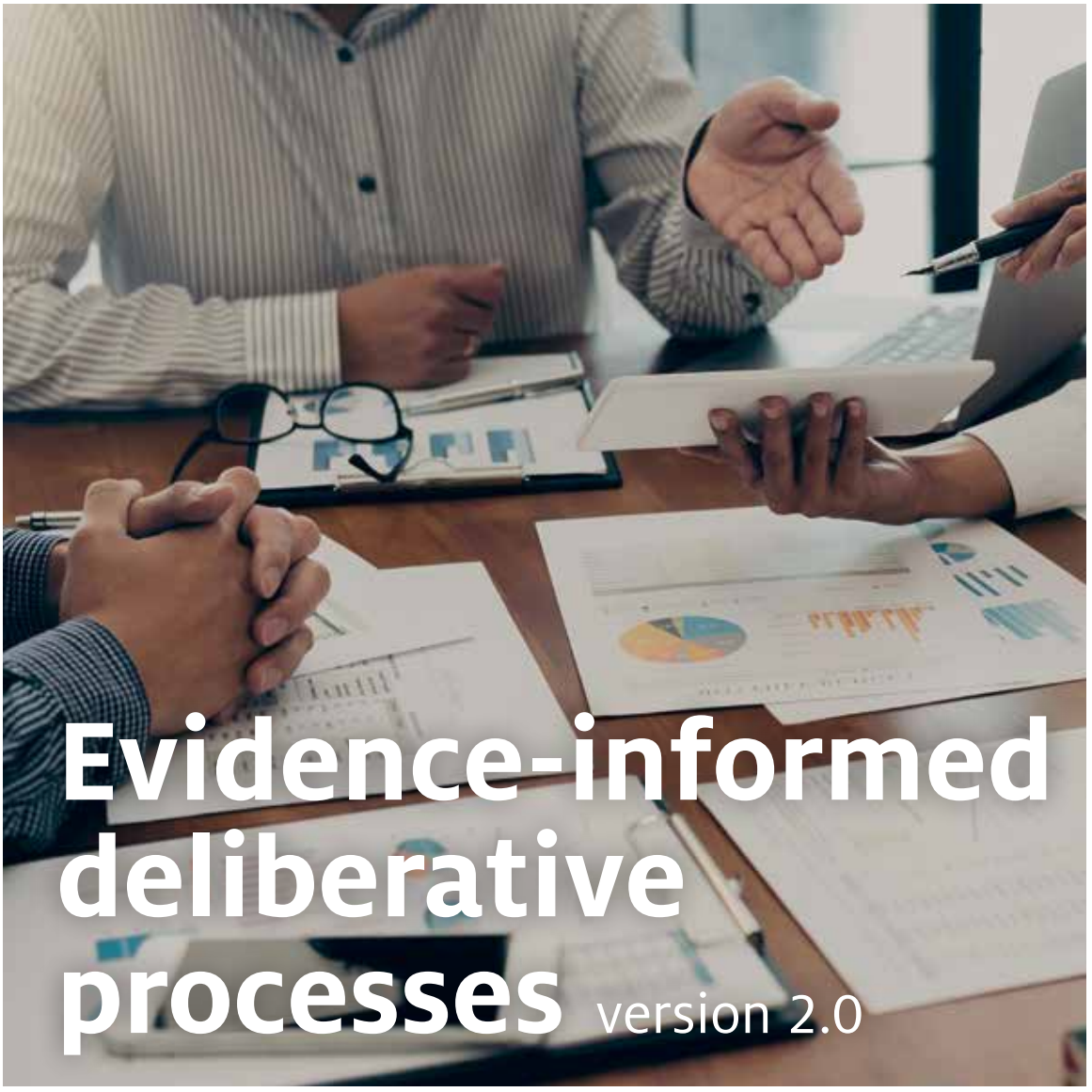

Evidence-informed deliberative processes version 2.0

A practical guide for HTA bodies for legitimate benefit package design

Wija Oortwijn, Maarten Jansen, Rob Baltussen

Evidence-informed deliberative processes

A practical guide for HTA bodies
for legitimate benefit package design

Wija Oortwijn, Maarten Jansen, Rob Baltussen | Nijmegen, 2021

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Copies of this guide can be downloaded from https://www.radboudumc.nl/global-health-priorities

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Glossary

The definitions provided here are mainly derived from the international HTA glossary and relevant toolkits.[1]

Cost-effectiveness analysis (CEA)	An economic evaluation consisting of comparing various options, in which costs are measured in monetary units, then aggregated. Outcomes are expressed in natural (non-monetary) units.
(Clinical) Effectiveness	The benefit of using a technology, programme or intervention to address a specific problem under general or routine conditions, rather than under controlled conditions, for example, by a physician in a hospital or by a patient at home.
Deliberation	The critical examination of an issue involving the weighing of reasons for and against a course of action. It can involve a single individual or a group of stakeholders.[2]
Disinvestment	The deliberate and systematic reduction of funding for a health technology of questionable or comparatively low value.
Evidence-informed deliberative processes (EDPs)	A practical, stepwise approach for HTA bodies to enhance legitimate benefit package design, based on deliberation between stakeholders to identify, reflect and learn about the meaning and importance of values, informed by evidence on these values.[3]
Health technology	An intervention developed to prevent, diagnose or treat medical conditions; promote health; provide rehabilitation; or organise healthcare delivery. The intervention can be a test, device, medicine, vaccine, procedure, program or system.
Health technology assessment (HTA)	A multidisciplinary process that uses explicit methods to determine the value of a health technology at different points in its lifecycle. The purpose is to inform decision-making in order to promote an equitable, efficient and high-quality health system.
Horizon scanning	A systematic examination of information to identify new or emerging health technologies that could be potential threats, risks, emerging issues and opportunities, allowing for better preparedness of health systems and informing policymakers, purchasers, and health care providers (for health service research prioritization, financial or operational planning) or facilitate early access (by facilitating controlled diffusion of technologies).[4] Furthermore, it may include health technologies that are becoming obsolete.

Institutionalisation	The embedding of certain rules and norms, and associated actions and processes, within a health system.[5]
Multi-criteria decision analysis (MCDA)	An umbrella term to describe a collection of formal approaches which seek to take explicit account of multiple criteria in helping individuals or groups exploring decisions that matter.[6]
Priority setting	The assignment of an order of priority based on explicit or implicit criteria for selection of health technologies for assessment.
Quality-adjusted life year	A unit of outcome of an intervention where changes to years of life subsequent to this intervention are adjusted according to the quality of life during those years.
Safety	A judgment concerning the acceptability of the risk (a measure of the probability of an adverse outcome and its severity) associated with using a technology in a given situation (e.g. for a patient with a particular health problem) by a clinician with certain training or in a specified treatment setting.
Stakeholder	An individual with an interest in the outcome of the HTA process final decision.[7]
Stakeholder involvement	An iterative process of actively soliciting the knowledge, experience, judgment and values of individuals selected to represent a broad range of direct interest in a particular issue, for the dual purposes of: creating a shared understanding, making relevant, transparent, and effective decisions.[8]
Systematic (literature) review	A synthesis that collates all empirical evidence fitting pre-specified eligibility criteria in order to answer a specific research question.
Universal health coverage	Ensuring that all people have access to needed health services (including prevention, promotion, treatment, rehabilitation and palliation) of sufficient quality to be effective, while also ensuring that the use of these services does not expose the user the financial hardship.[9]

Introduction

Why is HTA important to achieve universal health coverage?

Many citizens around the globe, including those in high-income countries, do not have access to high quality and affordable essential health services. This has led to many governments putting universal health coverage (UHC) high on the health agenda. Health technology assessment (HTA) guides governments in their choices on the public funding of health technologies and is as an important policy tool on the path towards achieving UHC.

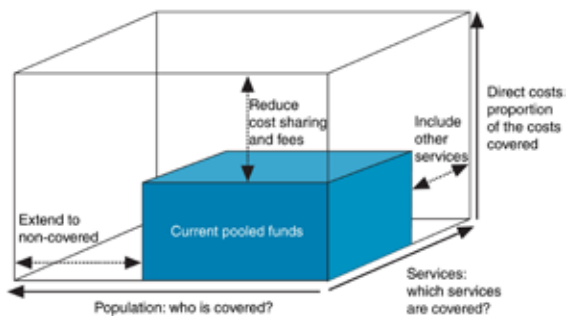
To achieve UHC, countries can advance in at least three dimensions, as reflected in the 'UHC cube'. These dimensions include putting more priority services in the essential benefit package, expanding coverage of existing priority services to non-covered populations and reducing out-of-pocket payments for existing priority services (Figure 1). For example, countries can decide to increase the coverage of skilled birth attendance by making it available to all rural populations or decide to reduce co-payments for antibiotic treatment of children with pneumonia. The decisions they make can have far-reaching consequences for the level and distribution of health services in a country and for financial risk protection.

HTA can be used by countries to inform decisions at different levels, e.g. reimbursement decisions on a single health technology or regarding larger parts of the benefit package (e.g. which health technologies should be provided in mother and child care).

Why a guide to enhance legitimate decision-making?

Policy makers are accountable to the populations they serve and have a duty to maximise the legitimacy of their decisions. Legitimacy here refers to the reasonableness, or fairness, of benefit package decisions as perceived by stakeholders. It is an important prerequisite for broad societal support for these decisions. The accountability for reasonableness (A4R) framework is an important academic work on the conditions of fair processes.[10] This framework identifies four key conditions for organising fair processes: (i) all relevant values should be taken into account; (ii) transparency must be ensured for all decisions; (iii) appeal opportunities must be organised; and (iv) these conditions must be enforced. However, there is little practical guidance on how these conditions should be implemented.

Figure 1. The UHC cube
Source: World Health Report 2010



The value of this guide is that it translates these A4R conditions into a practical, stepwise approach that HTA bodies can use to foster the legitimacy of their decision-making processes.

How is this guide different from other guides for benefit package design?

There are already several guides on benefit package design, such as the publication *What's in, what's out* by the Center for Global Development,[11] the WHO 'Making Fair Choices' report and its handbook *Strategizing national health in the 21st century*.[12,13], Specific guidance on the use of HTA for benefit package design is provided in the HTA Toolkit by iDSI[14] and the HTA roadmap by MSH.[15] All of these guides cover critical aspects of benefit package design, including institutional set-up, required decision-making processes, the necessity of stakeholder involvement, collection of evidence and monitoring and evaluation aspects.

We consider our guide complimentary to these, as we concentrate on the practical organisation of stakeholder participation through deliberation for benefit package design. That is to say, we provide a step-by-step and detailed guidance on various aspects, such as installing an advisory committee, organising stakeholder participation, making argumentation explicit in deliberation and coming to joint conclusions.

How can HTA bodies use evidence-informed deliberative processes?

An evidence-informed deliberative process (EDP) is a practical and stepwise approach for HTA bodies to enhance legitimate benefit package design based on deliberation between stakeholders to identify, reflect and learn about the meaning and importance of values, informed by evidence on these values.

The use of EDPs has far-reaching consequences for the organisation of HTA processes. These are discussed in detail in each step of the framework (in subsequent chapters of this guide) These are 'installing an advisory committee', 'defining decision criteria', 'selecting technologies for hta', 'scoping', 'assessment', 'appraisal', 'communication and appeal' and 'monitoring and evaluation'.

