OMERACT: Economic Evaluations and Health Policy

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ABSTRACT. The aim of this paper is to review how decisions are made about the delivery of health care. Limited resources and increasing health care costs have resulted internationally in a growing necessity to understand the economic consequences of health care delivery. Rheumatology remains underfunded because the societal impact of chronic conditions is not well appreciated in the current policy-making context. Although health policy development varies with the care delivery systems, economic evaluation can be used for incremental system-wide reform to prioritize spending across multiple diseases and interventions. It is important to understand how policy and funding decisions are made in order to see how they might be influenced. (J Rheumatol 2001;28:666–9)

Key Indexing Terms:
COST-EFFECTIVENESS ANALYSIS HEALTH POLICY HEALTH CARE ACCESS

Limited resources and increasing health care costs have resulted in a growing international necessity to understand the economic consequences of health care delivery. This has resulted in an imperative to include an economic perspective in clinical research. But unless the societal impact of chronic conditions is appreciated, these findings will not substantially influence the way health resources are distributed. This is unfortunate, because disciplines like rheumatology, whose proponents lessen this burden, remain underfunded and are undeveloped. It is important to understand how policy and funding decisions are made in order to see how they might be influenced.

Health policy development varies with the care delivery systems. Publicly funded systems and managed care organizations face different questions than do health insurers. But, worldwide, all funders of health care face the fact that rampant medical cost inflation means that, today, to remain viable, they must proactively bring to bear strategies to reduce their exposure to potential costs.

"For profit" health insurers traditionally manage their members by actuarially estimating their risk and loading potential high risk beneficiaries. While these methods ensure financial soundness of schemes, they pass costs accurately on to subscribers and do not affect rising costs, and thus, alone, cannot ensure the longterm affordability of access to health care for the majority.

Public insurers or state funders are "purchasers" of care with fixed budgets whose mandate is the provision of comprehensive services for large populations with a mixed risk profile. For them, cost containment strategies must focus on managing the range and extent of utilization of the benefits/therapies they provide. This is appropriate because it responds to the major causes of medical "inflation," namely the constant introduction into the marketplace of expensive health technologies and therapeutics that have some clinical benefits, but the overall effects of which are not adequately evaluated.

This new paradigm creates competition between therapies and interventions provided by different medical disciplines. However, when competition is implicit, rather than explicit, these decisions on resource usage are usually dominated by characteristics such as immediacy rather than a multifactorial process that includes a variety of factors such as cost-effectiveness. For clinical disciplines like rheumatology, whose focus is the diagnosis and treatment of chronic illness, explicit processes offer more hope, if the relative cost-effectiveness of their interventions can be clearly demonstrated.

Importantly, the credibility of studies on cost-effectiveness [i.e., demonstration of less cost for the same benefit/health effect (e.g., death averted)] or cost utility (demonstration of less cost for equivalent quantity and quality of life gained — this may be across various benefits, expressed as quality adjusted life years) requires that they be done as realistically as possible. Essentially, they constitute numerical models of actual situations. They must situate the pathology or intervention being explored in the fullest possible way, which means from the point of view of society, and not just the interests of purchasers or providers of care. (The development of models that include the full cost to society has been difficult to achieve, but the quality of these studies is improving with time and experience.)

POLICY MAKING AND IMPLEMENTATION STRATEGIES

Most interventions relying on cost economic evaluations are used in a piecemeal or episodic manner (typically undertaken at the point of introduction of a new therapy), reliance being placed on the cumulative effect of these to reduce costs. (Some of the mechanisms by which various health care systems do this are discussed below.) Recently, but very much more rarely, economic evaluations are driving system-wide reform, within "minimum benefit" packages (these are also discussed below).

The discussion covers some of the uses of "tools" of intervention: the evaluation, assessment, and decisions regarding the funding and provision of services. This can

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apply to: health technologies, drugs, remuneration of doctors, or the application of managed care tools that rely on sophisticated information to ensure value for money is obtained.

Industrialized countries differ in their chosen routes of health care delivery, and implementation strategies mirror these routes. A discussion limited to the health systems of a few countries follows.

