International Journal of Technology Assessment in Health Care, **20:1** (2004), 67–70.

Health technology assessment and policy from the economic perspective

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This article comments on the four country papers in this volume from an economic perspective. Different phases of the decision-making process, which can be supported by Health Technology Assessment (HTA), are considered. For each of these, there is large cross-country variation in the way in which HTA influences policy. Furthermore, economic themes regarding the relevance of HTA evidence for policy making, the position of cost-effectiveness in relation to other criteria vis-à-vis reimbursement decisions, the use of a cost per quality-adjusted life year threshold, and the incorporation of economic considerations in practice guidelines are discussed.

Keywords: Health technology assessment, Economics

Four interesting papers in this issue describe health technology assessment (HTA) and its role in health policy. They clearly show that the situation regarding HTA differs quite a lot between the four countries, with respect to both research and policy. HTA can play a role in various phases in the diffusion of a health technology, notably when the decision on reimbursement of the technology is taken (or revised) and when recommendations on its use are made to the professionals using the technology. It seems that HTA is not used to its full potential in the four countries and that this partly depends on whether the system is government run or more based on social insurance. Berg et al., in describing the Dutch system, even suggest that, in the latter type of system, the many stakeholders with different interests frustrate rational policy making.

Here, we concentrate on the typical economic issues, which can be associated with the use of HTA in policy and practice. Taking this economic perspective, we are especially interested in the cost-effectiveness issue, which is embedded in most HTA exercises. Typical questions addressed are the following: how does cost-effectiveness influence reimbursement status; is there a common societal cost per quality-adjusted life year (QALY) threshold; is there any revision of reimbursement status, and how does cost-effectiveness impact on that; and is cost-effectiveness incorporated in practice guidelines? However, before we consider these issues, the usefulness of HTA information as produced in the four countries will be considered.

CONSIDERING THE HTA EVIDENCE

The four countries probably stand out as rather favorable in Europe in terms of the quality of HTA studies. Nevertheless, some concerns can be raised on the usefulness of the material produced. An important asset is that, in all countries, guidelines on how economic appraisals should be performed exist, which show convergence on the most critical issues such as the perspective to be chosen, the principle of discounting, dealing with uncertainty, and the choice of comparator. Differences exist with respect to the specific details; for instance, on the choice of discount rate (1.5 percent on benefits and 6 percent on costs in the National Institute for Clinical Excellence (NICE) guidance, 4 percent for both costs and benefits in the pharmacoeconomic guidelines produced by the Dutch Health Insurance Executive Board). The existence of these guidelines, especially when issued by health authorities as in The Netherlands and the United Kingdom, will enhance comparability of study results across countries and opportunities to extrapolate from study results across different countries. Most guidelines, however, deal with methodologic principles rather than giving clear recommendations for their practical application (with some exceptions, like the

Dutch manual for costing in economic evaluation). In practice, large variations still exist in costing procedures, the use of modeling, handling of uncertainty, extrapolation of short-term benefits, and the incorporation of preference values (3). Also, the acceptance by health policy makers of concepts such as QALYs and "productivity costs" is not yet universal, leading to variation in practice across countries.

Another critical issue for the relevance of HTA studies is the degree to which they predict or describe health outcomes and expenditure in actual practice. Pharmacoeconomic studies, in particular, are based on data from controlled clinical trials, often without any attempt to "translate" the results of these trials into predictions of cost-effectiveness in daily practice. It is often the case that more information gradually becomes available, suggesting that compliance and persistence in the use of pharmaceuticals is poor. A recent estimate for The Netherlands based on pooled data from insurance companies and pharmacies was that more than 10 percent of pharmaceutical expenditure is wasted (2). Therefore, reported results on the cost-effectiveness of new technologies may provide a rather favorable picture of their relative efficiency. Unlike in the United States, health outcomes research, which is aimed at establishing cost-effectiveness in daily practice, is not yet prominent in Europe, and more resources should be invested in the development of databases that facilitate such analyses.