Whom is this guide for?

Many countries have organised their HTA activities in formal HTA bodies.[16] This guide is intended to improve the HTA processes of these bodies, but it is also relevant for countries that have not (yet) established such a body.

Our recent surveys, conducted in 2018–2019, among members of the International Network of Agencies for Health Technology Assessment (INAHTA)[17] and HTA experts in low- and middle-income countries (LMICs)[18] clearly show the need for country support on EDPs and the various steps involved (Table 1).

EDPs are presently employed by national health authorities in Ghana, Iran, Kazakhstan, Moldova, Pakistan and Ukraine for revision of their benefit packages. Its principles were used to design the health benefit package in Thailand,[19] the Netherlands[20] and the province of West Java in Indonesia.[21] EDPs are used as central framework in the guidance on HTA of the World Health Organization.[22]

Table 1. Need for guidance with respect to the steps of EDPs as expressed by INAHTA members (n=27) and HTA experts in LMICs (n=66)*

	INAHTA members	LMIC experts
A. Installing an advisory committee	46%	70%
B. Defining decision criteria**	n/a	n/a
C. Selecting health technologies for HTA	73%	85%
D1. Scoping	65%	81%
D2. Assessment	32%	82%
D3. Appraisal	64%	84%
E. Communication and appeal	52%	80%
F. Monitoring and evaluation	56%	86%

* INAHTA is the International Network of Agencies for Health Technology Assessment. Percentages refer to the element most in need of guidance, per step.
** Step B has not separately been addressed in the surveys.

How should this guide be used?

The guide takes the current decision-making context in a country as the starting point and offers practical guidance depending on the country's level of HTA development. As such, the guide is not meant as a blueprint, but as an inspirational and practical tool.

In this guide, we provide practical guidance to HTA bodies on how to apply each of the EDP steps both in terms on the practical organization and in terms of improving the legitimacy of the decision-making process. Additionally, for each step, we provide an overview on the approaches used by eight HTA bodies around the world (Australia, Brazil, Canada, France, Germany, Scotland, Thailand and the United Kingdom) and provide a detailed description of 1–3 HTA bodies with the best practice ('countries in the spotlight'). These examples may serve as inspiration for other HTA bodies. We also refer to key publications for further guidance. In Annex 1, we provide a checklist for HTA bodies to determine the level of implementation of EDPs in their country and identify areas to improve on this. Annex 2 provides a checklist to aid with stakeholder participation.
The mandate of an HTA body determines which steps are relevant. For example, the selection of health technologies for a specific hta may only be relevant for HTA bodies who have responsibility for it.

What is different in this second version of the guide?

This second version of the EDP guide now includes more detailed practical instructions on the implementation of EDPs, based on our recent country work, academic exchange, surveys among HTA bodies and further literature review. This guide also includes a chapter on monitoring and evaluation. We will continue to update this guide as we gain more experience in applying EDPs in different countries around the globe.

Can I get support to implement EDPs in my country?

We carry out projects to support countries in the application of EDPs in Ghana, Iran, Kazakhstan, Pakistan, Moldova and Ukraine. This involves workshops, short-term consultancy and/or long-term collaboration. Please contact us for more information (contact details are in the colophon).

Evidence-informed deliberative processes

Why do HTA bodies need evidence-informed deliberative processes?

Benefit package design is an intrinsically complex and value-laden political process that takes place in an environment of diverging social values and interests. Society includes a variety of relevant stakeholders, such as patients, the public, providers, payers, industry and policy makers. Each may also have differing social values and interests. This results in different perceptions of what makes health technologies valuable. In such pluralistic societies, stakeholders may reasonably disagree on what values can be used to guide benefit package design.

However, value frameworks currently employed by HTA bodies around the world to design benefit packages do not sufficiently account for this complex reality. They are ill fitted for considering the diversity of stakeholder values, which leads to insufficient sets of relevant information. Current frameworks are typically based on the use of 'substantive' criteria, which are believed to reflect the most important social values. This has led HTA bodies to use, for example, 'clinical benefit', 'safety' and 'cost-effectiveness' as important decision criteria.[23]

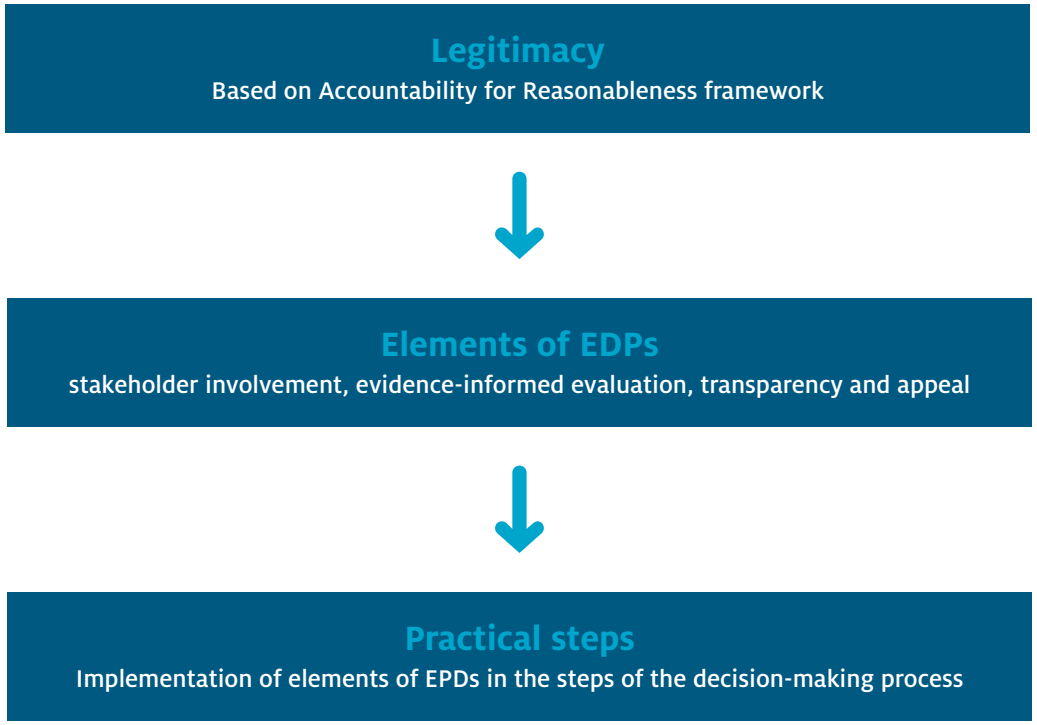
There is broad recognition that frameworks for benefit package design should be based on legitimate processes. Here, 'legitimacy' refers to the reasonableness, or the fairness, of benefit package decisions as perceived by stakeholders, which is an important prerequisite for broad societal support. The A4R framework recognises that stakeholders often justifiably disagree about the importance of specific social values in setting priorities and it argues that stakeholders are more likely to accept priorities that are the outcome of fair processes.[24] In other words, stakeholders may agree with certain decisions even though they may have preferred another outcome. The A4R framework identifies four key conditions for organising fair processes: (i) all relevant values should be taken into account; (ii) transparency must be ensured for all decisions; (iii) appeal opportunities must be organised; and (iv) these conditions must be enforced. However, there is little practical guidance as to how these conditions should be implemented.

EDPs respond to this and provide a practical stepwise approach for HTA bodies to implement the A4R conditions in their decision-making processes. As such, the use of fair processes, as embodied by the EDPs, can improve the legitimacy of decision-making processes.

What is an evidence-informed deliberative process?

EDPs are a practical, stepwise approach for HTA bodies to optimise the legitimacy of their decision-making processes. An EDP integrates four elements, all practical translations of the A4R conditions (Figure 2).[25] First, *stakeholder involvement* ideally operationalised through stakeholder participation with deliberation. This is the core element of EDPs and discussed in detail below. Second, the element of *evidence-informed evaluation*, which allows for the use of scientific evidence and contributions from stakeholders in terms of their experiences

Figure 2. The conceptual framework of EDPs



and judgments when further evidence is unavailable. Third, the element of *transparency*, which ensures that the deliberative processes, including their objectives, modes of stakeholder involvement, the decision reached and its related argumentation, is explicitly described and made publicly available. Fourth, the element of *appeal*, which ensures that a decision can be challenged and revised if new information or insights become available. As such, EDPs provide the best way to combine evidence, information, perspectives and values, while also allowing these aspects to be identified and openly discussed.

What are the practical steps?

We have translated these principles into a set of practical steps to support HTA bodies in the development of their processes (Figure 3). In steps A–C, we offer advice on the installation of an advisory committee, including the organisation of stakeholder participation; on how to define decision criteria, and on how to set up a process for identifying and selecting health technologies for hta. In steps D1–D3, we give advice on how to scope, assess and appraise a specific health technology. In steps E–F, we provide guidance on communication and appeal, and monitoring and evaluation respectively. Overall, the succesfull implementation of EDPs is strongly dependent on its context, i.e. the institutional design, policy environment and analytical HTA capacity. While the steps are presented as separate activities and in a linear fashion, in practice there is often deviation between them. To reiterate, when we speak of 'HTA' we refer to steps A–F, while 'hta' specifically refers to step D.

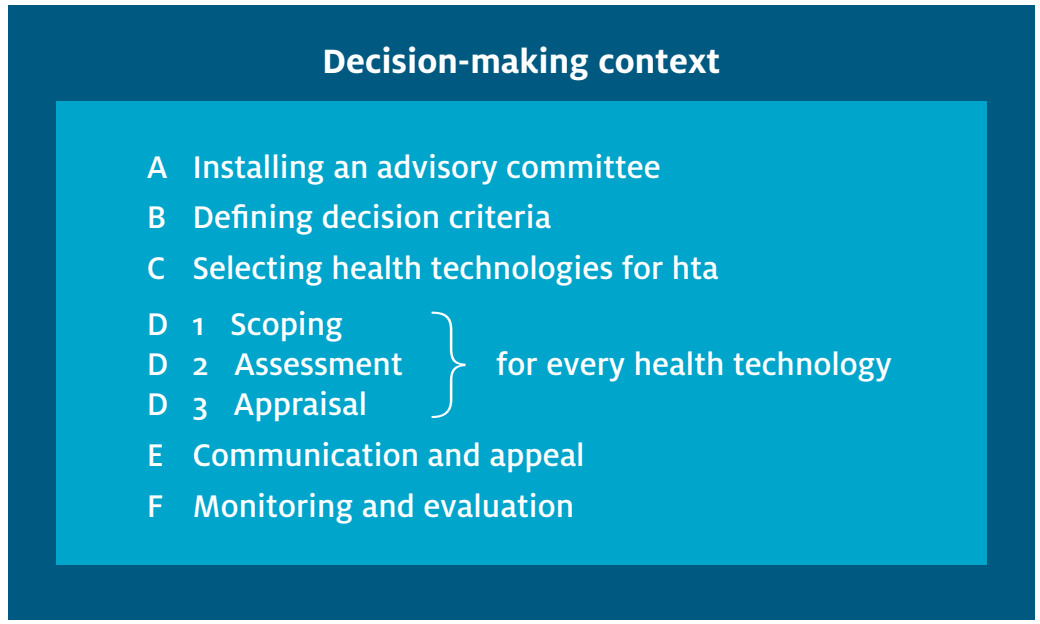
Why is stakeholder involvement important in EDPs?

