

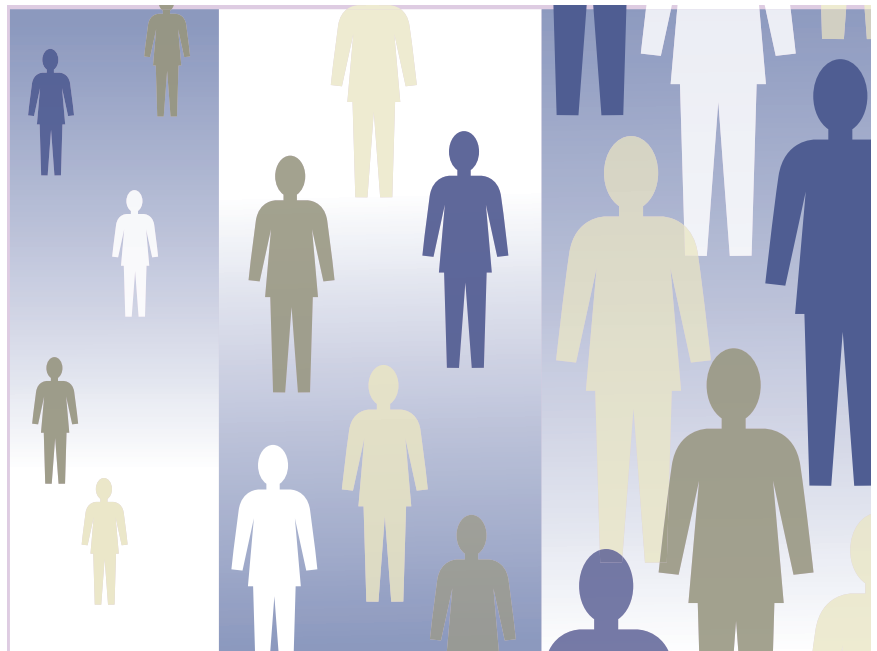
Demonstrating Value to Payers

When making coverage decisions, payers want data that demonstrate the value of new medical technologies.

Melissa Martinson and Rosemary Brekke

Talk about a crisis in U.S. healthcare—a crisis of cost, access, and quality—is widespread and common. In such an environment of concern, it is incumbent upon medical technology companies to concentrate their efforts on developing products that will improve the quality and value of healthcare. Medtech manufacturers will, of course, have to provide evidence of this benefit to payers. Success in doing so will be likely to expedite reimbursement, which in turn can increase the rate of adoption of innovative products. Savvy manufacturers, those long attuned to the need for including clinical outcomes data in regulatory submissions, now provide health economic data for payers too.

Health economics is the study of how resources are allocated to and within the health-related economy. Conducting health economic analysis helps medtech companies determine where the optimal value of a device might be found—for example, within which patient subgroups or care settings—or where the product fits



within the disease-treatment continuum. This discipline is increasingly integrated into the reimbursement process.

Reimbursement comprises the areas of coverage, coding, and payment. Health economics is becoming more important particularly for the area of coverage. Coverage is a con-

cept encompassing the process and criteria used to determine whether a product, service, or procedure will be paid for through a government or private insurance program.

This article discusses the question of how the value of new medical technologies can be measured and demonstrated. The full subject

includes consideration of how developers and manufacturers of new medical technologies can garner the appropriate information to use in such a demonstration. Also, the article examines the role that health economics plays in decision making with respect to coverage and payment.

Establishing Value in Healthcare

Value in the purchase of healthcare is no different from what a buyer seeks in the purchase of any other goods or services. Good value is getting much relative to the amount paid. Maximizing the value of healthcare ensures that, regardless of the level of funding for healthcare involved—5% of the budget or 50%—the care that is delivered provides the most benefit for the money.

It may be emotionally difficult for some people to talk about healthcare in financial terms, but that focus is necessary in order to quantify value. In healthcare, the term usually employed to signify value is cost-effectiveness, and a measure of this has been the cost-effectiveness ratio, that is, the dollar cost of a unit of effectiveness (see sidebar).

