

CHAPTER 10

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**Medicare's use of clinical and  
cost-effectiveness information**

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## Medicare's use of clinical and cost-effectiveness information

### Chapter summary

Policymakers are looking for ways to use Medicare's resources more efficiently and to address the long-term sustainability of the program. Cost-effectiveness analysis is a technique for comparing the costs and health outcomes of various clinical strategies. It shows the relative value of alternate services, including drugs, devices, diagnostic and surgical procedures, and medical services. Cost effectiveness has the potential to promote care that is more efficient and of higher quality.

Some researchers contend that the benefits from technological innovations more than justify the rising costs of health care (Cutler and McClellan 2001). By contrast, other researchers question whether spending more on medical care always leads to improved outcomes (Fisher et al. 2003). Skinner and colleagues (2006) found that regions experiencing the largest spending gains were not realizing the greatest improvements in patient outcomes. For at least one condition (acute myocardial infarction), survival gains have stagnated while spending has continued to increase since 1996.

### In this chapter

- Do cost-effectiveness ratios vary for colorectal cancer screening and ICDs?
- Improving the comparability of cost-effectiveness analyses
- Future issues

CMS considers clinical effectiveness information when making national coverage decisions and paying for some services. By contrast, Medicare does not consider the cost effectiveness of a new service. The variability of the methods used in cost-effectiveness studies was one of several issues that stakeholders raised when CMS unsuccessfully tried to include a service's cost effectiveness or value in the national coverage process in 1989 and 2000.

Different methods used in cost-effectiveness studies can produce disparate results from evaluations of the same services and illnesses (Pignone et al. 2002). Published recommendations for conducting and reporting such evaluations do not cover every aspect of a study's design. In the Commission's June 2005 report, we concluded that before Medicare could routinely use cost-effectiveness analysis, policymakers would need to address concerns about its methods, such as how to measure outcomes and costs (MedPAC 2005).

In this chapter, we consider the variability of the results across cost-effectiveness studies for the same service. We provide results of a review of the methods and findings from cost-effectiveness studies published in the medical literature for two Medicare-covered services—screening for colorectal cancer and implantable cardioverter defibrillators (ICDs) (Cohen et al. 2006).

This review shows some challenges and opportunities for the use of cost-effectiveness information by Medicare. Although some of the assumptions used across studies are consistent, differences in the models used, populations and comparators studied, and the clinical data and costs considered all contribute to the variation in the cost-effectiveness ratios for both services.

Despite the variation in the cost-effectiveness ratios for colorectal cancer screening, the literature suggests the service's clinical effectiveness and good value. By contrast, the literature for ICDs does not provide a clear indication

of the service's cost effectiveness because the results vary substantially across studies. The main reasons for this variation for this service are differences in the clinical characteristics of patients and the effectiveness of ICDs as measured by major clinical trials.

The Commission plans to explore ways for the Secretary to develop the infrastructure to consider information on both the clinical and cost effectiveness of a service. We will look at issues such as whether Medicare should solely sponsor and fund the research or whether a public-private partnership is appropriate. We will also examine how Medicare would set priorities for which cost-effectiveness analyses to sponsor.

The Commission also intends to explore other ways Medicare can use this information. Among these are: 1) providing cost-effectiveness information to beneficiaries and health professionals; 2) using cost-effectiveness analysis to prioritize pay-for-performance, screening, and disease management initiatives; and 3) using cost-effectiveness information in Medicare's rate-setting process. We also plan to explore the use of cost-effectiveness information by other payers in the United States and internationally. ■



Considering evidence about the clinical and cost effectiveness of health services might increase the return on society's investment in health care. Cost effectiveness evaluates the clinical effectiveness and resource costs of two or more alternate services, including drugs, medical devices, surgical and diagnostic procedures, and medical treatment strategies.

In this chapter, we consider the variability of results across cost-effectiveness studies for colorectal cancer screening and implantable cardioverter defibrillators (ICDs). Although some of the assumptions used across studies are consistent, differences in the models used, populations and comparators studied, and the clinical data and costs considered result in variation in the findings across studies. The two case studies help us understand why such variation occurs and assists us in thinking about how Medicare might use cost-effectiveness information.

The Commission plans to explore ways for the Secretary to develop an infrastructure to consider information on both the clinical and cost effectiveness of a service. We will look at issues such as whether Medicare should solely sponsor and fund the research or whether a public-private partnership is appropriate.

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## Background

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Cost-effectiveness analysis involves estimating the costs and health outcomes of a service and its alternatives, which may include no treatment. 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 alternate services. Services include drugs, devices, diagnostic and surgical procedures, and medical treatment.

Researchers can use different methods to assess the cost effectiveness of a service, which results in variation in cost-effectiveness ratios across studies. The term "methods" includes researchers' choice of:

- the costs and outcomes to be measured;
- the overall assessment approach (trial-based or modeling);
- the patient populations to be analyzed;
- the services and comparators to be analyzed;

- the time horizon to measure services' costs and outcomes; and
- the sources of clinical effectiveness, outcomes, and cost information.

The different design methods are not the only reason cost-effectiveness ratios for a specific service may vary across studies. The variation also stems from how analysts model the clinical course of an illness and from differences in the research questions. Finally, researchers' discretion may bias the results of studies when choices favor a pre-existing point of view.

Researchers have discretion in how they measure costs. They can count only those costs associated with medical treatment or define costs more broadly by including those associated with nonmedical services (e.g., transportation costs) and the value of lost productivity. 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 researcher's viewpoint influences the method of defining costs. A societal perspective includes all costs—medical, nonmedical, and indirect costs. By contrast, an analysis from an insurer's perspective includes only those health costs that affect that particular insurer.

Researchers often measure health outcomes in terms of life years gained or health-related quality-adjusted life years (QALYs). QALY is a measure of health outcome that assigns to each time period of a patient's expected remaining years of life a weight, ranging from 0 (death) to 1 (perfect health), that corresponds to the quality of life during that period. QALYs provide a common currency to assess the benefits that patients gain in terms of health-related quality of life and survival. Some issues remain about the use of QALYs to inform resource allocation decisions (Dolan et al. 2006). For example, some analysts question the robustness and stability of respondents' stated preferences.

