



Background Paper
November 29, 2006

Value-Based Coverage Policy in the United States and the United Kingdom: Different Paths to a Common Goal

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OVERVIEW— *This background paper traces the development within American health care of two interrelated trends and activities: an evidence-based approach to medical practice and the critical evaluation of new technologies with respect to their costs and effectiveness. Over the past 35 years each of these developments has increasingly shaped the coverage decisions of public and private health insurers, and their importance for coverage policy is certain to grow. The paper also contrasts the different approaches to such “evidence-” or “value-based” coverage policy in the mixed public and private U.S. health care enterprise with the approach taken in Great Britain’s single-payer National Health Service.*

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Whenever a third-party payer—someone other than the patient or the patient’s family—is involved, the question “Is this service covered?” can come up. Patients/consumers ask the question because the answer determines their out-of-pocket costs for the service. Health service providers ask because coverage affects the likelihood that they will be paid. The response has also been important to health care insurers and purchasers (typically employers or government programs) because it affects their profitability or solvency. With health care now accounting for 16 percent of the U.S. economy, the answer to “Is this service covered?” also matters for society overall. The value-for-money of the U.S. investment in health care is a growing concern. Yet how health plans—private insurers, employers, managed care organizations, Medicare, and Medicaid—decide which services should be covered is fraught with countervailing interests and considerations. “Value-based coverage policy,” which takes the financial consequences as well as the clinical implications of coverage decisions into account, is one approach to making these determinations that is increasingly advocated (and challenged) in technologically advanced economies around the globe.

Health care services paid for by health plans (private or public) are described in general terms by benefit categories, for example, physician services, hospital inpatient care, and physical therapy. Sometimes a plan will specify a maximum amount of a covered benefit, for instance, limiting the number of hospital days or physician visits that it will pay for within a certain time period. Experimental therapies have traditionally been excluded from insurance coverage. These kinds of provisions are referred to as limits to the amount, duration, and scope of covered benefits. Forty years ago, when private insurance for hospital and physician services was still a relatively young industry (and Medicare and Medicaid were in their infancy), whether or not a service was covered depended more on who provided the service or where the service was provided than it did on the particular service itself. As the array of medical services and technologies has burgeoned, however, naming broad categories of services and limiting coverage in terms of volume (the number of physician visits in a month

or the number of hospital days during a spell of illness) no longer suffice. Even blanket exclusion of care involving experimental therapies has been reconsidered as the costs of clinical research have risen drastically.

Coverage policies affect the use of services, the speed with which new interventions are adopted, the quality of health care provided, and ultimately health outcomes. They also affect the level of overall health care spending and help determine the value realized for dollars spent on health care. Health policymakers have a vital interest in how decisions are made about which health care interventions (see endnote)¹ will be paid for and under what conditions particular interventions are considered covered benefits.

Over the past four decades, health care decision-makers in the United States and the United Kingdom have taken distinctly different approaches to evaluating interventions and making coverage determinations. A comparison of these differences illustrates that, to paraphrase George Bernard Shaw, these are two nations divided by their responses to a common problem. Contrasting these responses may provide policymakers with valuable insights as they endeavor to promote effective medical practices and contend with ever-rising health care costs.

BACKGROUND

Forty years ago, the medical profession's best judgments about standards of care and appropriate interventions for particular medical conditions largely determined what constituted appropriate medical care. The basis of the Medicare program's coverage policy—statutory language borrowed from private health insurance policies—is essentially the same as it was when Medicare was enacted in 1965: Coverage and payment are limited to items and services that are deemed to be "reasonable and necessary" for the treatment of illness or injury. In the health care arena today, however, professional judgment no longer suffices as the arbiter of coverage policy. Medical care has become too complex and too expensive to rely on professional consensus alone. In 2004, health care accounted for 16 percent of the U.S. gross domestic product (GDP) or \$6,280, on average, for every man, woman, and child. In 1970, the corresponding figures were about 7 percent of GDP and \$480 per capita (in today's dollars).²

Some of this rapid growth in spending on health services can be attributed to general price inflation, increased use of health care by people with little or no access to services in the past, and an aging population. A significant component of the growth in health care costs, however, is due to increased intensity of services provided to each patient—in significant measure because of newer, more complex interventions. Yet physicians, insurers, public program managers, and large group purchasers of health care (employers and unions) do not know the extent to which the increased use of more technologically advanced health care, at growing cost, improves

health outcomes. More critically, there is often little or no scientific evidence for physicians to determine the likelihood of a particular patient's having an improved outcome with a certain course of treatment. And even when scientifically valid evidence supports the effectiveness of a particular intervention and identifies which types of patients are most likely to benefit from it, practicing clinicians are overloaded with information about advances in practice and may not apply new knowledge.

These trends and problems are not limited to the U.S. health care enterprise. Economically developed nations around the world, with health care delivery systems and financing structures that are quite different from those in the U.S., are experiencing similar growth in health care costs due to rapidly expanding and technologically sophisticated interventions—and greater use of services by their populations. Between 1999 and 2004, for example, the annual rate of growth in per capita health expenditures (in real terms) was 4.0 percent for Canada, 4.5 percent for New Zealand, 3.9 percent for Sweden, 5.4 percent for the United Kingdom, and 4.8 percent in the United States.³

Other nations also face the challenges of developing a better knowledge base for medical practice and ensuring that this information is accessible to and readily used by health care providers and patients. To the extent that health care practice is grounded in scientifically valid evidence, advances in practice have global relevance. The results of evaluations of the accuracy of a new diagnostic test or of studies of the clinical effectiveness of a pharmaceutical product can be shared across national borders, as long as any relevant differences among populations and professional practices are taken into account.

Over the past 35 years, the evaluation of health care interventions ("health technology assessment") and determinations of coverage in the United States have been dispersed among a number of public and private entities. In the United Kingdom, on the other hand, these same activities have occurred within that country's universal, centrally financed National Health Service (NHS), spearheaded by the NHS's National Institute for Health and Clinical Excellence (NICE).⁴ A comparison of the approaches taken by these two very different health care systems and the political and policy levers available within each system is revealing. For example, unlike most coverage decisions made in the United States, the more centralized technology assessment process in Great Britain considers information about an intervention's cost effectiveness when deciding whether that intervention should be provided within the NHS. At the same time, Great Britain's more systematic approach to assessing new health care interventions has prompted the more rapid introduction of effective new interventions throughout the NHS, particularly within local health authorities, or "trusts," that had been slow to introduce them.

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EVIDENCE-BASED MEDICINE AS THEORY AND PRACTICE

The rapid growth in clinical research in the latter half of the twentieth century spurred the development of a new aspiration for medical practice: “evidence-based medicine” (EBM). Progress in the biological sciences yielded insights into basic physiological processes and disease mechanisms. This new knowledge, combined with the statistical tools and methods of the emerging discipline of clinical epidemiology, produced an unprecedented volume of research literature relevant to medical practice.⁵ The increase in the number of randomized clinical trials was exponential; more than half of all the clinical trials conducted between 1954, when the first trial was conducted, and 1995 were completed in the last five years of that period. Over 10,000 new trials were reported in 1995.⁶ MEDLINE, the biomedical bibliographic database of the U.S. National Library of Medicine, contains approximately 13 million references; more than 500,000 of these were added in 2004 alone.⁷

The tools of EBM include literature reviews, criteria for assessing the quality of research evidence for the effectiveness of a health care practice or intervention, and explicit clinical practice guidelines. Faced with formidable amounts of research findings to assimilate and limited time to follow developments in medical science, clinicians have come to rely on medical journal survey articles that summarize the research relevant to a particular condition or medical intervention. Over time, the format and content of such survey articles themselves have been formalized and become more rigorous, with professional societies such as the American College of Physicians and the editors of the *British Medical Journal* establishing publications standards for systematic literature reviews.⁸

Systematic literature reviews employ strategies to reduce bias in the identification, appraisal, and synthesis of studies addressing a specific clinical question. These strategies include “grading” the quality of evidence produced with a given research design in a particular study. In some cases, the quantitative results of different randomized clinical trials are combined in meta-analyses, to create summary estimates of the impact of a particular intervention.

