Paying for Outcomes: Innovative Coverage and Reimbursement Schemes for Pharmaceuticals

Josh J. Carlson, PhD; Louis P. Garrison, Jr., PhD; and Sean D. Sullivan, PhD

“The customer really doesn’t want a drilling machine, he wants a hole-in-the-wall.”
—Theodore Levitt

R ecently, there has been significant interest in novel coverage and reimbursement schemes for medical products that involve the concept “pay-for-performance,” or “risk sharing.” The emergence of these schemes could have far-reaching implications for both payers and manufacturers, especially with the current policy emphasis on evidence-based methods for technology assessment, pricing and reimbursement reform, and cost containment. A few high-profile cases have brought the concept further into focus within the health care industry, including the U.K. National Health Services’ (NHS) scheme to reimburse drugs to treat multiple sclerosis in the context of evidence development, Johnson and Johnson’s scheme to refund the NHS for multiple myeloma patients who fail to respond after 4 cycles of bortezomib (Velcade), and UnitedHealthcare’s scheme to tie reimbursement of Genomic Health’s Oncotype DX assay to a program of data collection examining the link between test results and treatment choices in women considering adjuvant breast cancer therapy. These and several other schemes both in the United States and worldwide have been profiled in the news media and suggest this may be an emerging trend. Although the majority have been implemented outside the United States, a handful have been put into practice in the United States and may portend additional arrangements on the horizon. As these models continue to gain momentum, their potential impact on U.S. health care system increases. As such, a discussion of the underlying drivers, barriers, and potential implications of these new models for U.S. health care payers is warranted.

Why the Interest?
Performance-based reimbursement schemes appear to have arisen in response to a number of external factors such as (a) a limited evidence base and associated uncertainty for pharmaceuticals and other medical products at the time of market introduction owing, in part, to accelerated drug approval; (b) increasing cost pressures from those who fund and pay for health care; (c) the increased use of external reference pricing globally; and (d) an increased emphasis on policies to control new technologies such as health technology assessment programs. These schemes have an intrinsic appeal because they have the potential to alleviate some of the negative consequences that can arise from these health care trends. The relevant uncertainty arises in the transition from efficacy-oriented clinical trial estimates derived during product development to realized performance (effectiveness) in a given patient population. These uncertainties include those related to who will get treated (i.e., patient and subgroup heterogeneity), how much product will be used (both individually and at the population level), and what the impact of the intervention will be on long-term individual and population outcomes. These uncertainties are directly linked to a product’s ultimate budget impact and realized value. In addition, they are particularly salient at product launch when coverage and reimbursement decisions are typically made.

Cost pressures on pharmaceutical manufacturers and payers continue to grow with the rising cost of drug development; the emergence of new medical technologies, including diagnostics; and the general expansion of treatable conditions, which is exemplified by the potentially expanded indication for statin therapy to include healthy individuals with high levels of C-reactive protein. In this environment, health care payers are under great pressure to contain costs in order to remain competitive. As such, performance-based schemes that link payment to outcomes may be a means to create incentives for manufacturers to participate in targeting their products toward those most likely to benefit, since they would expect to receive a lower price per unit in less targeted patient populations. In addition, utilization-related schemes (e.g., price-volume and per patient utilization caps) limit total expenditures and budget impact.

Another motivation for performance-based schemes outside the United States can be to provide a means to give a discount to payers without changing the global price—the lowering of which can have negative impacts on revenue due to external reference pricing (i.e., benchmarking drug prices across countries). For

Performance-based reimbursement schemes
Reimbursement schemes between medical product manufacturers and payers in which the performance of the product is tracked in a defined patient population over a specified period of time and the level of reimbursement is tied by formula to the outcome.
example, the list price for bortezomib remains unchanged in the current performance-based scheme with the NHS, but because the NHS will be refunded for patients failing to respond, the net price paid per unit of drug will be lower than the list price—in effect, a discount.9

Finally, the increased worldwide emphasis on health technology assessment has created an environment in which payers and decision makers are requiring more and better evidence to inform coverage and reimbursement decisions. Performance-based schemes can add to the evidence base through the associated programs of data collection, especially if they are made publicly available.