There are two basic approaches to conducting a cost-effectiveness analysis. In trial-based studies, researchers collect economic data on resource use and quality of life in a clinical study, such as a controlled clinical trial. In modeling studies, researchers combine evidence from a range of sources in order to answer the research question. Modeling studies appear more frequently in the medical literature partly because they are less expensive to conduct than trial-based studies.

A simple modeling approach (a “statistical comparison”) directly compares the costs and clinical outcomes of two services. More complex approaches extrapolate beyond the period during which data are reported and extend results to a broader population by combining costs and outcomes with other assumptions. A Markov model is one such approach that specifies a set of health states (e.g., healthy, early cancer, late cancer, dead) and uses observed results to quantify the probability that people will move from one state to another during a given period of time. By assigning costs and outcomes to each of these states, Markov models can tabulate costs and outcomes for a population over time.

The incremental cost-effectiveness ratio shows the trade-offs and the value involved in choosing among services. For example, the cost-effectiveness ratio of a service is \$25,000 per year of life gained if that service costs an additional \$50,000 and extends life span by two years compared with its alternative. Services with low ratios are thought to provide greater value than services with higher ratios. If a service is less costly and improves outcomes compared with its alternative, then it “dominates” the alternative. Vaccinating beneficiaries for influenza dominates a strategy of not vaccinating (Coffield et al. 2001).<sup>1</sup>

Other methodological issues that researchers must address when designing cost-effectiveness studies include:

- ***The patient population.*** Researchers can include all patients in an analysis or a subset of interest to a payer—such as Medicare beneficiaries.
- ***The services and comparators to be analyzed.*** Researchers compare the service of interest to all or a subset of existing standards of care, which can include 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 the analyses extends into the future, researchers must convert future costs and outcomes to their current (present) value. In doing so, researchers adjust the cost-effectiveness ratio for the different timing of costs and outcomes. Researchers frequently use a discount rate of 3 percent to 5 percent.
- ***The sources of clinical outcomes and costs.*** Sources for clinical outcomes include randomized clinical

trials, comparative effectiveness studies, patients’ medical records, health care claims, and health care surveys. Sources for costs include health care claims submitted to a payer, charges of an individual provider, and health care surveys.

- ***The uncertainty of key variables.*** Sensitivity analysis varies the assumptions of the clinical events, costs, and other key variables.

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## **Do cost-effectiveness ratios vary for colorectal cancer screening and ICDs?**

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On behalf of the Commission, Cohen and colleagues (2006) reviewed the extent to which the assumptions, methods, and results varied across studies assessing the cost effectiveness of colorectal cancer screening and ICDs. We selected these two services because we identified many studies that assessed their cost effectiveness.

Even though there is some variation in the results for colorectal cancer screening, the cost-effectiveness ratios across all studies are relatively low, suggesting that screening is both clinically effective and provides good value compared with no screening. By contrast, the literature for ICDs does not provide a clear indication of the service’s cost effectiveness compared with pharmaceutical treatment because the results vary more widely across studies. This variation is due to differences in the clinical effectiveness reported in clinical trials of this service. The different populations and comparators examined across the cost-effectiveness studies also contributed to the variation in the results. Nonetheless, the literature on ICDs collectively sheds light on key areas of uncertainty where additional data collection might be helpful.

### **Cost-effectiveness ratios for colorectal cancer screening show some variation across studies**

Cohen and colleagues (2006) reviewed 26 studies that evaluated the use of colorectal cancer screening. The majority of the studies used similar assumptions to 1) model the discount rate for both costs and benefits (at 3 percent), 2) quantify benefits in terms of years of life gained, and 3) analyze the sensitivity of the model’s results. The studies used different modeling approaches to compare the effectiveness of colorectal cancer screening with no screening.<sup>2</sup> In addition, the studies modeled



different screening frequencies (every 2 years, 3 years, 5 years, and 10 years). They also compared different screening services (no screening to colonoscopy, virtual colonoscopy, double barium contrast enema, fecal occult blood testing, and sigmoidoscopy). Most studies conducted a univariate sensitivity analysis in which the researchers assessed the effect of varying values of key variables one at a time. Only one study conducted a multivariate sensitivity analysis.

The cost-effectiveness ratios of strategies of screening asymptomatic individuals for colorectal cancer compared with no screening show some variation across studies (Table 10-1). The cost-effectiveness ratios measuring the number of life years gained ranged from about \$1,400 to more than \$42,000 per life year gained. One study found that screening is both more effective and less costly than no screening because of lower medical spending over the study population's remaining life.

It is not surprising that the cost-effectiveness ratios vary across studies given that researchers assessed different populations and approaches to screen colorectal cancer. Researchers also assessed the cost effectiveness of screening for colorectal cancer for different time periods. Yet even with these different approaches, it is notable that the results are somewhat consistent across studies.

The coefficient of variation (CV), which is the ratio of the standard deviation of cost-effectiveness ratios across studies divided by the mean, suggests some consistency among the study findings. The CVs for colonoscopy screening every 10 years, fecal occult blood test screening every year, and fecal occult blood test screening every two years were 0.37, 0.44, and 0.45, respectively. CVs less than 1.0 imply that the standard deviation is less than the mean, which indicates the results do not vary substantially.

