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hai GLOBAL

Pharmaceutical Pricing Policies and Interventions

POLICY BRIEF NUMBER 6

THE ROLE OF HEALTH TECHNOLOGY ASSESSMENT IN MEDICINE PRICING AND REIMBURSEMENT

As part of the joint World Health Organization (WHO)/ Health Action International (HAI) Project on Medicine Prices and Availability, a series of in-depth reviews have been published on pharmaceutical policies and interventions that may improve medicine availability and affordability. This policy brief summarises the key points from the review on the role of health technology assessment (HTA) in medicine pricing and reimbursement with a focus on its use in low- and middle-income countries (LMICs). The review included a systematic literature review and a description of policy processes and requirements.

Page references to the review paper are given in parentheses.

WHAT IS THE BASIS FOR THIS POLICY BRIEF?

WHO/HAI Review Series on Pharmaceutical Pricing Policies and Interventions. Working paper 6: *The role of health technology assessment in medicine pricing and reimbursement* by Patricia Whyte and Cameron Hall, Deakin Health Economics, Deakin University, Australia. www.haiweb.org/medicineprices/policy/index.html

SUMMARY CONCLUSIONS

What are the main advantages of HTA?

HTA can increase value for money from scarce public resources and help to control medicine expenditure.

What are the main disadvantages of HTA?

It is costly to conduct and requires strong technical capacity. Decision-makers must have skills to interpret and apply HTA findings. The pharmaceutical industry, medical specialists and some patient groups for particular diseases often oppose HTA policies because they fear it may restrict marketing of, and access to, costly new medicines.

Is HTA appropriate for regulating all medicine prices?

HTA is most appropriate for innovative new medicines. For medicines which are off-patent or have close therapeutic substitutes, other policies may be more efficient.

Is HTA appropriate for all countries?

Low-income countries with very limited capacity and countries that face major challenges of lack of transparency in decision-making on medicines reimbursement are likely to face difficulty implementing HTA for medicines effectively. Use of international guidelines and model essential medicines lists can help such countries to incorporate HTA in their policies.

Are there any complementary policies that should accompany HTA?

Other policies can put downward pressure on prices for off-patent medicines and medicines with close therapeutic substitutes, including competitive tendering and therapeutic reference pricing. Policies to encourage doctors to prescribe formulary medicines and follow evidence-based guidelines are needed to complement HTA.

Are there any key pre-requisites for implementing HTA?

Capacity for HTA in LMICs should be established early and supported; prerequisites and barriers are extensive but not insurmountable and must be considered as HTA processes are developed.

What is the policy?

Health technology assessment (HTA) is a multi-disciplinary policy analysis of the medical, economic, social and ethical implications of development, diffusion, and use of health technology, including medicines, vaccines, medical devices and interventions. HTA for medicines typically uses clinical, pharmacological and pharmaco-economic analysis to assess whether a new medicine provides any additional benefit compared with current practice and at what additional cost, and may recommend that a medicine be used for specific indications or patient sub-groups. HTA often quantifies fiscal and population health impacts of increased use of the technology. HTA seeks to synthesise research evidence and develop recommendations for decisions by policy-makers, managers and clinicians. (p.1)

What is the policy used for?

HTA findings can be used to prioritise health care expenditure, increase value for money and control health expenditure. HTA of medicines is widely used in Organization for Economic Cooperation and Development (OECD) countries to inform the selection of medicines for national, regional or hospital drug formularies and the selection of medicines for reimbursement by health insurance systems. In many countries and global and national health organizations, HTA is used as an input into the development of evidence-based clinical guidelines and into advice to prescribers. A few countries use HTA in negotiation of medicine prices. (pp. 7-8)
In the case of medicines, HTA is usually carried out after the medicines regulatory authority has assessed quality, safety, and efficacy. The subsequent use of the HTA findings to inform decisions about formularies, pricing and reimbursement of medicines is usually a separate process from the assessment itself. There is considerable variation in how countries use HTA to inform policy, management and clinical decisions. (p.2)

How and where has it been implemented?

Australia was the first country to require companies to submit economic data to support applications for new pharmaceutical products to be reimbursed through its pharmaceutical benefits scheme (PBS) in 1993. Since then, most OECD countries have followed suit and now require HTA as part of their decisions on the reimbursement of new medicines. (pp. 7-8) Countries typically only mandate HTA for new products that are on-patent, not for new generics. Where countries use HTA to develop and update clinical guidelines, the assessment also reviews new evidence on existing medicines and other therapies.

