CHAPTER 8

Using clinical and cost effectiveness in Medicare

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In this chapter

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Policymakers are looking for ways to use Medicare's resources more efficiently. One way Medicare has done so is by using information about the clinical effectiveness of a service when making coverage decisions and setting payment rates. MedPAC supports CMS's recent effort in linking coverage with a requirement for collecting clinical effectiveness data. By contrast, Medicare does not explicitly consider the cost effectiveness of a service in either the coverage or payment process. Nonetheless, cost effectiveness potentially can promote care that is more cost efficient and higher quality. Before Medicare can routinely use cost effectiveness, policymakers will need to address valid concerns about its methods. The Secretary could play an important role in standardizing the methods used in these analyses. Medicare can begin considering cost effectiveness by collecting this type of information from manufacturers when making coverage decisions (when available), sponsoring cost-effectiveness studies, and using such studies to prioritize pay-for-performance and disease management initiatives.

Considering evidence about the clinical effectiveness and cost effectiveness of alternative health services might be another way to increase the return on society's investment in health care. Cost effectiveness evaluates the clinical effectiveness and resource costs of two or more alternative services, including drugs, medical devices, surgical and diagnostic procedures, and medical treatment strategies. The central function of cost-effectiveness analysis is to assess the relative value of alternative services for improving health. Currently, Medicare does not explicitly consider a service's cost effectiveness when making coverage decisions or setting payment rates. Medicare does, however, consider a service's clinical effectiveness when making coverage decisions and when making payments for certain services.

The first section of this chapter outlines how Medicare uses clinical information when making coverage decisions and setting payment rates. We find that CMS uses an open, evidence-based process when making coverage decisions and considers clinical effectiveness information in the rate-setting process for certain services. MedPAC supports CMS's recent effort in linking coverage with a requirement for prospective data collection.

Next, MedPAC begins to consider the use of cost-effectiveness information by Medicare. Cost-effectiveness analyses can potentially improve Medicare's ability to maximize beneficiaries' health and well-being and might enable the program to achieve better value for its expenditures. Medicare cannot use the dollars that it spends on services that are not cost effective for other important purposes—purposes such as providing other health benefits within and outside Medicare. Medicare, together with other payers and purchasers, is in a strong position to consider such information because it represents the interest of large populations.

Medicare could begin to consider cost-effectiveness analysis in the following specific ways:

- standardizing the methods used to conduct such studies,
- collecting cost-effectiveness information from manufacturers and providers in the coverage process (when available),
- sponsoring cost-effectiveness studies,
- providing cost-effectiveness analyses to beneficiaries and health professionals, and
- using available cost-effectiveness analysis to prioritize pay-for-performance and disease management initiatives.

However, before Medicare can routinely begin to use cost-effectiveness analysis, policymakers will need to address valid concerns about the methods that researchers use in current analyses. Policymakers and other stakeholders cite the lack of a common set of techniques in cost-effectiveness analysis as one reason for their limited use of such a method. The Secretary could play an important role in advancing the field of cost effectiveness by helping to standardize the methods in these analyses. In addition, the Secretary could develop the methods in an open process similar to the current process of making national coverage decisions.

Medicare's coverage and payment processes consider clinical effectiveness

Although Medicare's coverage process does not explicitly consider cost effectiveness, it does consider value by assessing the clinical effectiveness of new services. Medicare also considers clinical effectiveness when determining payment for new services paid through under certain prospective payment systems (PPSs) and for some services not paid through PPSs. However, the Congress recently limited the agency's use of such information when paying for certain services furnished in the hospital outpatient setting.

Making coverage decisions and using clinical effectiveness information

Medicare covers health care services when adequate evidence shows that these services improve health outcomes, regardless of the unit or aggregate cost. In practice, services that are high cost will receive greater scrutiny than other services (Tunis 2005).

Historically, CMS based its coverage determinations on descriptive information as well as scientific and clinical evidence. A general notice that the agency published in 1999 formalized the evidence-based process for making coverage decisions and made the process more transparent and understandable to the public. Using such an evidence-

based approach, CMS assesses whether a given service is reasonable and necessary by determining: (1) if it is safe and effective per the Food and Drug Administration (FDA) regulatory process; and (2) if adequate evidence leads CMS to conclude that the service improves net health outcome.

CMS may limit coverage to specified circumstances based on scientific evidence. For example, in a decision concerning carotid artery stenting, CMS extended coverage to patients who were at high risk of stroke and complications during surgery. To better ensure that patients receive care most appropriate for their needs, the coverage decision also delineated minimum standards that facilities must meet to treat high-risk patients—such as providing necessary imaging equipment, advanced physiologic monitoring equipment, and emergency management equipment and systems.

Recently, CMS is also linking national coverage with participation in comparative clinical trials and data registries in order to determine the effectiveness of new services for Medicare beneficiaries. The agency refers to these comparative clinical trials as "coverage with evidence development" or practical clinical trials.[1] CMS collects the data to ensure patient safety, evaluate the benefit of the service, and improve physician decision making. Ultimately, these data should improve the quality of the available scientific evidence because the current FDA regulatory process provides some but not all information needed for CMS to make evidence-based decisions. These trials can potentially enhance Medicare's ability to assess the effectiveness of new services while providing beneficiaries with access to these services. Information that CMS derives from these trials may enable the agency to refine coverage decisions based on high-quality evidence.