Benefit package design is an intrinsically complex and value-laden political process that takes place in an environment with a broad array of stakeholders. Stakeholders are often categorised as one of the 7Ps. These are:

- Patients and the public: can provide a unique insight into the experience of a particular disease or condition, such as explaining the advantages and disadvantages of a therapy.
- Providers/health professionals: can bring in new perspectives, such as expert views on the effectiveness of technologies and the feasibility of alternative implementation options, including organisational aspects.
- Purchasers: can provide information on the feasibility of alternative implementation options, including organisational aspects.
- Payers: can provide generic non-disease specific perspectives and are often the public.
- Policy makers: can provide information on the feasibility of alternative implementation options.
- Product makers (industry): can provide in-depth knowledge on the intervention under evaluation.
- Principal investigators (academia): can provide technical expertise on a variety of topics.

These stakeholders often have diverging social values and interests that result in different perceptions of what makes health technologies valuable. In pluralistic societies, stakeholders can be expected to disagree about what values can be used to include or exclude technologies from the package. For example, in decisions on

Figure 3. Practical steps of implementing EDPs



public funding of expensive cancer drugs, patients may argue that the best treatment should be made available, while other patients may argue that their treatment should not be displaced and taxpayers may reason that it is more important to use public resources efficiently. In comparison to all these other parties, health professionals may want to have access to the latest technological developments in their field. As a result, decisions are bound to be controversial because stakeholders likely disagree about what should be prioritised, who should benefit and who should not. Decision-making by health authorities on behalf of society, characterised by value pluralism, is only justified to the extent that their decision-making is carried out legitimately.

Stakeholder involvement can contribute to the legitimacy of decision-making and is formally defined as "an iterative process of actively soliciting the knowledge, experience, judgment and values of individuals selected to represent a broad range of direct interest in a particular issue, for the dual purposes of creating a shared understanding; and making relevant, transparent, and effective decisions."[26] It can improve the legitimacy of decision-making in three ways. First, stakeholder involvement can serve to identify the full range of interests that a society has in relation to a particular decision. Second, it can improve understanding among stakeholders by explicating the interests of all parties involved. Third, stakeholder involvement can contribute to improving the quality of decisions, as stakeholders contribute specific knowledge (e.g. about barriers to implementation or meaningful patient outcomes).

There are three approaches to stakeholder involvement: participation, consultation and communication. We discuss these approaches below.

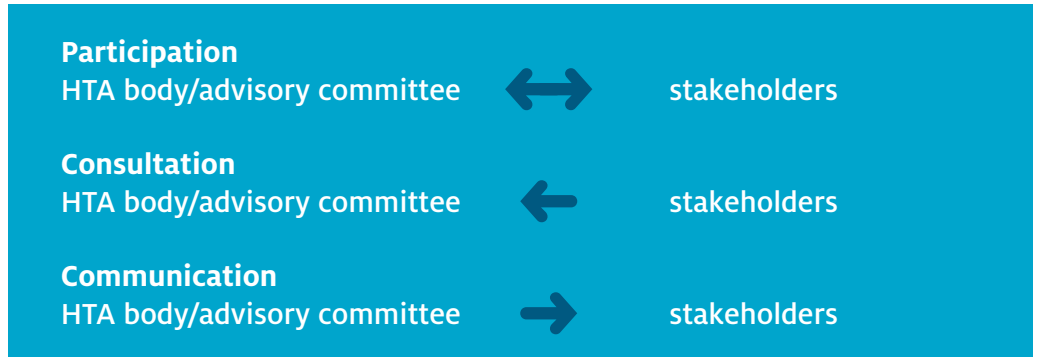
What is stakeholder participation?

Stakeholder participation means that stakeholders are actively engaged in deliberations and can openly exchange views on argumentation and evidence. Through this interaction and practical reasoning, stakeholders may deepen their understanding of their own preferences and those of others affected by decisions. They may replace uninformed opinions by views that are more rational and better supported by arguments and evidence, improving the quality of the decisions. There is good evidence that participants learn from deliberative engagement, including considering information that is contrary to their opinions and can change their opinions in line with this new information.[27] For these reasons, stakeholder participation can best realise the full benefits of stakeholder involvement and contribute to the legitimacy of decision-making. Accordingly, we strongly advise HTA bodies consider implementing stakeholder participation in their decision-making process.

Stakeholder participation on reimbursement decisions is organised through interaction with an advisory committee. Advisory committees have the mandate to formulate recommendations to decision-making bodies, such as the Ministry of Health. See the chapter on installing an advisory committee for more detail. Stakeholder participation in the advisory committee can be organised in two complementary ways (Table 2).

- HTA bodies may choose to include specific stakeholders as *formal members* of their advisory committee. Such stakeholders typically represent the general interest of patients and sometimes industry, and not a specific interest regarding certain health technologies. These members have voting power.
- Second, HTA bodies can also organise stakeholder participation by inviting specific stakeholders to participate in their meetings. There stakeholders are *not formal members* of the advisory committee and are not granted voting power. Such stakeholders typically represent interests or have specific expertise of the health technology being deliberated on. Nevertheless, this form of stakeholder participation does give some degree of influence and ownership of decisions to stakeholders.

Figure 4. Information flow between HTA body/advisory committee and stakeholders



There are several approaches to structuring the decision-making process to facilitate stakeholder participation and deliberation in committee meetings. We advise using a systematic approach that ensures all participants and members express their preferences and considerations, such as Nominal Group Technique (NGT). See the chapter on installing an advisory committee for further guidance. In addition, HTA bodies should take into account several general principles for stakeholder participation and deliberation when designing their decision-making processes.[28] These include:

- Transparency: the deliberative processes – including the objectives, modes of stakeholder involvement, the decision reached and its related argumentation – should be explicitly described and made publicly available.
- Inclusivity: all relevant values pertaining to decisions on a health technology should be taken into account. This requires that relevant stakeholders being meaningfully involved in the decision-making process. This means that barriers to effective participation should be removed.
- Learning: stakeholders are ideally provided opportunities to participate and interact in deliberations as this likely improves the understanding of preferences, arguments and/or evidence, thereby improving the quality of the decision-making process.
- Impartiality: the deliberative process used for each decision and those involved in it should be free from undue influences, both internal (e.g. from the agency supporting the HTA process) and external (e.g. from stakeholders with vested interests).

The use of these principles in the organisation of stakeholder participation is not without challenges. Ensuring transparency may be challenging in settings where there is little or no tradition of open decision-making. Ensuring inclusivity can be resource-intensive for HTA bodies who need to proactively identify affected stakeholders and involve them in decision-making processes and allocate adequately trained staff to the organisation of stakeholder participation. In addition, participating stakeholders may find it difficult to invest the time required to familiarise themselves with procedures of the HTA body. Ensuring learning takes place

amongst stakeholders can be challenging for HTA bodies, as this requires multiple perspectives being effectively represented by participating stakeholders and that all perspectives are accounted for in the deliberations. Ensuring impartiality may also be challenging in contexts in institutions with less experience in managing conflicts of interest. We provide practical guidance on how to deal with these challenges in the chapter on installing an advisory committee (Step A). In Annex 2, we provide a comprehensive checklist that HTA bodies can use to organise meaningful stakeholder participation. Table 2 illustrates how HTA bodies can organise stakeholder participation in the different steps of the decision-making process.

What is stakeholder consultation?

In many countries, meaningful stakeholder participation and actual deliberation between stakeholders is non-existent or in its infancy. Although not ideal, HTA bodies often rely on non-deliberative ways to involve stakeholder perspectives in their HTA processes such as through consultation.

Consultation refers to a structured process for collecting feedback from groups of stakeholders on specific issues without providing opportunities for meaningful deliberation with the HTA body's advisory committee. One example is the provision of oral or written patient testimonies to an advisory committee, as organised by HTA bodies in Australia, the Netherlands and the United Kingdom. Other examples include the use of surveys, stakeholder meetings and solicited feedback from stakeholders on HTA draft reports, including proposed recommendations (this latter approach is followed by HTA bodies in France, the United Kingdom, Germany, the United States and Brazil, among others). Table 2 illustrates how HTA bodies can organise stakeholder consultations in the different steps of the decision-making process.

The benefit of consultation is the large number of respondents that can be reached, however, there are some drawbacks. First, the timing and topic for input is predefined, which limits the scope of comments that stakeholders can provide. Second, consultation offers no opportunities for deliberation among stakeholders and the HTA body's advisory committee, which limits transparency and the facilitation of mutual learning. Third, the quantity of feedback can be significant, so time should be reserved for providing feedback. If the HTA body in question has limited capacity to do so, this can restrict the scope of public consultation and affect its ability to contribute to the overall legitimacy of the process.

What is stakeholder communication?

Stakeholder communication refers to efforts by HTA bodies to inform stakeholders of their activities and results by using communication platforms. Communication can be achieved through public meetings, by preparing plain-language versions of reports to increase accessibility, by dissemination to high priority groups using patients' organisations or by using social media. Communication is characterised by a very low-level of stakeholder involvement. This one-way flow of information means there is little chance of stakeholders influencing the decision-making process. Table 2 illustrates how HTA bodies can organise stakeholder communication in the different steps of the decision-making process.

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Table 2. An overview of options to involve stakeholders in each step of EDPs based on respectively stakeholder participation, consultation and communication.[29]

Table 2 continued

	Step A Installing an advisory committee	Step B Defining decision criteria	Step C Selecting health technologies for hta	Step D1 Scoping		Step D2 Assessment	Step D3 Appraisal	Step E Communication and appeal	Step F Monitoring and evaluation
Participation	Participation as **formal member** in advisory committee with voting rights					Participation as **formal member** in advisory committee with voting rights			
	Participation as **non-formal member** in advisory committee without voting right. Typically for stakeholders who represent interests of specific health technologies					Participation as **non-formal member** in advisory committee without voting right. Typically for stakeholders who represent interests of specific health technologies			
Consultation	Inviting stakeholders to nominate advisory committee members	Survey among stakeholders to assess their preferences vis-à-vis values and corresponding decision criteria	Inviting stakeholders to nominate health technologies	Interviews or focus groups, e.g. using nominal group technique		Invited submission of hta reports	Providing written or oral statements to advisory committee	Inviting stakeholders to review draft lay summaries and communication materials	Inviting stakeholders to review draft impact pathways and derived indicators, definitions and operationalisations
				Invited submissions		Inviting stakeholders to review evidence reports	Expert panel consultation		
		Publishing tentative criteria and soliciting stakeholder input, e.g. through citizen panels or patient advisory groups	Including stakeholder sources in horizon scanning	Multimedia analysis		Surveys	Citizen panels	Face-to-face meetings to discuss and address appeals and/or concerns, e.g. public hearings	
						Social media analysis	Face-to-face meetings with stakeholders		
						Primary research/ synthesis	Inviting stakeholders to review draft recommendations		
Communication	Publicly available document describing the advisory committee installation process and its procedures	Publicly available document describing the definition process and the decision criteria	Publicly available document describing the selection process and selected health technologies	Publicly available document describing the scoping process		Publicly available document describing the assessment process and outcomes	Publicly available document describing the appraisal process and outcome	Publicly available document describing the communication and appeal process	Publicly available document describing the monitoring and evaluation process
								Multimedia dissemination of final decision reports	
								Publicly available overview of appeals and relevant actions taken	

Understanding context

The successful implementation of EDPs depends on the institutional design and the policy context of an HTA body, as well as the availability of HTA capacity for carrying out HTA activities. This chapter describes these elements and how HTA bodies can assess and improve on them.