Clinical Practice Guidelines

In addition to increasingly rigorous systematic literature reviews, evidence-based medicine has been promoted through clinical practice guidelines. Long promulgated by professional societies in North America and Europe, such “systematically defined statements to assist practitioner and patient decisions about appropriate health care for specific clinical circumstances”⁹ have in the past few decades become better grounded in research evidence.¹⁰

It should not be surprising that the earliest evidence-based guidelines were for clinical preventive services. Preventive services aim to protect and

improve “statistical lives”—the estimated number of people in a population group of a certain size that faces a risk of disease or death without the screening services. Diagnostic and therapeutic services, in contrast, treat the problems of identified patients. The desire to do something rather than nothing for people presenting with illness or injury is strong. It can override the analytical and critical question of whether what can be done is actually effective or beneficial. Also, because preventive services (such

Evidence-Based Medicine and Value-Based Coverage Policy: Not Entirely New

Evidence-based medicine appears to be a relatively recent development; in fact, it simply represents the latest stage in the evolution of best practices in the art and science of medicine. Likewise, value-based coverage policy seems novel, yet it too is continuous, with activities going back at least 35 years. A number of related factors have contributed to these paradigms.

First, evidence-based medicine (EBM) grew out of the vastly improved information about clinical mechanisms and outcomes that became available during the second half of the last century. Revolutionary advances in the biological sciences and in epidemiology—particularly the innovation of the randomized clinical trial—not only put the practice of medicine on a sounder scientific footing, but also presented clinicians with an enormous amount of clinical evidence to assimilate and interpret.

Second, powerful and frequently expensive new technologies, including diagnostic equipment, surgical interventions, and pharmaceuticals, have proliferated over the past four decades (for example, CT (computed tomography) scanners in the early 1970s), and their startling costs and rapid spread have raised questions about their appropriate use.

Third, geographic variations in clinical practices and investments in medical technology have persisted since they were initially documented more than three decades ago, without evidence of corresponding impacts on health outcomes. This prompted researchers, health care purchasers,

and policymakers to investigate the relationship between particular clinical practices and health outcomes, beginning in the early 1990s.*

Fourth, and related to the previous point, concerns about the quality and safety of health care services persist, even as evidence-based best practices, in the form of clinical guidelines and in research publications, have been well established.† EBM has been propelled by a growing recognition that all medical interventions carry risks and that these risks must be managed in light of the benefits conveyed by the intervention.

Last, the rate of increase in health care spending, fueled in great part by health care innovations and the increasing intensity of services provided to each patient, has caused public and private payers and health plans to increase their scrutiny of new interventions before deeming them eligible for coverage or inclusion in the plan.

* John E. Wennberg, Elliott Fisher, and Jonathan Skinner, “Geography and the Debate over Medicare Reform,” *Health Affairs*, web exclusive, February 13, 2002, pp. W96–114, available at content.healthaffairs.org/cgi/content/full/hlthaff.w2.96v1/DC1; Elliott S. Fisher et al., “The Implications of Regional Variations in Medicare Spending. Part 2: Health Outcomes and Satisfaction with Care.” *Annals of Internal Medicine*, 138, no. 4 (February 18, 2003), pp. 288–298.

† E. A. McGlynn et al., “The Quality of Health Care Delivered to Adults in the United States,” *New England Journal of Medicine*, 348, no. 26, (June 26, 2003), pp. 2635–2645; Steven M. Asch et al., “Who is at Greatest Risk for Receiving Poor-Quality Health Care?” *New England Journal of Medicine*, 354, no. 11 (March 16, 2006), pp. 1147–1156.

as screening tests and immunizations) initially were not covered by private health insurance or Medicare, a justification for including them in health plans was needed.

The U.S. Preventive Services Task Force

In 1987, the U.S. Preventive Services Task Force (USPSTF) was established as an independent federal advisory committee under the auspices of the U.S. Public Health Service, with a mandate to review and synthesize evidence about clinical preventive services and to create practice guidelines for primary care physicians. The USPSTF developed standards for presenting evidence of effectiveness for specific services such as screening tests, patient education, and behavioral interventions. The first USPSTF published its *Guide to Clinical Preventive Services: Assessment of the Effectiveness of 169 Interventions* in 1989. The impact of this guide on the new discipline of EBM was significant. The explicit criteria for the quality and strength of research evidence underlying the task force recommendations helped to set a research agenda for filling in gaps in the existing evidence base. The guide also contributed to the trend away from expert consensus-based clinical guidelines and toward ones grounded in the systematic evaluation of clinical epidemiological research.¹¹

A second USPSTF was established in 1990 to review and incorporate more recent outcomes studies, and a third was convened in 1998. The recommendations of this latest task force incorporate, for the first time, information about the relative cost-effectiveness of particular services.¹²

DEVELOPMENT OF HEALTH TECHNOLOGY ASSESSMENT IN THE UNITED STATES

Evidence-based medicine and the economic evaluation of health care interventions are closely related to health technology assessment. Health technology assessment, once limited to the Food and Drug Administration's (FDA's) pharmaceuticals review and approval process focused on safety, has become an important analytic tool in the funding and coverage of health care services. The concurrent and intertwined development of evidence-based medicine and health technology assessment has led to the interest in and debates about value-based coverage today.

The FDA's Initial Role

The FDA is charged with determining the safety and efficacy of new drugs and devices as a condition of licensing them for sale. The Food, Drug, and Cosmetic Act (FDCA) of 1938 established the FDA with the mandate to determine the safety of new pharmaceuticals before they could enter interstate commerce. In 1962, an amendment to the act required manufacturers to establish the effectiveness of new drugs, in addition to their safety. A further amendment of the FDCA in 1976 extended the scope of the

FDA's technology assessment role to the regulation of new medical devices. (The original FDCA limited the FDA role regarding medical devices to removing any from the market that were proven to be dangerous or fraudulent.) The 1976 device provision of the FDCA required "valid scientific evidence" of new devices, while limiting the FDA to reviewing only high-risk devices. A crucial provision of the amendment exempts any new device from FDA review that is "substantially equivalent" to one that had been introduced prior to 1976. In fact, the great majority (90 percent) of all new devices enter the market under this provision.¹³

The standard of effectiveness employed by the FDA in evaluating new drugs is minimal. New therapeutic agents must be assessed in (usually at least two) randomized clinical trials against a placebo, to demonstrate the existence of a therapeutic effect, rather than to demonstrate greater or even equivalent therapeutic effect against the prevailing therapy (a drug already in use, for example). Following the introduction of approved drugs and devices into the health care marketplace, the FDA requires manufacturers to conduct post-marketing surveillance to develop additional information on adverse events, side effects, and contraindications associated with their use that did not surface in pre-approval trials and uses. The vast majority of the FDA's resources, however, are devoted to preapproval review rather than postmarketing surveillance.

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Congressional Office of Technology Assessment: Focus for a New Field

The genesis of the formal practice of health technology assessment (beyond the FDA's limited role) is often traced to the work of the Health Program within the U.S. Office of Technology Assessment (OTA).¹⁴ Congress established OTA in 1972 to serve the legislative branch as an independent source of information and analysis about complex scientific and technical issues. OTA construed health technology broadly, including "all elements of medical practice that are knowledge-based, including hardware (e.g., equipment and facilities) and software (e.g., knowledge skills)...the set of techniques, drugs, equipment, and procedures used by health-care professionals in delivering medical care to individuals and the systems within which such care is delivered."¹⁵ Over its 22-year life, OTA not only conducted in-depth studies of the effectiveness and cost-effectiveness of specific technologies and health care interventions, it also developed the broader framework to structure those individual assessments.