THE UNITED STATES

The US largely favors a market driven delivery system, resulting in most interventions relying on indirect influence via financial incentives and on sponsored studies to effect decisions. However, because Americans do not share a single set of values about health care and how it should be used, even with better information about the utility and cost-effectiveness of interventions, decisions are not obvious. Further, explicit use of cost-effectiveness criteria for allocating health care resources has been problematic because of the absence of a widely accepted cutoff of a level of effectiveness that demands or excludes coverage. Finally, the concept of cost-effectiveness is politically difficult to separate from health care rationing, which is roundly rejected by the US public.

Policy imperatives. In the absence of macro level policies that limit the adoption of new drugs and technologies, a variety of mechanisms have emerged that seek to distinguish effective technology from those that are ineffective, with varying degrees of effectiveness.

1. HCFA coverage standard

(a) The federal Health Care Financing Administration (HCFA) funds 40~50% of total spending within the US health care system. Its decisions thus have a major influence on the industry as a whole.

(b) The law underlying Medicare coverage policy in the USA prohibits payments for "items or service, which are not reasonable and necessary" — i.e., a service should be safe and effective, appropriate, and not experimental.

(c) Medicare's prospective payment system: Because Diagnosis Related Groups (DRG) payment (i.e., classification of all hospital cases into clinically and resource use homogenous groups that attract a prospectively defined single global hospital fee) does not increase when additional services are provided, the policy created new incentives to be efficient in the hospital care of Medicare patients. In theory, prospective payment should encourage the introduction of cost saving technologies, such as those that reduce the length of hospitalization or substitute for more expensive tests.

Two aspects of DRG updating process have important implications for new technology use. Individual DRG payments are updated in a regular schedule to account for new technologies associated with specific diagnoses. Therefore the decisions made by HCFA regarding the likely effect of the introduction of a new technology can send an important economic signal. There is also a specific increment to the system overall to encourage technological innovation.

2. Health care technology in the United States[1]

Technology assessment has burgeoned in the US over the past decade. There has been continually increasing support for technology assessment methods in both the public sector and, unlike many other countries, but consistent with the prevalent funding mechanism, in the private sector. In addition to federal programs, insurers, drug and device manufacturers, hospitals, and professional societies have developed their own capabilities and have fueled the growth of contract technology assessment organizations and university based research groups.

(A) US Federal Technology Assessment initiatives.

(i) 1978, National Centre for Health Care Technology created by Congress to advise Medicare and Medicaid on coverage decisions. This lasted until 1981, and was closed down largely owing to opposition from bodies such as the American Medical Association, concerned with the loss of clinician autonomy, and the medical device industry, concerned with a stifling of innovation. However, it seems likely that the industry's major concern was the potential for new devices to fail in the market after negative evaluation from a central government source.

(ii) 1984 to 1989: the Council on Health Care Technology was a "public/private" initiative that was ineffective (excessive fundraising effort was partly responsible) and was replaced.

(iii) 1989, the Agency for Health Care Policy and Research (AHCPR), an important body whose major responsibility was "medical effectiveness research." Clinical Guidelines and PORT (Patient Outcomes Research Teams) are the main mechanisms whereby the AHCPR funds outcome research, for example, PORT on total knee replacement and hip fracture repair and osteoarthritis.

(iv) Office of Healthcare Technology Assessment (OHTA)[2] within AHCPR. OHTA undertakes reviews and assessments and recommends coverage decisions to Medicare; private insurers often used these assessments in developing their own coverage policies.

(v) The Office of Health Technology Assessment (OHTA).

(1) OHTA evaluates the risks, benefits, and clinical effectiveness of new or unestablished medical technologies. In most instances, assessments address technologies that are being reviewed for purposes of coverage by federally funded health programs.

(2) OHTA's assessment process includes a comprehensive review of the medical literature and emphasizes broad and open participation from within and outside the federal government. A range of expert advice is obtained by widely publicizing the plans for conducting the assessment through publication of an announcement in the *Federal Register* and solicitation of input from federal agencies, medical specialty societies, insurers, and manufacturers. The involvement of these experts helps ensure inclusion of the experienced and varying viewpoints needed to round out the data derived from individual scientific studies in the medical literature.