Why is institutional design important and how should it be assessed?

The institutional design of an HTA body relates to its legal and regulatory context. A key principle for institutionalisation is independence, namely that the HTA body is not influenced by undue (political) pressure of any kind. For example, HTA bodies should be free from the influence of individual politicians, civil servants and pharmaceutical companies.

However, in reality, it is not easy for a national HTA body to be fully independent from the government.[30] Several HTA bodies have started as small departments in the Ministry of Health and many still have close ties. Moreover, the bulk of their funding often comes from the Ministry of Health. As a result, many HTA bodies are semi-independent and function 'at arm's length' of the Ministry, as is the case for ZIN in the Netherlands and NICE in the UK. Their independence is assured through legal and regulatory provisions, such as through stipulating that the HTA body has its own legal status, administrative autonomy and budget, and is only accountable to the Ministry in broad terms. Full reliance of a HTA body on private funding is similarly undesirable – in such situations, HTA bodies may become vulnerable to undue pressure from private interests.

Undue influence from Ministry of Health or other interest groups may jeopardise the functioning of a HTA body and reduce the potential benefits of EDPs. We advise HTA bodies to assess their independence, if there is reason to believe that this may be the case. This can be done by institutional review and making improvements where necessary and possible, for example.

Why is policy context important and how should it be assessed?

The policy context of an HTA body determines the successful implementation of EDPs to a large extent, and the subsequent use of HTA results. There are a number of aspects that deserve explicit attention:

- Is there a policy statement on the willingness to use HTA in policy and/or practice? A statement like this can play a pivotal role in convincing stakeholders to contribute to the implementation of EDPs, promotes the uptake of HTA results and is ideally issued by the Ministry of Health.
- Is there a formal process to link HTA results with policy making? Ideally, the Ministry of Health will have defined a clear pathway on how HTA results should feed into the decision-making process, including the role of different institutions. EDPs can be implemented in alignment with these pathways.
- Which stakeholders are involved in HTA activities? For the successful implementation of EDPs, it is vital to understand the various stakeholders' roles. Ignoring certain stakeholder groups may undermine societal support for the implementation of a health

technology, which may hamper its impact.
- Is there sufficient public funding for HTA activities available on an annual basis? The implementation of EDPs requires investment in analytical capacity and networks, and requires public funding to be sustainable. Reliance on private funding alone may jeopardise the independence of HTA activities.

We recommend that HTA bodies assess their policy context in terms of the above-mentioned aspects and they can do so in various ways (see 'Further reading' for full references):

- The policy process can be assessed through a review of policy documents (e.g. strategic health plans) and through the consultation of relevant stakeholders. Survey and interview tools for the latter are available elsewhere (e.g. Li, 2017).
- Stakeholders and their (potential) roles can be identified with a checklist (Vlad et al., 2018).
- The participation of stakeholders in the HTA process can be assessed with our checklist. The chekclist is provided in Annex 2 and published elsewhere (Jansen et al., 2018).
- The extent to which various steps of the EDPs process are implemented can be assessed using our survey on EDPs (Oortwijn et al, 2020).

For the successful implementation of EDPs, we advise HTA bodies to improve on the above aspects where needed. For example, they may wish to create formal linkages between the HTA body and the Ministry of Health, or to improve stakeholder participation in their decision-making processes.

What is current HTA capacity and how should it be assessed?

The implementation of EDPs and related HTA activities is largely dependent on the available HTA capacity. This relates to:

- An organisation's capacity for carrying out envisioned HTA activities, involving medical doctors, public health specialists, epidemiologists, statisticians, psychologists, biomedical engineers, ethicists and/or economists. This should include sufficient capacity to review and critically appraise international scientific literature.
- Access to international databases of scientific articles.
- Availability of domestic HTA training opportunities, such as short courses, workshops, Master's programmes and PhD training.
- Availability of an (inter)national networking strategies for collaboration between HTA bodies and relevant stakeholders.

We recommend that HTA bodies assess the HTA capacity in their setting and take action where needed.

Further reading

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Step A **Installing an advisory committee**

This chapter describes how to best install an advisory committee, with a view on its practical organization and the legitimacy of the decision-making process. The table at the end of this chapter provides an overview on how selected HTA bodies have installed their advisory committees.

What is the role of an advisory committee?

An advisory committee is typically organised as an impartial entity at an HTA body, with its role dependent on the mandate of the HTA body. In most countries, the mandate of a HTA body is to develop recommendations on reimbursement and pricing decisions.[31] In these contexts, the advisory committee is responsible for preparing such recommendations or decisions. However, a committee may also be involved in other steps, such as the selection of health technologies for hta (step C) or scoping (step D1). We recommend that HTA bodies involve an advisory committee in all steps in order to safeguard the consistency and continuity of the activities in question.

What should the composition of an advisory committee be?

As the advisory committee informs public funded decision-making, it should preferably reflect the broad public interest in its recommendations. This means that the composition of the committee should mirror the diversity of social values present in the population.

Countries have taken different approaches in realising this. HTA bodies often include two types of formal advisory committee members. The first type may include members on the basis of their professional or scientific expertise, such as clinicians, public health experts, ethicists, economists, or epidemiologists. The second type includes members on based on the interests they represent, such as patient- and carer-organisations or industries. Note that these latter members represent the general interests of patients and industry and not specific interests regarding specific health technologies. Generally speaking, all committee members have voting power, so have a say in the final recommendation of the advisory committee.

How can stakeholders get involved in the advisory committee?

There are different ways of involving stakeholders in the advisory committee. Ideally, stakeholders *participate* in the advisory committee; they are actively engaged in deliberations and can exchange their views on argumentation and evidence provided. There are two complementary ways to organise the stakeholder participation:

- HTA bodies may choose to include specific stakeholders as *formal members* of its advisory committee – as described in the section above. Such stakeholders typically represent the general interest of patients and industry occasionally, however, do not have specific interests regarding specific health technologies. These members have voting power.
- Second, HTA bodies may also wish to organise stakeholder participation by inviting specific stakeholders to participate in their meetings. These stakeholders are *not formal members* of

the advisory committee and are not granted voting power. Such stakeholders typically represent interests or have specific expertise of the health technology being deliberated upon.

Alternatively, stakeholders can be *consulted*; they can be involved in non-deliberative ways, such as through the provision of verbal comments at meetings or written testimonies prior to meetings. Another option is stakeholder *communication* in which stakeholders are only informed about the processes and/or decisions. For more detail on stakeholder involvement see the chapter on evidence informed deliberative processes.

How should the members of an advisory committee be identified and selected?

Ideally, the committee should consist of 10–15 members.[32] These members, including a chair and deputy chair, are typically installed for a period of three to five years, after which others replace them.[33] The secretary and deputy secretary of the committee are preferably affiliated with the HTA body.

The process for identifying and selecting committee members is preferably done by open advertising. It needs to be clearly described and publicly available. In some countries, the installation of an advisory committee is appointed by the Ministry of Health (e.g. Kazakhstan and the Netherlands).

Should an advisory committee be supported by sub-committees?

In settings where large parts of benefit packages are evaluated, a single advisory committee most often does not have the capacity to appraise all health technologies. In these instances, subcommittees can prepare preliminary recommendations for the advisory committee on the inclusion of certain health technologies pertaining to certain disease areas, which are then presented for endorsement to the advisory committee. This has the additional benefit that more stakeholders can be involved in the decision-making process. This provides them with a platform to express their perspectives and to provide advice to the advisory committee on the technology in question.

The Disease Control Priorities project in Pakistan was organised this way: several technical working groups (TWG) working in disease areas like communicable and non-communicable diseases prepared preliminary recommendations for the National Advisory Committee (NAC) on the inclusion of technologies in the benefit package (see Annex 3 for a detailed description of this process).

What is the role of the chair of the advisory committee?

In a deliberative process, stakeholders should be able to meaningfully share their views and the power differences among participants should be minimised. The chairperson of the committee, or an external facilitator, has an important role in this. Throughout the decision-making process, they should identify the preferences and underlying beliefs of all committee members, confront others with these and work towards a joint conclusion. The chair should check continuously if all members still agree with the joint recommendation being formulated.

Committee members may experience social pressure to conform to the wishes of the group and, means of doing so, settle for less satisfying results. A group decision may also be forced through by one dominant member. The chairperson has an important task to address these aspects. For more details on deliberative processes, see the chapter on evidence informed deliberative processes.

Should the advisory committee use a structured decision-making process?

An advisory committee requires a structured decision-making process in various steps of the EDP process, for example, in scoping (step D1) and appraisal (step D3). We advise the use of a systematic process that allows all committee members to express their preferences and considerations, such as NGT. This approach starts by asking all committee members individually to express their preferences and considerations. In a subsequent group phase, all individual contributions are listed on a chart and discussed in the order they appear. The chairperson invites all members to comment, ask questions for clarification or express their agreement or disagreement. Subsequently, all members are asked to make their judgment independently. An additional round of discussion may follow in which the judgements and the reasons behind them are discussed. Practical guidance on NGT is available elsewhere.[34]

How should a decision be reached?

An advisory committee can make several decisions throughout the different steps of the HTA process, for example, on the choice of decision criteria, the selection of technologies for hta and on the reimbursement of a technology. For the sake of legitimacy, these decisions should ideally be reached by consensus. It has been argued that a committee has reached consensus when it can agree on a decision and each member can say: 'I believe you understand my point of view; I believe I understand your point of view; I will support this decision when we leave this meeting because it was reached fairly and openly; and I believe this decision best represents the interests of society.'[35] However, the development of consensus is not always feasible because stakeholders may, for good reasons, continue to disagree. Also, from a theoretical perspective on legitimacy, it is not always necessary to reach a consensus. The objective of deliberation is to maximise understanding and support among involved stakeholders, realising that not all stakeholders necessarily need to agree with the decision. As such, an advisory committee can also reach a decision by majority voting in case consensus is not achievable.

How should undue influences in the process be avoided?

The advisory committee should be effectively independent and be free from undue influences. However, in reality, committee members may have interests in the outcome of the deliberations and may try to influence processes in a way that could lead to suboptimal recommendations, from a societal perspective. To be cognisant of this and to reduce the risk of undue influence, it is important that committee members sign a conflict-of-interest form before taking on their term *and* before every meeting. The latter also applies to all stakeholders involved in committee meetings. This is common practice in several countries (e.g. Poland, Australia and the United Kingdom).

Should committee meetings be public?

Some HTA bodies, such as ZIN in the Netherlands, have public meetings, which attendees need to register for themselves. The meetings are frequently attended by stakeholders, such as patient groups, and sometimes include more than 50 people. Most HTA bodies have closed meetings. The decision to have open or closed meetings may also affect the dynamics of the deliberation, as committee members may feel constrained to freely express their argumentation in the presence of an audience. Yet, open meetings may improve the transparency of the decision-making process and, thereby, its recommendations.

Should committee members and other stakeholders be trained?