In 1978, Congress also authorized creation of the National Center for Health Care Technology within the Department of Health and Human Services (DHHS). The center's role was advisory and informational: to provide information to state and local health care facilities planning authorities, to advise the Medicare program regarding coverage of new technologies, to set priorities for health technology assessment research, and

to develop improved methods for health technology assessment.¹⁶ The center operated for three years before it was reincarnated as the Office of Health Technology Assessment (OHTA) within the National Center for Health Services Research (NCHSR), another DHHS agency. OHTA's more circumscribed role was to advise the Medicare program on coverage of new technologies, helping to determine when a new intervention was no longer experimental but rather standard medical practice. Despite this limited charge, the influence of OHTA (and of its successor agencies) extended beyond Medicare; private insurers and health plans tended to follow Medicare's determinations about when interventions crossed the line from experimental to standard practice.¹⁷

Evolution from NCHSR to Agency for Healthcare Research and Quality

In 1989, Congress authorized the Agency for Health Care Policy and Research (AHCPR) to succeed the NCHSR (including OHTA), and invested the agency with a new charge: to develop, disseminate, and evaluate clinical practice guidelines. To this end, AHCPR established the Forum on Quality and Effectiveness in Health Care, which convened experts from both the public and private sectors to develop practice guidelines. AHCPR's original mandate emphasized reducing variations in medical practice and outcomes. The agency was given a much larger annual budget than its predecessors, almost \$100 million, of which two-fifths was earmarked for the development of clinical practice guidelines and medical effectiveness research.¹⁸ Although cost containment had not been explicit in its original charge, AHCPR's reauthorization in 1992 directed the agency to incorporate cost-effectiveness information into its technology assessments and to consider costs in developing practice guidelines.¹⁹ This expansion in the scope of AHCPR's evaluation activities to include cost effectiveness contributed to the circumstances that resulted in Congress's displeasure with the agency three years later.

The first of three Institute of Medicine (IOM) committees to address the development of clinical practice guidelines was tasked by AHCPR with helping to chart a course and propose methodological standards for the Forum's work, in accordance with the agency's legislative mandate. The IOM committee described the state of the art of practice guidelines development as progressing, but with "deficiencies in method, scope, and substance."²⁰ It noted a proliferation of guidelines without a coordinative mechanism to resolve inconsistencies and evaluative quality. Two years later, a second IOM committee issued recommendations for using clinical practice guidelines as evaluative tools to improve quality of care, involve practitioners in devising applications, and regularly review and update guidelines. The committee also outlined a research and evaluation strategy for the refinement of guideline development and implementation.²¹

A third IOM committee later recommended a strategy for AHCPR to set priorities for guidelines development and proposed that the agency establish a clearinghouse to identify and disseminate clinical practice guidelines developed by other organizations. The committee hesitated to assign AHCPR the added task of evaluating the soundness of such guidelines, however, and instead recommended further testing of assessment tools and criteria.²² In the same year that this third IOM report was issued, AHCPR's very existence was threatened, in part as a result of the politically effective opposition mounted by a well-organized group of orthopedic surgeons to practice guidelines for the treatment of low-back pain that the agency had issued.²³ Congress slashed the agency's 1996 budget by about 20 percent, reducing it to \$125 million.

AHRQ: A NEW APPROACH

In 1999, Congress reauthorized and renamed AHCPR, expanding the agency's charge to include (i) evaluation of the cost and use of health care services and access to services and (ii) promotion of evidence-based health care practices. At the same time, it redefined the role of the DHHS technology assessment agency with respect to clinical practice guidelines. The stance that AHCPR's successor, the Agency for Healthcare Research and Quality (AHRQ), takes in regard to clinical practice guidelines reflects the hesitation that the IOM committee expressed a decade ago and—even more—its near-death experience. The agency's role in guideline development now consists in the production of the underlying information about effective interventions through evidence reports and technology assessments. AHRQ also supports research on effective clinical practices and dissemination of guidelines through the National Guideline Clearinghouse.²⁴

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Evidence-Based Practice Centers

AHRQ's activities to promote evidence-based health care practices are organized around its support of 13 Evidence-based Practice Centers (EPCs), typically university-affiliated organizations that prepare "science syntheses," evidence reports and technology assessments, on specific clinical topics. These products can then serve as the basis for practice guidelines, performance measures, and educational materials devised by other agencies and organizations.²⁵ The first 12 EPCs were established by AHCPR in 1997, with five-year contracts. In 2002, a second round of contracts was awarded to 13 EPCs, including three first-time awards. Each of the EPCs has particular expertise in one or more area of clinical practice or evaluation methodology. Three of the EPCs specialize in conducting technology assessments for the Medicare program, and another one primarily supports the work of the USPSTF.

Topics for EPC reviews can be proposed by professional societies, health systems, insurers, employers, providers, and consumer groups. AHRQ solicits topic nominations annually through a notice in the *Federal Register*, but accepts them at any time. Evidence reports and technology assessments generally take about 15 months to complete and publish. These reports and assessments do not make clinical or coverage recommendations.²⁶

A New Collaboration with CMS: The Effective Healthcare Program

AHRQ oversees several programs in addition to the work of the EPCs. The Effective Healthcare Program, authorized by Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA), was launched in 2005 with a \$15 million budget. The Effective Healthcare Program encompasses a research network to generate evidence of clinical effectiveness, in addition to the synthesis of existing research by the EPCs. Section 1013 of the MMA directs AHRQ to conduct or support research, demonstrations, and evaluations to improve the quality, effectiveness, and efficiency of Medicare, Medicaid, and the State Children's Health Insurance Program. Research supported by the Effective Healthcare Program is to focus on health outcomes, comparative clinical effectiveness, and appropriateness of services and items such as pharmaceuticals, including their organization, management, and delivery. Research results are to be made widely available, through print and electronic media, to an audience that includes private health plans, pharmaceutical benefits programs, health care providers, and the general public, in addition to the federal and state health financing programs. As directed by the authorizing legislation, the Secretary identified ten priority conditions for initial study under this program.

This list was developed and published at the end of 2004, after considering testimony from the public and stakeholders at a "listening session" earlier in the year and written comments submitted to the Department of Health and Human Services.²⁷ It reflects the program's initial focus on the elderly and disabled Medicare population and on conditions involving drug therapies.