(3) OHTA analyzed and synthesized data and information received from experts and the scientific literature. The results are reported in this assessment. Each assessment represents a detailed analysis of the risks, clinical effectiveness, and uses of new or unestablished medical technologies. If an assessment has been prepared from the basis for a coverage decision by a federally financed health care program, it serves as the Public Health Service's recommendation to that program and is disseminated widely.

(vi) The Congressional Office of Technology Assessment (OTA) from 1972 to 1995 emphasized cost-effectiveness and randomized clinical trials, and included a critique of cost-effectiveness methods in benefit design, and an evaluation of the Oregon Medicaid system.

(vii) Private sector assessments

(1) Many professional bodies undertake some assessment including the American Academy of Paediatrics, the Clinical Efficacy Assessment Program of the ACP, and the American Medical Association Diagnostic and Therapeutic Technology Assessment Program.

(2) Blue Cross and Blue Shield established a medical necessity program in 1976 that includes the TEC (Technology Evaluation and Coverage) program. This program establishes: the status of regulatory approval; adequacy of scientific evidence about the effect of the technology on patients; net impact on health outcomes benefits compared with established therapies; and the effect obtained outside of research settings.

(3) University Hospital Consortium — since 1989. Reviews specific technologies and coordinates studies between member hospitals, assists with purchasing decisions, sets clinical guidelines, and selects drugs for formularies.

3. Physician remuneration

(A) Physician payment under Medicare. Payment to physicians increased in the mid-1980s, driven strongly by procedures such as cataract surgery, endoscopy, total knee replacements, hip replacements, hernia repair, and coronary artery bypass graft surgery, all of which were reimbursed at a high rate. Under the Resource Based Relative Value System cognitive services are now given relatively greater weight. The hoped-for effects are greater attention on the part of physicians to preventive and other primary care services, a gradual increase in the supply of generalists, and a decrease in the use of expensive technologies by specialists.

4. Managed care tools — use of data/information by managed care organizations.

(A) Utilization management involves (1) the collection of data on the pathology and therapy of patients, and (2) the application of preset algorithms to identify care that may not be appropriate.

(B) Physician profiling and "with-hold" mechanisms (a mechanism whereby some funds are withheld for payment on the successful achievement of targets) has in some cases been associated with significant changes in the use of medical technology. The Maine Medical Assessment Foundation brings physicians together to discuss the variation in the rates of use of common procedures, reported reductions in lumbar disc surgery, admissions for pediatric asthma, cesarian section, and hysterectomy using physician profiling and feedback.

DIRECTLY FUNDED HEALTH CARE SYSTEMS

Most European countries and Canada and Australia fund health care systems directly from funds derived from taxation. The tools of economic evaluations to funding decisions are consequently directly applicable by agencies that control these funds. Some examples include:

1. UK: The National Health Systems (NHS) National Institute for Clinical Excellence (NICE), the role of which is to enable control of the introduction of new technologies into routine service delivery within the NHS of England and Wales[3].

(A) Summary of the NICE Technology Appraisal Process

(i) The Department of Health (DH) and the National Assembly for Wales (NAW) will select technologies for appraisal by NICE based on one or more of the following criteria:

(1) Is the technology likely to result in a significant health benefit, taken across the NHS as a whole, if given to all patients for whom it is indicated?

(2) Is the technology likely to result in a significant effect on other health related government policies (e.g., reduction in health inequalities)?

(3) Is the technology likely to have a significant effect on NHS resources (financial or other) if given to all patients for whom it is indicated?

(4) Is NICE likely to be able to add value by issuing national guidance? For instance, in the absence of such guidance is there likely to be significant controversy over the interpretation or significance of the available evidence on clinical and cost-effectiveness?

(ii) NICE guidance to commissioners and clinicians:

(1) An assessment whether or not the intervention can be recommended as clinically effective and as a cost-effective use of resources, either in general or in particular circumstances.

(2) An assessment of wider implications for the NHS.

(3) A concise summary of the reasoning behind NICE recommendations.

2. Evaluating new drug therapies

Pharmacoeconomic studies (which are essentially cost-effectiveness studies regarding drug therapies) are required by Australian regulators to assist them making decisions about new drugs that manufacturers are applying to add or replace other drugs on the national list. This is a mechanism designed to put a ceiling on financial exposure to drug costs in these systems.