Committee members likely have different levels of knowledge and expertise and may not all be able to absorb the inherent complexity of

presented evidence without adequate training. Therefore, we advise that HTA bodies train all committee members in the principles of health research, including evidence-based medicine, economic analysis and health systems research.

With regard to training stakeholders, HTAi has developed important tools and guidance, such as guidance for patient organisations completing a patient group submission template (available for members via https://htai.org/interest-groups/pcig/resources/for-patients-and-patient-groups/). In Australia, the Patient Voice Initiative (PVI) has developed similar online tools for patient groups and communities for making submissions to the Pharmaceutical Benefits Advisory Committee (PBAC).

Should committee members be financially compensated?

HTA bodies typically follow governmental policies on financial compensation to serve at committees of public agencies in this respect. For example, in countries like Poland, Canada and the Netherlands, committee members are compensated for participating in committee meetings. However, while financial compensation is a necessary condition, it may not always be sufficient for motivating fruitful committee participation. The goal should be to recruit committee members who are not only motivated by money, but who have intrinsic motivations to act as committee members.

Further reading

Stakeholder involvement

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Countries in the spotlight

Brazil The National Committee for Health Technology Incorporation (CONITEC) advises the Minister of Health on inclusion, any changes to reimbursement and disinvestment of health technologies regarding the Brazilian Public Health System (SUS). The advisory committee of CONITEC (Plenary) represents 13 members with voting rights. The committee includes appointed representatives from seven different departments of the Ministry of Health and one representative of the following organisations: National Health Agency, National Health Surveillance Agency, National Board of Health, National Council of State Health Secretaries, National Council of Municipal Health Secretaries and Federal Board of Medicine. The term of membership is not fixed and the representatives need to declare any potential conflicts of interest. The committee meets every two weeks to formulate preliminary recommendations on each hta, based on majority of votes. The Chair has casting vote in case of equal division of votes. The recommendation is then submitted for public consultation. The contributions received are then discussed by the Plenary before the final recommendation is formulated. Following the meeting, the technical report with the recommendation is submitted to the Secretary of Science, Technology and Strategic Inputs of the Ministry of Health (SCTIE), who decides whether to accept the recommendation. If considered pertinent, the Secretary may request another public hearing on the topic before giving a decision.
Further information is available elsewhere.[36]

Canada The Canadian Agency for Drugs and Technologies in Health (CADTH) is an example of an HTA agency that has several specialised appraisal committees. The Canadian Drug Expert Committee (CDEC) is the advisory body for the Common Drug Review (CDR) process and makes recommendations on the inclusion of drugs in the federal, provincial and territorial publicly funded drug plan. The CDEC consists of 16 members including one chair, three patient representatives, one ethicist and 11 expert members who represent a variety of qualifications and fields of expertise, such as disease management, and evaluation of pharmaceutical products such as physicians, economists and pharmacists. All members are selected for a three-year term through a public call for nominations. Committee members must abide by the conflict-of-interest guidelines for CADTH expert committee and panel members, with conflicts of interest being handled by the chair in conjunction with a CADTH Executive Team member.
Further information is available elsewhere.[37]

Installing advisory committees in selected HTA bodies

Indicator	BRAZIL	FRANCE	GERMANY	THAILAND
HTA agency	**National Committee for Health Technology Incorporation – CONITEC**	**Haute Autorité de Santé – HAS**	**Institute for Quality and Efficiency in Health Care –IQWiG**	**Health Intervention and Technology Assessment Program HITAP**
Evaluated committee	Plenary	Transparency Committee (TC)	Federal Joint Committee ('Plenum')	Subcommittee for Development of Benefit Package and Service Delivery (SCBP)
Mandate of the advisory committee	Advisory	Advisory	Binding	Advisory
Accessibility of meetings	Open	Closed, although anyone can attend if approved by the chair.	As a rule, resolutions are passed in public sessions. Closed sessions or written voting is permissible only in clearly defined exceptions.	Closed
Number of members	13 members with voting rights	29 members with voting rights	13 members with voting rights	Not identified
Composition	Members from different departments of the Ministry of Health (7), National Health Agency, National Health Surveillance Agency, National Board of Health, National Council of State Health Secretaries, National Council of Municipal Health Secretaries and the Federal Board of Medicine.	One chair, two vice-chairs, 20 health practitioners, one methodologist, one epidemiologist, two patients & two consumer representatives.	One chair, two impartial members, members of Health Insurance Funds (5), Hospital Federation (2), Association of Statutory Health Insurance Physicians (2), Association of Statutory Health Insurance Dentists (1).	Multidisciplinary, members from different stakeholder groups. No industry representatives
Term	No term specified	Three-year term, renewable twice	Six-year term	Three-year term
Selection of members	Closed: appointed by stakeholder organisations.	Closed: appointed by HAS	Closed: appointed by stakeholder organisations.	Closed: selected by National Health Security Office.
Reporting of conflict of interest to become member	Yes	Yes	Yes	Not identified

Indicator	CANADA	UNITED KINGDOM	SCOTLAND	AUSTRALIA
HTA agency	**Canadian Agency for Drugs and Technologies in Health**	**National Institute for Health and Care Excellence**	**Scottish Medicines Consortium**	**Pharmaceutical Benefits Advisory Committee**
Evaluated committee	Canadian Drug Expert Committee (CDEC)	Technology Appraisals Committee	As above	As above
Mandate of the advisory committee	Advisory	Binding	Advisory	Advisory
Accessibility of meetings	Closed	Mixed: meetings are held in public, but the agenda is divided into two parts if the committee needs to discuss confidential information.	Mixed: meetings are open to the public, but occasionally, parts of the discussions may legally require a closed session to maintain the academic and commercial confidentiality.	Closed. Representatives from patient groups PBAC can participate by invitation only.
Number of members	16 members with voting rights	24 members with voting rights	23 members with voting rights	20 members with voting rights
Composition	One chair, three patient representatives, one ethicist, 11 experts members who represent a variety of qualifications and expertise; members are expected to have experience and knowledge related to HTA, reimbursement policy and/or epidemiology.	One chair; members represent the NHS, the public, academia and industry.	Members include clinicians, pharmacists, NHS board representatives, the pharmaceutical industry and the public.	Members include doctors, health professionals, health economists and consumer representatives
Term	Three-year term	Three-year term	Not identified	Four-year term
Selection of members	Open procedure: members are selected through a public call for nominations and appointed by CADTH.	Open procedure	Open procedure	Not identified: members are appointed by the Minister for Health.
Reporting of conflict of interest to become member	Yes	Yes	Yes	Yes

Step B **Defining decision criteria**

This chapter describes how to best define decision criteria, with a view on its practical organization and the legitimacy of the decision-making process. The table at the end of this chapter provides an overview on how selected HTA bodies have defined their decision criteria.

Why are decision criteria needed?

Decision criteria reflect the broad goals of a country's health system (such as maximisation of population health, equal distribution of health and financial protection) and underlying values (such as equity, solidarity and access to good quality care). The advisory committee employs decision-criteria for the assessment and subsequent appraisal of technologies. In this way, recommendations on the inclusion or exclusion of technologies in the benefit package are based on social preferences. We distinguish between generic and contextual decision criteria.

What are generic decision criteria?

First, HTA bodies should select a number of explicit criteria for that can be used to evaluate every technology. We label these criteria as 'generic criteria', as they are of generic relevance to technologies and should be used consistently.

Almost all countries with formal HTA bodies employ at least three common generic criteria. These are safety, effectiveness and quality of the evidence. This follows from the widely shared goal of improving population health through technologies that are proven to be safe and effective. Yet, the need for a broader set of generic criteria is being increasingly recognised; in the context of constrained budgets on one hand and the aspiration to achieve universal health coverage on the other.

- Cost-effectiveness as a criterion is important because it maximises the health benefits in a population to a given the budget. Ignoring cost-effectiveness and spending the budget on cost-ineffective technologies leads to substantial opportunity costs in terms of forgone healthy life years.
- Equity, or prioritising the worst-off, is important because it captures the value of fairness, which calls for providing technologies on the basis of need and reducing inequalities. The worst-off can be defined as: a) those with lowest standard health (or the most severe conditions) without the technology required to deal with it, or b) the poorest or otherwise disadvantaged (e.g. in terms of gender, area of living or marginalised groups). Since the most cost-effective services do not always benefit the worst-off, reimbursement decisions may sometimes consider whether health benefits for the worst-off should be assigned extra value. In practice, this implies that some technologies that are not considered cost-effective may still be reimbursed because they promote a fairer distribution of health and access to health care.
- Financial risk protection may also be considered important because some technologies may cause substantial out-of-pocket expenditures and may impoverish people. In cases where less cost-effective services may provide very high financial protection (at an acceptable cost), such services could be assigned extra value. This may imply that some

interventions that are not considered cost-effective may still be reimbursed because they provide substantial financial risk protection.
- Furthermore, additional decision criteria may be found to be relevant, such as budget impact or broad socio-economic impact.

We advise the advisory committee to create a list of relevant decision-criteria. In doing so they can consider existing lists of decision-criteria (see 'Further reading'). The best way to trade-off criteria is discussed in the chapter on appraisal (step D3).

What are contextual decision criteria?

In addition to the generic criteria, the advisory committee may also consider contextual criteria, which are specific to the technology under scrutiny. These may include many considerations like the 'unmet medical need' for rare diseases, or the 'impact on caregivers' for technologies related to homebased care. Since these contextual criteria can be many, we advise HTA bodies to develop a checklist of all potentially relevant criteria. These could be based on past evaluations or existing lists of decision-criteria. The advisory committee should ideally use this checklist to ensure evidence will be collected on all relevant criteria in the scoping step. Subsequently, the identified contextual criteria are then discussed in the appraisal step. This approach improves the quality and consistency of decisions.

How should decision criteria be selected?

The process of criteria selection involves the identification of broad health system goals and values and specifying these into decision criteria. This is a complex and abstract task, which should ideally be achieved through a broad consultation process with stakeholders, including members of the general population. We propose a stepwise process on the basis of similar work we carried out in Iran, Kazakhstan and Pakistan.

First, we advise doing a review of policy documents on national health strategies. This may lead to the identification of aspects or goals like improving equity, universal coverage, financial protection and taking care of the worst-off. These can be considered as important social values in health care. A dedicated working group can then group these values in meaningful categories and operationalise them in measurable criteria. For example, the social value 'taking care of the sickest' can be operationalised in the criterion 'severity of the condition', expressed in the number of healthy life years lost.

Second, we recommend undertaking a survey among a broad range of stakeholders, ideally including members of the general population, to assess their preferences vis-à-vis these values and corresponding criteria. If this is impossible, the survey can be limited to members of the advisory committee. Their preferences can best be recorded through a Likert-scale. Third, the results of the survey can be discussed in a meeting, again involving a wide range of stakeholders, to assess the definition of criteria and their relative importance. The result of this meeting should be to recommend a set of (generic) decision criteria. Fourth, the HTA body should ideally subject their list of decision-criteria to public scrutiny by means of a democratic process, for example, by publishing them online and soliciting comments. Finally, depending on the decision-making structure, the final list of decision criteria can taken to the next step in the process; being proposed to the Ministry of Health for endorsement, for example.

As an alternative process, HTA bodies may choose to organise a workshop in which stakeholders are asked to develop recommendations for a series of health technologies and, for every technology, to list the criteria they find relevant. They may first do so individually, then arrange a group discussion – this task should ultimately result in a comprehensive list of possible decision-criteria, which then need to be further classified into a final set of meaningful and coherent criteria.

