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Perspectives on health technology assessment: response from the patient's perspective

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Health technology assessment (HTA) involves values and judgments, but there have been few attempts to seek the views of members of the public or to ensure that they have access to the results. Patients and citizens can make an important contribution to HTA by determining priorities for assessment, designing and conducting assessments and appraisals, receiving and using the findings, and engaging in debates about policy priorities and rationing. Those responsible for HTA should make greater efforts to involve the public and ensure that the findings are accessible to patients for use when making treatment choices.

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The studies describing the approach to health technology assessment (HTA) in the four countries make interesting reading, but it is disappointing to see that the role of patients and citizens in HTA, both as contributors and as a key audience for its findings, is still peripheral to the process. I believe this failure to promote patient and public participation as a central part of HTA is a fundamental mistake. Unless greater effort is made to involve patients and citizens in determining priorities, in evaluating the efficacy and cost-effectiveness of health-care interventions, and, even more importantly, in using the results of these evaluations to make informed choices, HTA will have little chance of achieving its goals. It will also be hard to sustain public support for funding HTA if the public remains ignorant of its importance and relevance to them.

As the four country studies reveal, HTA is maturing and the dawning realization that it operates in a political context is an important part of the growing-up process. There is at last a recognition that HTA cannot continue to be viewed as a purely technical activity, safely left to experts. Nor can responsibility for decision-making simply be delegated to clinicians in the expectation that they will obediently follow guidelines drawn up by expert committees. The process of technology appraisal inevitably involves values and judgments. In a democratic society it is important that the public has a say in these essentially political decisions. To participate effectively, lay people must be helped to understand the limits of medical care, what it can and cannot do, and the costs, risks and side-effects of treatment as well as the potential benefits.

Public engagement in HTA and dissemination of the results to patients and the wider public has an important role to play in the process of public education and fostering democratic participation in health policy (3). As individual users of health services, patients need access to information on the effects of treatments so they can be actively involved in decisions that affect them directly. The views and preferences of citizens should be central to the policy process, and members of the public should be encouraged to express their views on spending priorities. In both roles, lay people need access to information generated by HTA, presented in a comprehensible and usable format.

Engagement of patients and citizens ought to be encouraged at all stages in the HTA process, in particular the following:

- Determining priorities for assessment.
- Designing and conducting assessments and appraisals.
- Receiving and using the findings from HTA.
- Engaging in debates about policy priorities and rationing.

DETERMINING PRIORITIES FOR ASSESSMENT

The task of assessing all new health technologies is probably beyond the resources of individual national HTA agencies, let alone tackling the backlog of existing "old" technologies that have not yet been subjected to rigorous assessment. It is necessary, therefore, to prioritize candidate technologies for assessment and to agree on criteria for selecting those that will be the focus of major evaluations. The descriptions of the selection process in the four countries indicate that patients and the public are rarely, if ever, involved in the process in a direct way, although the involvement of politicians in setting the agenda for HTA suggests scope for influence by energetic lobby groups. So, for example, in Sweden, SBU projects are initiated by the Swedish parliament and the county councils, ANAES in France and the National Institute for Clinical Excellence (NICE) in the United Kingdom have some patient representatives on their committees, and presumably there are also some public representatives among the plethora of bodies involved in the HTA process in The Netherlands. But none of this amounts to much if there is no formal requirement to consult more widely and to take account of the views of citizens. The impression one gets is of a closed process, controlled by a few representatives of the clinical professions (especially doctors), academic researchers, and civil servants.

It does not have to be like this. Much of the work of HTA is of necessity highly technical, but evidence has shown that lay people can make an active and useful contribution to the priority-setting process (18). Stevens and Milne describe the process for identifying research priorities in the National Health Service's Research and Development program (the NHS R&D program) in England. The R&D priority-setting process may not be perfect, but the efforts made to involve a wide range of stakeholders, including patient groups, give it a legitimacy that is noticeably absent from the first, very important, stage of the selection of treatments and procedures for consideration by NICE. Delegating the selection of topics to an internal committee in the Department of Health without lay involvement undermines efforts made by NICE during the later stages of assessment and appraisal to ensure their processes are transparent and participatory. The NHS R&D program has demonstrated that wide consultation and public engagement in priority setting is possible. It is a great pity that the Department of Health has ignored this in its approach to setting the agenda for NICE.