**Performance-Based Schemes in the United States**

There are 4 performance-based schemes between payers and manufacturers in the United States of which we are aware (Table 1). There have been other schemes aimed at patients covering products for blood pressure, hair loss, smoking cessation, and the ever popular erectile dysfunction—but these were more akin to money-back guarantees for other common products (e.g., brake pads).5,10 The first scheme in the United States to include insurers was an older scheme implemented in 1998 that is no longer in practice in which Merck offered to refund both patients and insurers up to 6 months of their prescription drug costs if simvastatin (Zocor) plus diet did not help lower LDL cholesterol to target concentrations identified by their doctors. The scheme was offered as part of Merck’s “get to goal” campaign and appears to have been more of a patient-focused marketing plan rather than a major shift in the coverage and reimbursement policy for simvastatin.5 Nonetheless, it predates many of the current examples and indicates that although interest and uptake of these schemes may be relatively new, the concept itself has been around for a while, even in the United States. The other 3 U.S. examples are more recent and include schemes between UnitedHealthcare and Genomic Health for the Oncotype DX genomic test used to predict recurrence of breast cancer;9 a scheme between CIGNA and Merck regarding 2 related products, sitagliptin (Januvia) and sitagliptin + metformin (Janumet), in the treatment of type 2 diabetes;11 and a scheme between the manufacturers of risdroronate (Actonel, Proctor & Gamble, and sanofi-aventis) and Health Alliance in the treatment of osteoporosis.12 The details that underlie these schemes are worth exploring.

UnitedHealthcare agreed to reimburse the Oncotype DX test at list price ($3,460 per test) for 18 months while it and Genomic Health track the results to determine if the genetic test is having the anticipated effect on actual clinical practice. If the number of women receiving chemotherapy exceeds an agreed-upon threshold, even if the test suggests the patients would not benefit from therapy, the insurer will receive a pre-negotiated lower price.3 The savings for the payer, and that which underlies Genomic Health’s value message and pricing, is in women who would have received expensive (approximately $15,000, including costs associated with infusion, patient time, use of colony-stimulating factors to prevent myelosuppressive complications, and management of chemotherapy-related side effects)13 adjuvant chemotherapy under current treatment guidelines, but for whom this assay suggests it is not necessary. If, however, women and their doctors do not follow the test results and proceed with adjuvant chemotherapy, the payer receives no cost savings. In fact, they are worse off because they have also paid for a relatively expensive genomic test. It therefore appears that UnitedHealthcare wanted to ensure that they were getting sufficient value and cost offsets to warrant the coverage and reimbursement amount for the assay by implementing an internal study on the proportion of women whose treatment choice coincides with their genetic test results. If this proportion is not in line with the agreed-upon estimates, a pricing change would then be warranted to align the reimbursement amount with the actual value received.

The details for the scheme between Merck and CIGNA involve the coverage and reimbursement for two type 2 diabetes drugs—sitagliptin and sitagliptin + metformin. Under the scheme, CIGNA will assess the blood sugar levels using hemoglobin A1c lab values for patients on any oral antidiabetic medications. If the A1c values, in aggregate, have improved by the end of the agreement period, the discounts offered by Merck on sitagliptin and sitagliptin + metformin will increase by a pre-agreed amount. In addition, CIGNA will use claims data to determine if patients are taking sitagliptin and sitagliptin + metformin as prescribed by their physicians. If so, Merck will further increase the discounts it offers CIGNA on the 2 products. Finally, the agreement also includes better placement for sitagliptin and sitagliptin + metformin on CIGNA’s formulary tiers with a lower copayment versus that for other branded drugs.11,14 This agreement differs from many other schemes we have encountered in that there is a deeper discount when patients on any diabetes drug improve their A1c lab values. Previous schemes have linked discounts to poorer performance for patients on the specific medical product in question (e.g., bortezomib in the United Kingdom).9 The scheme provision that ties the improved adherence to larger discounts makes intuitive sense because it can benefit all the key parties—payers, manufacturers, and patients. Diabetes patients who are more adherent tend to have better outcomes.15 Furthermore, patients with better adherence and outcomes tend to utilize fewer resources, which can provide a cost savings for the payer who is already receiving discounts on the cost of the drug.16 This may in part be attributable to a healthy adherer effect.16 Finally, manufacturers can improve sales volumes with better patient adherence because patients will be filling a greater proportion of their prescriptions. This improved sales volume will likely offset the lost revenues related to the per unit discount offered by Merck. As a recent New York Times article stated, “Merck is betting not only that its drugs prove superior but that CIGNA’s incentives to reap the benefits of the deeper Januvia and Janumet discounts will prompt the insurer to try to keep patients on those drugs.”13 This scheme is
Barriers to Implementation