Healthcare effectiveness, of course, can be manifested in a lowering of blood pressure, an increase in joint mobility, a reduction in pain level, or in other types of physiological improvement as different as apples and oranges. A common denominator is needed to make possible value comparisons of healthcare services with different beneficial outcomes. The usual denominator for this purpose is the quality-adjusted life year (QALY), a measure that combines quality of life with quantity of life. Quality of life is measured on a scale of 0 (death) to 1 (perfect health). One year in perfect health yields 1 QALY. When a health state is

Quantifying the Healthcare Value of New Technology

One technique that has been employed to determine the economic value of a medical therapy is the calculation of its cost-effectiveness ratio, which is the dollar cost of a defined unit of effectiveness.

For example, if joint-repair surgery costs \$20,000 and improves pain-free mobility in that joint by 20° of motion, then the cost-effectiveness ratio is \$20,000 divided by 20°, or \$1000 per degree of additional pain-free movement. The chief alternative, joint-replacement surgery, might cost \$45,000 and improve pain-free mobility by 70°. The incremental cost-effectiveness ratio for this therapy is calculated by dividing the extra \$25,000 in cost by the 50° of greater freedom of movement that results. The additional range of mobility costs \$500 per degree. Thus, the more expensive procedure is the better buy in terms of cost per unit of improvement in outcome.

Most new technologies provide a less clear-cut benefit than in this example because the incremental cost for each additional unit of effectiveness is greater than that of the cheaper existing technology. If the 50° mobility improvement with joint replacement costs an additional \$75,000, then each additional degree of pain-free motion would have a \$1500 price tag.

When the healthcare budget is fixed, choosing among therapies is a straightforward matter with this means of measuring value. If there is enough money in the budget to pay \$1500 each for additional degrees in a range of painless motion, then the more expensive therapy can be provided. If not, then either more money must be budgeted for healthcare or coverage must be limited to the less expensive therapy.

However, there are problems with this method. Not all healthcare benefits are measured in terms of range of motion. There has to be a way to compare the value of a 10-mmHg reduction in blood pressure with the value of a 20° improvement in mobility range. One good solution is the quality-adjusted life year (QALY), a measure of healthcare benefit used in some industrialized nations to change apples and oranges into fruit, each type of which bears a QALY label that allows meaningful comparison.

assigned a quality of 0.5, it is because people have been judged to value two years living in that state only as much as one year in perfect health ($0.5 \times 2 = 1$ QALY).

This academic-seeming system is used in various places around the world. The British National Health Service (NHS) has a research arm, the National Institute for Clinical Excellence (NICE), which makes explicit coverage decisions based on such criteria. Currently, NICE uses a cutoff of approximately \$53,000 per QALY because, given the NHS budget and the volume of services demanded,

this is effectively what the country has chosen to pay for.¹

The recent implantable cardioverter-defibrillator (ICD) coverage decisions by the Centers for Medicare and Medicaid Services (CMS; Baltimore) offer a U.S. comparison. The value of this technology is about \$51,000 per QALY, according to a Blue Cross Blue Shield Technology Evaluation Center (TEC) assessment.² This figure applies to a patient for whom the probability of arrhythmic death is 10% with conventional therapy; other types of patients are calculated to cost significantly more.

There is much discussion in the UK about raising revenues for the NHS so that the cutoff cost-effectiveness ratio can be higher. This would allow more types of patients to be treated with ICDs and other technologies.

The degree of rationality brought to the making of coverage decisions in Great Britain, Europe, and Canada has been impossible for the United States so far. CMS has tried twice in the past decade to define coverage criteria, without success. A top official of the agency's coverage division believes that cost-effectiveness has been one of the most contentious issues thwarting these efforts, and claims that competing interests among all of the stakeholders—CMS, Medicare beneficiaries, physicians, hospitals, product manufacturers, and others—have made it impossible to reach consensus.³

CMS has been inching toward a definition, however. In a recent guidance document, the agency says that it may issue a coverage decision with the qualification that more data be collected. This is the language it will use in such a case: "Assessment of important outcomes has not been evaluated in the available clinical studies. These outcomes may include, but are not restricted to, long-term risks and benefits, quality of life, utilization, costs, and other real-world outcomes."⁴ Note the inclusion of quality of life, costs, and utilization among the important outcomes.

Collecting Health Economic Data

Until now, CMS has covered some services without conditions. However the agency has stated that it does not intend to continue this practice. For medtech companies to ensure that their products receive the broadest coverage possible, they will have to provide evidence of the value

of those products in the form of health economic data. Health economic data are any data pertaining to the resources—labor, supplies and materials, facilities, money, and so on—used in producing healthcare.