Horizon scanning is now accepted as an important tool in the selection of topics for formal assessment, but there are risks in this approach. If you spend too much time gazing at the horizon, you do not see what is directly under your feet. While it is clearly important to improve the system for monitoring and regulating the introduction of new technologies, there is considerable evidence that existing "old" technologies are often used inappropriately. When patients or clinicians are involved in identifying priorities for assessment, their starting point is usually a series of health problems rather than a list of technologies. This can lead to a very different view of priorities in which current technologies are likely to be accorded greater importance than new ones.

Ensuring appropriate use of commonly used treatments could have greater impact on the quality of patient care than attempts to control the use of new technologies. In some cases, the scope for efficiency improvements might be even greater. Studies of practice variations reveal inconsistencies that have more to do with irrational practice styles and clinical uncertainty than with differing rates of morbidity (20). Systematic utilization review coupled with assessment of patients' needs and preferences should be incorporated into the priority-setting process to balance the emphasis on new technologies.

DESIGNING AND CONDUCTING ASSESSMENTS AND APPRAISALS

Involving patients in the design of trials or reviews and choice of outcome measures can greatly increase the relevance of the final product (1). If HTA is to produce practical information that can be readily applied in clinical practice, it should start from the type of "real world" questions that patients ask of clinicians (14). For example, What are the characteristics of the diagnosis/disease/disorder and what are the different ways in which it can be treated? What kinds of side effects can happen and what are the chances of each? What are the trade-offs between length of life and quality of life? If length of life is not affected, what trade-offs have to be made between the inconveniences and costs of treatment, and the chance of side effects to gain a benefit in symptom relief?

Patients' views on the relative importance of different outcomes may differ from those of clinicians or researchers (10). For example, they are often more concerned with quality of life and psychosocial issues than with physiological indicators of health status. The methods for measuring these outcomes are available—there are plenty of well-validated instruments to measure patient-assessed health status—but they are still under-used in HTA. Ensuring that the patients' perspective is incorporated in the design stage of assessments and appraisals could greatly increase the chance of producing relevant, and actionable, results.

A patient-focused approach to HTA would start by determining the types of questions that patients want answers to and relevant outcomes and would involve them in appraising protocols, recruiting and preparing information for study participants, undertaking research, and interpreting research findings (8). In addition to direct involvement of lay representatives, the research process should include a variety of methods to determine the experience, views, and preferences of wider groups of patients. These could include qualitative studies such as focus groups or in-depth interviews, cohort studies to track patients' experience along care pathways, and analyses of routine data to determine side effects,

complication rates, or readmission rates after existing treatments for a health problem, as well as randomized controlled trials to evaluate treatment efficacy. The focus would be on appraising all the various treatment and management options for patients with specific conditions, instead of looking independently at the clinical effectiveness of specific drugs, physical therapies, or surgical operations.

Receiving and Using the Findings from HTA

Patients are, or should be, one of the main audiences for HTA, yet little has been done by European HTA agencies to disseminate their findings in an accessible way or to ensure that the information is available to patients when they need it. There are some honorable exceptions to this rule. The NHS Centre for Reviews and Dissemination in England has carried out some useful pilot projects (9) and SBU Alert in Sweden produces information for policy makers and the public, but why is the Alert advisory board composed of medical experts only? Surely there must be many lay people who could provide valuable advice on techniques for effective public dissemination.

In a recent update to their definition of evidence-based medicine, some of its main proponents argued that eliciting patients' preferences and offering them choices should be central to clinical decision-making (12). Shared decision-making is frequently advocated nowadays, but implementation requires training and support and it is rarely practiced in an effective manner (7). In shared decision-making, patients and health professionals share both the process of decision-making and ownership of the decision made. Shared information about values and likely treatment outcomes is an essential prerequisite, but the process also depends on a commitment from both parties to engage in a negotiated decision-making process. The clinician has to be prepared to acknowledge the legitimacy of the patient's preferences, and the patient has to accept shared responsibility for the treatment decision.

If decision-making is to be shared, the information to inform decisions must also be shared. Given the short consultation times experienced in most busy clinics, it is often unrealistic to expect clinicians to provide full information about the risks and benefits of all treatment options. This information is not always readily available to clinicians, let alone lay people. Patients cannot express their preferences or make informed choices unless they are provided with clear information about the risks and benefits of treatment options. These should be provided in the form of user-friendly evidence-based patient decision aids.