Differences in the cost-effectiveness ratios also stem partly from the assumptions used to model the effectiveness of a screening strategy, including the biological behavior of colon cancer, the effectiveness and adverse effects associated with each screening strategy, and the likelihood that patients will actually complete the tests required for a given screening strategy. For example, assumptions about the duration of the precancerous and early cancer detectable phases (dwell time) affect the results. If the dwell time is long, strategies that involve a highly accurate test at a less frequent interval (e.g., screening colonoscopy every 10 years) will appear to perform well compared with

**TABLE  
10-1**

**Cost-effectiveness ratios for colorectal cancer screening compared with no screening vary across studies**

Screening strategy	Frequency	Cost-effectiveness ratio (Dollars per life year)	
		Low end	High end
Colonoscopy	Every 3 years	\$21,763*	
	Every 5 years	17,316	\$36,612
	Every 10 years	10,633	26,693
Fecal occult blood testing	Annually	4,643	25,860
	Every 2 years	2,942	10,861
Sigmoidoscopy	Annually	1,391*	
	Every 3 years	16,318	20,727
	Every 5 years**	14,384	42,310
	Every 10 years	24,226*	

Note: The cost-effectiveness ratio is given in 2004 dollars per life year gained.  
 \*Based on the results of one study.  
 \*\*One study reported that sigmoidoscopy screening every 5 years was dominant (lower costs and better outcomes) compared with no screening.

Source: Cohen et al. 2006.

a more frequent but less accurate test, such as annual fecal occult blood testing.

**Cost-effectiveness ratios vary substantially across ICD studies**

Cohen and colleagues (2006) reviewed 14 studies assessing the cost-effectiveness of ICDs. The majority of studies used similar assumptions to 1) conduct the analysis from the perspective of the health care payer; 2) model the discount rate for both costs and benefits (at 3 percent); 3) quantify benefits in terms of life years gained; and 4) analyze the sensitivity of the model's results. The studies varied somewhat in the modeling approaches they used to estimate the incremental benefits and costs of ICDs versus pharmaceutical treatment or no treatment.<sup>3</sup> Most studies conducted either a univariate or multivariate sensitivity analysis; 4 of the 14 studies did not analyze the effect of varying the assumptions of key variables.

The cost-effectiveness ratios vary widely across studies. The cost-effectiveness ratios of ICDs versus pharmaceutical treatment range from \$18,000 to \$569,000 per year of life gained (Cohen et al. 2006). Three studies found that the use of ICDs was less effective and more

costly than pharmaceutical treatment. The ratios of ICDs versus no treatment range from \$60,000 to \$258,000 per life year gained. One study found that ICDs reduce costs and improve outcomes compared with no treatment.

It is not surprising that the range of cost-effectiveness ratios is large because these studies analyzed patient populations with different clinical characteristics obtained from multiple clinical trials. The costs and benefits of ICDs vary depending on patients' risk of mortality. Factors affecting risk include:

- whether the patient has experienced a life-threatening arrhythmia (secondary prevention patients) or not (primary prevention patients);
- the extent of heart damage as measured by the pumping capacity of the heart's left ventricle—the ejection fraction; and
- other factors, such as whether the patient was undergoing concomitant bypass surgery or had an acute myocardial infarction.

For example, among primary prevention patients, the cost effectiveness of ICDs varied based on the patient's ejection fraction. ICDs were more cost effective for patients with an ejection fraction of less than 30 percent than for patients with an ejection fraction greater than 40 percent (\$53,000 versus \$230,000 per year of life gained (Hlatky et al. 2005)).<sup>4</sup> Another study showed that ICD therapy had higher costs and worse clinical outcomes compared with non-ICD medical therapy among primary prevention patients who were also undergoing concomitant bypass surgery or who had an acute myocardial infarction (Sanders et al. 2005).

The disparity in the results from multiple clinical trials is due to differences in their design. There are at least 10 major trials comparing ICDs to a control group (Hlatky et al. 2005). Sanders and colleagues (2005) linked the variability in ICD clinical trial results to: 1) differing characteristics of the populations studied, 2) differing quality of the non-ICD medical therapy given to the control groups, and 3) differing competing risks of death from causes not due to ICD implantation. Clinical trials of ICDs in patients with a higher mortality risk will show a worse incremental cost-effectiveness ratio (higher costs and poorer outcomes) for the ICD strategy compared with clinical trials of ICDs in patients that have a lower mortality risk.

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## Improving the comparability of cost-effectiveness analyses

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Not all researchers follow the existing standards for conducting cost-effectiveness studies. Still, the methods that researchers employ have improved over the past 15 years. Nevertheless, stakeholders have raised concerns about the variability and lack of transparency in the methods.

The Panel on Cost-Effectiveness in Health and Medicine developed standards for conducting and reporting cost-effectiveness analysis (Gold et al. 1996). The U.S. Public Health Service convened a panel of 13 nongovernment scientists and scholars in 1993. The panel recommended the use of a reference case (a standard set of methods and assumptions) to improve the comparability, reporting, and transparency of cost-effectiveness analyses. For example, they recommended that the reference case:

- discount costs and health outcomes at the same rate,
- use quality-adjusted life years to measure the effectiveness of the service,
- use a time horizon that is long enough to capture all relevant future effects of the service,
- reflect the marginal costs consumed, and
- use a micro-costing approach to determine health care costs.

Despite the panel's recommendation for a reference case, variation in the methods, results, and reporting persists across studies as evidenced by Cohen's review and others (Drummond and Sculpher 2005, Jefferson et al. 2002). Valid comparisons of cost-effectiveness ratios across studies require that researchers derive the numerators and denominators of the ratios using comparable methods and assumptions and report them in similar terms. The reference case lays out the broad assumptions that researchers should use to construct these models. As already mentioned, researchers have discretion in designing the analysis. The text box provides some common methodological and reporting flaws of cost-effectiveness analyses.