The characteristic features of practical clinical trials are that they: (1) select clinically relevant alternative services to compare; (2) include a diverse population; (3) recruit participants from heterogeneous practice settings; and (4) collect data on a broad range of health outcomes (Tunis et al. 2003). Recent examples of these trials include:

- ***FDG-PET (2-deoxy-2- [F-18] fluoro-D-glucose positron emission tomography) scans for the diagnosis of patients who have mild cognitive impairment or shows signs of early dementia.*** CMS will collaborate with the National Institute on Aging, the Agency for Healthcare Research and Quality (AHRQ), the Alzheimer's Association, manufacturers, and other experts to develop a large practical clinical trial.
- ***Percutaneous transluminal angioplasty of the carotid artery with stenting.*** CMS will cover this technology when medical providers furnish it in accordance with FDA-approved protocols that govern postapproval studies.
- ***Off-label uses of four anticancer drugs: xaliplatin (Eloxatin®), irinotecan (Camptosar®), bevacizumab (Avastin®), and cetusimab (Erbitux®)***. CMS will cover these drugs for beneficiaries in certain clinical trials sponsored by the National Cancer Institute.

What do these services have in common? They are either new or a new use of an existing service, they are costly, they have the potential for high use, and current scientific evidence is inadequate for certain populations of interest. For example, the four anticancer drugs are costly. One of the new drugs to treat colorectal cancer costs about $30,000 when used with other agents for an eight-week course of treatment (Schrag 2004).

Finally, paying for the costs of routine care for patients in FDA clinical trials—which began in September 2000—is another way in which Medicare has strengthened its clinical evidence base. CMS pays the routine costs of care for patients who enroll in trials that meet certain criteria.[2] From the information collected in clinical trials, Medicare can begin to learn about the effectiveness of new services. In addition, the MMA authorizes the AHRQ to conduct and support research studying the outcomes, comparative clinical effectiveness, and appropriateness of health care items and services.

Setting payment rates and use of clinical effectiveness information

Some of Medicare's PPSs consider the clinical effectiveness of new technologies in the rate-setting process. For example, for a new technology to be eligible to receive a pass-through payment in the inpatient PPS, it must represent an advance in medical technology that substantially improves (relative to services previously available) diagnosis or treatment. For new-technology pass-through payments under the hospital outpatient PPS, medical devices must meet the same criteria.

For services not covered under PPSs, CMS has set a new service's payment rate the same as that of an existing service after concluding that both services are clinically

comparable. In 2003, CMS set the payment rate for a new service (a biological) at the same rate as that of an existing service after concluding that both services were functionally equivalent. The new service was darbopoetin alfa (Aranesp®), and the existing service was erythropoietin (Procrit® and Epogen®). Specifically, the agency concluded that both products were functionally equivalent because they used the same biological mechanism to produce the same clinical result—stimulation of the bone marrow to produce red blood cells.

Section 622 of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) limits the use of the functional equivalence standard. The Congress prohibited the use of this standard for drugs and biologicals in the hospital outpatient setting. However, the MMA did not preclude the agency from setting the payment rate the same for other clinically comparable services in other settings. Under the "least costly alternative" policy, Medicare's contractors (carriers and fiscal intermediaries) may deny coverage for the additional cost of a more expensive service if a clinically comparable service costs less.[3]

Carrier policies related to two drugs illustrate how this policy works. During the last several years, many carriers have implemented a least costly alternative for two drugs used to treat prostate cancer—leuprolide acetate (Lupron®) and goserelin acetate (Zoladex®)—administered in physicians' offices. Current payment for Lupron is $226.66 versus $192.68 for Zoladex. According to the Office of Inspector General (OIG), carriers implemented a least costly alternative in 47 of 57 jurisdictions in 2003 (OIG 2004). Thus, in these jurisdictions, contractors paid physicians the payment amount for Zoladex when they furnished Lupron. In some instances, contractors paid the higher payment amount if the physician documented why the more costly treatment option was medically necessary. The OIG recommended that all carriers apply a least costly alternative for Lupron.

Understanding cost-effectiveness analysis

For more than 25 years, researchers have used cost-effectiveness analysis as a technique for economic evaluation in health care. This tool is used by some commercial health plans and purchasers, most frequently for understanding the value of new drugs. Many medical directors believe that cost-effectiveness analysis can and should play a greater role. Nonetheless, some stakeholders fear that the explicit use of cost effectiveness by public and private payers could harm patients' access to care, negatively affect the innovation of new services, and lead to the rationing of care.

What is cost-effectiveness analysis?

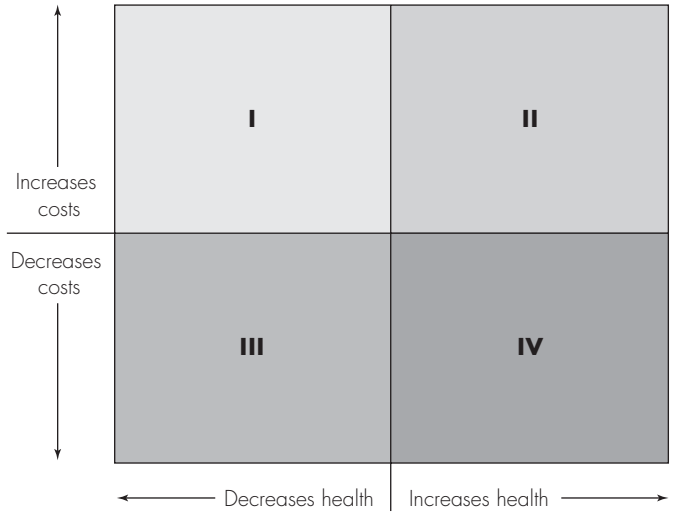
Cost-effectiveness analysis involves estimating the costs and health outcomes of a service and its alternatives. Researchers usually summarize their results in a series of cost-effectiveness ratios that show the cost of achieving one unit of health outcome for different kinds of patients and alternative services.