The Piggybacked Clinical Trial. Medtech manufacturers have sometimes been reluctant to gather health economic data because of the cost that this adds to product development. An economic study can be just as expensive as a clinical trial conducted to achieve FDA approval. However, with a little planning, it is

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usually possible to piggyback the economic study onto the clinical trial for only modest additional cost. Piggybacking an economic study may add 10–25% to the clinical trial expense, while a stand-alone economic study could cost \$500,000 to \$1 million or even more.

A medical device manufacturer will need to make five major decisions in designing a piggybacked clinical trial. These involve considerations of:

- Whose perspective the cost-effectiveness analysis should assume.
- Which comparison groups should be included in the trial.
- The time horizon of the new medical technology in terms of realization of benefits.
- How much detail to pursue in

collecting cost data.

- What healthcare effectiveness data to collect, and when to collect it.

Analytical Perspective. Whose costs and whose benefits should be included in the cost-effectiveness analysis is the first question to be decided. Health economists often recommend adopting a societal perspective.⁵ This entails estimating the costs borne by everyone affected, from patients through employers (who lose the productivity of the employee who is a patient).

Because of the way the U.S. healthcare system is segmented, payers have an incentive to control their own costs rather than society's. Payers thus are generally more interested in analyses undertaken from their own perspective, not a societal perspective. If the payer is concerned only with clinical outcomes, then the trial sponsor (the manufacturer) should collect data relating only to direct medical costs—that is, those borne by the healthcare payer. If the payer is a self-insured large employer or workers' compensation system, then back-to-work data may become an important factor.

Following the relevant payer's perspective marks a path toward determining which economic data to include in a study. Data gathering may still be a challenge, but this course of action is much simpler than trying to collect all costs.

Comparison Groups. While FDA frequently approves clinical studies with a placebo or inactive control, CMS is interested in trials that include comparisons to the best available therapy or standard of care. This usually means that any control group used in the study must represent active therapy. FDA generally will approve trials of this design as well. The sponsor should be aware

that, whereas comparison with a placebo requires a demonstration of superior efficacy, a comparison with active controls will often require only a demonstration of outcome equivalence. The statistical test is different in the latter case, as those familiar with bioequivalence tests in drug trials already know.

Time Horizon. Many new medical technologies, especially implantable devices, require considerable up-front expenditure. Benefits from the technology, such as improved quality of life or future healthcare cost savings, are realized later. The trial sponsor should determine how long these benefits will have to accumulate to justify the up-front costs. Sometimes this will be longer than the duration of a traditional clinical trial. However, because FDA often requires postmarketing studies that are really a continuation of the original trial, the longer time horizon will not add substantially to a clinical trial's cost in many cases.

Cost-Data Detail. If a new therapy is expected to reduce hospital readmissions substantially, and if it is a credible assumption that outpatient costs, pharmacy costs, and other postrelease costs will not be increased, then hospital readmission rate and hospital charge data may be all the trial sponsor has to collect. Hospitals are sometimes reluctant to provide this information; therefore, it is a good idea for the sponsor to include a stipulation in the study contract. Hospital charges do not reflect hospital costs very closely, however, so it is usually also a good idea to get the hospital's CMS cost-to-charge ratio as well.

On the other hand, if outpatient costs are important, extra care may need to be taken in designing the study. This is because follow-up costs are often part of the structure of the trial. There are two common approaches to calculating these costs. One is the utilization approach: vis-

its, special procedures, prescription drugs, and lab tests are simply counted, with government and commercial databases then being used to provide a typical cost per unit of healthcare delivered. The other is the accounting approach, in which the kind and amount of each type of healthcare labor, material, supply, facility, and so on, used during the trial is tallied. Here again, the cost of each unit is determined from healthcare payer prices. The units involved in the accounting approach are much smaller than those costed in the utilization approach.

What Data to Collect, and When. Payers sometimes make a distinction between efficacy and effectiveness. Efficacy is the maximum potential benefit from a device that is realized in patients who are very compliant, closely monitored, and intensively treated in a clinical trial. Effectiveness is a measure of what happens in the real world. Some therapies are more inclined toward a difference in efficacy and effectiveness than others. If the sponsor is developing a therapy that has a potentially large efficacy–effectiveness divergence, then it may need to include in the trial patients who are treated under real-world conditions.