For reasons of transparency and legitimacy, it is important to clearly describe the above-mentioned elements in a publicly available document.

Further reading

Conceptual frameworks

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Examples of decision criteria based on surveys

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Countries in the spotlight

The Netherlands Equality, solidarity and equity are considered to be important principles of the Dutch health system. With regard to coverage decision-making, the Ministry of Health makes the final decision, using the recommendations of the HTA body (i.e. the National Health Care Institute – ZIN). Even though ZIN is transparent about the way in which coverage decision-criteria (necessity, effectiveness, cost-effectiveness and feasibility) are defined, they are not explicitly derived from the relevant health system values and their application, in practice, has proven difficult to understand. The interpretation of the necessity criterion has, for example, been used differently in coverage decisions, as testified by the cases of Viagra (favourable cost-effectiveness ratio, but not reimbursed) and Myozyme and Fabrazyme (unfavourable cost-effectiveness ratio but reimbursed). This has led to ZIN recognising the need to operationalise the necessity criterion, as well as the need for public debate on the reimbursement of expensive drugs (for rare diseases) and about how to address social values in coverage decision-making.
Further information is available elsewhere.[38]

Sweden The Health and Medical Services Act has described the goals of the Swedish health system. The 'ethical platform' has translated these into principles to guide national and local health decisions. The principles include human dignity (i.e. all individuals have equal value), which precedes the principle of needs and solidarity (resources should be primarily allocated to the areas of greatest need) and the principle of cost-effectiveness (a reasonable incremental cost-effectiveness ratio). With regard to HTA, the Dental and Pharmaceutical Benefits Agency (TLV) makes decisions regarding pricing and reimbursement of new prescription drugs and are obligated to consider these ethical principles. However, the use of these principles is not always transparent in practice: although the government has made clear that severe diseases and significant impairments in the quality of life should be prioritised, even at a higher societal cost, it is not clear how high those costs may be. In addition, the New Therapies Council develops recommendations for city councils regarding the use of new drug therapies on the basis of cost-effectiveness analysis of TLV. Yet, the county councils are highly autonomous and there are examples that councils have been using the principles differently.
Further information is available elsewhere.[39]

Defining decision criteria by selected HTA bodies

Indicator	BRAZIL	FRANCE	GERMANY	THAILAND
HTA agency	**National Committee for Health Technology Incorporation – CONITEC**	**Haute Autorité de Santé – HAS**	**Institute for Quality and Efficiency in Health Care –IQWiG**	**Health Intervention and Technology Assessment Program HITAP**
Evaluated committee	Plenary	Transparency Committee (TC)	Federal Joint Committee ('Plenum')	Subcommittee for Development of Benefit Package and Service Delivery (SCBP)
Reimbursement decision criteria	Efficacy, accuracy, effectiveness, safety, cost-effectiveness, budget impact.	Actual clinical benefit, likely clinical added value compared to treatment alternatives, target population.	Level of additional patient benefit versus the appropriate comparative therapy defined as recovery, relief from pain or discomfort, improvement in quality of life, extension of life, reduction of side effects.	Cost effectiveness as main criterion, budget impact, equity and moral aspects of access to health services.
Stakeholder involvement	Not identified	Not identified	Not identified	Not identified

Indicator	CANADA	UNITED KINGDOM	SCOTLAND	AUSTRALIA
HTA agency	**Canadian Agency for Drugs and Technologies in Health**	**National Institute for Health and Care Excellence**	**Scottish Medicines Consortium**	**Pharmaceutical Benefits Advisory Committee**
Evaluated committee	Canadian Drug Expert Committee (CDEC)	Technology Appraisals Committee	As above	As above
Reimbursement decision criteria	Unmet need, efficacy, effectiveness, safety, cost-effectiveness, budget impact,may include ethical, legal and social implications.	Clinical effectiveness and health-related factors, cost-effectiveness, non-health factors (social value judgments), cost (savings) outside NHS and non-health gains. Additional criteria for end-of-life medicines.	Clinical effectiveness and cost-effectiveness.	Comparative health gain (effectiveness and safety), comparative cost-effectiveness, other relevant factors (e.g. patient affordability, predicted use in practice and financial implications, equity and severity of the medical condition treated).
Stakeholder involvement	Not identified	Not identified	Not identified	Not identified

Step C **Selecting health technologies for hta**

HTA bodies have limited budgets for their activities, so important choices need to be made on which health technologies are evaluated. Making such choices involves two steps: i) the identification of health technologies in need for hta; and ii) among those, the selection of technologies that are most important to evaluate.

In some countries, such as France, the Ministry of Health is responsible for both steps. In other countries the responsibilities are divided, such as the UK, where the National Institute for Health Research Innovation Observatory *identifies* technologies for hta, while its HTA body, NICE, *selects* the technologies. In other countries, responsibilities are organised differently, per disease domain. Here, for ease of argumentation, we refer to the 'responsible body' as being responsible for both steps unless stated otherwise.

This chapter starts by presenting different approaches used by responsible bodies for the identification and selection of health technologies. It then provides recommendations on which approach the responsible body can best use in their own context, on both reimbursement and disinvestment decisions.

What approaches are available for identifying and selecting technologies for hta?

The responsible body may employ of broad array of approaches for the identification and selection of health technologies for hta, ranging from ad-hoc requests and nomination procedures to horizon scanning systems. These approaches can all be characterised by how proactive the HTA body is in the identification and selection of technologies for hta and by what sources of information are used (only stakeholders or also other sources).

1. Ad-hoc requests

When 'ad-hoc requests' are used, the responsible body is not proactive concerning the identification and selection of technologies for hta. It relies on stakeholders (e.g. decision-makers, industry, medical societies and hospitals) to submit topics; they can typically do so throughout the year. This reflects current practice in several countries, such as in Germany and the Netherlands, where industry can submit pharmaco-economic dossiers to the HTA body to obtain reimbursement decisions.

2. Nomination procedures

In the use of 'nomination procedures', the responsible body proactively identifies and selects technologies for hta.

- *Closed procedure*
  In a closed procedure, the responsible body is not transparent in the way technologies are identified and selected. It determines the topics and number of htas that need to be conducted annually. There is little or no public documentation available on how this is decided.
- *Targeted procedure*
  In a targeted procedure, the responsible body first defines priority areas and, subsequently, identifies and selects technologies for hta within these priority areas. This process may

explicitly involve stakeholder consultation. For example, in Ukraine, the Ministry of Health defined four priority areas for service inclusion for the benefit package: childbirth, neonatal care, stroke and myocardial infarction. These priority areas are laid down in a separate law (2019) and new ones may be selected each year. For each of the areas, the Ministry of Health selects health technologies for hta in consultation with stakeholders.

- *Open procedure*
  In an open procedure, the responsible body proactively consults several stakeholders to identify and select technologies for hta. For example, the HTA body in Thailand, HITAP, invites representatives of payers, health professionals, academics, patient associations, citizen groups and industry to submit potential topics for hta twice a year. From the identified topics, a 'short-list' is defined and a panel of representatives of four stakeholder groups (health professionals, academics, patients and civil society) make the final selection.

3. Horizon scanning systems

Horizon scanning is formally defined as "a systematic examination of information to identify new or emerging health technologies that could be potential threats, risks, emerging issues and opportunities, allowing for better preparedness of health systems and informing policymakers, purchasers, and health care providers (for health service research prioritization, financial or operational planning) or facilitate early access (by facilitating controlled diffusion of technologies). Furthermore, it may include health technologies that are becoming obsolete and that have the potential to effect health, health services and/or society."[40]

The responsible body can scan and monitor various health information sources (including reports from companies and regulatory agencies, scientific literature, presentations, newspaper articles and online information/ portals) to identify promising technologies not yet widely used in their health system. Horizon scanning is frequently done in high-income countries and to some extent in middle-income countries (e.g. Brazil). A horizon scanning system must be clear about the time horizon taken, the independence of those who are identifying health technologies and should have a clear dissemination strategy. Most of the governmental horizon scanning systems use a two to three year time horizon for scanning health technologies.

Which approach is best for identifying and selecting technologies for hta?

As described above, there are different approaches to the identification and selection of technologies for hta, all consisting of different elements. This makes it difficult to recommend a single approach, so countries may opt to choose several (e.g. ad-hoc for extramural pharmaceuticals and a horizon scanning system for all other technologies, as done in the Netherlands). Here, we define a number of steps that the responsible body should follow in this process and we provide best practice advice for each of the steps.

As a first step, the responsible body should identify health technologies for hta. We advise that it should preferably use the information from the targeted or open nomination procedure, or from a horizon scanning system. As a second step, the responsible body should ask the question 'which of the identified health technologies are most important from a societal perspective?' Here, 'importance' should be interpreted as the impact that a health technology has on society. To answer this question, we advise the responsible body to employ criteria that reflect societal impact, such as potential population health benefits, budget impact and impact on health policy. This step results in a list of health technologies that have a potential high impact on society, which may or may not in needed for hta.

In the third step, the responsible body should ask the question 'which of these health technologies need to be selected for hta?' In other words, is the required evidence to make a decision already available, or is more evidence collection necessary for the relevant decision-making criteria? For answering this question, it is necessary to know the criteria for reimbursement decision-making (see step B in this guide). These criteria often include safety, effectiveness, quality of the evidence and cost-effectiveness.

Some studies aimed at selecting technologies for hta mix criteria on societal impact and reimbursement decision-making in the second step. We consider these studies conceptually flawed. Only criteria that reflect societal impact of health technologies are relevant here. This means that clinical effectiveness, by itself, is not always relevant in the second step, as it does not necessarily reflect societal impact. However, clinical effectiveness can be relevant when combined with other criteria to reflect societal impact, such as the size of the target population. This leads to our recommendation that the responsible body should employ, among others, the criterion '(potential) population health benefit', which combines clinical effectiveness and size of target population.

Which approach should be used for choosing to identify and select health technologies for hta for disinvestment decisions?

There are a number of steps that the responsible body should follow when it comes to disinvestment decisions. As a first step, the responsible body should use a systematic approach to identify health technologies with suspected low or no added value among the reimbursed health technologies. They can do so based on an analysis of practice variation of the current use of health technologies or invite stakeholders to nominate health technologies with no or low added value for disinvestment (see above for nomination procedures). Alternatively, the responsible body may consider the whole benefit package as the basis for selecting health technologies in need of assessment.

In the second step, based on the candidate health technologies, the responsible body should ask the question 'which of the identified health technologies with suspected low or no added value should be selected for disinvestment?' To answer this question, we advise the body to employ criteria that reflect societal impacts, such as budget impact or suspected low value (as indicated by the criteria for reimbursement decisions such as low effectiveness or high cost-effectiveness – see step B in this guide) in combination with a large target population. This step results in a list of low-value health technologies that are candidates for disinvestment, and which may needed for hta.

In a third step, the responsible body should ask the question 'which of the identified health technologies need to be selected for hta?' In other words, is the required evidence to make a decision already available, or is more evidence collection needed for the relevant decision-making criteria? When answering this question, it is necessary to know the criteria for reimbursement decision-making (see step B in this guide).