Cost-effectiveness analysis is closely related to cost–benefit analysis. Although both types of analyses consider costs and benefits, the key difference is how researchers measure benefits. In cost–benefit analysis, researchers express benefits in monetary terms, whereas cost-effectiveness analysis presents benefits in terms of health outcomes.

Researchers often measure health outcomes in terms of years of life gained, cases of a particular disease prevented, or improvements in functional status. Researchers also commonly use improvements in health-related quality-of-life years as a measure. The quality-adjusted life year (QALY) is a measure of health outcome that assigns to each time period a weight (ranging from 0 to 1) that corresponds to the quality of life during that period. It is the arithmetic product of life expectancy and a measure of the quality of the remaining life years. QALYs provide a common currency to assess the extent of the benefits that patients gain from a variety of services in terms of health-related quality of life and survival. Although use of QALYs in cost-effectiveness analysis is widespread, some researchers are concerned that these measures do not adequately reflect societal values (Nord et al. 1999).

By providing estimates of outcomes and costs, cost-effectiveness analysis shows the tradeoffs involved in choosing among services. That is, the analysis provides information about the opportunity cost of each service. We can think of the value of services—in terms of their net costs and net outcomes—as a grid, with four quadrants showing the impact of services as either increasing or decreasing health and either increasing or decreasing costs (Figure 8-1).

FIGURE 8-1 **The impact of services on outcomes and costs**



Note: This figure shows the impact of services in terms of their net costs and net outcomes as a grid, with four quadrants showing the impact of services as either increasing or decreasing health and either increasing or decreasing costs.

Researchers refer to a service that is more effective and less costly than its alternatives as "dominant." In Figure 8-1, dominant services fall into the lower right quadrant (IV). A service that is more costly and more effective than its alternatives falls into the upper right quadrant (II). Table 8-1 presents the cost-effectiveness ratios of selected services that beneficiaries use; we present this table for illustrative purposes only. Among the selected services we include in the table, influenza vaccination is "dominant." The remainder of the services fall into quadrant II—they improve health but increase costs. The cost-effectiveness ratios range from less than $10,000 per QALY for beta blocker after acute myocardial infarction to over $500,000 per QALY for left ventricular assist devices and positron emission tomography (PET) for Alzheimer's disease (Gillick 2004, Neumann 2005a).

How have cost-effectiveness analyses evolved in health care?

In the 1960s and early 1970s, policymakers applied cost-effectiveness analysis to a variety of health issues, including kidney disease and maternal and child health programs. Beginning in the 1970s, cost-effectiveness analyses of health issues began to appear in major medical journals. Since then, researchers have developed models to compare costs and outcomes for services ranging from:

- ***drugs***—(e.g., those used in combination antiretroviral therapy for HIV disease);
- ***preventive services***—(e.g., vaccination against pneumoccocal pneumonia);
- ***screening***—(e.g., for HIV and different types of cancers and chronic diseases, such as chronic kidney disease);
- ***services***—(e.g., early hospital discharge after uncomplicated acute myocardial infarction and smoking-cessation services); and
- ***procedures***—(e.g., bypass surgery for coronary artery disease and lung-volume-reduction surgery (Boulware et al. 2003, Eddy 1989, Paltiel et al. 2005, Willems et al. 1980).

The number of cost-effectiveness analyses has grown steadily (Elixhauser 1998). General medical, medical specialty, public health, and policy journals publish more than 100 studies per year (Gold et al. 1996). Neumann (2005b) reported that about 40 percent of all published cost-effectiveness studies assess the value of pharmaceuticals (Neumann 2005b). This investigator found that fewer studies are published assessing the cost

TABLE 8-1 **Cost effectiveness of selected services in the Medicare population**

Technology	Cost-effectiveness ratio (2002$/QALY)
Influenza vaccine	Cost saving
Beta blocker after acute myocardial infarction	Under $10,000
Cholesterol management, secondary prevention	$1,000–$50,000
Dialysis for ESRD	$50,000–$100,000
Lung volume reduction surgery	$100,000–$300,000
Left ventricular assist devices	$500,000+
PET for Alzheimer's disease	$500,000+

Note: QALY (quality-adjusted life year), ESRD (end-stage renal disease), PET (positron emission tomography). The cost-effectiveness ratio is expressed in 2002 dollars spent for each additional year of life at full quality gained.

Source: Gillick 2004, Neumann 2005a.

effectiveness of other types of services, such as surgical interventions, screening services, and medical and diagnostic procedures. The availability of efficacy data on drugs from FDA clinical trials partly accounts for the higher proportion of published studies assessing drugs. In addition, manufacturers' need to show the value of a new drug to formulary committees and other purchasers may also play a role, as discussed below. Manufacturers also use cost-effectiveness analysis to predict the price that purchasers will be willing to pay for a new drug (Neumann 2005b).

Over the years, pharmaceutical manufacturers have sponsored an increasing proportion of cost-effectiveness analyses. Neumann (2005b) estimates that their share increased from 14 percent between 1976 and 1997 to 20 percent between 1998 and 2001, while government- and foundation-sponsored studies decreased from 54 percent to 43 percent. About one-third of all studies did not report the funding source during each time period.

