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Economic Analysis in Healthcare Decisions

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The potential use of economic analysis in healthcare decision making has strong natural appeal as it becomes clear how an individual's healthcare decisions can affect, through insurance coverage, the ability of others to obtain the care they need. Healthcare spending in the United States continues to rise faster than inflation and worker salaries. Financial pressures force difficult decisions about whether to increase premiums, reduce benefits, or reallocate resources. Using analytic methods that are designed to maximize the value of the health services purchased for a given level of spending makes intuitive sense. The failure to adopt such a framework given the current economic realities in healthcare seems, at best, unwise. The two articles by Neumann¹ and Bloom² that appear elsewhere in this issue illustrate this point quite well.

The application of economic analysis in healthcare decision making, however, particularly with regard to medical necessity, has proven to be highly controversial. Evidence varies regarding the extent to which decision makers actually use economic analysis, a point that Bernard Bloom discusses in this issue of the *Journal*.² The reluctance to explicitly address financial issues in this context may well reflect a "deep-seated abhorrence of limits and of the corporate or government officials who impose them," and Peter Neumann's article cogently presents this case.¹ In addition, the discussion of the use of cost-effectiveness analysis (CEA) can benefit from a broader understanding of other important sources of tension within the policy process through which decisions are made about coverage of specific items and services. Significant disagreement exists over the appropriate locus for medical decision making, the integrity of the process used by payers to make these decisions, and the proper role of scientific evidence.

Locus of Decision Making

One important source of tension regarding decisions about medical necessity is the degree of deference accorded to judgments and preferences of patients and their physicians. The idealized view is that clinicians and patients arrive at optimal choices by carefully considering the pros and cons of the existing alternatives, free of all factors other than clinical risks and benefits and individual patient preferences. In this model, clinicians attempt to identify the clinical intervention that best meets the patient's need, separating this clinical decision from any consideration of what the indirect impact of this decision might be. Only at the level of these individual decisions, it is argued, can all the subtle variations in clinical presentation and social context be thoroughly understood and factored into determinations about what is the best medically necessary care for the patient.

Unquestionably, clinical decisions are best made by informed patients consulting with their clinicians. However, many outside factors that have little to do with clinical benefits and preferences also influence healthcare decisions. Patients are exposed to an increasing volume of direct-to-consumer advertising and to healthcare information from the Internet and other sources that are of variable objectivity and quality. Clinicians also receive a large volume of information from commercial entities and the media that may not reflect a carefully balanced perspective on the value of medical alternatives. Several decades of health services

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research, documenting widespread underuse, overuse, and misuse of healthcare technologies and services, demonstrate that reliance on judgments at the level of individual patient encounters does not ensure that medically necessary care will be consistently provided.³

The persistent discomfort with payers as a locus for making decisions about medical necessity is manifested in a range of social and legal developments. The backlash against managed care in the 1990s was primarily driven by the powerful concern of patients and policy makers over the idea that those who pay for healthcare services could override the decisions of clinicians and patients regarding diagnosis, treatment, and specialist referral. In legal proceedings, challenges to noncoverage decisions are generally received with considerable sympathy by judges and juries who see sick patients with limited options for getting well having their doctors overruled by organizations whose primary functions are administrative, not clinical. States continue to pass laws mandating that specific services be covered in order to prevent insurers from limiting access to certain technologies and services, and requiring independent appeals mechanisms outside the control of the payer for medical necessity decisions. In the past few years, the US Congress passed federal laws that allow beneficiaries to appeal local and national medical necessity policies, and that require Medicare to issue draft coverage decisions for public comment. These changes reflect steady pressure from industry advocates and a demand for additional accountability when making policy that blocks payment for medical services deemed to be medically appropriate by patients and their physicians.

Process for Medical Necessity Decision Making

A second important source of tension over coverage decisions made by public and private payers is the process for making these decisions, particularly with respect to explicitness, consistency, transparency, and opportunity for outside input. With a few exceptions, payers have been rightfully criticized on each of these elements of the process. Historically, the process by which payers made these decisions was not described in detail; few payers provided any clear description in their contracts or other documents about how these decisions were made, by whom, with what information, and in what time frame. Prior to 1999, the Medicare program communicated coverage decisions through brief statements in the policy manual describing what would or would not be covered. Furthermore, no formal process was in place for making these decisions, and outside stakeholders had limited opportunity to participate in or even track the development of specific policy decisions.