Consider the discretion researchers have in modeling the clinical course of an illness. The lack of consistency of the clinical assumptions can result in economic evaluations of the same disease showing different results. Eddy (2005)

## Common methodological and reporting flaws of cost-effectiveness analyses

**D**rummond and Sculpher (2005) noted 11 methodological and reporting shortcomings of cost-effectiveness analyses, focusing on those flaws that are likely to be most important when deciding on payment for, or coverage of, a service:

- omitting important costs and outcomes,
- omitting one or more alternate services,
- imprecisely comparing the clinical effectiveness of alternate services by using information from more than one clinical trial,
- not using all available clinical evidence,
- incorrectly modeling outcomes beyond the period observed in clinical studies,
- relying on assumptions rather than data,
- inadequately assessing the impact of uncertainty on the results,
- not sufficiently reporting all of the results such as the costs and health effects of each service,
- reporting average cost-effectiveness ratios (total costs divided by total health effects for the two services being compared) rather than the incremental ratio (the difference of the total costs divided by the difference of the total health effects between two services),
- not sufficiently reporting on the generalizability of the results, and
- selectively reporting results and placing undue emphasis on certain results. ■

found that 5 models produced widely different estimates of the likelihood a diabetic would have a heart attack in 20 years. Even though each model used the same medical treatment costs and quality weights, the cost-effectiveness ratios varied substantially (–\$10,000 to nearly \$40,000 per QALY) because of the different ways each evaluation modeled the clinical course of diabetes.

The variation in the methods and potential bias of researchers is not unique to cost-effectiveness studies. The design of randomized clinical trials—including the population of patients studied, the method of randomizing patients, and the study time frame—can vary across studies for any given service. Consequently, the clinical effectiveness found in clinical trials can vary. Some researchers are also concerned that the reporting of randomized clinical trials in the literature is not transparent and needs improvement (Moher et al. 2001). For example, a review of 122 recently published randomized clinical trials found that only one paper described randomization adequately (Hotopf et al. 1997). Bekelman and colleagues (2003) showed that industry-sponsored studies were significantly more likely to reach conclusions that were favorable to the sponsor than were nonindustry-sponsored studies.

While the methods of cost-effectiveness studies vary, the quality of some studies has improved over time. Neumann and colleagues (2005) noted improvement in the methods used in studies published between 1998 and 2001 compared to studies published from 1976 to 1997. Studies published in the later period presented the study perspective more clearly, discounted both costs and outcomes, and reported incremental ratios. Jefferson and colleagues (2002) concluded that modest improvements occurred in the quality and methods of studies published from 1990 to 2001.

The variability of the methods and assumptions is not the only concern that stakeholders have raised about Medicare's use of cost-effectiveness information. Some stakeholders are concerned that Medicare's use of cost-effectiveness information might:

- impair beneficiaries' access to certain services,
- ration care rather than promote appropriate care,
- slow innovation, and
- interfere with the practice of medicine.

Some researchers contend that resistance to cost-effectiveness analysis may be cultural: Americans do not believe that resources are limited, accept limits imposed upon them by payers, or want to consider the trade-offs between health costs and benefits (Ginsburg 2004, Neumann 2004).

### **Potential strategies to improve the comparability of results across studies**

Cost-effectiveness analysis will be most useful to policymakers when the results are comparable across studies and services. The Commission highlights four strategies that may improve the comparability of results across studies:

- improving transparency in the reporting of studies,
- validating clinical models against real-world outcomes,
- using validated and accepted instruments for quality assessments, and
- increasing the availability of information about the effectiveness of alternate services.

Improving the transparency in the reporting of both publicly and privately funded studies might alleviate concerns about methods and potential biases. One option is for researchers to post their economic models and data on the internet (Rennie and Luft 2000). Doing so would permit all interested users to test the sensitivity of results to different assumptions and data. Posting the models might permit users to change multiple elements by inserting their own data. The Academy of Managed Care Pharmacy's guideline for submission of clinical and economic data calls for manufacturers to submit an electronic version of their cost-effectiveness model (AMCP 2005). However, some researchers may be concerned about the public availability of their models because of proprietary reasons. Mandatory posting might undercut researchers' incentive to develop new models.

Validating clinical models used in cost-effectiveness analyses against real world outcomes might reduce some of the variability of the results across studies. Some of the variation in cost-effectiveness ratios stems from researchers using different clinical models that use different assumptions about the course of disease progression. Posting validated clinical models on the internet would provide access to all users and might reduce

the variability of clinical models used in cost-effectiveness studies.

Using validated and accepted instruments for measuring health outcomes, such as QALYs, might also reduce some of the variability of the results across studies for the same service. Differences in the methods used to estimate QALYs could lead to variation of the findings across cost-effectiveness studies for the same illness.

Increasing the availability of evidence about the effectiveness of alternate services may enhance the quality of cost-effectiveness research. Comparative effectiveness reviews assess the clinical effectiveness of one treatment compared with its alternatives by reviewing the medical literature. One valuable source of this information is the Agency for Healthcare Research and Quality (AHRQ). The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) required that AHRQ conduct and support research with a focus on outcomes, comparative clinical effectiveness, and appropriateness of pharmaceuticals, devices, and health care services. AHRQ is currently studying 10 conditions that affect Medicare beneficiaries including arthritis, cancer, chronic obstructive pulmonary disease, asthma, diabetes, and dementia (e.g., Alzheimer's disease).<sup>5</sup>

Another group conducting comparative effectiveness reviews is Oregon's Center for Evidence-Based Policy. The Center's Drug Effectiveness Review project is a collaborative effort of 15 organizations (13 of them are states) to obtain the best available effectiveness comparisons between drugs in the same class through reviews of the existing medical literature.

Another promising data source for effectiveness information is Medicare's administrative claims database. Medicare's inpatient, outpatient, and drug claims together offer analysts the ability to: 1) focus on the elderly and disabled populations, 2) compare real-world outcomes across different providers and settings, and 3) analyze side effects that may go underreported in small clinical trials (Hunter 2006). Analysts will need to address limitations and general lack of limited clinical information available within administrative claims data. For some analyses, analysts may also need to obtain additional information not reported on claims, such as lifestyle factors that may affect treatment outcomes. One concern about using administrative claims data is that patients who received a specific service may have different demographic or clinical characteristics than patients who received one of

the comparators studied. Researchers can use statistical methods (e.g., multivariate regression) to control for differences in characteristics between treatment groups.