Designing cost-effectiveness analysis

When measuring the clinical effectiveness, outcomes, and costs of alternative services, researchers must construct a conceptual model. Such models range from the simple (such as decision trees) to the complex (such as Markov models).[4] A cost-effectiveness analysis typically addresses the following methodological issues:

- ***The perspective of the analysis.*** The findings of a cost-effectiveness analysis vary depending on the viewpoint of interest to the researcher—society, purchaser, insurer, or another party. A cost-effectiveness analysis from a societal perspective includes everyone who is affected by the service; it also includes all associated health outcomes and costs (Gold et al. 1996). By contrast, a cost-effectiveness analysis from an insurer's perspective would include only those outcomes and costs that affect that particular insurer.
- ***The sources of clinical effectiveness and outcomes data.*** Researchers can use data from numerous sources, including FDA clinical trials and practical clinical trials, patients' medical records, health care claims submitted to insurers, and health surveys.
- ***The method of defining costs.*** Costs include direct medical (e.g., cost of medical services), direct nonmedical (e.g., transportation costs), and indirect (e.g., value of lost productivity). For example, lost productivity measures the costs associated with lost or impaired ability to work or to engage in leisure activities, and lost economic productivity due to death.
- ***The selection of comparison services.*** Comparative groups can include pharmaceutical, medical, and surgical services, or no treatment.
- ***The time horizon.*** Researchers must choose the period of time to measure a service's costs and outcomes.
- ***The discounting of costs and outcomes.*** When the time horizon of cost-effectiveness analyses extends into the future, researchers must convert future costs and future health outcomes to their present value. In doing so, researchers appropriately adjust the cost-effectiveness ratios for the different timing of cost and outcomes. The discount rates that researchers use to convert health outcomes and costs to a present value can differ.
- ***The uncertainty of the clinical events and costs.*** Sensitivity analysis varies the assumptions of the clinical and cost data.
- ***The measurement of outcomes.*** As we mentioned earlier, researchers measure outcomes in terms of QALYs, cases of a particular disease prevented, or improvements made in functional status.

Recognizing the complexity of cost-effectiveness analysis, several groups have published guidelines designed to ensure and improve the quality of such analyses. In 1993, the Public Health Service convened the U.S. Panel on Cost Effectiveness in Health and Medicine to address methodological concerns about cost effectiveness. The panel reviewed the state of the health and medicine field and developed recommendations to improve the quality and comparability of these types of studies.[5] In addition to this panel, some peer-reviewed journals have also developed publication standards for cost-effectiveness studies.[6]

One study shows that more recently published cost-effective analyses are adhering to the guidelines of the panel (Neumann 2005c). Comparing studies published in 1998 to 2001 with those published in 1976 to 1997, studies improved in almost all categories, including: clearly presenting the study perspective (73 percent versus 52 percent, respectively); discounting both costs

and outcomes (82 percent versus 73 percent, respectively); and reporting incremental cost-effectiveness ratios (69 percent versus 46 percent, respectively).

Who uses cost-effectiveness analysis?

The experience of public and private entities regarding cost-effectiveness analysis varies. Cost-effectiveness analysis is not explicitly used by Medicare, and is used by some organizations to develop clinical guidelines and—on a limited basis—by health plans and purchasers. Other countries use cost-effectiveness analysis more widely than the United States.

Use of cost-effectiveness analysis by public and nonprofit entities

At least two organizations consider cost-effectiveness analysis when developing guidelines—the recent recommendations of the third U.S. Preventive Services Task Force (USPSTF) and the *U.S. Guide to Community Preventive Services*.[7] USPSTF, an independent panel of private-sector experts in primary care and prevention, considered cost-effectiveness studies in its recommendation concerning screening for abdominal aortic aneurysms, coronary heart disease, and bacteriuria. The *U.S. Guide to Community Preventive Services* examines population-based health promotion and disease prevention services. This group considered selected economic evaluations, including cost-effectiveness analysis, for the following topics: diabetes, oral health, physical activity, and tobacco. The task force used these analyses to make the case that the intervention was valuable and should be incorporated routinely into primary medical care.

The National Committee for Quality Assurance (NCQA) has recognized the importance of cost-effectiveness information. In selecting a measure for a particular clinical condition, the committee considers cost-effectiveness information. For example, in its *State of Health Care Quality Report*, it includes estimates of the incremental cost effectiveness of conducting conventional pap screening every three years, compared with conducting no pap screening (NCQA 2004). This effort shows how policymakers can use cost-effectiveness analysis in prioritizing which measures to use in pay-for-performance programs and how frequently providers should furnish these services to patients.

One state—Oregon—experimented with using cost-effectiveness analysis to help reform its Medicaid program. The state attempted to rank different services based on their cost effectiveness and cover only those services that fell above a line established by the state's budgetary resources. Ultimately, policymakers considered information on cost effectiveness less formally in the plan Oregon eventually adopted because of disputes surrounding its use. Specifically, stakeholders criticized the initial priority list that ranked services based on their cost effectiveness as being counterintuitive, assigning higher priorities to some services that seemed less important than other lower ranked services (Eddy 1991).