Many public and private payers have taken a very different approach over the last several years, investing considerable resources to make the medical policy process more scientific and transparent. The Blue Cross and Blue Shield Association has conducted rigorous scientific reviews of medical technologies for nearly 20 years and applies well-defined criteria in evaluating existing evidence. Initially available only to subscribing health plans, the technology assessment reports have been made available to the public since June 2003. More recently, the Medicare program has made significant changes to improve the process for making medical necessity decisions.⁴ A description of the process now appears in the Federal Register and can be found on the Medicare coverage Web site (www.cms.gov/coverage). Clinical studies are evaluated through the well-described framework of evidence-based medicine, and decision memos are now posted on the coverage Web site, providing detailed explanations of what evidence was reviewed and how the coverage policy was derived from the evidence. Substantial opportunity for outside input exists, with numerous calls and meetings with stakeholders taking place for each coverage decision. An independent expert Medicare Coverage Advisory Committee was also established to provide a public forum in which particularly complex or controversial decisions can be discussed. While these process improvements have not eliminated significant differences of opinion regarding specific decisions, the aggregate effect has been to substantially reduce the frequency and intensity of conflicts with stakeholders, and to allow for an orderly and informed exchange of views on a better-defined and more tractable set of issues.

While considerable progress has taken place, there remains a high level of discomfort with many aspects of the process for making coverage decisions. Significant variations in medical necessity policy still exist between health plans that cannot be easily explained. Many health plans continue to develop these policies through mechanisms that are neither reliable nor transparent.⁵ Medicare has not yet been successful in publishing regulations or guidance documents providing a definition of medical necessity, or articulating the criteria that are applied to medical technologies in order to determine whether they are “reasonable and necessary.”

The Role of Evidence-based Medicine in Medical Necessity Decisions

While an increasing number of organizations have adopted evidence-based medicine as the analytic framework for making medical necessity decisions, considerable discussion continues regarding the appropriate application of this framework. First and foremost is

the question of how to determine when the overall quality of evidence is adequate to demonstrate medical necessity for individual technologies and services. While the framework provides a well-defined set of rules to guide the appraisal of evidence from clinical studies, the translation of this assessment into categorical decisions of medical necessity may require subtle judgments about study methods.⁶

A second critical and challenging issue is how different sources of information about risks and benefits should be weighed when making medical necessity decisions. Specifically, different views exist about the relative importance of local expert opinion as reflected in community standards of care, national expert clinical consensus as articulated in clinical practice guidelines and consensus documents, and conclusions about clinical effectiveness derived directly from high-quality, peer-reviewed clinical research. Many stakeholders maintain that the state of the art in clinical practice may sometimes be ahead of the state of the science in the published literature, and that the clinical community determines the value of clinical interventions through experience long before confirmation is provided in published studies. Others raise questions about the reliability of professional consensus by pointing to the large geographic variations in care,⁷ high rates of inappropriate care, and underuse of services of proven effectiveness.⁸

Beyond these fundamental issues, numerous secondary issues must also be addressed. Should evidence requirements be applied differently for different categories of technologies (eg, drugs versus devices)? Should diagnostic technologies be evaluated based on diagnostic accuracy alone, or should there be evidence to support the impact of the technology on patient management and health outcomes? What is the optimal point in time to assess new technologies? Should technologies in widespread use be reviewed to determine whether they are supported by good clinical studies, and should they be held to a different standard of evidence than new technologies? Who is responsible for generating evidence regarding the effectiveness of existing and new technologies? These and many other issues related to the use of evidence-based medicine in healthcare policy making are the subject of highly spirited debate, again highlighting the fact that the challenges and resistance attributed to the use of cost-effectiveness analysis must be viewed as part of a broader set of challenges.

Challenges Specific to CEA

In addition to the 3 categories of issues discussed above, several critical challenges must be confronted regarding the use of CEA in medical necessity deci-

sions. These major challenges include concerns about validity and transparency of CEA methods, uncertainties about motivations for use of CEA, and the difficulties inherent in applying results to individual patients.

Neumann correctly notes that tremendous progress has been made in developing and standardizing the methods of economic analysis in healthcare over the past 25 years. The number of such studies published in first-tier peer-reviewed journals provides strong validation of the credibility and usefulness of these reports. Whether or not the methods are sufficiently robust or transparent to be used in medical necessity determinations is a separate question. Economic models are inherently more complex and less transparent than analyses of the clinical studies that comprise the effectiveness component of the models. Many important assumptions are embedded in decisions about the model structure, and it is challenging to ensure that every important decision is fully disclosed and explained. This complexity poses particular problems because CEA sponsors' motivations may be suspected of guiding the analysis in ways that may be difficult to detect. Given the critical role of transparency and comprehensibility in the legitimacy of policy decisions about medical technology, CEA raises special challenges over and above those faced when weighing clinical risks and benefits.