Interest in HTA has expanded internationally and now a number of middle-income countries have established HTA agencies or commission HTA from academic and other institutions, including Argentina, Brazil, Chile, China, Colombia, Croatia, Malaysia, Mexico, Poland (now a high-income country), South Africa, Thailand, Turkey and Uruguay. A number of these countries have introduced HTA as part of their decision processes for medicines reimbursement policy. But HTA is developing at uneven speeds across countries and the role of HTA differs from country to country. (p.12) It seems that many LMICs have no HTA processes, and information is hard to obtain about the development of HTA in LMICs. Many LMICS draw upon guidelines developed by global health institutions that are informed by HTA, such as the WHO model essential medicine lists. (p.22)

Key aspects of designing HTA processes for medicines

An international working group proposed the following key principles when designing HTA processes: consideration of evidence and outcomes, consideration of the full societal perspective, generalisability of findings, timely assessments, transparency of the HTA process and transparency of the link between HTA findings and decision making. (1) (p. 8)

Legislation is normally necessary to require HTA processes for defined categories or types of new medicines before decisions are made to include them in a national formulary or list of reimbursable medicines. The legislation should clearly define what is expected in HTA by referencing guidelines, how the analysis will be evaluated, decision-making criteria, roles and composition of various committees that should be established, whether the opportunity exists to appeal if unsuccessful, requirements to make the findings of the evaluation available to the public, and fees that would be levied for each application. It is good practice for legislation to include provisions to ensure that participants in HTA and subsequent decisions do not have any conflict of interest. Legislation should allow for decisions of the national organisation to be challenged under certain circumstances and also for recourse against pharmaceutical suppliers if they have not met contracted terms. (p.19)

The manufacturer usually lodges the submission with the national organisation responsible for managing the national formulary. The national organisation then assigns the submission for evaluation, which may be carried out in-house or outsourced to an organization with suitable expertise to conduct independent evaluation. A multidisciplinary team including a health economist will conduct the evaluation, which is then presented to a committee of experts for discussion and recommendation to accept or reject (or defer pending future data) the pharmaceutical for listing on the formulary. (p.16)

What institutional and technical capacity and information is required to implement the policy effectively?

HTA requires expertise in a number of areas including health economics, biostatistics, pharmacoepidemiology and a range of fields of clinical medicine. It is vital to have experts with the ability to critically review and apply statistics for the proper interpretation and application of economic evaluations. LMICs need to offer competitive salaries or other incentives to attract and retain staff with the necessary expertise. A 2001 survey of HTA of pharmaceuticals in 11 OECD countries found that the staffing of these agencies ranged from 5 to 23 full time positions, depending on the number of assessments to be conducted and whether external consultants were used. (2) The head of the HTA agency is usually appointed by the Ministry of Health. Most countries use external consultants or outsourcing to academic or other research institutions for conducting HTA. It is also important for decision-makers who consider the HTA reports and recommendations on formulary listing or pricing to have the skills to objectively assess the evidence presented to them, or to have access to policy advisers with these skills. However, some studies of country experience suggest that technical expert capacity can be built up over time by making the technical requirements less stringent initially. (pp.16-17)

The resources to establish an HTA organisation may have to come from government initially. However, the operating costs could be funded at least in part through a submission fee applying to manufacturers. (p.16)

Sharing of HTA guidelines and information across countries can reduce the burden on individual countries. However, the adaptation of HTA across countries is a key concern, particularly for LMICs. Some HTA information will be relevant in every setting, for example, evidence from systematic literature reviews and evidence from randomised controlled trials regarding the effectiveness of an intervention. However, factors such as the epidemiological profile of disease, models of clinical practice, relative prices and unit costs, the availability of healthcare resources and budget constraints, as well as the choice of health benefit, comparator, comparability of treatment patterns and populations, are more country-specific and hence may limit the generalisability of results. While the technical aspects of best practice within HTA, such as transparent reporting of methods and findings, can be applied across countries of all economic levels, other aspects of HTA are best developed specific to each setting. However, countries with limited HTA capacity can prioritise assessments based on the practice of high-income countries (HICs) where HTA is well established. For example, if an HIC finds that a particular technology is not cost-effective, it will likely not be cost-effective in an LMIC setting either. (pp. 19-20)

What specific challenges were encountered and what lessons can be learned?