Head-to-head clinical trials are important sources of effectiveness information, but they are not conducted as frequently as placebo-controlled trials. To gain approval to market a drug or device by the Food and Drug Administration (FDA), most manufacturers conduct trials of a service's efficacy by comparing it with a placebo (an inactive treatment), which is not evidence of a service's effectiveness relative to another service. The National Institutes of Health is the largest federal sponsor of head-to-head trials. Other federal agencies that fund these trials include AHRQ and the Veterans Health Administration. Manufacturers of medical products also sponsor these trials. However, conducting head-to-head trials is not the primary mission of any public or private organization (Tunis 2003). The Commission plans to examine total spending and the share of each agency's budget devoted to conducting head-to-head trials.

CMS is beginning to gather information about a service's effectiveness in the national coverage process. Under coverage with evidence development (CED), CMS extends national coverage to a service that, in the past, the agency might not have covered due to lack of data about its clinical appropriateness. National coverage of a service may be limited to providers who participate in and beneficiaries who enroll in a prospective data collection activity. The goal of CED is to ensure that patients are receiving care that is reasonable and necessary given their specific clinical condition (CMS 2005).<sup>6</sup> CED may ultimately provide patients, providers, and researchers an opportunity to learn about a service's value in real-world settings. The agency may require CED for services:

- that are in new classes with new mechanisms;
- that may be effective for only certain types of patients;
- that have demonstrated major advances over prior treatments, suggesting that they could benefit patients with other conditions; and
- that may have substantial consequences for treating the wrong patients.

Services for which CMS has required CED include ICDs; carotid artery stenting; off-label, unlisted uses of drugs approved for colorectal cancer; and certain types of imaging services for cancer diagnosis, staging, and monitoring.

Increasing the availability of comparative effectiveness data would overcome one potential limitation of cost-effectiveness studies—using data from more than one randomized clinical trial to estimate the clinical effect of alternate services (Drummond and Sculpher 2005). Researchers use data from multiple clinical trials because comparative effectiveness information is not always available. Using information from more than one clinical trial might lead to inaccurate comparisons if 1) patients enrolled in the various trials are not equivalent in terms of baseline risk, 2) the settings for the trials are not comparable, and 3) the clinical endpoints are measured differently. Thus, an apparent superiority for one service versus another, derived from using data from more than one clinical trial, might be due to differences in the trials rather than differences between the therapies.

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## Future issues

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The Commission plans to consider the issues associated with Medicare sponsoring new research and developing the infrastructure needed to review cost-effectiveness information from the existing literature. Key questions include:

- Who would sponsor the research?
- Who should pay for the research?
- What services could Medicare focus on?
- What methodological issues might Medicare consider?
- How could Medicare use cost-effectiveness information?
- Are there any lessons to learn from other payers and providers in the United States and from other countries that are using cost-effectiveness information?

## Who would sponsor the research?

Medicare, alone or with other public payers and private groups, may need to sponsor additional research. This additional research could entail reviewing the medical literature and designing studies (models and head-to-head trials) assessing services' cost effectiveness.

One option is for the Department of Health and Human Services (HHS) to sponsor effectiveness research. Potential agencies include CMS and AHRQ. Both agencies

already sponsor and conduct reviews about the clinical effectiveness of services. AHRQ has taken some steps in looking at cost effectiveness through its evidence-based practice centers.

Alternatively, HHS, other public payers (e.g., the Veterans Health Administration and state Medicaid agencies), and private plans, payers, and purchasers could jointly sponsor clinical and cost-effectiveness studies. Private sector groups already sponsor comparative clinical effectiveness studies. For example, 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 the clinical effectiveness of services (Garber 2001).

The increased role of the federal government in sponsoring clinical and cost-effectiveness research may be warranted because this research is a public good. Effectiveness research has generally not been forthcoming from private health plans and providers. Sponsoring this research may not be in any single plan's or payer's interest because it is problematic to keep the information proprietary, and it might be difficult to capture the full return on the investment (Neumann 2005). Cost-effectiveness analysis may have a more important role for Medicare because the program covers patients over a longer time period (from age 65 to death) than do most private payers.

A public-private partnership may more effectively address concerns raised by stakeholders about the use of cost-effectiveness analysis than a noncollaborative process. Private payers may be reluctant to make extensive use of cost-effectiveness information out of fear that patients will criticize them about being more concerned about profits than about patients' health. Litigation risks may also dissuade some private payers from using cost-effectiveness information (Jacobson and Kanna 2001). As discussed earlier, stakeholders raised a number of concerns about Medicare's use of cost-effectiveness analysis, including that it could harm beneficiaries' access to care and reduce innovation of new services. Public payers may also be reluctant to use cost-effectiveness information out of fear that beneficiaries will perceive their care as being second rate. A public-private partnership may also be advantageous because it would send a clear and effective signal to researchers to improve their methods and develop valid and transparent cost-effectiveness analyses.

## **Federal agencies or independent groups could conduct cost-effectiveness analyses**

CMS already assesses the clinical effectiveness of services when making national coverage decisions and paying for some services.<sup>7</sup> In some cases, CMS supplements its research by sponsoring outside groups, such as AHRQ, to conduct technology assessments and consulting with the Medicare Coverage Advisory Committee (MCAC). A technology assessment studies the medical, social, ethical, and economic implications of the development, diffusion, and use of services. The MCAC advises CMS on whether a specific service is reasonable and necessary under Medicare by reviewing and evaluating medical literature, reviewing technology assessments, and examining data and information on the effectiveness and appropriateness of the service under consideration (CMS 2006a).<sup>8</sup>

AHRQ has taken several steps in constructing an infrastructure to conduct comparative effectiveness reviews of health care services. AHRQ created evidence-based practice centers in 1997 to synthesize existing scientific literature about health care topics and to promote evidence-based practice and decision making. There are currently 13 centers, which include academic institutions and private research organizations.<sup>9</sup> The centers are conducting comparative effectiveness reviews of 10 health conditions affecting older people including dementia, arthritis, and diabetes. This research fulfills the MMA mandate that AHRQ conduct and support research with a focus on outcomes, comparative clinical effectiveness, and appropriateness of pharmaceuticals, devices, and health care services.