Even under the assumption of robust and generally accepted CEA methods, the application of these techniques to payers' decisions remains problematic. Some discomfort with these techniques may be explained by a general public resistance to recognizing that healthcare resources are limited, but the discomfort is heightened when decisions to limit care are made by organizations for whom reduced spending may be linked to improved financial performance. Use of economic analysis under the premise that resources are limited raises questions about who is actually benefiting from any savings, particularly when applied by for-profit entities.⁹ Greater acceptance of economic analysis for decision making in the United Kingdom (and other countries with a publicly funded national health service) may reflect in part a greater cultural emphasis on equity, but may also emerge from the structure and financing of the British healthcare system. Because virtually all healthcare in the United Kingdom is paid for through a single national program in which all individuals participate, the reality of a fixed budget is more self-evident, and the logic of applying CEA to use resources efficiently becomes less suspect. While fundamental differences in history and values may explain why the United States and United Kingdom have such different approaches to financing and deliv-

ering healthcare, the substantial differences that now exist between these systems could themselves explain observed differences in receptivity to the use of CEA in decision making.

Finally, and possibly most significant, the application of economic analysis in making decisions about medical necessity becomes highly problematic whenever it is translated to decisions about the care of individual patients. The determination that a particular technology or service is not cost effective means that specific individuals, someone's parent, spouse, child, or friend, may be unable to obtain that potentially beneficial intervention because it has been deemed not to be worth the price. Confronting such dilemmas on the individual level invariably raises virtually impossible objections to the use of CEA for individual yes/no decisions about what will and won't be covered. When a decision not to pay for a particular service is made by weighing the clinical risks against the benefits, the underlying logic is to avoid exposing the individual to possible harm. Using CEA for such decisions can be translated to mean that a clinical benefit must be foregone because the price is too high.

Putting Resistance to CEA in Context

It has become a widely held view that the inability to complete a federal rule-making process to define the term "reasonable and necessary" results primarily from discomfort with the proposed inclusion of cost effectiveness as one of the criteria. Neumann's article explains this in the context of our unwillingness as a society to accept that healthcare resources are limited. I would agree that the public desire for healthcare services exceeds the allocated resources. However, successful completion of rule-making related to coverage decisions, with or without inclusion of a cost-effectiveness criterion, would also require the development of some consensus regarding the locus of decision making, transparency of the process, and the appropriate use of evidence in decision making.

Despite the challenges facing the use of economic analysis in decisions about technology and procedures, it does have a critical role to play in healthcare. While it may not be a perfected tool, it provides a robust methodology to support efforts to purchase the greatest amount of health for any given level of healthcare spending. Because of the special challenges raised by using economic methods to make categorical decisions, it may be preferable to further develop policy approaches that include cost outside the realm of medical necessity. Medicare has recently taken some steps

in this direction with respect to 2 very high cost technologies with marginal, operator-dependent benefits: lung volume reduction surgery for end-stage lung disease and left ventricular assist devices for destination therapy for end-stage heart failure. In each case, Medicare limited coverage of these procedures to centers with evidence of unique expertise, both to increase the likelihood that patients would have improved outcomes and to promote appropriate use of these high-risk, costly interventions. The pricing of a new immunoassay used to screen for colorectal cancer was derived in part from an economic model comparing this test to alternative colorectal cancer screening technologies. In this case, the statutory language authorizing Medicare to add coverage for the new colorectal cancer screening test explicitly directed the Secretary to include costs as one of the factors to be considered.

Obtaining good value from healthcare spending is an urgent policy priority, made more so by the accelerating pace of medical discovery. Perhaps the most important benefit of expanding the use of economic analysis in health policy decision making would be to support more informed public dialogue about how best to achieve a healthcare system that produces high-quality and safe healthcare, allows patient freedom of choice in healthcare decisions, maintains healthy innovation, and expands access to care while remaining affordable. A decision-making framework that explicitly includes economic analyses would enable us to adopt explicit and consistent reimbursement guidelines that link healthcare benefits to the amount paid.¹⁰ Without such a framework, linkage between quality and cost is considerably more difficult. In an environment of increasing pressure on healthcare resources, important opportunities to improve public health may be missed.

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