If effectiveness is to be reported in QALYs, patients can complete quality-of-life instruments or other assessments at baseline and then periodically during the follow-up. The frequency will depend on the nature of the condition and of the intervention that are the focus of the study. However, a score on a quality-of-life instrument often cannot be translated into quality, or utility, on a 0-to-1 scale. It is important to measure quality either by a method that yields utility directly or by using an instrument for which a crosswalk between the instrument score and a degree of utility has been developed.

Benefits of Piggybacking.

Gathering health economic data as part of an FDA clinical trial results, as mentioned, in a cost savings due to avoiding a stand-alone outcomes study. A second benefit is that piggybacking maximizes the chances of the technology achieving coverage for at least some patients. Clinical trials generally include very complete data on patient health characteristics, so it is possible to determine which patients benefit most, which cost the least, and for which the technology is the most cost-effective. CMS is interested in this information. If some category of patients seems to benefit significantly more than others, that fact can be helpful in making the case for coverage.

A good example is the story of Medicare's coverage of ICDs (see Figure 1). Initially, coverage was limited to the treatment of life-threatening ventricular tachyarrhythmias. Then, in 1999, coverage was expanded to comprise additional types of cardiac patients. Coverage was expanded again in 2003 and in 2005, each time to encompass indications for which clinical evidence—balanced with costs—demonstrated value.

The lesson to be taken away is that CMS did not automatically allow blanket coverage for ICDs. Instead, it prodded manufacturers into providing clinical evidence for various indications. Once clinical evidence was available, cost-effectiveness analysis informed the coverage determination; coverage was allowed for those subgroups of patients who would benefit most. Those are usually the patients with whom the therapy is the most cost-effective.

Some product developers may find that a piggybacked trial is no longer an option for them because the safety and efficacy trial is already well under way or has been completed. These companies could consider developing an economic model that brings together data from many