AHRQ has also developed the infrastructure to conduct technology assessments that CMS requests when making national coverage decisions. These technology assessments examine the clinical outcomes of one or more health care services. AHRQ conducts technology assessments in-house or collaborates with its evidence-based practice centers.

The agency also assists other federal agencies with developing cost-effectiveness analyses. For example, CMS requested that AHRQ assess the cost effectiveness of drugs used to treat rheumatoid arthritis and multiple sclerosis under a MMA-mandated demonstration (CMS 2006b). This demonstration, which began on September 1, 2004, and ended on December 31, 2005, paid for selected drugs for cancer, multiple sclerosis, and rheumatoid arthritis that replaced drugs covered under Part B. (In 2006, the Part D

program provides coverage for the drugs paid for under the demonstration.) As another example, in 2003 AHRQ completed an assessment of the cost effectiveness of fecal occult blood tests for CMS.

Since 1985, almost 10 percent of AHRQ's extramural research grants have included a clinical economic component (AHRQ 2006). For example, AHRQ funded a study to determine the cost effectiveness of lung-volume reduction surgery for patients with severe emphysema. This study paralleled a trial sponsored by CMS and the National Heart, Lung, and Blood Institute comparing lung-volume reduction surgery to medical therapy for severe emphysema.

Alternatively, sponsoring entities could create an independent agency to conduct the effectiveness analyses. The National Institute for Health and Clinical Excellence (NICE) in the United Kingdom is an independent group that develops guidance for the National Health Service (NHS) in England and Wales on the clinical and cost effectiveness of medical services. Established in 1997, NICE relies on academic centers to assess the effectiveness of drugs, medical devices, diagnostic techniques, medical and surgical procedures, and the clinical management of specific conditions (Sculpher 2005).

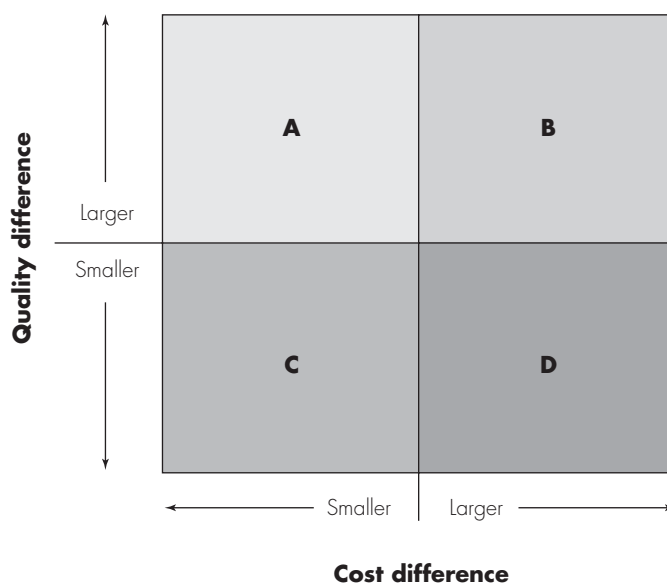
### Who should pay for the research?

One option for funding is for the Congress to appropriate funds to a public agency (e.g., HHS) to conduct clinical and cost-effectiveness analyses. Doing so would require policymakers to annually consider the priority of such research compared with other health programs. However, variations in the level of federal appropriations may reflect the budget cycle rather than the priority of the research. Another option is to dedicate some percentage of general revenues to fund effectiveness research.

Discretionary funding from private groups—such as private plans and payers and manufacturers of drugs, biologics, and medical devices—could also be vulnerable to budget uncertainties. Private sponsors might decide to withhold or withdraw funding for any number of reasons, such as disagreeing with the selection of a service for consideration. In addition, this mechanism might be open to conflict of interest. The influence of private groups who directly fund the research on a study's design and findings could be a concern.

**FIGURE 10-1**

### Selecting services for cost-effectiveness analysis based on differences in their costs and quality



Another alternative is a method that is not linked to either annual federal appropriations or discretionary funding from private groups. For example, one analyst suggested that a specified percentage of sales from drug manufacturers, health plans, and pharmacy benefit managers may be an appropriate and available mechanism for funding needed effectiveness research (Reinhardt 2004).

### Which services could Medicare focus on?

Medicare could select services based on disease prevalence, high per unit cost, high expenditures, or other factors. One option is to use the same criteria that CMS uses in its national coverage process. CMS initiates such a review if the service: 1) represents a significant advance, and no similar service is currently covered under Medicare; 2) is the subject of controversy among medical experts as to its medical effectiveness; 3) is currently covered but is widely considered ineffective; and 4) may be either significantly underutilized or overutilized.

Another option is to consider both the differences in cost and quality of alternate services (Figure 10-1). Medicare could begin to look at groups of services used to treat a specific illness that have small differences in quality but large differences in cost (quadrant D in Figure 10-1). Focusing on these services might increase the return on

society's investment in health care. By contrast, formal cost-effectiveness analyses may not be as needed for services with small differences in quality and costs. Medicare may find it most difficult to consider the cost-effectiveness of services with large differences in cost and quality. It may be a matter of judgment to decide where given services fall in the continuum of cost and quality differences.

It is worth noting that cost-effectiveness analysis may not save the Medicare program money. Wider use of cost-effective, underutilized services might result in increased Medicare spending, which might not be offset with savings elsewhere. For example, McGlynn and colleagues (2003) reported on the underuse of clinically effective treatments. Promoting the use of such services could increase Medicare spending. On the other hand, over the long run, cost effectiveness could save the Medicare program money if it encourages manufacturers to develop services that are more cost effective than current ones or helps inform providers and influences their patterns of care.

### **What methodological issues might Medicare consider?**

Medicare will need to consider the procedures for evaluating cost-effectiveness information and the methods for conducting cost-effectiveness analyses. For example, should studies limit the population to Medicare beneficiaries or patients of all ages? Should costs be limited to Medicare payments? Should the model include all costs—taking the societal perspective? Should the analysis measure outcomes using QALYs or another method such as life years gained?