Use of cost effectiveness by commercial health plans and purchasers

Health plans, pharmacy benefit managers (PBMs), and hospitals have used cost-effectiveness analysis, along with other types of pharmacoeconomic analysis, for the past two decades (Neumann 2005b). Pharmacoeconomic data include cost-effectiveness analysis and other types of health economic analyses, such as cost–benefit, cost-of-illness, and cost-of-care studies.

The 1997 Food and Drug Administration Modernization Act codified rules governing the health economic information that manufacturers can disseminate to plans and purchasers. The act permits manufacturers to disseminate health economic information, provided that this information directly relates to an approved indication of a service and results from reliable scientific evidence.

Commercial plans and purchasers frequently consider evidence about a new service's cost rather than its cost effectiveness when making coverage decisions. Cost information supplements the clinical effectiveness information that plans use in making these decisions (Project HOPE 2002). A survey of medical directors of 228 managed care plans in 2001 indicates that 90 percent of the plans consider the cost of a new service (Garber 2004). The survey results also indicate that:

- Nearly all plans (93 percent) will cover a more effective service, even if it is more costly.
- Plans use cost information the most frequently (58 percent) to design policies that require the use of less costly (but equally effective) services first.

By contrast, plans consider formal cost-effectiveness analyses to assess new services less frequently. In one survey, only 40 percent of the plans reported using cost-effectiveness analysis (Garber 2004). Another survey found that 51 percent of private payers used either cost-effective or cost–benefit analysis (Bloom 2004).

The Blue Cross Blue Shield Evaluation Center, which provides technology assessments to subscribing commercial health plans and provider groups, uses an evidence-based process for assessing services but generally excludes explicit considerations of cost and cost effectiveness (Garber 2001). Instead, the center relies primarily on clinical evidence.[8]

Purchasers more frequently consider cost-effectiveness information to inform coverage decisions about drugs than about other services. To consider such information appropriately, formulary managers have increasingly adopted the Academy of Managed Care Pharmacy's (AMCP's) new evidence-based formulary guidelines, which call for drug manufacturers to submit clinical and economic evidence about their products to support the listing of new pharmaceuticals (AMCP 2005). These guidelines, the *Principles of a Sound Drug Formulary System*, were developed by a coalition of national organizations and lay out the essential components of a drug formulary system. AMCP supports the consideration of pharmacoeconomic factors when making formulary decisions, after establishing a drug's safety, efficacy, and therapeutic need.

Why do commercial health plans not use cost-effectiveness analysis more widely? Concerns about potential litigation may discourage them from explicitly using such analysis in coverage decisions. In one survey of health plan officials, most respondents said that they would cover equally effective but costlier treatments for fear of litigation or backlash (Singer et al. 1999). To date, very little litigation has directly raised or challenged the use of cost-effectiveness analysis (Jacobson and Kanna 2001). Researchers also note that if the medical profession begins to accept cost-effectiveness analysis underlying its standards of care, the courts could incorporate the information by deferring to professional custom.

Lack of understanding about the value and applicability of cost-effective analysis may also limit its use. Issues surrounding the methods used to conduct studies may be another factor. We discuss some of these issues later in this chapter. Prosser and colleagues (2000) report that plans may not use cost-effectiveness analysis because their members may view such analysis as a tool to ration care.

Use of cost effectiveness internationally

The international experience sharply contrasts with that of the United States. For a number of years, health systems in Australia, the United Kingdom, and other countries have incorporated cost-effectiveness considerations explicitly into their processes for making coverage and pricing decisions about drugs and other services.[9] For example:

- Since 1992, Australia requires drug companies to submit evidence on the comparative cost effectiveness of new pharmaceuticals before listing them on the national formulary, and this information guides the government's decisions on paying for new drugs. Companies cannot list new drugs on the national formulary unless an independent statutory body (the Pharmaceutical Benefits Advisory Committee) recommends it. Between 1993 and 2000, the pharmaceutical industry in Australia submitted more than 300 studies (Hill et al. 2000).
- In the United Kingdom, the National Institute for Health and Clinical Excellence (NICE) provides nonbinding guidance to the National Health Service (NHS) on treatments and care for people who use the NHS in England and Wales. NICE develops technology assessments on the use of new and existing services and clinical guidelines on the appropriate treatment of specific diseases and conditions. NICE's technology assessments consider both clinical effectiveness and cost-effectiveness information.

However, the consideration of clinical effectiveness and cost-effectiveness information internationally has not proceeded without some disputes. For example, some patient groups and manufacturers have raised concerns about a January 2005 preliminary recommendation by NICE that did not support the use of three drugs for treating mild to moderate Alzheimer's disease (NICE 2005). NICE will release its final guidance in July 2005.

Some concerns surrounding Medicare's use of cost-effectiveness analysis

Numerous stakeholders—drug and device manufacturers, providers, beneficiaries, and health economists—have raised issues and concerns about Medicare's use of cost-effectiveness information in the coverage process. Stakeholders have also raised some of these same concerns about the use of such information by other public and private payers and purchasers.

- ***Use of cost effectiveness might impair beneficiaries' access to certain services and will lead to rationing.*** For example, a policy that covers only those services that have cost-effectiveness ratios below a specific threshold would result in beneficiaries not having

access to all services. Critics are concerned that Medicare will use cost-effectiveness information for cost containment purposes only, not for promoting appropriate care.

- ***Some policymakers, providers, and beneficiaries may not understand cost-effectiveness methods.*** Cost-effectiveness analysis requires a kind of abstract thinking that might be counterintuitive to some individuals because it ranks treatments by their cost-effectiveness ratios instead of by their benefits (Eddy 1992).

