front costs—particularly when manufacturers have more confidence than payers do about benefits or have significant capacity to influence outcomes. As in coverage with evidence development, manufacturers can assist in tracking value measures (for example, in conjunction with postmarket study requirements of the Food and Drug Administration) and can help providers identify and effectively treat patients who are likely to benefit from the product. For such contracts to work, it must be feasible to reliably measure treatment impact or markers of impact. Category 3 contracts can encourage manufacturers to improve complex treatments using evidence derived from real-world measurement. Examples include complex biologics that have more impact when better targeted to specific subgroups of patients; advanced diagnostics that use artificial intelligence based on patient data to optimize algorithms; new medical devices that can be modified over time based on patient experience, such as robotics with artificial intelligence features; and gene and cell therapies that may be modified to become more potent and safer over time.[36]

Category 4 payment models based partially (category 4A) or primarily (category 4B) on per person payments for a covered population may be useful when evidence shows that volume-based payments are not leading to satisfactory outcomes for a population. Many proposed payment reforms for antimicrobials include delinking payment from volume by shifting to a partially or fully population-based payment. This approach discourages overuse while still recognizing the social value of an antibiotic to protect a population against contagion involving broadly resistant organisms. Category 4 models may also be useful when both the marginal manufacturing cost of a product is low relative to its volume-based price and a large potential population is untreated. Examples could include a small-molecule drug or a one-time therapy expected to have high value relative to production cost. If fee-for-service access is very restricted at a high price, it may be possible for the manufacturer and the payer to use a population-based payment to increase overall value in a way that both find worthwhile. For example, such a reform for certain potent but costly cholesterol-lowering agents could enable both better population health outcomes and additional medical cost savings as a result of fewer complications from heart disease.

As in other value-based payment models, negotiations would influence how additional value is shared. For example, state Medicaid programs may recognize the downstream health benefits of broader access to a drug, but tight budget constraints may nonetheless limit their ability to pay more now—even if the up-front payments are linked to long-term reductions in Medicaid costs from avoided complications. Louisiana's hepatitis C model does not link manufacturer payments to performance measures such as higher hepatitis C detection and cure rates or lower long-term costs from reduced hepatitis complication rates or avoided liver transplants. Consequently, there is little manufacturer incentive to promote access through assisting with patient identification and adherence. The achievement of improved population hepatitis outcomes and fewer complications will consequently depend on enhanced screening and management by providers and public health programs, rather than manufacturer incentives.

## Conclusion

Value-based payment reforms for medical products are understandably gaining attention, but they are diverse and differ in terms of their suitability for the range of challenges in medical product innovation, access, and affordability. Our framework aims to provide structure to this diversity; enable tracking of the prevalence and growth of value-based payment models; and facilitate more rigorous evaluation of their impact on access, outcomes, and costs.

Such rigorous assessment is needed to help guide related policy reforms. Manufacturers, payers, and others have cited policies such as off-label communication restrictions at the Food and Drug Administration, anti-kickback regulations, and Medicaid best price requirements as barriers to implementing value-based payment models for medical products.[37] These policies serve critical roles in fee-for-service payment environments, preventing inappropriate financial relationships between health care entities while ensuring that government programs can receive substantial discounts. In contrast, such regulatory restrictions are less likely to be beneficial in value-based payment reforms that shift payments substantially away from fee-for-service.

Indeed, communicating about spending and outcome evidence and sharing resources to improve performance may be critical to the success of value-based payment reforms.

Finally, our framework can help align medical product payment with broader shifts in health care toward a focus on demonstrated value for individual patients. Currently, the operational capacity for all types of value-based care reforms is limited by the availability of longitudinal data on patient experience and analytic infrastructure.[14] This technological capacity is increasing but still is typically not readily available for assessing product performance on outcomes, such as the reliable and routine conduct of postmarket studies of medical products that receive accelerated approval from the Food and Drug Administration. More clarity on the opportunities for medical product payment to help advance value-based care and the evidence needed to support it could accelerate the adoption of new payment models for medical products. Given the increasing concerns about the affordability of new treatments and the potential for innovation in medicines and how they are used to address unmet medical needs, it's time for a systematic and comprehensive approach to medical product payment reform. ■

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