them to avoid such patients. Although this concern may be valid, the new rule only exacerbates an inherent side effect of prospective payment in the absence of perfect risk adjustment: to the extent that certain observable patient characteristics are associated with higher costs and are not accounted for in the payment formula, the DRG system already rewards hospitals for avoiding patients with these risk factors. On the other side of the issue, some stakeholders proposed substantial expansions of the list of nonreimbursable conditions, given the extent of injuries, the number of deaths, and the magnitude of the expense associated with patient-safety problems in hospitals; the approval of a much larger set of conditions would have ratcheted up the financial implications and made a more compelling case for more fundamental and comprehensive reform of inpatient care. In the end, however, CMS ruled out a number of candidate conditions, either because they could not be identified through existing DRG codes or because of a lack of proven strategies for preventing them.

In the same regulatory ruling, Medicare has also refined the DRG system to increase the extent of payment differentiation according to the severity of illness. Thus, along with emerging pay-for-performance initiatives, the new policy appears to be part of a larger reform of the Medicare payment scheme. The current reform rests on the following three principles: payers should pay more for the treatment of conditions that require more resources and that the provider could not reasonably have prevented; they should pay more when evidence-based or consensus-based best practices are followed; and they should pay less or not at all for low-quality care. Naturally, the last will be the most controversial.

The conditions for which Medicare will cease to pay hospitals as of next October have been shown to be within the control of hospitals, so there is a relatively compelling case that their costs should fall on the provider rather than the purchaser. It is unclear how Medicare will generalize the principle of refusal to pay for poor-quality care beyond this initial and largely symbolic effort. As inadequate as the store of evidence-based beneficial practices appears to advocates of pay for performance, there is even less empirical support or consensus for the identification of inappropriate or clearly contraindicated services and care patterns.

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Satisfaction Guaranteed — “Payment by Results” for Biologic Agents

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As increasing numbers of promising but expensive biologic agents are introduced for use as medical treatments, drug pricing has become a high-profile issue. Earlier this year, pricing practices took a new turn in Britain, when the National Institute for Health and Clinical Excellence (NICE), the evaluative agency that applies cost-effectiveness analysis in making recommendations concerning drug coverage, declined to support coverage of the proteasome inhibitor bortezomib (Velcade) by the British National Health Service (NHS) for the treatment of multiple myeloma. It concluded that the price was too high relative to NICE’s estimates of its average benefits for the population to be covered. Rather than reducing bortezomib’s price, its manufacturer, Johnson & Johnson, offered to forgo charges for patients who do not have an adequate response to the drug. Although many details remained to be negotiated — including the criteria defining a response — the proposal reflected the recognition that the time has come to consider new approaches to drug pricing.

Payment based on results, including “pay for performance,” has been touted as a way to avoid waste and increase value in health care. In a conventional pay-for-performance contract, a small part (typically, 1 to 10%) of the reimbursement to providers is tied to measures of the quality and cost
of the care they give. Johnson &
Johnson’s proposal takes the con-
cept of pay for performance to its
logical extreme, offering a money-
back guarantee that risks the loss of
100% of the drug price when the
desired results are not achieved.

Furthermore, most performance
incentives reward effort — often
measured in terms of a provider’s
compliance with practice guide-
lines — more than outcomes.
Providers have resisted payments
based on outcomes, which are in-
fluenced by difficult-to-measure
factors they cannot control, such
as patient compliance and disease
severity. In results-based payment,
the manufacturer supplies an un-
varying product, and the response
to it depends heavily on the se-
lection of patients — a decision
made by physicians and patients,
not the company. Why, then,
might it make sense to accept
payment by results rather than
simply lowering the drug’s price?
And how broadly can this pric-
ing model be applied?

In Britain, without a favorable
determination by NICE, a costly
new intervention is unlikely to be
adopted by the NHS. Although
NICE does not make decisions
solely on the basis of cost-effec-
tiveness, in the past it has indi-
cated that it generally accepts
interventions whose cost-effec-
tiveness ratio it estimates to be
below about £30,000 per quality-
adjusted life-year for the intend-
ed population, though there have
been exceptions.

For drug manufacturers, fail-
ure to obtain NICE’s approval can
mean the loss of a substantial
market. For most patients in Brit-
ain, it means paying for the drug
out of pocket or through the small
but growing private-insurance
market.

Replacing a per-unit price with
an outcome- or results-based price
shifts financial risk from the pay-
er to the manufacturer. If NICE
applied the definitions of re-
response used in major clinical tri-
als, and if response rates were
similar to those in trials, bortezom-
bib would sell at an effective dis-
count rate of about 60% when
used to treat relapsed multiple
myeloma. However, the reper-
cussions are different from those
of a simple price cut. To maxi-
mize revenues, the manufacturer
has a stronger incentive to maxi-
mize the number of patients with
a response, not merely the num-
ber of patients treated or doses
sold. A focus on responders might
encourage redoubled efforts to ad-
dress the undertreatment of indi-
cations for which a drug is effec-
tive. As a result, use of the drug
may increase.