Research into the use of decision aids for patients has shown that they can be an effective solution to these problems (17). Decision aids are designed to help people make specific deliberative decisions about disease management and treatment options, prevention, or screening. They use a variety of media to present the information in an accessible form to patients. The content is based on reviews of clinical research and studies of patients' information needs. They are very different from standard health education materials, because they are not didactic or prescriptive—they do not tell people what to do. Instead they help patients clarify their own values and preferences and weigh up the potential benefits and harms of alternative courses of action.

There are now more than 200 patient decision aids recorded on the Cochrane inventory, almost all of which were developed in North America (http://www.ohri.ca, accessed 24 February 2003). In the process of developing and testing these, a considerable body of knowledge has been developed on how to use them to involve patients in treatment decision making (6). Yet the lessons continue to be ignored by those responsible for HTA. This is very short-sighted, because providing patients with information about best practice may prove to be the most effective way to ensure that it is implemented. For example, NICE produces patient versions of its clinical guidelines and assessment reports, but these fail to comply with the carefully elaborated design principles for effective decision aids (16), being little more than plain language summaries of the information produced for purchasers. The templates for evidence-based patient choice have been developed (14). What is lacking is the willingness to use them to make the evidence available to patients at the time they need them.

ENGAGING IN DEBATES ABOUT POLICY PRIORITIES AND RATIONING

Politicians have been very unwilling to accept a leadership role in rationing debates because they prefer to propagate the idea that all demands for health care could be met if only efficiency could be increased. As the studies from the four countries clearly illustrate, the potential for medical technologies to achieve beneficial effects for larger numbers of people with a wider range of conditions and ailments is increasing faster than the public's willingness to pay for them. The key issue for the future is how to ensure a fair and equitable distribution of health-care resources. Which essential services should be available free at the point of use or at low cost and which should not? Decisions to restrict or ration services are not amenable to a technical fix. They can and should be informed by evidence on clinical effectiveness, but they also involve values. The views of experts must be weighed alongside the opinions and experience of lay people.

The American philosopher, Norman Daniels, has argued that policy-makers should seek public legitimacy for rationing decisions by meeting four criteria for "reasonableness" (5):

- the rationale for decisions to restrict access should be clearly and publicly stated,
- it should be contestable and be acceptable to "fair minded" people,

- there should be a mechanism for appeal,
- the process should be enforceable and defensible.

These principles are applicable to the process of technology appraisal and underline the importance of ensuring public buy-in to the recommendations arising from HTA.

In Sweden, Norway, Denmark, and The Netherlands, high-profile efforts to develop criteria for deciding on priorities in health care predated the current phase of the HTA programs and were largely disconnected from them. Both the Swedish parliamentary committee on priority setting in health care and the Dunning committee in The Netherlands succeeded in raising the level of public debate, both nationally and internationally. This was an important step toward the first of Daniels' principles: promoting public understanding of the rationale for rationing decisions. But subsequent experience has revealed the limitations of relying on short-term committees to oversee the process (4). A continuous approach is required to widen and deepen the public debate, with adequate institutional support and well-developed dissemination mechanisms, including a strategy for engaging the interest and support of the mass media.

The national committees in the Nordic countries found it difficult to reach agreement on the main purpose and scope of their health systems (13). Competing priorities included maximizing health gain, minimizing health inequalities, helping disadvantaged groups, or promoting solidarity and social inclusion. Without agreement on these fundamental issues, it was hard to devise universal principles that could be applied in practice. Experiments on devising lists of treatments for inclusion in or exclusion from reimbursable healthcare packages have also proved problematic (2). Exclusions have almost always been fiercely resisted by powerful interest groups, few treatments can be completely excluded, and public pressure to extend the lists usually results in increased funding.

The emphasis has now shifted downward to the clinical application of HTA results and the development of evidence-based guidelines but as several studies in this issue have demonstrated, this professionally controlled, technical approach has so far failed to accommodate the complexities of clinical decision-making in the real world. What is needed is a better synthesis between the different ways of deciding on priorities, with explicit principles publicly debated and agreed at the macro-level, greater transparency and more public involvement at the meso- or organizational level, and sufficient flexibility at the micro-level to avoid the rigidities of the "one-size-fits-all" approach to treatment decision-making, which tends to downplay the importance of clinicians' experience and patients' values and preferences (11).