The network of 13 research centers contracted with AHRQ to address these priorities and complement the work of the Evidence-based Practice Centers is called DEcIDE (Developing Evidence to Inform Decisions about Effectiveness). These 13 centers include academic, clinic-based, and practice-based research groups that have access to electronic health information databases, primarily electronic health records, with which to address questions of health outcomes and comparative clinical effectiveness. The research centers may engage in methodological studies, comparisons of health outcomes using secondary databases, evaluations of formulary structure on outcomes, analysis of existing disease registries or prescription databases, prospective studies of health outcomes, and evaluation of drug therapy decision-making tools.²⁸

Initial Priority Conditions of the Effective Healthcare Program

- Ischemic heart disease
- Cancer
- Chronic obstructive pulmonary disease/asthma
- Stroke, including control of hypertension
- Arthritis and non-traumatic joint disorders
- Diabetes mellitus
- Dementia, including Alzheimer's disease
- Pneumonia
- Peptic ulcer/dyspepsia
- Depression and other mood disorders

Pharmaceutical Outcomes Research

AHRQ and its predecessor agency have funded research relating to drug therapies since 1992. AHRQ's pharmaceutical outcomes program now consists of the Centers for Education and Research on Therapeutics (CERTS), 11 academically based research centers and a Coordinating Center (at Duke University). The CERTS program was first authorized as part of the Food and Drug Administration Modernization Act of 1997 and then expanded in AHRQ's authorizing legislation in 1999. The CERTS research program has three basic aims: (i) to increase awareness of the uses and the risks of new drugs, combinations of drugs, and devices, and of ways to promote their appropriate use; (ii) to provide clinical information to all interested parties, including patients, providers, and third-party payers; and (iii) to improve quality and reduce the cost of health care by increasing appropriate drug use and safety.²⁹

AHRQ's Delivery-System-Based Research and Demonstration Networks

In addition to the programs and activities described above, AHRQ also supports a number of practice-based, delivery-oriented research, demonstration, and evaluation projects. These include the Integrated Delivery System Research Network and its follow-on program, Accelerating Change and Transformation in Organizations and Networks (ACTION), with 15 collaborative initiatives, and the Primary Care Practice-based Research Networks. The focus of these initiatives is applied research and the dissemination and prompt adoption of innovative practices and evidence-based products, tools, and strategies.³⁰

MEDICARE COVERAGE POLICY

Medicare has dual systems for determining coverage policy: local decisions made by the roughly 50 regional fiscal intermediaries and carriers that pay hospitals, physicians, and other service providers on behalf of Medicare, which may vary in detail and timing of coverage from place to place, and decisions made by the Centers for Medicare & Medicaid Services (CMS) that apply nationally. CMS reports that about 90 percent of all coverage determinations are made at the local level; only about 18 to 24 national coverage decisions are made in a given year.³¹ This structure for coverage policy is rooted in the original design of the Medicare program to limit the influence and role of government in medical practice. It has survived because of the medical technology industry's support of local coverage determinations, which are perceived as more responsive and timely than the national coverage determination process. However, a comprehensive analysis of local Medicare coverage decisions as of 2001 concluded that the local process was not necessarily quicker than national coverage decisions and recommended greater standardization of coverage policies, at least for new technologies and technology extensions.³²

CMS describes Medicare's national coverage process as follows:

Medicare coverage is limited to items and services that are reasonable and necessary for the diagnosis or treatment of an illness or injury (and within the scope of a Medicare benefit category). National coverage determinations (NCDs) are made through an evidence-based process, with opportunities for public participation. In some cases, CMS' own research is supplemented by an outside technology assessment and/or consultation with the Medicare Coverage Advisory Committee (MCAC). In the absence of a national coverage policy, an item or service may be covered at the discretion of the Medicare contractors based on a local coverage determination (LCD).³³

Medicare Coverage Advisory Committee

Before 1999, Medicare followed no formal process or standard for publication or comment in national coverage determinations.³⁴ Coverage decisions were conveyed through updates to the program's policy manual, which governed the actions of its contractors, the Medicare Part A (hospital) fiscal intermediaries and Part B (physician) carriers. Since April 1999 all coverage decisions have been posted on the program's Web site, along with background information on the issue and the rationale for the decision.³⁵

The MCAC was chartered late in 1998 to advise the Secretary of Health and Human Services whether specific items and services are reasonable and necessary under Medicare law.³⁶ Only a few national coverage issues are referred to the MCAC for a recommendation. The MCAC serves as a forum in which complex or controversial issues, proposals, and questions can be examined as openly as possible. It provides a setting and process for the presentation of evidence, deliberation, and a public exchange of views about a given coverage issue.

The MCAC was originally designed to operate with a number of specialized panels, with representatives from each panel serving on the MCAC executive committee. In the absence of explicit guidance from the Health Care Financing Administration (CMS's predecessor) on how the MCAC should operate, the executive committee tasked a working group to create interim operating principles. The document that the working group presented to the executive committee articulated the MCAC's role as determining whether there was adequate evidence to conclude that a treatment was effective. The standards of evidence outlined in the document, however, created concern among the medical technology industry, which led to congressional action to define the MCAC's role and to eliminate the structure of specialized panels.³⁷

Under its current charter, the committee may consist of up to 100 members, who are selected for their expertise in a variety of health-related fields.³⁸ A maximum of 88 members are voting members, and the additional 12 non-voting members (equally divided) may represent consumer or industry interests. No more than 15 members, including one representative each

from industry and consumer groups, attend a given meeting, based on their particular knowledge regarding the matters under consideration. The MCAC meets between four and eight times during a given year.

Assessing New Technologies for the Medicare Program

Another resource (in addition to seeking the advice of the MCAC) that CMS can employ in making national coverage determinations is to commission a technology assessment from another agency or organization. Whether conducted in-house or commissioned externally, technology assessments to support a national coverage determination always include a systematic review of the published evidence about health outcomes related to the intervention in question. In cases in which CMS concludes that it needs assistance because the body of evidence to review is extensive, conflicting, or requires special expertise not available from staff, it typically turns to AHRQ to either conduct the technology assessment directly or assign the task to one of AHRQ's Evidence-based Practice Centers.

Technology assessments to support a national coverage determination always include a systematic review of the published evidence about health outcomes.

National Coverage Determinations

Requests for a coverage decision can originate with anyone, including practitioners, manufacturers, beneficiaries, or contractors. The MMA revised and codified several aspects of the Medicare national coverage determination process, effective January 2004. It established timeliness standards for the issuance of coverage determinations following receipt of a formal request. For requests that do not require an externally conducted technology assessment or review by the MCAC, the deadline for making a draft of the proposed national coverage determination available to the public is six months following the date of a request. If Medicare program administrators decide that an external technology assessment or MCAC review is needed to inform a coverage decision, CMS has nine months in which to make a draft of the proposed national coverage determination available to the public. A 30-day public comment period follows publication of the proposed coverage determination on the CMS Web site. Sixty days following the close of the comment period, the Medicare program issues a final decision.

Coverage with Evidence Development

Over the past several years, Medicare has adopted several policies to promote the development of evidence for coverage decisions involving new technologies. Since September 2000, Medicare has paid for the costs of routine care for patients in clinical trials, making the undertaking of such studies less costly for those supporting the innovative research. Medicare

will also pay for certain experimental devices during the period over which they are being evaluated in clinical trials under an investigational device exemption (IDE) policy.³⁹

In some cases of promising technologies that do not meet Medicare's standards for national coverage, Medicare has construed "reasonable and necessary" to make coverage conditional on providing the intervention in the context of a clinical research study. The most notable example of this is the National Emphysema Treatment Trial (NETT), in which the benefits, costs, and risks of lung volume reduction surgery for patients with severe emphysema were evaluated over a seven-year period (1997 to 2003). The clinical trial itself was sponsored by the National Institutes of Health and AHRQ, but Medicare paid for all the clinical costs of care associated with it.⁴⁰ The results of this study ultimately led to Medicare coverage of lung volume reduction surgery for only that subset of patients for whom benefit had been demonstrated.

More recently, Medicare has made several additional national coverage decisions in favor of new technologies contingent on further evidence development. These include the following:

- Off-label uses of colorectal cancer drugs within several clinical trials sponsored by the National Cancer Institute
- PET scans for patients with suspected dementia who are part of a clinical trial (not necessarily randomized)
- Implantable cardioverter defibrillators, with submission of data to a clinical registry to ensure the defibrillator was provided in a reasonable and necessary manner and for subsequent analysis of risks, benefits, and indications for use

In July 2006, CMS issued a guidance document that describes those instances in which CMS may issue a national coverage determination that provides coverage for an item or service only in the context of additional data collection—a concept labeled coverage with evidence development (CED).⁴¹ This guidance document defines two variations of CED. The first, coverage with appropriateness determination (CAD), describes instances in which CMS determines that an item or service is nationally covered but requires more information to ensure that it is provided in the manner prescribed in the national coverage determination. The guidance document mentions the following four concerns that may result in CAD as a condition of coverage: (i) "if the newly covered item or service should be restricted to patients with specific conditions and criteria," (ii) "if the newly covered item or service should be restricted for use by providers with specific training or credentials," (iii) "if there is concern among clinical thought leaders that there are substantial opportunities for misuse of the item or service," and (iv) "if the coverage determination significantly changes how providers manage patients who use this newly covered item or service."