(A) Criteria for evaluating new drugs applying for entry to the national register in Australia:

(i) Is there a current alternative drug or is it a "breakthrough"?

(ii) Is it more clinically effective?

(iii) What are the financial outcomes? (For example, not "has the tumor size diminished," but "did the patient survive?")

(iv) How have these outcomes been modeled?

(v) What quality of life measures have been used?
(vi) What method of deriving utility was used? European Quality of Life instrument, etc.
(vii) What type of economic analysis was used?
(viii) Was an economic analysis of current alternative therapies performed?
(ix) What is the present (discounted) value of the net costs and outcomes?
(x) What is the cost per unit of the drug and what is the marginal cost of achieving each unit of benefit?
(xi) What effects will the drug's usage have on other drugs?
(xii) What are all the financial implications for the government?

Comment on the relative effectiveness of the different cost containment methods used. Countries with state funded health systems use direct strategies of containing high costs. The excessive use of expensive technology by hospitals is constrained by tight global budgets, and capital outlays for major equipment or facilities requires government approval. In some countries the numbers of surgeons and other specialist physicians are directly controlled. In sophisticated democracies these decisions often are political, sparking intense public debate, and governments have fallen for failure to gain societal agreement with interventions. Looking back, results are mixed. Some of these countries have rationed excessively, denying their populace useful cost effective therapies. Others have been unable to deny provision of obviously unnecessary care, resulting in excessive health care spending with little health gain and loss of important opportunities in other sectors. As discussed, the US uses a more indirect mechanism, relying on market forces using available data appropriately. Comparison of these strategies versus the US strategies "suggest that measures to control health spending in the US, whether legislative changes to programs such as Medicare and Medicaid or market driven approaches such as employers' shift to managed care, have not been excessive but are roughly in line with other countries. On the negative side, they provide no evidence that US strategies are working better than methods used by other countries." The inability to ration sensibly remains probably the most important reason behind the ongoing and again accelerating health care cost increases in the US.

USING ECONOMIC EVALUATIONS TO REFORM THE WHOLE HEALTH CARE DELIVERY SYSTEM

System-wide reform. The challenge for policy makers working towards this aim is to strike a reasonable balance between health care access that is still "comprehensive" and affordability.

The World Bank's 1993 "World Development Report — Investing in Health," suggests a methodology to improve government spending be introduced, based on epidemiological and economic analyses leading to a recommended essential public health and clinical services package for low and middle income countries. Central to this are the estimation of the burden of disease and the cost-effectiveness of interventions[4].

The critical issue thus becomes defining which therapies to include in a package of services that fits this definition. Service provision may be defined as positive inclusions or negative exclusions, and may be further refined by the application of additional factors such as age or comorbidity. The common starting point of defining a package of care is "league tables" of services, stacked by cost per gain, such as quality adjusted life year league tables. This was the basis of the Oregon Health Plan, and also forms the basis of the South African government's health system reforms. In both these situations this approach has helped to define a "minimum benefit package" that purchasers of care must, by law, provide.

This is important from a rheumatological viewpoint, as it essentially represents a comparison of cost utility study results across pathologies and interventions performed by specialists in a variety of disciplines — an explicit competition for funding. Both Oregon and SA began with an intention to prioritize acute care over the provision of care for chronic conditions, which substantially negates the aim of spending limited health budgets in the best interests of society, and not just hospital systems or health budgets. Cost utility analyses that demonstrate the reduction of the burden to society of, for example, keeping a rheumatoid arthritis sufferer actively working and self-sufficient for a decade may be more beneficial than both simple palliation or acute interventions such as a coronary bypass graft.

This approach is beginning to affect the privately funded delivery of care, as shown in this conference abstract: "Use of the Original Oregon Plan Prioritization Methodology for Decisions in Private Health Plans. In 1988 and 1989, the State of Oregon piloted a prioritization of health care benefits methodology with the design facilitation of the Bioethics Consultation Group. The group has since utilised a revision of this methodology for health benefits decisions with various private and public sector clients throughout USA and Canada. In particular, the group has assisted large non-profit but private health plans, such as Kaiser Permanente, Group Health Co-operative of Puget Sound, and Community Health Plan of New York, to design and implement processes for prioritising health care and prevention benefits for members."

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