Medicare, the federal health insurance program that covers some forty million elderly and disabled Americans, has grown rapidly since its inception in 1965. Medicare spending increased from $33.9 billion in 1980 (1.3% of GDP) to $252 billion in 2002 (2.5%).

Spending growth has been driven in part by inflation and the aging of the Medicare population (older beneficiaries spend more than younger ones) but mostly by the incorporation of new and sometimes expensive medical technologies. For the purposes of this paper, technology is defined broadly to include drugs, devices, medical and surgical procedures, and the organizational and supportive systems within which such care is provided.

While some new technologies may reduce costs by replacing more expensive alternatives or preventing expensive health consequences, the overall effect of new technology is an increase in costs. Thus, how Medicare decides to pay for new medical technology has profound implications for beneficiary access to health-enhancing medical advances, as well as for the fiscal well-being of the program. Over the years, Medicare has been criticized on grounds that its procedure for covering medical advances is ill-defined, unpredictable and opaque. In response, policy makers have recently attempted to make the process more transparent, consistent and evidence-based. Notably, they have also tried, without success, to incorporate cost-effectiveness analysis as an explicit criterion for coverage in an effort to obtain a better value for the dollars spent on health care. This paper describes Medicare’s ongoing efforts to pay for efficient and cost-effective medical technology, the barriers the program faces in this endeavor and prospects for the future.

Medicare Coverage of New Medical Technologies

Medicare’s authorizing legislation prohib-

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its payment for any expenses incurred for “items and services that are not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member.” The statute restricts coverage by excluding, for example, cosmetic surgery, personal comfort items, custodial care, routine physical checkups and outpatient drugs.

The question of how to cover new medical technology has long challenged the program. Part of the problem is that it is not always clear what constitutes an illness or injury. As Stanford Professor Alan Garber has noted, the original statute applies to diagnosis and treatment of infirmities and implies that Medicare would pay, for example, to treat memory loss but not to improve memory.

Even more challenging has been how to interpret which items and services are “reasonable and necessary.” United States law stipulates that the Federal Drug Administration (FDA) assess the benefits and health risks of scientific advances but that Medicare determine whether to pay for the innovation. FDA approval of a drug or device, therefore, does not determine if it is “reasonable and necessary.” A drug could be safe, effective and FDA-approved, but not “reasonable and necessary” if, for example, it cost substantially more but offered no more benefits than an existing Medicare-covered drug.

Ultimately, it is the Center for Medicare and Medicaid Services (CMS) within the U.S. Department of Health and Human Services that decides whether Medicare will cover and pay for a new technology.

In practice, most Medicare coverage determinations are made by local contractors, known as carriers and fiscal intermediaries, who pay claims for the program. This policy reflects an argument that decentralized decision making is pragmatic because it would be difficult for the central Medicare office to respond to the dynamic world of medical innovation. Medicare has historically made ten to twenty national coverage decisions each year for technologies that have a major impact on the program or are deemed controversial. These decisions are binding on all local contractors.

The precise criteria used for Medicare coverage decisions, whether on a local or national basis, have never been codified. In Medicare’s early years, technologies were covered based largely on whether they were generally accepted in the medical community. Following the broader movement towards “evidence-based” medicine, Medicare has amended its process over the years with the goal of grounding decisions in solid clinical studies. It has also tried to move towards explicit considerations of cost-effectiveness or “value for money” arguments in its deliberations.

Medicare and Cost-effectiveness Analysis

In recent years, cost-effectiveness analysis has emerged as the recommended analytic technique for conducting economic evaluations of health and medical interventions. This approach is appealing because it allows a convenient means to quantify both economic and health benefits in a single ratio. Cost-effectiveness analyses involve comparisons between two alternatives or between the presence and absence of an intervention: the cost per effect (C/E) ratio reflects the increment or difference in the interventions’ costs divided by the difference in their health effectiveness.
One might expect that cost-effectiveness analysis (CEA) would occupy an important place in the Medicare policy-making toolkit. CEA offers decision makers a structured, rational approach to prioritize resources for health care more efficiently and improve the return on resources expended. Many thousands of cost-effectiveness analyses have been published over the years on a wide array of treatments and conditions. Furthermore, CEA has been used by many countries to guide coverage and reimbursement decisions.

Over the years, CEA has helped challenge prevailing wisdom and has brought clarity to large health policy debates. It has underscored, for example, that prevention programs usually do not produce cost savings, that modern, cost-increasing interventions can sometimes be worth the money spent, and that an additional diagnostic test can be enormously expensive relative to the clinical benefits conferred.