Although these agreements have an intrinsic appeal, tying reimbursement to a product’s actual performance, there are many barriers to their implementation. Apparent major barriers are: (a) the associated transaction and administration costs; (b) the limitations of current information systems in terms of tracking performance; (c) agreeing on the scheme details (e.g., the appropriate outcome measure or the financial adjudication process); (d) physician push-back; (e) the “free-rider” problem—other manufacturer or payer competitors may benefit from the information or schemes developed; and (f) a lack of trust between payers and developers. The primary transaction costs include the development of processes to track product performance, personnel time to administer the schemes, and the costs of negotiating these deals. For a payer to consider implementing a novel reimbursement mechanism, the expected transaction costs must be substantially outweighed by the potential benefits—including sufficient compensation to overcome institutional inertia. As such, these schemes may be reserved for high-cost drugs with limited evidence of efficacy. Currently, some health systems give many such drugs or other medical technologies conditional approval pending further trials to confirm clinical benefit (e.g., Health Canada and Centers for Medicare & Medicaid Services).10,21 For the manufacturers, conditional approval allows early market access. However, payers may view conditional approval as shifting the costs for some Phase III-IV work onto the payer community. Thus, performance-based schemes may be another mechanism for addressing evidence uncertainty at product launch but at a reduced level of payer investment.

Many payer information systems remain underdeveloped in their ability to track clinical outcomes as electronic medical records still remain a goal rather than a reality for the majority of U.S. health care payers. Without adequate information systems, these schemes will be limited as to the types of performance in line with the concept of “value in use,” where value is defined as “the benefit the customer obtains through use, and compels the firm to bring in customer usage as part of its responsibility to deliver the outcome.”17

Finally, the scheme between Proctor & Gamble, Sanofi-Aventis, and Health Alliance for the use of risedronate in osteoporosis has its own unique aspects that differentiate it from other performance-based schemes.12 In this scheme, the 2 companies that jointly sell the osteoporosis drug agree to reimburse the insurer for the costs of treating nonspinal fractures suffered by patients who consistently take the medication. This appears to be the first published example of a manufacturer agreeing to cover the cost of disease-related sequelae as opposed to discounting or refunding the cost of their product. This scheme will certainly lower the medical costs to Health Alliance, since hip fractures and wrist fractures cost approximately $30,000 and $6,000, respectively. The benefit to the manufacturers will be in keeping patients from switching to cheaper generic versions of alendronate and through maintaining a lower copayment level than their competitor, ibandronate. This scheme further illustrates one of the proposed motivations for the implementation of performance-based schemes—decreasing the risk to the payer related to product uncertainty—in this case, the uncertainty about the benefit of risedronate in terms of reducing nonspinal fractures. This uncertainty was revealed in the clinical trials of risedronate, which failed to show a statistically significant reduction in nonspinal fractures, whereas some of its competitors have been able to demonstrate this benefit in their trials.18,19 In essence, the makers of risedronate are betting that their product will be able to reduce these nonspinal fractures in actual practice and/or the cost of treating them will be offset by maintaining or even expanding their market share in a highly competitive market in which it may not be the market leader.