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**Stymied Attempts
to Incorporate
Cost-Effectiveness
Criteria in Medicare
Coverage Policies**

In 1987, as a consequence of a legal settlement with a Medicare beneficiary who sued for reimbursement of an angioplasty procedure performed prior

to a coverage determination, the Medicare program published its first notice explaining its coverage process. Two years later, a second *Federal Register* notice in the form of a proposed rule expanded upon the earlier one. The 1989 notice proposed, for the first time in Medicare's history, that a cost-effectiveness criterion be applied. Further development of the policy was immediately subject to delays as a result of opposition from professional and industry groups. An effort to issue the proposed rule in final form in 1996 met with substantial opposition, and the 1989 notice was finally withdrawn in 2000.

In 2000, a Medicare notice of intent was published that proposed criteria for determining which items and services are "reasonable and necessary." The criteria included demonstrable medical benefit and added value. The notion of "added value" implied that, in the case of two or more similar technologies used for the same purpose that are equally beneficial, only the lowest-cost approach would be covered.* This criterion of added value proved controversial, however, and CMS announced, in a September 2003 *Federal Register* notice, that it did not intend to develop a proposed rule based on the notice of intent.† Forestalling any further efforts to issue regulations to govern coverage decisions, Section 731 of the Medicare Pre-

scription Drug, Improvement and Modernization Act of 2003 directed CMS to publish factors considered in the process for making national coverage determinations as "guidances."[‡]

CMS complied in 2006 with publication of guidance on the circumstances under which a national coverage determination is sought and when an external technology assessment is commissioned. It includes the following disclaimers:

Cost effectiveness is not a factor CMS considers in making NCDs [national coverage determinations]. In other words, the cost of a particular technology is not relevant in the determination of whether the technology improves health outcomes or should be covered for the Medicare population through an NCD.[§]

TAs [technology assessments] are, and will continue to be, focused on clinical factors pertinent to beneficiaries' health outcomes. Furthermore, while economic considerations may be a factor discussed in a technology assessment, the primary purpose of a TA is to evaluate the clinical and scientific evidence pertaining to the clinical benefits and risks of the technology, and cost is not a factor in our review or decision to cover a particular technology.[¶]

Although Medicare's administrative policies governing coverage determinations have not included cost-effectiveness criteria, cost-effectiveness has been important when legislative action has been required to expand Medicare coverage for specific preventive services, such as influenza and pneumococcal immunizations, colorectal cancer screening, and osteoporosis screening.

* Sean R. Tunis and Jeffrey L. Kang, "Improvements in Medicare Coverage of New Technology," *Health Affairs*, 20, no. 5, (September/October 2001), pp. 83–85.

† U.S. Department of Health and Human Services (DHHS), "Notice of Revised Process for Making National Coverage Determinations," *Federal Register*, vol. 68, no. 187 (September 26, 2003), pp. 55634–55641.

‡ DHHS, "Procedure for Producing Guidance Documents Describing Medicare's Coverage Process," *Federal Register*, vol. 69, no. 185 (September 24, 2004), pp. 57325–57326.

§ Centers for Medicare & Medicaid Services (CMS), "Guidance for the Public, Industry, and CMS Staff: Factors CMS Considers in Opening a National Coverage Determination" April 11, 2006; available at www.cms.hhs.gov/mcd/npc_view_document.asp?id=6.

¶ CMS, "Guidance for the Public, Industry, and CMS Staff: Factors CMS Considers in Commissioning External Technology Assessments," April 11, 2006; available at www.cms.hhs.gov/mcd/npc_view_document.asp?id=7.

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A second type of CED described in the guidance document is termed coverage with study participation (CSP). Under CSP, CMS will allow coverage of certain items or services for which the evidence is not adequate to support national coverage and where additional data would further clarify the health impact of these items and services for Medicare beneficiaries. In the past, this level of evidence would have prompted non-coverage decisions. The document lists some of the evidentiary findings that might lead to this decision:

- Available evidence may be a product of otherwise methodologically rigorous evaluations but may not have evaluated outcomes that are relevant to Medicare beneficiaries.
- The available clinical research may have failed to address adequately the risks and benefits to Medicare beneficiaries of off-label or other unanticipated uses of a drug, biologic, service, or device.
- Available clinical research studies may not have included specific patient subgroups or patients with disease characteristics that are highly prevalent in the Medicare population.
- New applications may exist for diagnostic services and devices that are already on the market, but there is little or no published research that supports a determination of reasonable and necessary for Medicare coverage at the time of the request for an NCD.
- Sufficient evidence about the health benefits of a given item or service to support a reasonable and necessary determination is available only for a subgroup of Medicare patients with specific clinical criteria and/or for providers with certain experience or other qualifications. Other patient subgroups or providers require additional evidence to determine if the item or service is reasonable and necessary.

PRIVATE-SECTOR AND STATE INITIATIVES

Technology assessments have a public good aspect that works against private-sector endeavors in this field. Investments in developing new knowledge about effectiveness and cost-effectiveness of specific services and coverage policies cannot easily be kept confidential (and thus monopolized) to preserve the competitive advantage of the organization making the investment in new information. In a centrally financed and administered or regulated health services sector, economies of scale can be realized with a coordinated technology assessment and evidence development process. This model is exemplified in the British National Health Service. Still, despite potential “free riders,” both private-sector organizations and state agencies in the United States support technology assessment activities.

Blue Cross Blue Shield Association's Technology Evaluation Center

Established in 1985, the Blue Cross Blue Shield Association's Technology Evaluation Center (TEC) was one of the first private-sector agencies devoted to assessing evidence of the clinical effectiveness of new technologies. The center conducts 20 to 25 assessments each year evaluating specific technologies in terms of their clinical effectiveness and appropriateness. Assessments are produced by TEC's core staff and reviewed and authorized by a Medical Advisory Panel of nationally regarded experts. The advisory panel of 19 members meets three times yearly. In 1993, TEC began a collaboration with Kaiser Permanente. As part of this joint effort, Kaiser experts have served as advisors to TEC and sit on its Medical Advisory Panel. TEC has also served as an AHRQ-supported Evidence-based Practice Center since 1997.

TEC employs a set of five criteria to assess health interventions, including drugs, devices, and procedures:

- A technology must be approved by the appropriate governmental regulatory body (typically FDA).
- Scientific evidence must support judgments about the intervention's effect on health outcomes.
- The intervention's beneficial effects should outweigh any harmful effects, and thus improve the net health outcome.
- The intervention must be as beneficial as any established alternatives.
- The intervention's benefits must be achievable under the usual conditions of medical practice.⁴²

Assessment reports follow a specific format. While they may include analyses of cost-effectiveness or compare the relative effectiveness of different clinical approaches, they always evaluate whether the intervention improves health outcomes such as length of life, quality of life, and functional abilities.

Until 2003, the center's assessments were available only to subscribing members. Since then, however, final technology assessment reports have been posted publicly. As with the recommendations of the MCAC, reports of the TEC are advisory only; TEC evidence evaluations are separate from coverage decisions, which are made by individual health plans.