The fixed costs of bringing a
pharmaceutical product to market
can reach hundreds of millions
of dollars, but the cost of produc-
ing each additional unit is very small
in relation to the total cost and
unit price. If the marginal cost of
producing the drug is one tenth
of the price, with payment by re-
results, a manufacturer will profit
by extending the treatment to any
population of patients with a re-
sponse rate of at least 10%.

Payment by results also repre-
sents an innovative approach to ad-
dressing one of the central dilem-
as in the allocation of drugs to
patients — the fact that a treat-
ment’s benefits vary greatly, often
in predictable ways, from one pa-
tient to another. Survivors of myo-
cardial infarction have larger short-
term survival benefits from statins
than do young women with hyper-
cholesterolemia but with no oth-
er risk factors for heart disease.
A much greater survival benefit of
imatib mesylate (Gleevec, Novar-
tis) has been demonstrated among
patients with gastrointestinal stro-
mal tumors than among those
with malignant glioma. Physicians
may be aware of many other char-
acteristics that determine wheth-
er a patient is likely to benefit
more or less than the enrollees
in a study. If the price of the com-
ound is uniform, access is
likely to be limited to patients in
the population groups known to
derive the greatest benefit (if the
cost is paid by insurance) or will-
ing to pay the most (if patients
spend their own money). Patients
in groups that are not expected to
derive particularly large benefit
are less likely to receive treatment,
often because payers will create
barriers to such use of the drug.
In results-based payment, payers
face much less financial risk from
treating such groups.

Manufacturers with a monopo-
ly often address variation in bene-
fits by charging different patients
different prices (price discrimina-
tion). Prices might be lower for
low-income patients or for those
not expected to receive large ben-
efits. This approach makes the
treatment available to more pa-
tients who might benefit while
increasing sales and profits. How-
ever, in many circumstances, price
discrimination is difficult to im-
plement and unpopular, and both
marketing considerations and po-
itical pressure limit its use.

Payment by results can extend
access to drugs while avoiding
some of these disadvantages. A
manufacturer can earn more if
nearly everyone in whom the per-
formance goal might be achieved
receives treatment. It’s true that
simply reducing the drug’s price
would also increase the number of
patients for whom the cost-effec-
tiveness ratio was favorable, but
the reduction would need to be deep to increase volumes by a similar amount, and it would not create similar incentives for ensuring that the patients likely to have high benefit received the drug.

Moreover, the payment-by-results approach obviates the need to calculate different cost-effectiveness ratios for different groups of patients: as long as benefit is captured well by the response measure, cost-effectiveness can be calculated the same way for everyone. In effect, payment by results creates a personalized rather than a population-based cost-effectiveness ratio, and the NHS need not restrict access for certain classes of patients to ensure that the cost-effectiveness test is met.

With such advantages over simple unit pricing, why has results-based pricing seldom been used? As the bortezomib negotiations suggest, there are many practical challenges. First, there must be a clearly defined, objective measure of results, and it must closely correspond to the desired treatment effect — that is, to a valid health outcome measure. Furthermore, the outcome measure must not be heavily confounded by patient characteristics or by other treatments. Survival, for example, is perhaps the most important outcome of treatment, but it is not usually a practical measure, because it is heavily influenced by underlying disease and by any other treatments given and because the timeline for observing changes in survival may be impractically long. The use of a surrogate measure, such as the monoclonal protein level in bortezomib’s case, will be most successful if it is a good biomarker or predictor of clinically meaningful outcomes and if it is not influenced by other treatments.

Johnson & Johnson proposed to accept payment by results for patients in whom at least one other treatment had failed — a policy that reduces the likelihood that the outcome measure would be confounded by other aspects of care. In such patients, the monoclonal protein level is an accepted measure of treatment response. Results-based pricing is most likely to be successful in other settings in which response can be defined with similar objectivity and reproducibility and among patients in whom other therapies have failed and whose condition is unlikely to improve if the compound is not used.

Any criterion used for payment should also be predictable, reliable, and difficult to manipulate. If a test result is highly variable or subjective, well-defined criteria and audits would be crucial to ensure that the measure worked as intended. For example, should a transient decrease in the monoclonal protein level be considered a response? Are multiple measures necessary? If there is room for judgment, how will disagreements over the interpretation of the performance criteria be adjudicated? If the performance measure is confounded by the underlying health of the patient, it could result in overpayments for healthier patients, creating incentives for focusing on lower-risk patients rather than on those most likely to benefit. Addressing this problem would require either a different price or different outcome criteria for low-risk patients, either of which would complicate the approach.

Although Johnson & Johnson and NICE reached agreement on an indicator, they differ over the appropriate threshold for payment — whether an adequate response should be defined as a 50% or just a 25% reduction in the serum monoclonal protein level. In the United Kingdom, the cost-effectiveness standard will be determined by the NHS, not by competitive or other mechanisms.

Clearly, difficulties of implementation will limit the adoption of payment by results. Experience in overcoming the practical barriers, however, may well address both payer and industry concerns about pricing for potentially valuable drugs while increasing the number of patients who can receive effective treatments.

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