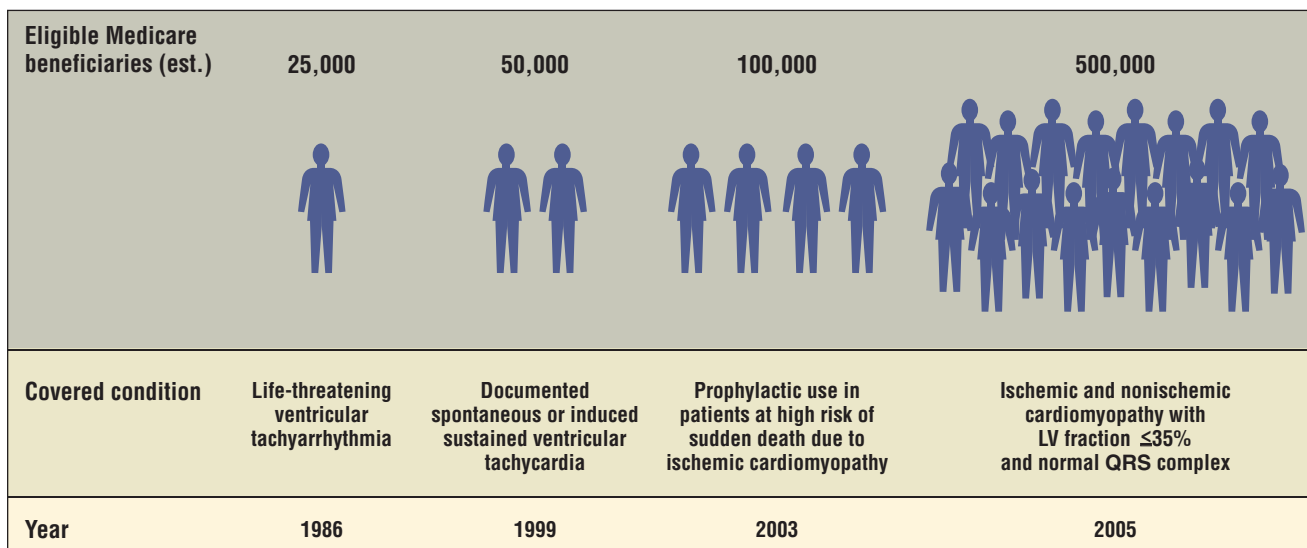


Figure 1. As more clinical evidence has become available, the Centers for Medicare and Medicaid Services (CMS) has gradually expanded its coverage of implantable cardioverter-defibrillators (ICDs) to additional types of cardiac patients. Initially, Medicare coverage was limited to the treatment of life-threatening ventricular tachyarrhythmias. In 1999, coverage was expanded to include patients with documented instances of sustained ventricular tachycardia, adding approximately 25,000 beneficiaries. In 2003, CMS approved the prophylactic use of ICDs in patients at high risk of sudden death due to ischemic cardiomyopathy, expanding coverage to approximately 100,000 patients. And earlier this year CMS expanded coverage again, bringing the total number of eligible patients to approximately 500,000. Each expansion of coverage was made to encompass treatment indications for which clinical evidence—balanced with costs—demonstrated value. Sources: Medtronic Inc. (Minneapolis); McClellan and Tunis.⁶

sources, including the clinical trial. Cost and effectiveness data on alternative therapies could be gathered from the literature, from commercial and government databases, and from other sources. The thing such manufacturers should not do is ignore the payers’ need for cost-effectiveness data simply because they are not able to collect them during the FDA trial.

Economics and Decision Making

The use of economic incentives and disincentives to influence behavior is commonplace, in healthcare as elsewhere. In 1984, the Hospital Inpatient Prospective Payment System was implemented to pay hospitals for inpatient care of Medicare beneficiaries. The hospital received a fixed payment for each discharge according to its designated diagnosis-related group (DRG) regardless of the actual costs it incurred. The payment methodology was based on the economic law of averages, which

might be put simply as “you win some, you lose some, it all averages out in the end.” Increased efficiency was the provider behavior this system was intended to elicit. However, faced with a fixed payment, the hospital focused on cost. Medical device manufacturers found themselves on the receiving end of new pricing pressure.

Cost Minimization. In some circumstances, the use of health economic data to develop a cost-minimization argument has been a successful strategy for mitigating pricing pressure. Vascular closure devices provide an illustration. The cost of a closure device was offset by a decrease in consumption of such resources as nursing time and post-catheterization recovery time. Although the hospital did not receive a higher DRG payment for procedures in which the closure devices were used, device companies, using economic data, were able to demonstrate a derivative cost benefit within the parameters of the fixed payment. This removed a sales barrier

to the devices.

Such a cost-minimization argument is not always possible, especially for devices costing thousands of dollars. But Congress did pass legislation, the Benefits Improvement Protection Act of 2000, allowing hospitals to be paid additionally for qualifying technology.⁷ The application for a new technology add-on payment does not explicitly require cost-effectiveness data, but it does request cost data and proof of significant clinical improvement (that is, effectiveness).

Coverage and Payment. The coverage component of reimbursement is also favorably influenced by health economic data. A survey of 228 managed-care plans nationwide revealed that 90% of the plans consider costs in some form when evaluating new interventions.⁸ The survey was conducted in 2001. Even at that time, 40% of the plans were using formal cost-effectiveness analysis to inform coverage decision making.

Health economics often is a route

to coverage, but it does not necessarily lead to better payment. This is especially true with respect to physicians for whom the dominant method of payment is a fee schedule. The Physician Medicare Fee Schedule reflects a resource-based relative value system. The components of work, practice expense, and malpractice expense determine the value of one service in relation to other services. Under this system, physicians do not receive additional compensation as a reward for performing a cost-effective procedure, even if the service negates a need for more-costly treatments. Clearly, to improve the value of healthcare in some areas necessitates changing some incentives.

Payers. CMS's position on the use of health economic data bears watching. Currently, health economic data play an important, albeit implicit, role in Medicare coverage decision making. After repeatedly failing to add cost-effectiveness explicitly to its coverage criteria, CMS relegated that measure of value to a lower level of importance, using it only implicitly to inform coverage decisions. In its April 2005 guidance document, the agency avoided using the term *cost-effectiveness*, but the draft is peppered with such terminology as "value of new technologies," "most benefits at the lowest possible cost," and "reliable information on the risks, benefits, and costs of various treatment alternatives."⁴ CMS can hardly look at these factors without addressing cost-effectiveness. Meanwhile, a sister agency, the Agency for Healthcare Research and Quality, is undertaking systematic cost-effectiveness research.

This will have implications for private payers as well. Even though private payers are not required to emulate Medicare coverage or payment methodologies and policies,

some health plans do generally look to Medicare as the leader in setting policy for new technologies.⁹ If CMS can explicitly add cost-effectiveness to its coverage criteria, Medicare coverage decisions will become even more influential in private-payer coverage determinations.