Drug Formularies

In 1998, Regence Blue Shield in Washington State became the first U.S. health insurance organization to require economic evidence as a condition

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of formulary review.⁴³ This initiative became the precedent for guidelines promulgated by the Academy of Managed Care Pharmacy in 2001 for drug manufacturers' submissions of evidence of effectiveness, safety, and value for formulary inclusion. Such standards not only serve to signal the kinds of information that health care plans consider essential for making formulary decisions, they also reduce the uncertainties and costs that manufacturers face in preparing documentation for coverage reviews by multiple health plans.

In recent years, the federal-state Medicaid program has accounted for roughly 20 percent of national spending for prescription drugs: almost \$34 billion in 2003. As of 2005, 34 state Medicaid programs used some form of preferred drug lists or restrictive formularies in administering their prescription drug benefit.⁴⁴ The Oregon Medicaid program led the way with the use of evidence in formulary design with the Drug Effectiveness Review Project (DERP). Since 2001, Oregon has commissioned systematic analyses of drug effectiveness by therapeutic classes from the Evidence-based Practice Center at Oregon Health and Science University (OHSU) to inform decisions about the inclusion of specific therapeutic agents in its Medicaid drug formulary. Only therapeutic agents within a class for which there is evidence of effectiveness in actual clinical practice (and not just in research settings) are included.⁴⁵ In 2003, other states were invited to join with Oregon in supporting DERP. As of 2006, 15 states are participating in support of DERP, primarily through their Medicaid agencies.⁴⁶

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DERP reviews are now produced not only by the Oregon EPC, but also by others in California and North Carolina. The project plans to complete review of 25 therapeutic classes over a three-year period. Each review is updated for new evidence every 7 to 24 months. The reviews answer questions in three broad categories: (i) how drugs within a given class compare in overall effectiveness, (ii) how drugs within a given class compare in terms of safety and adverse events, and (iii) how safety and effectiveness profiles for a specific drug may differ for subpopulations.

DERP reports do not include cost information. Reports are made publicly available without charge on the project's Web site. AARP and Consumers Union have created consumer-oriented summaries of DERP reviews and have posted these summaries on their open Web sites.⁴⁷ Just this year, a companion program to DERP, the Medicaid Evidence-based Decisions Project (MED) was launched at OHSU to provide states with access to systematic reviews, technology assessments, an interactive Web-based information clearinghouse, and information and analyses tailored to the state's particular circumstances.⁴⁸

THE BRITISH NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE (NICE)

By the late 1990s, the British National Health Service, probably the most popular British institution since its establishment in 1948, was experiencing a crisis of public confidence in the way resource allocation decisions were made.⁴⁹ First, the public became increasingly aware of disparities in the availability of new pharmaceuticals and other technologies across regions—dubbed “postcode prescribing”—as a result of differences in funding priorities and decisions by local health authorities with fixed annual budgets for all NHS services within a given area. Second, the budgetary pressures created by continued advances and growth in health care technologies appeared to threaten the financial sustainability of the NHS. These distinct yet related concerns provided the impetus for the creation of NICE in 1999.⁵⁰

NICE was constituted as a Special Health Authority within the NHS to promote clinical excellence and the effective use of available resources in the health service. Its original charge was to

(i) conduct technology appraisals of drugs, devices, diagnostic techniques, surgical procedures, and health promotion interventions; (ii) develop clinical practice guidelines, taking into account both the effectiveness and cost-effectiveness of particular interventions with respect to specific health conditions; (iii) determine the safety and effectiveness of interventional (that is, invasive and radiological) procedures; and (iv) develop clinical audit methodologies. In 2005, NICE was also tasked to develop public health guidance on the effectiveness and cost-effectiveness of individual interventions and programmatic strategies to improve population health.⁵¹

NICE has quasi-independent status within the NHS. The directors of the institute’s governing board are elected by an independent appointments commission. The institute employs about 200 staff in all capacities. NICE reported a budget of 27.7 million pounds for its most recent annual period, 2005–2006.⁵² Three centers within the institute develop guidance for NHS programs and providers: the Centre for Public Health Excellence, which develops guidance on public health programs and interventions; the Centre for Health Technology Evaluation, which conducts technology appraisals and evaluates interventional procedures; and the Centre for Clinical Practice, which develops clinical guidelines.

Technology Appraisal Process

NICE’s technology appraisal process affects the adoption of new technologies, including pharmaceuticals, by local health authorities of the NHS. The topics that NICE addresses in its technology appraisals are selected by the Department of Health, the agency that administers the NHS. Topics

“Postcode prescribing” and budgetary pressures, distinct yet related concerns, provided the impetus for the creation of NICE.

may be proposed by a variety of individuals and groups, including health professionals, patients and the general public, clinical directors within the Department of Health, manufacturers, and the National Horizon Scanning Centre of the University of Birmingham, a group that tracks emerging technologies.⁵³ Together, NICE and the department define the scope of the technology assessment.⁵⁴

Technology appraisals are conducted by multidisciplinary committees convened by NICE. These committees include experts from academia and the NHS, with the support of core institute staff. Organizations representing stakeholders (for example, patients, health professionals, and manufacturers) can be involved in the appraisal process as “consultees” to provide information to the Appraisal Committee and review draft guidance. A technology assessment report (TAR), a systematic review of evidence, serves as the starting point for a technology appraisal. NICE commissions such assessment reports from one of eight independent academic groups.⁵⁵ External contracts, including payments to NICE’s collaborating centers, account for 38 percent of NICE’s annual budget.⁵⁶ Preparing a TAR takes from six months to one year.

Recently, however, NICE initiated a fast track for single products (the single technology assessment, or STA, process), which has cut the time needed for review by half. In an STA, the manufacturer’s submission serves as the principal source of evidence. Evidence review groups (the same academic groups that produce TARs) review and critique the submission.

After receiving the TAR, the Appraisal Committee considers it, along with additional information supplied by manufacturers and other interested parties. In addition to the findings of the systematic evidence review, the ultimate technology appraisal reflects equity considerations (such as fair treatment of people across the age spectrum and with different health conditions) and political and institutional judgments.⁵⁷ After reviewing all materials submitted to NICE, the Appraisal Committee issues a provisional determination. This is sent to the interested parties for comment. The committee then reviews comments and makes a determination. This determination can be appealed before NICE issues its guidance to the NHS.

Shortly after its establishment, NICE published guidelines for manufacturers and sponsors submitting evidence. These guidelines covered the presentation of information about clinical effectiveness, cost-effectiveness, and wider impacts on the NHS of a funding decision. The guidelines were later revised to further standardize submissions of evidence. The guidelines now specify that the perspective from which economic evaluations are conducted be that of the public decision maker, that is, as valued for the population as a whole, and that the intervention (for example, drug) being assessed should be compared to the most commonly used alternative intervention.⁵⁸

NICE conducts 30 to 50 appraisals annually. In its appraisals and recommendations NICE is not responsible for considering affordability, that is, the overall budgetary impact of a newly introduced intervention on a

health authority. Regional administrators of the NHS have budget caps that affect their decisions about purchase and deployment of expensive equipment. Since 2002, if NICE determines that a new, expensive technology is cost-effective, NHS authorities in England and Wales must make resources available to finance the provision of that intervention for its approved indications within three months of NICE's determination (unless a specific exception is issued by the Minister of Health). However, this directive is not always followed.⁵⁹ Since 2001, all evidence considered by NICE is made public.