For reasons of legitimacy, the responsible body should make efforts to ensure that the applied processes and criteria follow societal preferences and values. One way to achieve this is through the inclusion of stakeholders throughout the process. In addition, we advise the use of a transparent process, i.e. to describe the various steps including its selection criteria in a publicly available document. The results of this step should be approved by the relevant authority (e.g. the Ministry of Health).

Further reading

Horizon scanning

- Oortwijn, W. on behalf of the HTAi Global Policy Forum. Facing the dynamics of future innovation: The role of HTA, industry and health system in scanning the horizon. Background Paper 2018 Global Policy Forum. Available from: https://htai.org/wp content/uploads/2018/02/HTAi_Global_Policy_Forum_2018_Background_Paper.pdf
- Methods Toolkit from EuroScan International Network. Available from: https://www.euroscan.org/index.php/en/tools/methods-toolkit-2 (includes chapters on evaluation of horizon scanning methods and systems, examples of networks and collaborations as well as hospital-based horizon scanning systems and activities).

Selection of health technologies in need of HTA

- Pichon-Riviere A, Augustovski F, Alfie V, García Martí S. Mechanisms for identification and prioritization of health technologies to be assessed by HTA agencies. Identification and selection of health technologies in need for HTA for reimbursement decisions. HTAi 2020 Latin America Policy Forum on Health Technology Assessment. Background document. Brasilia, April 2020.
- Galician Health Technology Assessment Agency (avalia-t), a member of the Spanish horizon scanning (GENTECS) network, has developed the PriTEC prioritization tool for prioritizing health technologies for assessment. The tool is available from:

http://pritectools.es/index.php?idioma=en

- Polisena J, Varela-Lema L, Gutiérrez-Ibarluzea I, Godman B. A Disinvestment Toolkit: The Prioritization Of Technologies Of No Or Low Added ValueInternational Journal of Technology Assessment in Health Care , Volume 34 , Special Issue S1: Conference Theme: Strengthening the Evidence-to-Action Connection , 2018 , pp. 159 DOI: https://doi.org/10.1017/S0266462318003355
- Lauvrak V, Arentz-Hansen H, Di Bidino R, Erdos J, Garrett Z, Guilhaume C, Migliore A, Scintee SG, Usher C, Willemsen A. Recommendations for Horizon Scanning, Topic Identification, Selection and Prioritisation for European Cooperation on Health Technology Assessment. EUnetHTA WP4 Deliverable 4.10, Oslo, 2020. Available from: https://eunethta.eu/wp-content/uploads/2020/04/200305-EUnetHTA-WP4-Deliverable-4.10-TISP-recommendations-final-version-1.pdf
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Criteria for selection of health technologies for hta

- Henshall CH, Oortwijn WJ, Stevens A, Granados A, Banta HD (eds.). Priority setting for health technology assessment: theoretical considerations and practical approaches. In: EUR-ASSESS report. Int J Technol Assess Health Care. 1997;13(2):144-85.
- Specchia ML, Favale M, Di Nardo F, Rotundo G, Favaretti C, Ricciardi W, et al. How to choose health technologies to be assessed by HTA? A review of criteria for priority setting. Epidemiol Prev. 2015;39(4 Suppl 1):39-44.
- Varela-Lema L, Atienza-Merino G, López-García M. Priorización de intervenciones sanitarias. Revisión de criterios, enfoques y rol de las agencias de evaluación [Priority setting of health interventions. Review of criteria, approaches and role of assessment agencies]. Gac Sanit. 2017;31(4):349-357. doi:10.1016/j.gaceta.2016.09.015

Countries in the spotlight

United Kingdom The National Institute for Health Research (NIHR) Innovation Observatory (IO), an independent research team based at Newcastle University in the UK, provides timely information on new and emerging health technologies that may have a potential significant impact on patients or the provision of health services in the near future. The IO produces horizon scanning reviews and pipeline analysis reports. These aim to identify all new and emerging health technologies being developed within a specified time-frame, in a technology area, across a disease area or at a specific point on a patient pathway.

The NIHR IO uses advanced data systems that scan both open and confidential data sources. Data sources include trial registers, as well as secondary sources, such as scientific literature, regulatory agencies, clinical experts, patients/patient organisations, media and other horizon scanning organisations/trade data. The reviews can incorporate patient, expert and company feedback on identified technologies. The NIHR IO mainly identifies pharmaceuticals and cell therapies, followed by diagnostics and imaging, devices and biotechnology.

Public and patient consultation is a critical element of the NIHR IO horizon scanning capability. It hosts a national public and patient forum called VOICE: Valuing Our Intellectual Capacity and Experience. This forum can be used to assist prioritisation, obtain consultation on an innovation (by both companies and horizon scanning organisations) or educate members on topics.

NICE undertakes additional filtration and conducts the actual selection of health technologies for HTA. They request NIHR IO to provide technology briefs, including information on target groups, information about the technology, patient groups, patient pathways, efficacy, safety, estimated costs and impact. These technology briefs are sent to industry and one or two clinical experts for review. Subsequently, NICE selects technologies by using the following criteria: significant health benefit, significant impact on health-related policy, significant impact on the NHS resources, evidence on significant variation in use and the added value of national guidance.
Further information is available elsewhere.[41]

Thailand There are two main platforms of coverage decision making in Thailand: the Subcommittee for the development of the Benefits Package and Service Delivery (SCBP) for non-pharmaceutical products, and the National List of Essential Medicines subcommittee (NLEM) for pharmaceutical products. Here, we focus exclusively on the SCBP.

The SCBP provides recommendations on which non-pharmaceutical products should be included in the Universal Coverage Scheme (UCS) benefits package, the largest health plan managed by the National Health Security Office (NHSO). Before 2009, any stakeholder could propose new technologies to the NHSO. This led to a large number of proposals and submissions. Concerns on inconsistent levels of evidence quality and undue influence of high-level authorities prompted the SCBP to develop a more systematic process for identifying and selecting technologies for hta.

The current identification and selection process falls under the authority of SCBP and is coordinated by HITAP. The Health Economics Working Group (HEWG) determines the criteria for topic identification and selection.

These are: size of population affected; severity of disease; effectiveness; variation in practice; economic impact on household expenditure and equity/ethical and social implications. The working group on topic nomination consists of three to four representatives from each of the seven stakeholder groups (policymakers, health professionals, academics, patient associations, civil groups, industry, civil society, lay-citizens, plus other subcommittees and working groups under the NHSO). Annually, these stakeholders identify technologies that are potential candidates for hta. Subsequently, the working group on topic selection involves other representatives from four stakeholder groups (health professionals, academics, patients and civil society) then selects the nominated technologies for hta using a scoring system based on six criteria.
Further information is available elsewhere.[42,43]

Selecting technologies for hta by selected HTA bodies

Indicator	BRAZIL	FRANCE	GERMANY	THAILAND
HTA agency	**National Committee for Health Technology Incorporation – CONITEC**	**Haute Autorité de Santé – HAS**	**Institute for Quality and Efficiency in Health Care –IQWiG**	**Health Intervention and Technology Assessment Program HITAP**
Evaluated committee	Plenary	Transparency Committee (TC)	Federal Joint Committee ('Plenum')	Subcommittee for Development of Benefit Package and Service Delivery (SCBP)
Health technology identification procedure	Horizon scanning; open procedure	Open procedure by the Ministry of Health	Open procedure with annual submissions	Open procedure with annual submissions (non-drugs); ad-hoc requests (drugs)
Health technology selection procedure(s) to prioritise for hta	Explicit	Explicit	Explicit	Explicit
Stakeholder involvement in the identification of health technologies	Yes, with consultation. Stakeholders can submit technologies for assessment.	Yes, with consultation. Patient and/or carer organisations may identify health technologies.	Yes, with consultation. Industry and providers can submit dossiers and the public can provide topics.	Yes, with consultation. The working group on topic nomination, consisting of seven stakeholder groups, identifies non-drug technologies for hta.
Stakeholder involvement in the selection of health technologies	Yes, with consultation – internal and external review with expert involvement.	Not identified	Yes, with participation. IQWiG selects up to five topics based on 15 preselected topics by a committee of public members, patients and one representative of the Commissioner for Patients' Affairs.	Yes, with consultation. The Working group on Topic Selection, consisting of four stakeholder groups, selects non-drug technologies for hta.

Indicator	CANADA	UNITED KINGDOM	SCOTLAND	AUSTRALIA
HTA agency	**Canadian Agency for Drugs and Technologies in Health**	**National Institute for Health and Care Excellence**	**Scottish Medicines Consortium**	**Pharmaceutical Benefits Advisory Committee**
Evaluated committee	Canadian Drug Expert Committee (CDEC)	Technology Appraisals Committee	As above	As above
Health technology identification procedure	Horizon scanning; targeted procedure; ad-hoc requests	Horizon scanning; open procedure	Horizon scanning; ad-hoc requests	Ad-hoc requests
Health technology selection procedure(s) to prioritise for hta	Explicit	Explicit	Explicit	Not identified
Stakeholder involvement in the identification of health technologies	Yes, with consultation. Interested individuals or entities can submit topics for consideration.	Yes, with consultation. Clinicians, patients and the public, and other organisations, such as the NHS, can also identify potential topics.	Yes, with consultation. SMC consults a network of clinical experts to support their horizon-scanning function.	Not identified
Stakeholder involvement in the selection of health technologies	No. The final decision about the HTA topics to be undertaken by CADTH will be done quarterly and is based on a ranked list, the resource needs for the topics, and CADTH's capacity.	Yes, with consultation – internal and external review with expert involvement.	Not identified	Not identified

Step D1 **Scoping**

This chapter provides guidance on scoping, with a view on its practical organization and the legitimacy of the decision-making process. The table at the end of the chapter provides an overview on how selected HTA bodies have organised scoping.

What is scoping?

Scoping concerns the explicit definition of the objective and research questions of an hta. These need to be discussed and defined prior to conducting the assessment in order to produce htas that really address a relevant policy problem. Scoping requires the systematic exploration of the relevant aspects of a health technology under evaluation from multiple perspectives (e.g. patients, informal carers, health professionals, decision-makers). Scoping provides important input for the assessment of health technologies, in the sense that it defines what evidence needs to be collected. This may differ from health technology to health technology.

Who should be involved in scoping?

HTA bodies are often responsible for scoping, but policy makers, Ministries of Health or external committees, in consultation with relevant stakeholders and/or experts, can also do this.

How should scoping be conducted?

We advise to define the research question using the PICO or TICO format. The PICO format describes which population is targeted, which intervention is evaluated, which comparator is used and what the relevant outcome measures are. The TICO format describes which technology is being evaluated, for which indications (in terms of target diseases, population and intended use), which comparator is used, and what the relevant outcome measures are.
A more flexible approach may be required in the context of complex health technologies.[44]

The choice of outcome measures relates closely to the selection of decision criteria, as described in step B. These decision criteria include, by default, the generic criteria such as safety, effectiveness and quality of the evidence. In addition, these may also include a number of contextual criteria, namely those that are specific to the technology under evaluation. Scoping these criteria, and the questions in general, is ideally done in consultation with relevant stakeholders or via qualitative approaches to stakeholder involvement (e.g. using NGT).

It is important to clearly describe the above-mentioned elements in a publicly available document for transparency and legitimacy.