Countries have encountered many barriers to the implementation of formal HTA processes, including shortage of expertise; timeliness of availability of necessary information; linking the different participants and organizations involved in HTA and subsequent decisions, including pharmaceutical companies, experts, researchers, and decision makers; lack of decision-maker capacity; limited acceptance of external data; transparency and comprehensible communication of HTA reports and decisions; and incentives to use HTA evaluations. These barriers are substantial, but a growing number of countries have demonstrated that they can be overcome. The evidence from the literature search addresses possible solutions to each of these challenges. A review of how HTA has evolved in several European countries over the past decade found that countries have generally strived to modify their methods to improve the impact of assessments on policy and practice, meet national objectives and the various needs of stakeholders, and achieve greater transparency, legitimacy and relevance. (3) (pp. 9-10,21) This suggests LMICS initiating HTA should plan to review and improve their HTA processes and implementation over time.

The pharmaceutical industry perceives HTA as a barrier to gaining market access. Consequently, the industry is typically resistant to the introduction of HTA before medicines are reimbursed. Companies may have to employ new staff or contract a consultancy at considerable expense to prepare submissions to the HTA agency. Public reimbursement of a medicine, however, offers a strong incentive for industry to cooperate and the skills requirement is not insurmountable, particularly for multinational companies which are the main producers of new medicines requiring HTA. (p.17)

Doctors, particularly specialists, may also have concerns about the risk that HTA may restrict their ability to prescribe new medicines if rejected for public reimbursement. Patient groups may share the same concerns. Early and meaningful consultation with specialists in the field before decision-making is necessary to address this issue. Consultation with patient and consumer groups before decision-making, or the establishment of a forum, can ensure the needs and experiences of patients are well understood before decisions are made and can help to ensure effective communication of HTA findings. Effective participation of civil society organisations can also provide a check or balance for the organisation that makes funding decisions based on HTA.

However, some patient groups are fragmented and/or source their funding from interested pharmaceutical companies. (p. 18)

How has the policy been monitored and evaluated?

There is little comparative and quantitative evidence on the effect of the application of HTA on medicine prices, availability and affordability. This is especially true in the setting of low- and middle-income countries. There is a body of descriptive evidence on the role of HTA and its implementation which enables some tentative conclusions to be drawn on the likely impact that HTA would have. As one commentator noted, whereas the last 10 years have been well spent on building the HTA infrastructure and evidence base, the next decade should focus on ascertaining outcomes. (p.22)

What effect does the policy have on prices and availability?

There is some evidence that HTA can be used to manage medicines expenditure growth effectively while maintaining availability, when it is combined with other tools such as restricting reimbursement to pharmaceuticals on a national formulary. (p.21)

Only a small number of HICs appear to use HTA for negotiating value-based prices for innovative new medicines, including Australia, Canada, the UK, France and Germany. In these countries, the negotiation of the reimbursement price involves wider considerations in addition to HTA. One study compared the effects of using HTA in price negotiation with using therapeutic reference pricing for selected groups of medicines in four countries that had used one or both policies. (4) HTA did not result in any clear pattern of impact on pricing. Reference pricing was found to be effective in lowering prices of medicines that are therapeutically equivalent in cases when there was a big difference in prices among equivalent medicines – for example when one medicine in the group went off-patent and attracted competition from generics. HTA is, in theory, a superior method for obtaining value for money because it addresses not only price, but also the appropriate indications for the use of the medicine and the relation between additional value and additional costs. However, conducting HTAs is more costly than reference pricing, so the most efficient approach might be a combination of both policies. (p. 39)

Are there risks associated with the policy?

Even high-income countries with strong HTA capacity still face challenges, generally relating to the evidence presented. One review of HTA decisions for medicines in the UK, Canada and Australia found that significant uncertainty around clinical effectiveness, usually resulting from inadequate study design and the use of inappropriate comparators, was a key issue affecting decisions the countries made to reimburse new medicines. (5) As well, the results of the evaluation process in the different countries appeared to be influenced by the context, agency processes, ability to engage in price negotiation and perhaps differences in social values. It is expected that this would also be true in LMICs. (pp. 11-12)

Reimbursement decisions often consider information other than just cost-effectiveness results, but the full range of decision criteria leading to a reimbursement price is often not transparent. Transparency is vital to convince stakeholders that the decision is made based on sound and consistent principles. This is of particular importance in countries – both LMICs and higher income countries – that face challenges in managing medicines policies arising from conflict of interest and corruption. (pp. 9-10) Clear and objective criteria for decision-making and publication of HTA recommendations and reasons for reimbursement decisions can contribute to transparency of the process and stakeholder and public acceptance. (pp. 17-18)

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OTHER USEFUL RESOURCES

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