- ***Some policymakers, providers, and beneficiaries may mistrust the methods used to conduct cost-effectiveness analysis.*** Researchers have noted that the methodological approach varies from study to study. Evaluations of the same services and diseases can show different results. In assessing the cost effectiveness of treating patients with diabetes, Eddy (2005) compared five models that used the same quality weights and cost per treatment. He found that cost-effectiveness ratios varied from about –$10,000 per QALY to nearly $40,000 per QALY.

  Although the U.S. Panel on Cost Effectiveness in Health and Medicine recommended that published studies include a reference case that uses a standard set of methods, many published analyses do not do so. The lack of clear reporting on methods has led to concerns from some stakeholders that cost-effectiveness analysis is not transparent and that analyses are "black boxes." Finally, some stakeholders are concerned that analyses contain the biases of the sponsors who fund the studies and the researchers who conduct them.

- ***Cost-effectiveness analysis might slow innovation.*** Medicare's coverage policies strongly influence the medical care that beneficiaries receive for services not covered under PPSs. (For services paid through PPSs, providers serve as the purchaser and make decisions about which services to furnish to beneficiaries.) Because Medicare covers more than 40 million beneficiaries, a negative coverage decision could have an enormous effect on manufacturers' revenues. Manufacturers have noted that a noncoverage decision by Medicare has a much greater impact on them than the coverage decisions of individual commercial health plans. In addition, other payers—including commercial health plans and Medicaid—often follow Medicare's policies.

- ***CMS may not have the statutory authority to consider costs.*** Section 1862 of the Social Security Act gives the Secretary the authority to cover items or services that are "reasonable and necessary" for the diagnosis or treatment of illness or injury or that improve the functioning of a malformed body member. Some stakeholders question whether the Secretary has the authority to consider the value of a service—in terms of its costs or cost effectiveness—when making a determination of reasonable and necessary.

- ***Cost effectiveness may not capture public preferences for allocating limited resources.*** Some stakeholders contend that cost effectiveness might be an aid to decision making, but it is not a complete procedure for making resource allocation decisions because it cannot incorporate all the values relevant to such decisions.

Medicare's coverage and payment processes do not explicitly use cost-effectiveness analysis

Although the national coverage process considers clinical effectiveness, it generally does not consider clinical and cost information together—that is, cost effectiveness. Only in one instance—for a colorectal screening test—has CMS explicitly considered the cost effectiveness of a service when making a national coverage decision and setting the payment rate (see text box, p. 188).

On several occasions, CMS tried to interpret the statute's requirement that Medicare only pay for services that are reasonable and necessary by including either cost effectiveness or added value considerations. In 1989, the agency published a proposed regulation stating that for purposes of coverage, the medical community would have to accept a technology as safe, effective, noninvestigational, and appropriate. CMS also included cost effectiveness as an explicit criterion. Stakeholders criticized the proposal, particularly for its cost-effectiveness provision, and the agency withdrew the proposal in 1999.

Later, in 2000, CMS published a notice of intent outlining the criteria the agency would use when making national coverage decisions. The criteria considered the cost only for services that provided equivalent benefits to an

CMS's use of cost effectiveness for a new service

The Balanced Budget Act of 1997 extended coverage of colorectal screening tests to Medicare beneficiaries. To carry out the law, CMS first asked the Agency for Healthcare Research and Quality (AHRQ) to conduct a technology assessment of immunochemical fecal occult blood tests (iFOBTs) to:

- compare iFOBT to guaiac-based fecal occult blood test (gFOBT) in terms of cancers detected, cancer deaths averted, and costs;
- assess cost effectiveness; and
- estimate payment levels of iFOBT at which cost effectiveness would equal that of gFOBT at current Medicare payment.

Based on information from this technology assessment and other sources, CMS concluded that there was adequate evidence for Medicare to cover iFOBT. The results of this analysis demonstrate that CMS can use cost-effectiveness information in developing payment rates. ■

existing covered alternative but that were more costly (Figure 8-2). Again, because of strong opposition, CMS never issued a proposed regulation. Foote (2002) noted that resistance by affected interest groups was one element that delayed action.

The future of cost-effectiveness analysis in Medicare

In recent years, Medicare is using its resources more efficiently by assessing the clinical effectiveness of services when making coverage decisions and when setting payment rates for certain services. MedPAC supports CMS's efforts in using an evidence-based, transparent process when making coverage decisions and, more recently, in implementing practical clinical trials and data registries as a means to obtain better scientific evidence.

Might cost-effectiveness analysis also improve Medicare's ability to obtain better value for its expenditures? Cost effectiveness has the potential to favor medical services that are more likely to improve patient outcomes and to discourage the use of services with fewer benefits. Cost-effectiveness analysis may not save the Medicare program money. Wider use of cost-effective, underutilized services might result in increasing Medicare spending, which might not be offset with savings elsewhere.

On the other hand, cost effectiveness could save the Medicare program money in the long run if its use by the program encourages manufacturers to develop services that are more cost effective than current ones. Manufacturers might bring more cost-effective products to the market, if doing so could allow them to increase their share of Medicare's market.

Medicare could begin to consider cost-effectiveness analysis in four ways. First, the program could begin to collect cost-effectiveness information during the coverage process. If feasible, CMS could collect the data via data registries and practical clinical trials after the agency agrees to cover a service. In addition, manufacturers that have already prepared cost-effectiveness analyses could share these analyses with the agency. Such analyses could help the agency better understand the value of a new service. Almost all large drug and medical device companies have formalized the conduct of cost-effectiveness analysis within their firms (DiMasi et al. 2001).