However, after repeated attempts to incorporate cost-effectiveness analysis formally as a criterion for covering new medical technologies, Medicare abandoned the approach, even as it moved to strengthen its coverage process. Medicare’s retreat from cost-effectiveness analysis also defied the federal government’s broader movement to subject new regulations to formal cost-benefit and cost-effectiveness tests.

The Health Care Financing Administration (HCFA), which administered the Medicare program, first sought to clarify coverage criteria and its national coverage process in proposed regulation in 1989. Medicare’s retreat from cost-effectiveness analysis also defied the federal government’s broader movement to subject new regulations to formal cost-benefit and cost-effectiveness tests.

The Health Care Financing Administration (HCFA), which administered the Medicare program, first sought to clarify coverage criteria and its national coverage process in proposed regulation in 1989. To be eligible for coverage, technology had to be accepted by the medical community, safe, effective, non-investigational, and appropriate. Emboldened by what the agency called an “explosion of high-cost medical technologies,” HCFA proposed to add cost-effectiveness as a criterion for coverage. Asserting that costs were relevant in deciding whether to expand or continue coverage of technologies, HCFA explained, “We believe the requirement of section 1882(a)(1) that a covered service be ‘reasonable’ encompasses the authority to consider cost as a factor in making Medicare coverage determinations.”

HCFA argued that a disciplined effort to assess systematically the cost-effectiveness of technologies under coverage review would “vastly improve our knowledge base and be a deterrent to coverage of procedures that may be costly, but have little or no impact on improving health outcomes.” It added that the provision would codify its authority not to cover a service viewed as marginal with respect to safety and effectiveness but expensive in comparison with available covered alternatives.

In the proposed regulations, HCFA defined cost-effectiveness and put forth the analytic steps it would follow, stating that they were “well accepted by economists.” Specifically, HCFA said that cost-effectiveness would give the program authority to cover services in the following categories:

1. Very expensive to the program but provides significant benefits not otherwise available
2. Less costly and at least as effective as alternative covered intervention
3. More effective and more costly but added benefit justifies cost
4. Less effective and less costly but a viable alternative

Finally, HCFA noted that it was aware that cost-effectiveness analysis was a complex
The proposed regulation – and particularly the cost-effectiveness provision – proved very controversial. As a front page New York Times story noted in 1991, the plan signaled a fundamental shift in U.S. health policy, giving Medicare the authority to weigh cost as a factor in reimbursing new technologies. Despite internal support within the Department of Health and Human Services, the 1989 proposed regulation was never released in final form. Opposition came from the medical device industry and consumer groups, such as the American Association of Retired Persons, which argued that the policy would lead to “rationing.”

HCFA’s attempt in the mid-1990s to resurrect the plan and to publish the 1989 proposed regulation as a final rule also met stiff opposition. In a 1996 letter to HCFA Administrator Bruce Vladeck, a broad-based group of medical and industry professionals contested the idea, citing the elapsed time since the 1989 regulation and the controversial nature of various elements, particularly the cost-effectiveness provision.

The letter noted, for example, that:

> with respect to the cost-effectiveness issue, the U.S. Public Health Service has recently published a report titled ‘Cost-Effectiveness in Health and Medicine,’ which raises a number of significant questions with respect to the methodology associated with many cost-effectiveness analyses… How HCFA plans to conduct cost-effectiveness analyses[…]is an issue that should be subject to the public comment process.

The letter also stated that “failure to seek and incorporate public comment may result in a rule that could inappropriately deprive Medicare beneficiaries of access to life-saving, life-enhancing medical technology.” Signers of the letter included: the American College of Physicians, the American Medical Association, the American Society for Internal Medicine, the Biotechnology Industry Organization, the College of American Pathologists, the Council on Radionuclides and Radiopharmaceuticals, Inc., the Health Industry Manufacturers Association, the National Electrical Manufacturers Association and the Pharmaceutical Research and Manufacturers Association of America.

At a Congressional hearing in 1997, HCFA Administrator Vladeck acknowledged that:

> It is apparent that cost-effectiveness analysis is extremely controversial for beneficiaries, providers, and suppliers. It raises fears of rationing based on cost. HCFA has not and does not intend to make coverage decisions based solely on cost-effectiveness, and we will not refuse to cover services merely because they are costly. Moreover, manufacturers or providers do not need to submit formal cost-effectiveness analyses to HCFA in order to have a service considered for coverage.

In 1999, HCFA formally announced that it was withdrawing its 1989 proposal. By then, in response to persistent criticisms that its process needed to be more consistent, transparent, and evidence-based, HCFA had established the Medicare Coverage Advisory Committee.
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(MCAC) to advise the program about the evidence of new medical technologies for coverage considerations.\textsuperscript{28} The MCAC was created to counsel HCFA as to whether specific medical items and services were reasonable and necessary under Medicare law using unbiased contemporary considerations of “state-of-the-art” technology and science.