GETTING FROM HERE TO THERE

This demand for more patient and public involvement is not simply baying for the moon. There are several problems to be tackled, but the essential building blocks are already in place, and if the will is there, the difficulties could be overcome. The HTA system in the United Kingdom provides a good example of the potential for transforming the system. Despite denials that they are part of a rationing process (19), NICE has largely adopted Daniels' principles. Efforts have been made to ensure transparency, and public engagement and patient groups are actively involved in all stages of the process except the first (see above), albeit to a limited extent. Appraisals are published in paper and electronic format, and a formal appeal mechanism has been established. NICE recommendations are often controversial, and there has been fierce public debate about some of them, with accusations of impartiality or capitulation to particular vested interests being bandied about. Nevertheless, adherence to Daniels' criteria for public accountability ensures a level of legitimacy that would not have been possible without the commitment to openness and patient involvement.

To date, however, public involvement has been limited to representatives of special interest groups, particularly groups representing patients with specific diseases or conditions. Some of these organizations are large and professionally run, while others are small self-help groups operating with limited funds. It is much easier for the larger groups to find people with the time and commitment to serve on committees. The best funded groups are usually those representing people with chronic conditions affecting large numbers of people. There are also some generic patient associations that concern themselves with issues common to all patients, not just those with particular diseases, but the single-issue groups generally find it easier to attract committed participants than do the generic groups.

Groups that lack a large constituency of people with long-term needs willing to pay membership fees usually have to depend on governmental, charitable, or commercial sources for funding. The pharmaceutical industry has shown considerable interest in funding patient groups, particularly those groups campaigning on behalf of patients who might be persuaded to consume their products. Direct-to-consumer advertising is currently prohibited within the European Union, but organizations such as the European Federation of Pharmaceutical Industry Associations (EFPIA) have aimed to foster close relations with patient groups as a key plank of their public relations strategy. Some patient groups were established with funding from pharmaceutical companies as part of their "disease awareness" campaigns while others were set up by clinicians to support their efforts to raise funds for research. Although many reputable groups are scrupulous about avoiding any strings that might be attached to industry funding, not all are so fastidious. Some so-called "patient groups" are vehicles for industry public relations rather than genuine user groups. The representativeness of many of these groups is questionable, and a system that relies solely on members of organized patient groups to represent the views of the public is vulnerable to accusations of bias.

What is needed is a systematic attempt to engage the views of citizens, to balance those of the specific interest groups. The response of the UK government has been to establish a Citizens Council attached to NICE that "will provide advice to the Institute on topics relating to social, ethical or moral questions which arise in the Institute's work." The first report of the Citizens Council was published in December 2002 (15). Membership of the group explicitly excludes people working in the NHS or private medicine, the health-care industries, or in groups or organizations whose function is to act in support of patient or industry groups or to lobby on their behalf. Among the thirty participants at the first Council meeting were an electrician, a store assistant, a make-up artist, a milliner, a scaffolder, a retired pilot, a teacher, and a taxi-driver. It will be interesting to see if this brave attempt to secure involvement of 'ordinary citizens' is successful. The British have been dilatory in attempting it—Oregon and New Zealand led the way (4)—but they have the advantage of being able to learn from experience elsewhere.

It could be argued that public involvement is even more crucial in countries with highly centralized health systems such as the United Kingdom and France than in countries such as Sweden where regional control of health care ensures that local politicians are actively engaged in the process, or The Netherlands with its tradition of engaging a range of stakeholders. The British system is often accused of having a democratic deficit at its heart, so the establishment of the Citizens Council is especially welcome. The government is taking this tentative step into the dangerous waters of explicit rationing, because the political risk of not keeping the public informed about the choices that are being made on their behalf is beginning to look more dangerous than the traditional alternative of muddling through with decisions taken implicitly and covertly instead of in an open and transparent manner. The next very important step will be to devise a way of ensuring that the principles elucidated by the Citizens Council have wider legitimacy and can inform the specific recommendations arising from the appraisal process.

Those responsible for health-care budgets must ensure that individual clinical decisions fit within the wider context of resource availability and public priorities. Clinical freedom and patient choice cannot be divorced from its societal context, and when individual expectations clash with population priorities, there must be a mechanism for resolving the conflict. The challenge is to harness the potential of HTA to improve the effectiveness of medical care, while ensuring that public confidence in health-care systems is maintained. The balancing act of individual needs versus population requirements cannot be left to "experts" alone. Patients and citizens need to understand the choices confronting policy makers and need to be involved in determining priorities and trade offs.

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