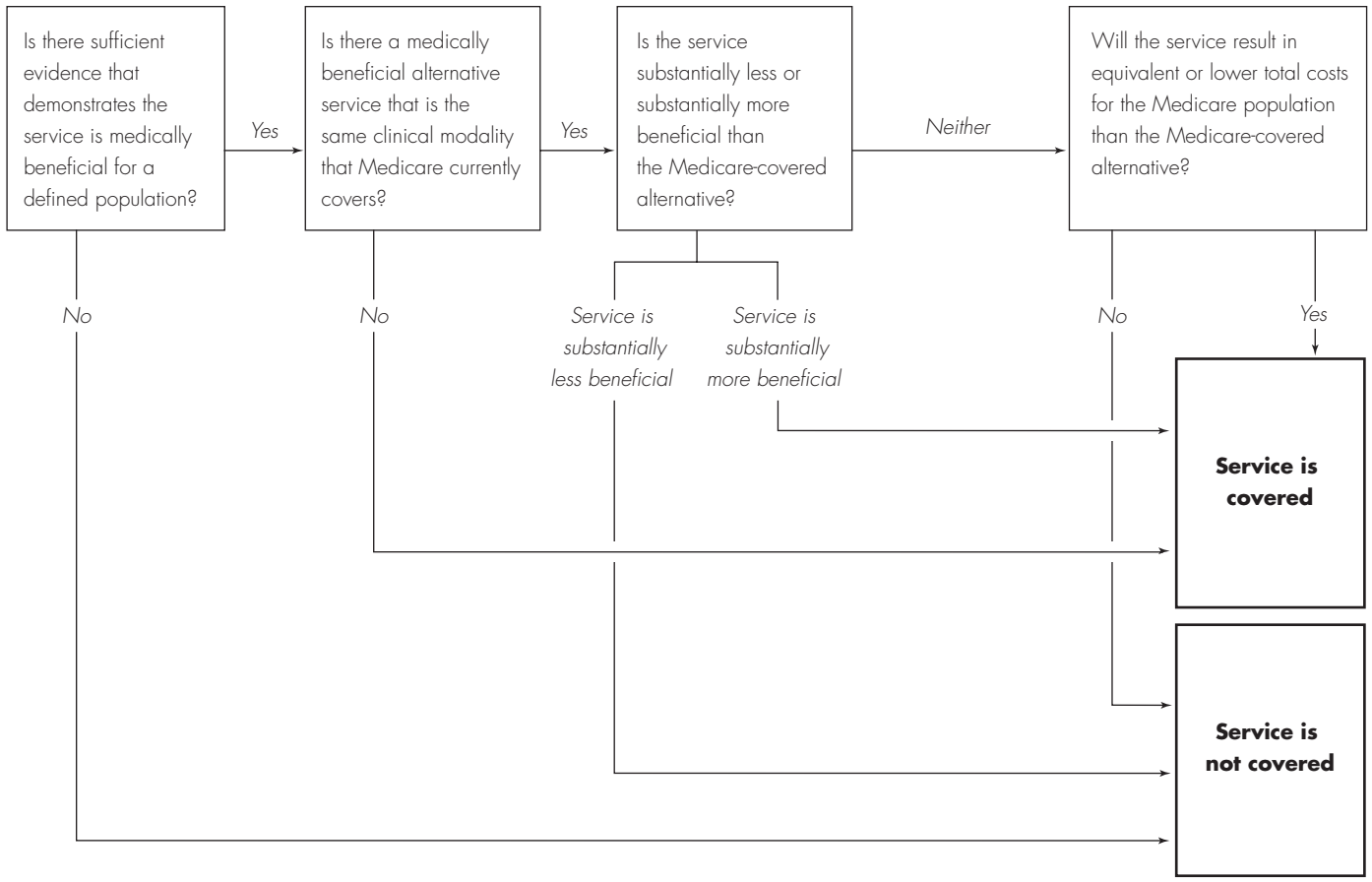
Second, the Secretary could sponsor cost-effectiveness studies—but these studies will be successful only if the research is independent. The Secretary could conduct the studies or could sponsor other organizations—such as quasi-public entities or independent private organizations—to do so. AHRQ has already conducted cost-effectiveness studies and technology assessments

for CMS to use in the national coverage process. In addition, AHRQ—along with its predecessor agency, the National Center for Health Services Research—has sponsored methodological work in this field (Power and Eisenberg 1998). The National Institutes of Health have also sponsored internal and external research on cost effectiveness. Neumann (2004) raises the possibility of contracting with quasi-public entities, such as the Institute of Medicine, to conduct such studies. Alternatively, Reinhardt (2001, 2004) suggests that independent research institutes conduct cost–benefit analyses on drug therapies. The Secretary would also need to determine the services on which to focus—for example, services with high costs and the potential for high use versus services for which little cost-effectiveness information is available (such as surgical and diagnostic procedures and medical devices).

Third, Medicare could provide cost-effectiveness analysis to beneficiaries and health professionals. Both are potential audiences for information about the relative value of treatment alternatives that cost-effectiveness analysis can provide. The traditional Medicare program does not encourage providers and beneficiaries to weigh the costs and benefits of a service when making health care decisions. Medicare does provide some clinical effectiveness information about certain providers—

FIGURE 8-2

Proposed criteria for making coverage decisions



Note: CMS never officially implemented this set of criteria, which was issued in a notice of intent.