### Table 1: Performance-Based Coverage and Reimbursement Schemes in the United States

<table>
<thead>
<tr>
<th>Source</th>
<th>Disease area</th>
<th>Product</th>
<th>Manufacturer</th>
<th>Payer</th>
<th>Description</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Moldrup (1998)5,10</td>
<td>High cholesterol</td>
<td>simvastatin</td>
<td>Merck</td>
<td>Patients and insurers</td>
<td>Merck promised to refund patients and insurers up to 6 months of their prescription costs if simvastatin plus diet did not help them lower LDL cholesterol to target concentrations identified by their doctors.10</td>
<td></td>
</tr>
<tr>
<td>Pollack (2007)3</td>
<td>Breast cancer</td>
<td>Oncotype Dx</td>
<td>Genomic Health</td>
<td>United-Healthcare</td>
<td>UnitedHealthcare agreed to reimburse the Oncotype Dx test for 18 months while it and Genomic Health monitor the results. If the number of women receiving chemotherapy exceeds an agreed upon threshold, even if the test suggests they do not need it, the insurer will negotiate a lower price.</td>
<td></td>
</tr>
<tr>
<td>Shearer (2009)11</td>
<td>Type 2 diabetes</td>
<td>sitagliptin, sitagliptin + metformin</td>
<td>Merck</td>
<td>CIGNA</td>
<td>Merck has agreed to peg what the insurer CIGNA pays for the diabetes drugs sitagliptin and sitagliptin + metformin to how well type 2 diabetes patients are able to control their blood sugar.</td>
<td></td>
</tr>
<tr>
<td>Anonymous (2009)12</td>
<td>Osteoporosis</td>
<td>risedronate</td>
<td>Proctor &amp; Gamble, sanofi-aventis</td>
<td>Health Alliance</td>
<td>Two companies that jointly sell the osteoporosis drug risedronate agreed to reimburse the insurer Health Alliance for the costs of treating nonspinal fractures suffered by patients taking that medicine.</td>
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</tbody>
</table>
measures that can be tracked and will also add to the administrative cost barriers as they rely more heavily on human effort and resources. This barrier may be more easily overcome by large payers with better developed information technology (and larger resources) and integrated health systems. Agreeing on the many details of a proposed scheme both large and small can be a substantial barrier as well. The most important details likely relate to the appropriate outcome measure(s) and the process for the financial reconciliation of the scheme. Additional financial barriers might include Medicaid best-price considerations, especially given the uncertainty in the final price per unit for a new scheme.

Physician resistance is another possible concern. Physicians, already pressed for time, are loath to take on any more responsibilities, paperwork, and effort unless appropriately compensated. Physicians may also have concerns about being pressured into prescribing certain products either by their patients, looking for lower copays, or by the payer, seeking to maximize its discounts or related incentives. Also, information can be a public good, and both private payers and manufacturers might be reluctant to enter into these schemes if competitors will benefit from the information. First-mover advantage and other network effects may mitigate this barrier to some extent. The number and nature of the identified barriers makes it likely that performance-based contracts will be limited to specific situations in which not only the incentives are aligned, but also the parties can come to agreement on the key process components under which the contract operates.

**Future Prospects**

Performance-based schemes appear to have arisen as a way to decrease some of the potential negative impacts of uncertainty in the provision of and payments for medical care by altering the balance of associated risk between manufacturers and payers. These potential negative impacts grow as products become more expensive—a continuing trend, especially in the area of specialty pharmaceuticals such as those under development in oncology.

Though it is too early to say whether performance-based schemes will become a lasting trend, they attempt to address key health policy issues related to increasing cost pressures, uncertain effectiveness, and value in health care spending. In fact, the emergence of performance-based schemes may be seen as another step in the evolution of health care toward one based on value—in this case, realized value—and the desire to improve the level of patient benefit per dollar spent. Recent health care financing reform initiatives, including value-based insurance design and value-based pricing, are reflective of this movement toward value-based health care.22,23 In addition, major health care payers, including UnitedHealthcare, CIGNA, and the NHS in the United Kingdom as well as large pharmaceutical manufacturers such as Merck, Novartis, and sanofi-aventis, have demonstrated a willingness to engage in performance-based schemes. The ultimate impact of these schemes in the United States will likely be limited by the identified barriers, especially those related to administrative costs. But given the interest shown by the involved parties to date and the potential to meet the goals of individual stakeholders—patients, manufacturers, and payers—performance-based coverage and reimbursement schemes have the potential to become a more widespread practice in health care.

**Authors**

JOSH J. CARLSON, PhD, is a Senior Postdoctoral Fellow; LOUIS P. GARRISON JR., PhD, is Professor of Pharmacy and Associate Director; and SEAN D. SULLIVAN, PhD, is Professor of Pharmacy and Health Services and Director of the Pharmaceutical Outcomes Research & Policy Program, University of Washington, Seattle, Washington.

AUTHOR CORRESPONDENCE: Josh J. Carlson, PhD, Senior Postdoctoral Fellow, Pharmaceutical Outcomes Research & Policy Program, University of Washington, Box 357630, Seattle, WA 98195-7630. Tel.: 206.685.6065; Fax: 206.543.3833; E-mail: carlsojj@u.washington.edu.

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