The MCAC has helped HCFA shore up the evidence base for national coverage decisions. Medicare officials argue that since its creation, the MCAC has helped to ensure a more consistent analytic framework and wider participation— not just of physicians, but also methodologists, industry representatives, and consumer advocates. The MCAC has also ensured a more consistent, explicit and open process, under which the MCAC adheres to Federal Advisory Committee Act rules by holding meetings that are open to the public.\textsuperscript{29}

However, the MCAC, like CMS in general, has essentially been prohibited from using cost-effectiveness as a criterion in judging evidence. To this day, the de facto Medicare policy is that Medicare will pay for any new medical technology that confers some positive health benefit, even if it is hugely expensive, to gain marginal health effects. CMS still does not want, or cannot afford politically, to have the program seen as an agent of cost-containment rather than a vehicle to improve care.\textsuperscript{30} Former Administrator Tom Scully stated, “It’s tough to say no to a cancer drug no matter how slender the benefits.”\textsuperscript{31}

It is important to emphasize that Medicare’s failure to incorporate CEA was caused by political pressures rather than a lack of statutory or regulatory authority. A full policy and legal debate on whether the “reasonable and necessary” language imparts Medicare the statutory authority to use CEA has never taken place. Nevertheless, the 1989 proposed regulation, as well as attempts to revise the cost-effectiveness debate in the 1990s, signaled a belief by Medicare officials that the existing statute provided sufficient cover. While courts have not ruled on Medicare and cost-effectiveness per se, there is good reason to believe that courts would defer to the government’s position on the matter, as long as the policy were not handled in an arbitrary or capricious manner.\textsuperscript{32}

Anticipating the Medicare Drug Benefit

The recently enacted Medicare drug legislation raises the stakes. Drug company pipelines are full of promising and expensive drugs. The recent FDA approval of avastin for the treatment of colorectal cancer provides just one example. At $4,400 per month, this intervention alone could add billions of dollars to Medicare expenditures.\textsuperscript{33} Critical questions regarding coverage are left largely to private health plans contracting with Medicare. Thus, cost-effectiveness analysis might be incorporated at a local level by competing plans or Pharmacy Benefit Managers. A growing number of organizations have begun implementing formulary submissions guidelines, which call for health plans to request formally that drug companies present a standardized “dossier” containing detailed information not only on the drug’s effectiveness and safety, but also on its economic value relative to alternative therapies.\textsuperscript{34}

But again, there is likely to be resis-
tance to using CEA too explicitly. When asked in surveys, for example, many health plan managers have stated that cost-effectiveness does not play a role in decision-making, or that it is a minor, secondary consideration after clinical factors. Explanations accompanying this answer include a lack of understanding about the approach, a belief that analyses are not relevant to their “perspective,” a mistrust of the data or the motives of study sponsors, and a fear that using economic evaluations explicitly to withhold care risks litigation or negative publicity.

Health plans have historically been more comfortable using other ways to manage pharmacy budgets, in particular by implementing incentive-based formulations whereby enrollees face higher co-payments for using expensive, brand-name drugs rather than less expensive generics or other alternatives. Other policies include requiring prior approval before physicians can prescribe certain medications, targeting physicians who prescribe more than recommended doses, and profiling physicians to monitor high cost prescribers. They will likely tread carefully with CEA, using analyses not to accept or reject a drug for a formulary but to guide questions about a drug’s place in therapy: Is it on the preferred drug list? To which patient subgroups is it targeted? To which formulary tier does it belong?

Conclusions

Cost-effectiveness analysis offers an important approach for getting value for money in health care. Despite this, CEA has not yet occupied the important place at the health policy table that many experts have anticipated. Medicare’s push towards evidence-based medicine has not carried CEA along with it. This leaves the problem of rising Medicare spending and the prospect of new and costly medical technology on the horizon — policy makers will find ways, implicitly if not explicitly, to set limits. They would benefit from using cost-effectiveness analysis as a more formal input into the process.

References

5. 1862(a)(1)(A), Social Security Act, italics added.
7. Ibid.
9. For convenience, I use the term “cost-effectiveness analysis” in this paper generally to mean a formal economic evaluation of a healthcare technology, service, or program. Many political and policy discussions about the use of economic evaluations refer generically to cost-effectiveness analysis without restricting the focus to analyses that use standard metrics such as cost per life-year or cost per quality-adjusted life years (QALYs).
11. Ibid.
14. Weinstein MC. High-priced technology can be good
18. Federal Register. Medicare program: criteria and procedures for making medical services cover