ROLE OF HTA IN REIMBURSEMENT POLICIES

The countries differ regarding the role of HTA in reimbursement policy, depending on the finance and organization of their system. Regarding general technologies in the United Kingdom (NICE) and The Netherlands, the authors of the corresponding papers report that several important new programs and technologies were assessed in terms of their cost-effectiveness. The assessments translated into consequent decisions on introduction and reimbursement. This took place in The Netherlands in the mid-1980s, when several transplant programs and in vitro fertilization (IVF) were assessed in terms of costs and benefits and led, for instance, to the postponed introduction of liver transplantation and restrictions on the reimbursement of IVF, based on economic arguments. In the United Kingdom, systematic consideration of cost-effectiveness became more formalized with the emergence of NICE in 1999. SBU assessments in Sweden appear to be less directly connected to policy but aim at informing providers, the public, and policy makers at the county level. In addition, in France, several organizations perform HTA studies as part of other activities with the main purpose of providing information and advice to various customers.

It is interesting to note the differences between the countries in the character of the HTA studies performed for this purpose. Using the useful research categorization of Stevens and Milne, it can be observed that most of the Dutch HTA studies are in the category of primary research (primary data collection in the context of clinical trials), while in the United Kingdom, the establishment of the research and development (R&D) program gave a tremendous boost to secondary research. Also, the work commissioned by NICE consists of mainly systematic review and some de novo modeling of cost-effectiveness. In addition, the SBU reports in Sweden are predominantly based on systematic literature review. This strategy seems to be related to the process of setting priorities for HTA. In the United Kingdom, this process is more policy driven (for instance, the systematic priority setting in the R&D program and the prioritization process by NICE) as is the case in Sweden (the SBU Alert advisory board). In The Netherlands, researcher curiosity now drives the program (submitted proposals for HTA from the research community), although the government chose the topics of the first HTA studies mentioned earlier.

In France, the picture is mixed as is nicely illustrated in Table 3 of Orvain et al. Priority setting among health care interventions for HTA seems to be most explicit at ANAES. From an economic perspective, it is interesting to note that, in many cases, HTA reports by the different organizations listed in Table 3 do not incorporate cost-effectiveness considerations. Table 6 of Orvain et al. suggests that effort is made at ANAES to include this aspect in their HTA reports.

Regarding the reimbursement of new pharmaceutical products, the picture is changing rapidly. Sweden and The Netherlands will demand cost-effectiveness information from manufacturers as a necessary condition for considering reimbursement. Sweden has established a new governmental agency for negotiating prices and reimbursement of drugs, effective from October 2002, and in The Netherlands, a system of demanding submissions that incorporate statements on cost-effectiveness and budget impact is already in place and will become compulsory in 2005. Although not formally connected to reimbursement, NICE in the United Kingdom is increasingly considering medicines for review and provides guidance for their use. In France, the role of HTA in decisions by the transparency committee and the pricing committee, both connected to the process of reimbursement and pricing of pharmaceuticals, is still limited. Orvain et al. note that manufacturers have to report on safety, effectiveness, and "usefulness from a public health standpoint," but do not mention cost-effectiveness explicitly.

Furthermore, it is interesting to see that only France has a policy in place to revise reimbursement status of pharmaceutical products periodically. The criterion to support decisions here is budget impact rather than cost-effectiveness. Other countries are considering such a policy, and the United Kingdom recently granted reimbursement to various products for multiple sclerosis patients given that they can demonstrate cost-effectiveness of at least £36,000 per QALY within a certain period.

Compared with authorities in The Netherlands and Sweden, NICE has been more explicit about a cost per QALY

threshold (£30,000–£40,000 per QALY) as a criterion for reimbursement or for the inclusion of a technology in the health insurance package. Although NICE officials publicly deny adopting any particular threshold, the range mentioned above can be inferred from various HTA reports on technologies. However, in all jurisdictions, multiple criteria appear to be used to arrive at reimbursement decisions. As is extensively discussed by Berg et al., several criteria are inputs in this decision process, and HTA or, more specifically, cost-effectiveness may not be the dominant factor. In The Netherlands, a discussion is held about an ethical framework based on both efficiency and equity criteria. For health care interventions, different thresholds for cost-effectiveness may apply according to the severity of the disease (4). The higher the severity of illness of patients, the more society may be willing to accept lower levels of relative treatment efficiency for those patients. However, budget impact also has an important influence on decisions, especially when budget silos are in place, as is the case in most social insurance-type systems.