The NHS maintains an Economic Evaluation Database that identifies, summarizes, and critiques evidence from published studies that include economic information about clinical interventions. This database is employed in technology appraisals. The preferred outcome measure for reporting cost-effectiveness results is cost-per-quality-adjusted-life-year (cost per QALY). Although students of NICE have observed that recommended interventions tend to have a cost per QALY at or below roughly \$31,000 to \$46,000, NICE does not impose an explicit cost-effectiveness cutoff value.⁶⁰ Factors that are considered in addition to the intervention's estimated cost-effectiveness include, for example, the availability of any other interventions for the condition involved and equity concerns.⁶¹

NICE has operated, in its relatively short tenure, during a period of steady growth in NHS funding.⁶² At some point, however, the rate of increase in NHS resources almost inevitably will slow and NICE will have to answer for its funding recommendations to local health authorities facing tighter budget constraints. The NICE technology appraisal process has earned credibility because of its rigorous scientific review process and the transparency of its procedures and deliberations. A World Health Organization review of the NICE program over its first four years concluded that NICE had led the field internationally in innovations in technology appraisals—but also noted that NICE had been operating in a fiscal and policy environment that supported the approval of new, cost-effective medicines and technologies.⁶³

The transparency of NICE's appraisal decisions has increased opportunities to challenge the legitimacy of NHS funding decisions.⁶⁴ NICE has been criticized for being too quick to approve expensive interventions.⁶⁵ At the same time, it has been accused of slowing the adoption of new drugs and technologies by declining to recommend that local health authorities pay for them.⁶⁶ Glaxo-Wellcome sought coverage approval for a new drug to treat the symptoms of influenza (zanamivir, or Relenza) prior to the 1999–2000 flu season. NICE initially recommended against funding zanamivir because of its relatively high cost for a small benefit. Health technology agencies around the world followed NICE's lead and declined to cover the drug.⁶⁷ NICE reversed its decision for specific high-risk patients in

The NICE technology appraisal process has earned credibility because of its rigorous scientific review process and the transparency of its procedures and deliberations.

November 2000, when additional clinical and economic evidence demonstrating the cost-effectiveness of zanamivir in these patients became available. NICE can err either by determining that an intervention is valuable (and cost-effective) when it is not or by determining that an intervention should not be funded when, in fact, it would be cost-effective to provide it. Both types of errors carry risks of patient welfare and misallocated resources.

Several strategies have been proposed to reform the operation of NICE. Some of these would strengthen the institute's ability to constrain cost growth within the National Health Service. One such proposal is that NICE be given a fixed "growth" budget for the net cost of new technologies. This would require the institute to consider the aggregate budgetary impact of its recommendations for funding new technologies.⁶⁸ A second proposal would require that NICE first approve all new technologies that seek NHS funding through local health authorities, rather than just a subset of new interventions, as is now the case.⁶⁹ A third approach would extend NICE's reviews to existing interventions that may be ineffective or inefficient—defunding them to make room in local health budgets for effective and efficient services.⁷⁰ Finally, some have urged that NICE pay more attention to the equity implications of its recommendations and address their distributive impacts more explicitly, particularly with regard to funding services used by people of different ages.

A STUDY IN CONTRASTS (AND SIMILARITIES)

The ideal of medical practice grounded in scientifically valid evidence of effectiveness is embraced on both sides of the Atlantic. Likewise, standards and processes for the appraisal of new interventions have grown more demanding in both American and British settings. Advances in the methods and application of EBM and technology assessment are the result of experiences and knowledge shared internationally.

A review of the history of technology assessment and coverage decisions in the United States shows that these issues received attention relatively early (late 1960s and early 1970s) as health insurance coverage expanded and costly new technologies entered the market. It also reveals a typically American ambivalence about economic evaluations of new technologies and attempts to standardize medical practice during the past several decades. More recently there has been a renewed interest in establishing both a scientific basis for medical practice and the comparative effectiveness of alternative interventions. The use of economic information, including cost-effectiveness analysis, to evaluate innovative technologies and practices for coverage decisions remains suspect. Nevertheless, cost-effectiveness analysis has been applied selectively in U.S. settings—in Veterans Administration formulary decisions and in legislation requiring Medicare coverage of preventive services, for example.

Great Britain's adoption of systematic technology assessment and economic evaluation of new interventions was a response to a different set of

pressures and concerns. NICE was established to spur the diffusion of effective new interventions throughout the NHS. Its performance has demonstrated that economic evaluation is not a synonym for cost control; cost-effectiveness analysis can help to demonstrate the underuse of effective interventions as well as inappropriate or inefficient use. NICE has also enjoyed a degree of political insularity that U.S. agencies tasked with similar functions (OTA and AHRQ's predecessors) did not have. It is unclear whether or how this independence from political pressures could be achieved on this side of the Atlantic.

One development evident in both Great Britain and the United States is adherence to standards of transparency and public participation in coverage decisions within public programs. Over the past decade, Norman Daniels, a moral philosopher, and James Sabin, a physician, have developed ethical principles for health care coverage decision making—in both the public and the private sectors.⁷¹ They argue that decisions that limit coverage and therefore access to expensive health care interventions can be ethical or fair if the decision-making process meets certain conditions: that coverage decisions are based on information and reasoning to which all stakeholders—patients, clinicians, payers, patent holders, and manufacturers—have access (that is, the decision process is transparent); that the decisions and the process by which decisions are reached are held accountable for being reasonable to those whose interests are at stake in the decision; and that an appeals process is in place. These criteria for fair coverage decisions appear to have gained wide acceptance, explicitly or implicitly, among public and private health plans. Increased publicity is of course facilitated by Web-based information and communication capabilities. Nevertheless, the commitment of Medicare (with AHRQ) and NICE to explain their decisions, consider appeals, and create opportunities for public participation throughout their review processes signals a genuine reform in public policymaking.

A second significant trend is the movement toward greater consistency in and harmonization of informational and analytic standards in technology assessments internationally. The Academy of Managed Care Pharmacy standard format for submitting economic information on drugs for inclusion in formularies has contributed to this trend. Perhaps it is not surprising that manufacturers have promoted efforts to standardize such requirements. Compliance with disparate documentation requirements for coverage decisions by various purchasers has been frustrating and costly for pharmaceutical and medical device companies. They have become allies in efforts to define a standard approach to technology assessment—including evidence of cost effectiveness—so that they can more efficiently prepare research and analytic results for coverage reviews in multiple national markets.

Pharmaceutical and medical device companies have become allies in efforts to define a standard approach to technology assessment.

BARRIERS TO AND OPPORTUNITIES FOR VALUE-BASED COVERAGE

It may seem obvious why economic evaluation has become an integral part of technology assessment and funding decisions within the British National Health Service while it has remained controversial and suspect in the United States—for public as well as for private payers:

- Historically, the British have been more willing than Americans to accept limits to health care, and the NHS is a trusted public institution.
- A single-payer system allows for a unified perspective on cost effectiveness: the payer is society as a whole, benefits are calculated for the entire population, and the population remains in the system over entire lifetimes. In contrast, the multipayer, mixed public and private American health care enterprise means that each payer has a different perspective on the cost effectiveness of a particular investment or coverage decision. The characteristics of each payer's enrollees may differ in ways that affect the results of a cost-effectiveness analysis, and turnover in enrollment within health plans makes it difficult to capture longer-term health payoffs.

It is perhaps more remarkable how similar the activities of NICE and its affiliates are to those of U.S. centers of evidence-based practice and technology assessment. The difference is that in the United States the various parts have not been joined together to form a unified whole; rather, each piece functions relatively autonomously and decision making remains dispersed among many payers. A proposal to create a national center to develop and disseminate comparative effectiveness information has recently been forwarded, in recognition that this information is a true public good that is undersupplied in a market economy.⁷² Such a center could, conceivably, knit together the technology assessment and effectiveness research and synthesis that now is conducted by various public and private-sector organizations.