Health plans such as Blue Cross Blue Shield and Kaiser Permanente constitute a private-payer exception. These plans already conduct cost-effectiveness analysis, although they decline to state that such analyses are used in coverage decisions. The Blue Cross Blue Shield TEC, in a recent evidence report on wound-healing technologies, instead says this: "Given significant costs of chronic wounds, future comparisons of the cost-effectiveness of various strategies for preventing wounds, managing wounds, and improving quality of care would be of value to clinical decision-makers."¹⁰

Other organizations have also indicated that they would like to see economic data used to demonstrate the value of technology. One is the Medicare Payment Advisory Commission (MedPAC), a congressional advisory group charged with reviewing Medicare payment policies and making recommendations concerning them. MedPAC is a proponent of pay-for-performance, a strategy it believes will improve care. According to the commission, pay-for-performance is only an initial step toward inducing providers to use resources more efficiently in delivering high-quality care. "Efficiency can then be extended to include how the actions of providers, such as physicians and hospitals, may in one episode of care affect beneficiaries' health and use of services over time and across settings," wrote MedPAC in a report to Congress. "We will build on this work to identify further strategies to bring value to Medicare purchasing."¹¹

Determination of Coverage

Health economics and reimbursement intersect at the point of coverage. At present, payers review the literature to make a determination that a healthcare technology is reasonable and necessary, then factor in its cost-effectiveness. The indications and limitations of coverage are written on the basis of those considerations. Often, the cost-effectiveness analysis is used to limit coverage to the patients who will benefit most from a treatment. What is not said—but is usually true in these circumstances—is that these are the patients for whom the therapy will be applied most cost-effectively.

CMS has made several such decisions recently. The example of the implantable cardioverter-defibrillator is useful to consider again here.

In May of 2002, Guidant Corp. (Indianapolis) applied for expanded coverage based on clinical results from the Multicenter Automatic Defibrillator Implantation Trial II (MADIT II). Those results showed a modest benefit for patients with a previous myocardial infarction and compromised cardiac pumping ability, and the Medicare Coverage Advisory Committee (MCAC) recommended coverage for all of those patients.¹² CMS did not immediately provide that coverage, however. It is likely that the total budget impact of coverage—up to \$3 billion annually—caused concern. Since Medicare is budget-neutral, to come up with the necessary funds would have required severely restricting coverage of other services or drastically reducing payments. Instead, using data not available to MCAC, CMS determined that a small subgroup of patients accounted for most of the benefit received by the full patient population. It was able to offer coverage to this subgroup without

breaking the bank.

The Blue Cross Blue Shield TEC also looked at the cost-effectiveness of ICD therapy in the MADIT II population. Its report showed that ICD implantation improved life expectancy by 1.85 years relative to medical therapy, or about 1.33 QALY. The cost-effectiveness advantage was estimated at about \$51,000 per QALY.² Although the report did not specifically address the subpopulation that Medicare covers, it is likely that the cost-effectiveness is greater—that is, the cost per QALY is lower—in that covered population.

This would appear to be a not overly generous coverage policy in light of the fact that Britain's NHS covers therapies up to \$53,000 per QALY. But CMS also decided to cover left-ventricular assist devices (LVADs), which Blue Cross Blue Shield evaluated at more than \$800,000 per QALY.¹³ This is a generous policy that involves trade-offs; for example, for every patient who receives an LVAD, 15 other patients could have received an ICD.

Conclusion

Although the lack of formal policy makes it difficult to indicate the relationship between health economics and reimbursement definitively, it is clear that payers want, and in some instances already use, health economic data in making decisions.

As today's healthcare crisis worsens, cost-effectiveness analysis in itself will not be enough to control costs. Only a national commitment

to spend a certain strictly limited percentage of the national budget can do that. But within that constraint, cost-effectiveness analysis can help healthcare dollars to be allocated so as to achieve the maximum possible health benefit.

Most healthcare payers will have reasonable incentives to both control costs and maximize benefits—at least for their own enrollees. To attain those ends, they will be looking to companies developing new medical technologies to provide them with information demonstrating the value of products being introduced to the market. Favorable coverage decisions will be awarded more readily to those developers that can show convincingly that their products do indeed offer good healthcare value.

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