To help frame the methodological issues, Medicare—along with other public and private groups—could review the guidelines developed by the Panel on Cost-Effectiveness in Health and Medicine. It might also be useful to examine current standards developed by other groups such as the formulary guideline developed by the Academy of Managed Care Pharmacy for submission of clinical and economic data (AMCP 2005). The goal of the academy's guideline is to standardize the set of clinical and economic evidence that manufacturers submit to health plans. The guideline includes the layout for the submission of clinical and economic data to health plans and recommends that manufacturers include unpublished studies, data on off-label indications, related disease management strategies, and an economic model to provide evidence of the product's value.

Bringing together users and researchers might foster a more collaborative relationship between all parties. Nonetheless, different payers may have different perspectives and needs, which may result in variations in some aspects of the design of studies across payers. For example, a payer may ultimately decide to limit the study population to the patients it covers (not all patients) and only include the costs of services that it pays for (not societal costs).

### **How could Medicare use cost-effectiveness information?**

Cost effectiveness has the potential to identify medical services that are more likely to improve patient outcomes and discourage the use of services with fewer benefits. As the field of cost effectiveness evolves and as Medicare and others address methodological issues, Medicare could use cost-effectiveness information in a variety of ways.

The program could use cost-effectiveness information to cover a service for all Medicare beneficiaries or for beneficiaries with specific clinical or demographic characteristics. However, the coverage process may not be the area to begin to use this information. As we mentioned earlier, stakeholders raised many concerns when CMS tried to use the information in the national coverage process. Rigid use of cost-effectiveness information in the coverage process may not be consistent with Americans' fear of limits set by public and private organizations and affinity for new medical technology (Neumann 2005, Neumann 2004). Rather, Medicare might want to begin to use cost effectiveness to inform providers and patients about the value of services and to develop payment policies that account for a service's value.

Medicare could provide cost-effectiveness information to beneficiaries and health professionals. Both are potential audiences for information about the relative value of treatment alternatives. Currently, the traditional Medicare program does not encourage providers and beneficiaries to weigh the costs and benefits of a service when making health care decisions. The program does provide some clinical effectiveness information about certain providers—dialysis facilities, hospitals, home health agencies, and nursing homes—but not cost-effectiveness information.

There is some evidence that providers and patients might consider cost-effectiveness information as they weigh treatment options. 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). Anecdotal reports also suggest that some physicians examined both the cost and outcomes of lung-volume reduction surgery when considering this procedure for their patients (Kolata 2006).

Medicare might use the information to prioritize pay-for-performance measures, target screening programs, or prioritize disease management initiatives. A pay-for-performance program could link providers' bonuses to the provision of cost-effective services. Medicare might weight performance bonuses higher for the most cost-effective services furnished by providers. Medicare could consider cost effectiveness when choosing measures for pay-for-performance programs; there are usually more potential measures than are practical to use.

Cost-effectiveness analysis could measure the value of alternative screening strategies in different patient populations in order to focus provider education or performance incentives. Cost-effectiveness analyses could help inform policymakers about which subpopulations to target for screening, such as screening diabetics for chronic kidney disease. Medicare already varies coverage of certain screening tests (e.g., colorectal cancer and glaucoma) according to the risk of developing the illness. Covered colorectal cancer screening tests for prevention include: 1) an annual fecal occult blood test for beneficiaries age 50 and older, 2) flexible sigmoidoscopy every 4 years for beneficiaries age 50 and older, 3) colonoscopy for high-risk beneficiaries every 2 years and for other beneficiaries every 10 years, and 4) screening barium enemas every 4 years for beneficiaries age 50 and older who are not at high risk of developing colorectal cancer or every two years for beneficiaries who are at high risk.

Once the analyses become more rigorous, Medicare could use cost-effectiveness information in the payment and rate-setting processes. For example, Medicare might require manufacturers to enter into a risk-sharing agreement, which links actual beneficiary outcomes to the payment of a service based on the service's cost effectiveness. Manufacturers might rebate the Medicare program for services that do not meet expectations for their effectiveness. The program is already holding some

providers at risk for their performance. Under Medicare's Chronic Care Improvement Program, contractors assume risk for achieving savings and quality targets. CMS is adjusting contractors' fees based on whether they achieve targets for program savings, clinical outcomes, and satisfaction.

Alternatively, Medicare could base the payment for a service at the level that the cost-effectiveness analysis suggests that the service is effective and provides value to the program and beneficiaries. In its comparison of the cost effectiveness of fecal occult blood tests conducted for CMS, AHRQ determined the payment level for which the cost effectiveness of two tests would be equal. Medicare could also consider a tiered cost sharing structure that requires higher cost sharing for those services that show less value to the program and includes a beneficiary appeal process. Many drug formulary programs tier copayments. Part D plans also could use the results of these studies in this way.

## **How do other payers and providers within the United States and internationally use cost-effectiveness information?**

The Commission will review in greater detail the different ways that other payers and providers within this country and elsewhere use cost-effectiveness information. The approaches vary from group to group, as we show in the three examples in the rest of this section by summarizing the use of cost-effectiveness information by the Veterans Health Administration (VHA), in England and Wales, and in Washington state. We are interested in looking at the different ways other groups select services for review and how they use the information (e.g., vary the level of payment of services). We anticipate that this review will inform us about the advantages and disadvantages of different approaches for Medicare.

### **Use of cost-effectiveness information by the VHA**

The VHA has recently emphasized the use of cost-effectiveness information for newer, costly drugs for inclusion in its formulary (Aspinall et al. 2005). Since 1994, the VHA has required a formal cost-effectiveness analysis from manufacturers of drugs that have small differences in quality but large differences in cost compared with their alternatives. The VHA routinely requests manufacturers to submit clinical and economic data using the Academy of Managed Care Pharmacy

format and incorporates this information into the drug reviews used in the formulary decision making process.

The VHA reviews a drug's clinical and cost effectiveness to determine its status on the drug formulary. The VHA also uses effectiveness studies to develop criteria for patients who are most likely to benefit clinically from a drug. The VHA does not use a cost-effectiveness threshold to determine whether to include a drug in the formulary because of the controversy about trying to determine what constitutes good value.