The continuing challenges facing evidence- and value-based coverage policy are also the same internationally. Data from well-designed clinical trials are expensive to collect and too scarce, and useful results often become available only after coverage decisions have been made. Furthermore, innovation in medical practice is a dynamic process. The population that receives a particular intervention changes and expands. Adaptations in the intervention and in accepted practice may take place without explicit documentation or reexamination. These shifts in application once a new technology enters medical practice make definitive assessments of efficacy, risk, and cost elusive.⁷³

The data and analytic practices that underlie evidence-based medicine and economic evaluation of health care interventions inevitably yield results that entail a considerable degree of uncertainty.⁷⁴ The range of plausible values (confidence intervals) around point estimates of cost

effectiveness has led some to conclude that cost-effectiveness analysis is not helpful in making coverage decisions. The uncertainty inherent in cost-effectiveness results is one reason why such analyses should never be the sole basis of coverage decisions. Yet decisions about what technologies should be covered, and for whom, must and will be made by those who pay for or administer health benefits. Others argue that considering the quality of evidence of effectiveness in a disciplined and explicit fashion—and juxtaposing the estimate of effectiveness with an estimate of the resources needed to achieve the beneficial results—improves on current implicit judgments and unsystematic coverage decisions.

The United States has avoided coming to terms with escalating health care costs and possibly diminishing returns on investments in health care. Perhaps more than anyone else, Americans value technological progress in medical care. The national bias in favor of innovation, along with the dispersion of responsibility for coverage decisions in American health care, makes it particularly difficult to focus public attention on the consequences of our “default” approach to allocating health care resources. When the United States is ready to take on these issues, AHRQ’s Evidence-based Practice Centers and Effective Healthcare Program offer an infrastructure for developing the kind of information needed. Medicare’s national coverage decisions process offers a framework for transparent and interactive policy development. And a model for citizen involvement in making hard choices in health care can be found in the British NHS.⁷⁵ However value-based coverage fares, the United States and Great Britain are likely to continue addressing their common problems in distinctive ways.

ENDNOTES

1. “Interventions” are defined here as diagnostic services, treatments—including but not limited to drugs, surgeries, and medical devices—and preventive and screening services.
2. Author’s calculations, based on Centers for Medicare & Medicaid Services (CMS), “Health Care System: Facts and Figures 1980-2000,” available at www.cms.hhs.gov/TheChartSeries/03_Facts_Figures_1980_2000.asp#TopOfPage; and Laurence J. Kotlikoff and Christian Hagist, “Who’s going broke? Comparing healthcare costs in ten OECD countries,” NBER working paper 11833, Cambridge, MA, 2005.
3. Author’s calculations, based on Organisation for Economic Co-operation and Development, “OECD Health Data 2006”; available at www.oecd.org/document/16/0,2340,en_2649_34631_2085200_1_1_1_1,00.html.
4. While many other nations with technologically developed health care systems are active in the fields of technology assessment, evidence-based medicine, and the economic evaluation of health care interventions, this review is limited to models and activities in the United States and Great Britain. Canada (Province of Ontario) and Australia, in addition to Great Britain, have been leaders in developing value-based coverage policies for pharmaceutical products in particular.

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6. Mark Chassin, "Is Health Care Ready for Six Sigma Quality?" *Milbank Quarterly*, 76 (1998), pp. 565–591.
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10. Steven H. Woolf *et al.*, "Potential Benefits, Limitations, and Harms of Clinical Guidelines," *British Medical Journal*, 318 (February 20, 1999), pp. 527–530.
11. Steven H. Woolf and David Atkins, "The Evolving Role of Prevention in Health Care: Contributions of the U.S. Preventive Services Task Force," *American Journal of Preventive Medicine* 20, suppl. 3 (2001), pp. 13–20.
12. The work of the third task force is supported by AHRQ's evidence-based practice centers, and its guidelines are available through the online National Guideline Clearinghouse (www.guideline.gov). For a comprehensive overview of the USPSTF, see Eileen Salinsky, "Clinical Preventive Services: When Is the Juice Worth the Squeeze?" National Health Policy Forum, Issue Brief No. 806, August 24, 2005; available at www.nhpf.org/pdfs_ib/IB806_ClinicalPrevServices_08-24-05.pdf.
13. Center for Devices and Radiological Health, "CDRH Annual Report, Fiscal Year 1997," U.S. Food and Drug Administration, October 14, 1997; available at www.fda.gov/cdrh/annrep97.html.
14. In fact, health technology assessment also has roots in the health planning and certificate of need programs of the early 1970s. Enacted in 1972, Section 1122 of the Social Security Act required that hospitals receive prior approval for large expenditures for the acquisition of new equipment and capital improvements in order to receive cost-based reimbursement from Medicare. Office of Technology Assessment (OTA), *Development of Medical Technology: Opportunities for Assessment*, (Washington, DC: U.S. Congress, August 1976); and Egon Jonsson, "Development of Health Technology Assessment in Europe: A Personal Perspective," *International Journal of Technology Assessment in Health Care*, 18, no. 2 (2002), pp. 171–183.
15. OTA, *Development of Medical Technology*, p. 4.
16. John M. Eisenberg and Deborah Zarin, "Health Technology Assessment in the United States," *International Journal of Technology Assessment in Health Care*, 18, no. 2 (2002), pp. 192–198.
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18. Eisenberg and Zarin, "Health Technology Assessment."
19. Neil R. Powe and Robert I. Griffiths, "Clinical-Economic Trials, Background Paper 5," *Tools for Evaluating Health Technologies: Five Background Papers* (Washington, DC: Office of Technology Assessment, 1994).

Endnotes / continued ►

Endnotes / continued

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The National Health Policy Forum is a nonpartisan research and public policy organization at The George Washington University. All of its publications since 1998 are available online at www.nhpf.org.

Glossary

Clinical Practice Guidelines — Systematically developed statements to assist practitioner and patient decisions about appropriate health care for specific clinical circumstances.

Cost-Effectiveness Analysis — An economic analysis in which all costs are related to one common measure of effectiveness. Results are usually presented as a ratio of the increase in costs associated with an increase in effectiveness.

Coverage Policy — Coverage policy is intended to promote value in medical care by using payment to encourage the use of effective care and by withholding payment for ineffective care. *Evidence-based coverage policy* promotes the adoption of medical practices of demonstrated effectiveness through coverage decisions and conditions of coverage.*

Economic Evaluation — Considering information about the economic costs of a health care intervention along with information about its benefits in a technology assessment or coverage decision. An economic evaluation can take the form of a cost-effectiveness analysis, a benefit-cost analysis, or a budgetary impact analysis.

Efficacy — The desirable effect of an intervention under ideal circumstances.

Effectiveness — The desirable effect of an intervention under real-world circumstances

Evidence-Based Medicine (EBM) — Both a framework for evaluating medical benefit and an approach to the practice of medicine. As defined by those who coined the term: “The conscientious, explicit, and judicious use of current best evidence in making decisions about health care.”†

As one policymaker characterized the aspirations of EBM, “Evidence-based medicine involves increased

reliance on formal, systematic analysis and synthesis of the research literature to determine clinical effectiveness. It challenges consensus-based judgments and applies critical assessment of the available research to decide if there is methodologically sound evidence that the outcomes of a clinical option are favorable, and it identifies types of patients for whom the service is most effective.”‡

Health Outcome — The ultimate consequences of a health care intervention, including, for example survival, quality of life, ability to function, and freedom from pain.

Health Technology — The drugs, devices, and medical and surgical procedures used in medical care and the organizational and supportive systems within which such care is provided.

Health Technology Assessment (HTA) — A multidisciplinary field that studies the medical, social, ethical, and economic implications of the development, use, and diffusion of health technologies. HTAs commissioned by the Medicare program to support national coverage determinations generally focus on the safety and efficacy of the intervention.

Medical Necessity — Term first used in insurance contracts after World War II, generally understood as medical care that most physicians considered appropriate.

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