Source: CMS 2000.

dialysis facilities, hospitals, home health agencies, and nursing homes—but not cost-effectiveness information.

A consortium of health-related organizations conducted a project in which consumers participated in discussion groups and physicians responded to a survey and participated in discussion groups on the use of cost effectiveness. The results suggest that the former are interested in obtaining better information and that the latter consider cost effectiveness when making clinical decisions (Ginsburg 2004; Sacramento Healthcare Decisions 2001). This project included the following key findings:

- Physicians vary in how often they discuss cost effectiveness with their patients: 50 percent do so occasionally, 30 percent do so frequently or always, and 20 percent report that they never do.
- Most physicians (90 percent) either agreed strongly or agreed somewhat that it is appropriate for them to consider cost effectiveness when making clinical decisions.
- Many consumers accept cost effectiveness as a reasonable criterion when doctors consider treatment alternatives for individual patients.
- Consumers also indicated that they need to take more responsibility in their role as health care recipients, to improve their individual well-being as well as to reduce costs.

This research shows that patients and providers can—and sometimes do—consider cost-effectiveness information. Nonetheless, Medicare, together with other payers and purchasers, is in a strong position to disseminate such information because it represents the interest of large populations.

By using cost-effectiveness analysis, Medicare might promote other organizations' use of this analysis. For example, more commercial health plans might begin to consider cost-effectiveness analysis; as mentioned earlier, less than half of the surveyed plans consider cost-effectiveness analysis. Medical organizations and federal agencies might also consider using cost-effectiveness analysis to develop clinical guidelines.

Fourth, Medicare could begin to use available cost-effectiveness analysis to prioritize pay-for-performance and disease management initiatives. Consider the screening of chronic kidney disease among the Medicare population: Cost-effectiveness analyses could help inform policymakers about which subpopulations (such as beneficiaries who have diabetes) would generate the most favorable ratios of health gain to spending. USPSTF has demonstrated the usefulness of cost-effectiveness analysis to determine optimal interventions for screening and to identify the different target populations or risk groups who might be suitable for preventive services (Saha et al. 2001).

Before Medicare can routinely use cost-effectiveness analysis for any of these purposes, it will need to address valid concerns about the methods used in current analyses. The measurement of costs and outcomes differ from study to study. As we mentioned earlier, evaluations of the same services and diseases can show different results. The lack of a standardized method of cost-effectiveness analysis has limited its use by policymakers (Gold et al. 1996).

The Secretary could play an important role in advancing the field of cost effectiveness. As mentioned earlier, the U.S. Panel on Cost Effectiveness in Health and Medicine made important contributions in addressing methodological concerns. The Secretary could help standardize the methods used to conduct these analyses in an open process similar to the current national coverage process. This action will improve the quality of cost-effectiveness studies, in turn increasing their usefulness to patients, private and public payers, policymakers, and health professionals.

As the field of cost effectiveness evolves and as Medicare and researchers address methodological issues, Medicare could begin to apply cost-effectiveness analysis in its rate-setting process. This method might augment the tools that Medicare now uses in the rate-setting process, such as the "least costly alternative" policy. ■

Endnotes

1 Practical clinical trials address questions about a service's risks, benefits, and costs as they would occur in routine clinical practice (Tunis et al. 2003). In practical clinical trials, researchers select clinically relevant interventions to compare, include a diverse population of study participants, recruit participants from a variety of practice settings, and collect data on a broad range of health outcomes. Researchers conduct these trials in "real-world settings" with minimal intrusion on care.

2 CMS will pay for beneficiaries' routine costs in clinical trials for those trials that: (1) evaluate a service included in a Medicare benefit category; (2) assess the clinical efficacy of a service; and (3) enroll patients with a diagnosed disease rather than healthy volunteers.

3 CMS contracts with companies, known as fiscal intermediaries and carriers, to process and reimburse Part A and Part B claims.

4 Decision-tree models represent the sequence of chance events and decisions over time for an intervention and each comparative service. A Markov model is a special type of state-transition model in which the transition probabilities depend only on the current state, not on the previous states.

5 Among the panel's recommendations is one regarding the use of a reference case, in which researchers should use a standard set of methods. For example, the reference case analysis should compare the proposed service of interest to existing practice.

6 For example, the *New England Journal of Medicine* developed a policy for the review of cost-effectiveness analyses intended to preclude financial conflicts of interest that might affect the choice of methods or data that researchers use in an analysis (Kassirer and Angell 1994). The journal announced that it would not publish cost-effectiveness analyses if an author has a financial relationship with a sponsoring company.

7 The USPSTF, convened by the Public Health Service, evaluates clinical research to assess the merits of preventive measures, including screening tests, counseling, immunizations, and preventive medications.

8 The Blue Cross Blue Shield Technology Evaluation Center uses five criteria for evaluating services: (1) the technology must receive final approval from the appropriate government regulatory bodies; (2) the scientific evidence must permit conclusions by the Commission concerning the technology's effect on health outcomes; (3) the technology must improve the net health outcome; (4) the technology must be as beneficial as any established alternatives; and (5) the improvement must be attainable outside the investigational settings.

9 Other countries that consider cost-effectiveness information include Canada, Denmark, Finland, France, Hungary, Ireland, Italy, New Zealand, the Netherlands, Norway, Portugal, Spain, and Switzerland.

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