### **Use of cost-effectiveness in England and Wales**

NICE develops guidance for the NHS in England and Wales on the clinical and cost effectiveness of medical services. NICE's process for developing recommendations takes about 14 months to complete. The Secretary of State for Health formally refers technologies for guidance to NICE. Advisory committees identify potential services using criteria that include 1) high clinical need, 2) potential for significant health gain, and 3) potential for significant cost impact. The NHS uses a National Horizon Scanning Centre to identify significant new and emerging health technologies.

NICE commissions independent academic groups to conduct technology assessments, which are usually completed in six months. Technology assessments include 1) a systematic review of clinical and economic evidence, 2) a cost-effectiveness analysis, and 3) a review of the manufacturer's submission. An independent committee—the Appraisal Committee—prepares NICE's recommendations about the use of services within the NHS. Manufacturers, patients, and health professionals can comment on the scope and findings of the technology assessment, submit evidence to the Appraisal Committee, comment on the Appraisal Committee's draft recommendation, and appeal the Appraisal Committee's final decision.

The Appraisal Committee does not use a threshold to guide its recommendations. Nevertheless, NICE's "Guide to the Methods of Technology Appraisal" states that for services with an incremental cost-effectiveness ratio greater than £30,000 (about \$53,000) per QALY, "the case for supporting the service has to be increasingly strong" for the Appraisal Committee to recommend its use. The Appraisal Committee does consider factors other

than clinical and cost effectiveness, such as equity, in its recommendations.

### **Use of cost-effectiveness information in Washington state**

The governor of Washington state signed into law on March 29, 2006, a health technology assessment program to consider evidence about the safety, efficacy, and cost effectiveness of services. The Commission intends to track the implementation of this program.

The administrator of the Washington State Health Care Authority, consulting with participating agencies and a health technology clinical committee, will select services that the health technology committee will review. The newly passed law gives priority to the review of services for which:

- concerns exist about safety, efficacy, or cost effectiveness—especially relative to existing alternatives—or significant variations in use;
- actual or expected state expenditures are high due to demand, cost, or both; and
- adequate evidence is available to conduct the review.

The health technology committee will consist of six practicing physicians and five other health professionals. The administrator of the Washington State Health Care Authority, consulting with participating agencies, selects the committee's members. The committee will review the safety, efficacy, and cost effectiveness of up to six services the first year of the program and up to eight services thereafter. Evidence-based practice centers (designated by AHRQ or another appropriate organization) will conduct the technology assessments. The committee will determine the conditions under which the service will be included as a covered benefit in programs of participating agencies, and if covered, the criteria that the participating agency administering the program must use to decide whether the technology is medically necessary or proper and necessary treatment.<sup>10</sup> Finally, the law requires that the administrator develop a centralized internet site that provides information about the technology assessment.

### **Other issues to consider**

Policymakers will need to consider a number of overarching issues when setting up the infrastructure for Medicare to consider clinical and cost-effectiveness information: ensuring transparency of the study methods

and results to stakeholders and the timing of assessing a service's cost effectiveness.

Some stakeholders mistrust cost-effectiveness analysis because the methods of some studies are not transparent and the results are not reproducible. Ensuring the transparency of the process will be a key issue for policymakers to consider if Medicare begins to use cost-effectiveness information. It will be important that Medicare offer stakeholders the opportunity for comment and participation in the process.

In recent years, CMS has developed a more open and predictable process for scrutinizing clinical evidence on which to base national coverage decisions. This process

could be a model for future deliberations on the use of cost-effectiveness analysis.

Another key issue is the timing of assessing a service's cost effectiveness. Researchers could study a service when it is not widely used by providers (before or at FDA approval for devices and drugs) or wait until it diffuses into medical practice. The results of a cost-effectiveness analysis could change as providers adopt the service into their practice. On the one hand, providers may become more proficient in furnishing a service over time, which would lower its costs while still resulting in the same outcome. Or, researchers may become more aware of the side effects of a service over time, which would increase its costs and result in poorer outcomes. ■

## Endnotes

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- 1 If a service is more costly than its alternative but not does improve outcomes, then it is “dominated” by the alternative.
- 2 The types of models used to assess the cost effectiveness of colorectal screening include Markov models and static models.
- 3 Some studies assessed the cost effectiveness of ICDs by statistically comparing the experience of cohorts receiving ICDs to the experience of control groups, while other studies used a Markov model or a static model.
- 4 A normal ejection fraction ranges from 55 percent to 70 percent.
- 5 The other conditions are: depression and other mood disorders, ischemic heart disease, peptic ulcer disease and dyspepsia, pneumonia, stroke, and hypertension.
- 6 CMS issued draft guidance for national coverage determinations with evidence development in 2005.
- 7 Currently, CMS considers patient’s hematocrit level when paying for erythropoietin and darbepoetin alfa for dialysis patients. In 2003, CMS set the payment rate for a new biologic at the same rate as an existing biologic after concluding that both were functionally equivalent. The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 limits the use of the functional equivalence standard in the hospital outpatient setting.
- 8 The MCAC meets about six times each year. The MCAC functions on a committee basis by reviewing and evaluating medical literature, reviewing technology assessments, and examining data and information on the effectiveness and appropriateness of medical items and services that are covered or are eligible for coverage under Medicare. Each committee generally includes 13 to 15 members.
- 9 The 13 evidence-based practice centers are Duke University; ECRI; Tufts University–New England Medical Center; the Blue Cross Blue Shield Technology Evaluation Center; John Hopkins University; McMaster University; Oregon Health & Science University; RTI International–University of North Carolina; Southern California–RAND; Stanford University–University of California, San Francisco; University of Alberta, Edmonton, Alberta, Canada; University of Minnesota; and University of Ottawa. The first three centers (Duke, ECRI, and Tufts) focus on technology assessments for CMS.
- 10 Participating agencies include the Department of Social and Health Services, the state health care authority, and the Department of Labor and Industries.

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