IMPACT OF HTA AT THE NATIONAL LEVEL

Related with the different degrees of using HTA evidence in the countries is its actual impact on the systems in the various countries. Although first seen as guidance, (positive) NICE appraisals are now "compulsory," meaning that local British National Health Service (NHS) commissioners need to make funding available to support NICE decisions. One should take into account, however, that only a small percentage of all important topics can be addressed by NICE. It is interesting that Stevens and Milne seem to suggest a relationship between this factor and the slow uptake of new technologies in the United Kingdom compared with other countries. In The Netherlands, there is evidence that both the top-down HTA program (selection of topics by the government) and the bottom-up program for "investigative medicine" (driven by researcher curiosity) have been influencing policy and practice, although no formal evaluation has been carried out to assess their contribution to efficiency. Berg et al. note that the list of excluded services is "minimal and highly eclectic," suggesting that there are few cases where a technology was rejected or recommended for restricted use largely on the basis of costs.

The proposed systems for deciding on reimbursing medicines in Sweden and The Netherlands will have an impact, although it remains to be seen how cost-effectiveness is balanced with budget impact, which will also be explicitly considered in each manufacturer's submission. The latter also goes a fortiori for France, where budget impact has been the dominant factor to date. A EUR-ASSESS study suggested that, in Sweden, six evaluations by national agencies have influenced decisions on insurance coverage, but the examples given show the expansion rather than the balancing of costs and effects (1). Carlsson quotes another EUR-ASSESS study suggesting that, in the area of invasive cardiology therapy, HTA influenced coverage decisions. As Orvain et al. comment, the impact of HTA in France very much depends on the implementation by its various customers in the French system.

HTA AT THE REGIONAL AND LOCAL LEVEL

Actors at regional and local levels of decision making, who use HTA results in their daily decisions, seem to be most prominent in the United Kingdom. Since the 1991 internal market reforms, appraisals, not all of which can be termed HTAs, have been made and used by local and regional agencies to support their policies. However, these actors are increasingly informed by HTA guidance from several bodies (see Stevens and Milne). In The Netherlands, insurance organizations, using cost-effectiveness evidence, are supposed to be involved in selecting efficient providers, negotiating contracts with incentives for efficiency (which involves adopting protocols for "appropriate care"), and encouraging competition to generate lower drug prices. However, there is little evidence of the emergence of these activities. Nonetheless, the Government is determined to stimulate these practices, and as a first step, cholesterol-lowering drugs and gastrointestinal drugs have been removed from the Dutch reference price system in 2003. Health care insurers may purchase these drugs directly from pharmaceutical companies or contract in bulk from wholesale companies. As patents expire for some of the products in these two therapeutic areas, insurers can be expected to cash in as much as possible due to expected price reductions and, thus, contribute to a more cost-effective use of these drugs.

In addition, hospital management have little expertise to interpret and use HTA information for their purposes. The situation in France may be even further away from applying HTA evidence at this level, at least as far as the economic component is concerned. Finally, in Sweden, county councils and major hospitals seem to make limited use of HTA results. As Carlsson notes "only 23 percent of selected respondents used economic evaluations." A greater involvement of these actors in the HTA cycle may enhance efficiency in the various systems.

Practice guidelines that incorporate cost-effectiveness information is scarce in all countries. Their status is variable, and incentives to use them are lacking, even in the NHS management structure. In The Netherlands, a special program was funded to develop seventeen practice guidelines incorporating cost-effectiveness information, but the medical community heavily debated its consideration of economic arguments (Berg et al.). The program has since been discontinued through lack of government funding. Furthermore, in most countries, there seems to be little support for the implementation of these types of guidelines.

CONCLUSIONS

As Stevens and Milne observe in their concluding paragraph, the necessity to prioritize cannot be escaped as there is a trend toward new interventions that are both expensive and effective. HTA can be expected to support prioritization especially if it is customer-driven, includes an economic component, and is used in a rational policy environment. The evidence from the four countries shows that there is great scope (and necessity) for enforcing the role of HTA in health policy and practice. Perhaps the situation in the United Kingdom, although criticized by Stevens and Milne, may act as an example of more systematic attention to economic arguments in health policy and inspire developments elsewhere. However, the UK institutions and processes cannot be translated straightforwardly and have to be tailored to the characteristics of the other systems to provide suitable solutions for other countries.

The authors are not very clear about the actual impact of HTA on policy and practice because of lack of formal evaluation studies in all countries (but expected soon for NICE). It seems that many arguments play a role in deciding on reimbursement, especially when many actors are involved, as is the case in the insurance-based systems. The more direct the relationship between policy and HTA evidence is, the more likely it is that the latter will have an influence. Moreover, if HTA does have an influence, it is often not in the form of an outright rejection of a technology but rather through limiting the technology's use to those subgroups for which it is most cost-effective.

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