Further reading

- Practical guideline for scoping of an HTA report (for hospital based HTA – using PICO and/or TICO questions). Available from: http://www.adhophta.eu/toolkit/assets/tools/AdHopHTA_toolkit_tool3_document.pdf
- Hailey, D., Babidge, W., Cameron, A. et al., 2010. HTA agencies and decision makers. An INAHTA guidance document. Available from: http://www.inahta.org/wp-content/themes/inahta/img/HTA%20%20Decision%20Makers.pdf
- Brereton L, Ingleton C, Gardiner C, Goyder E, Mozygemba K, Lysdahl KB, et al. Lay and professional stakeholder involvement in scoping palliative care issues: Methods used in seven European countries. Palliat Med. 2017;31(2):181-92.
- EUnetHTA. An analysis of HTA and reimbursement procedures in EUnetHTA partner countries: final report, 2017. Available from: http://5026.makemeweb.net/outputs/analysis-hta-and-reimbursement-procedures-eunethta-partner-countries-final-report

Countries in the spotlight

The Netherlands In its scoping activities, the ZIN requests insights, expertise and experiences from relevant stakeholders, including health professionals (associations), insurers and patient associations. Examples of topics that can be covered during a scoping exercise are relevant outcome measures, estimated number of patients, expected market penetration and appropriate use of agreements, such as start and stop criteria (in the case of specialised drugs). Scoping is also explicitly mentioned in the Dutch guideline for conducting economic evaluations, where determining the PICOT in collaboration with medical and patient organisations is recommended prior to conducting the hta.
Further information is available elsewhere.[45]

United Kingdom At NICE, the scoping process takes place six times per year, in which several technologies are considered at the same time. Scoping defines the issues of interest (for example, population, comparators and health outcome measures) and sets the boundaries for the work undertaken by the independent academic groups and the manufacturer(s) or sponsor(s) of the technology who produce reports for the advisory committee. There are two opportunities for consultees (patients and carers) and commentators (stakeholders) to get involved in scoping:

1. The scoping consultation (written consultation). Consultees and commentators are invited to comment on the draft scope (PICO), draft remit given to NICE by the Department of Health (e.g. to evaluate the clinical and cost effectiveness of (technology x) within its licensed indication for treating (disease y)) and the provisional matrix of all stakeholders that should be involved in the appraisal. For this purpose, NICE provides a form that consultees have 20 working days to complete.
2. The scoping workshop (oral consultation). This is held if the consultation responses received need further discussion and consultees and commentators are invited to attend this workshop. A recommendation is then sent to the Department of Health to make a final decision about whether a topic should be referred to NICE for appraisal.

Further information is available elsewhere.[46]

The organization of scoping by selected HTA bodies

Indicator	BRAZIL	FRANCE	GERMANY	THAILAND
HTA agency	**National Committee for Health Technology Incorporation – CONITEC**	**Haute Autorité de Santé – HAS**	**Institute for Quality and Efficiency in Health Care –IQWiG**	**Health Intervention and Technology Assessment Program HITAP**
Evaluated committee	Plenary	Transparency Committee (TC)	Federal Joint Committee ('Plenum')	Subcommittee for Development of Benefit Package and Service Delivery (SCBP)
Scoping procedure	Yes	Yes	Yes	Yes
Stakeholder involvement in scoping	No, although industry request private scoping meetings prior to submission.	Yes, with participation of experts and stakeholders.	Yes, with participation. Selected patient representatives are involved in the determination of an appropriate comparator therapy.	Yes, with consultation. Experts, health practitioners and key stakeholders are invited to comment on the scope of the research in a stakeholder meeting.

Indicator	CANADA	UNITED KINGDOM	SCOTLAND	AUSTRALIA
HTA agency	**Canadian Agency for Drugs and Technologies in Health**	**National Institute for Health and Care Excellence**	**Scottish Medicines Consortium**	**Pharmaceutical Benefits Advisory Committee**
Evaluated committee	Canadian Drug Expert Committee (CDEC)	Technology Appraisals Committee	As above	As above
Scoping procedure	Yes	Yes	Not identified	Not identified
Stakeholder involvement in scoping	Yes, with consultation. Input from stakeholders, such as patient groups, clinical experts, drug programs and expert committee members, is considered.	Yes, with consultation. Patients, carers and stakeholders are invited to comment on the draft scope in a written consultation. An oral consultation may follow.	No	No

Step D2 **Assessment**

What is assessment?

The assessment of health technologies includes various activities: systematic evidence collection on the selected decision criteria; synthesising evidence, including quality analysis; independent review of evidence and reporting findings and implications. This guide does not provide detailed methodological guidance on the various activities, as these already exist elsewhere (see the 'Further reading' section). However, it does discuss issues in the assessment of health technologies that may affect the legitimacy of the decision-making process. The table at the end of the chapter provides an overview on how selected HTA bodies have organised assessment.

This guide presents assessment and appraisal as subsequent activities. However, in reality, they may not always follow. For example, an advisory committee may consider the available evidence base insufficient to adequately appraise a certain health technology and may request additional evidence collection.

Who does the assessment?

The collection and provision of evidence is ideally carried out by an independent party, such as an academic organisation, to avoid undue influence of any kind. In some countries, it is also done by the HTA body itself or by the manufacturers of the health technology. In the latter case, it is important to have measures in place to safeguard impartiality, for example by using strict guidelines on evidence collection and provision.

Ideally, the HTA body synthesises the evidence, including an analysis of its quality and issues an evidence report. This report includes standardised evidence summaries for each criterion, a critical evaluation of the available evidence and related uncertainty and an overview of where evidence is missing. The evidence report should be subjected to an independent review and discussed by relevant stakeholders, which may lead to revisions and the final report.

For reasons of transparency and legitimacy, we recommend that HTA bodies clearly describe how the following assessment activities are organised in a publicly available protocol:

- evidence collection;
- synthesis of the evidence, including analysis of its quality;
- independent review of the evidence, including stakeholder consultation;
- report of findings and implications.

How should hta findings from another setting be adapted?

HTA bodies, particularly those in low- and middle-income countries with limited analytical capacity, may consider the adaptation of HTA findings from other settings.

The adaptation of hta study findings requires a careful process in which HTA bodies firstly need to judge whether the study context is sufficiently similar to their context, and secondly, whether they need to make adjustments. Some hta information will be relevant in every setting, for example, evidence from systematic literature reviews and evidence from randomised controlled trials regarding the efficacy and effectiveness of an intervention. However, factors like the epidemiological profile of diseases, models of clinical practice, relative prices and

unit costs, the availability of healthcare resources and budget constraints, the choice of health benefits, comparators, and the comparability of treatment patterns and populations are more context-specific, so may limit the generalisability of results.

The EUnetHTA has developed a short guide for countries to assess whether existing hta studies can be adapted and transferred to different contexts. Their adaptation toolkit aims to help HTA bodies to assess the relevance of the report; the reliability, and transferability. Please see the 'Further reading' section for references.

In some instances, HTA bodies may also choose to directly adopt study findings from other settings. For example, if a high-income country finds that a particular technology is not cost-effective, it will likely not be cost-effective in a low- and middle-income setting either.

Further reading

Guidelines for data collection

- The HTA Core Model® is a methodological framework for production and sharing of HTA information. Available from: https://www.eunethta.eu/hta-core-model/
- INTEGRATE-HTA for the assessment of complex health technologies. Available from: https://www.integrate-hta.eu/downloads/#results
- MedTech HTA – methodological guidance regarding comparative effectiveness, economic evaluation and organisational impact of medical devices. Available from: http://www.medtechta.eu/wps/wcm/connect/Site/MedtecHTA/Home/Publication/

Guidelines for assessing the quality of evidence

- Corabian P, Tjosvold L, Harstall C. Evidence grading systems used in heath technology assessment practice. Edmonton (AB): Institute of Health Economics, 2018. Available from: https://www.ihe.ca/publications/evidence-grading-systems-used-in-health-technology-assessment-practice
- Scott AM, Hofmann B, Gutiérrez-Ibarluzea I, Bakke Lysdahl K, Sandman L, Bombard Y. Q-SEA – a tool for quality assessment of ethical analyses as part of health technology assessments. GMS Health Technol Assess. 2017;13.
- Higgins JPT, Thomas J, Chandler J, Cumpston M, Li T, Page MJ, Welch VA (editors). Cochrane Handbook for Systematic Reviews of Interventions, 2021. Available from www.training.cochrane.org/handbook.
- AMSTAR - MeaSurement Tool to Assess systematic Reviews. Available from: https://amstar.ca/index.php

Guidance on reporting the evidence

- Busse R, Orvain J, Velasco M, Perleth M, Drummond M, Gurtner F, et al. Best practice in undertaking and reporting health technology assessmwents. Working group 4 report. Int J Technol Assess Health Care. 2002;18(2):361-422.
- Watts RD, Li IW. Use of Checklists in Reviews of Health Economic Evaluations, 2010 to 2018. Value Health. 2019;22(3):377-382.
- Hailey D. Toward transparency in health technology assessment: a checklist for HTA reports. Int J Technol Assess Health Care. 2003;19(1):1-7.

Guidance on adapting evidence reports to other settings

- EUnetHTA. Adaptation Toolkit, 2011. Available from: https://www.eunethta.eu/wp-content/uploads/2011/01/EUnetHTA_adptation_toolkit_2011_version_5.pdf
- Goeree, R., et al., Transferability of health technology assessments and economic evaluations: a systematic review of approaches for assessment and application. ClinicoEconomics and Outcomes Research, 2011. 3: p. 89–104.

Countries in the spotlight

Scotland The New Drugs Committee (NDC) is composed of clinicians, pharmacists and pharmaceutical industry representatives. It meets monthly to assess the clinical and economic evidence presented by companies for each new medicine. The evidence presented is supplemented by testimonies from their network of clinical experts across NHS Scotland. Following this technical assessment for identifying the strengths and weaknesses of the case being presented, the NDC offers preliminary advice to the company, allowing them to provide feedback and address uncertainties before the medicine is considered by the Scottish Medicines Consortium (SMC). Once the medicine reaches the SMC, additional evidence from patient groups is also considered. If the recommendation is negative, the manufacturer can request a Patient and Clinician Engagement (PACE) meeting to gather further information on the added value of a medicine.
Further information is available elsewhere.[47]

Argentina HTAs are conducted by several universities and private organisations at the request of different entities, including the Ministry of Health and private insurance. There is no formal scoping procedure in place and there is no involvement of stakeholders in the assessment phase. The focus of assessing health technologies lies on reviewing the clinical evidence. IECS (Institute for Clinical Effectiveness and Health Policy), a member of the INAHTA, delivers different types of HTA reports, depending on the research question: Full HTA report (6–12 months), Rapid Response Reports (4–8 weeks) and Brief Technical Reports (6–12 weeks). The latter report can be followed by a full HTA report.

IECS applies the Consolidated Health Economic Evaluation Reporting Standards (CHEERS) with regard to reporting on economic evaluations. IECS has also developed a tool to inform the 'evidence-to-decision making' process to be applied in HTA. This consists of evaluating: the quality of the evidence (using GRADE); the magnitude of net benefit (considering both benefits and adverse effects); the economic and organisational aspects (cost-effectiveness or expected budgetary impact).
Further information is available elsewhere.[48]

The organization of assessment by selected HTA bodies

Indicator	BRAZIL	FRANCE	GERMANY	THAILAND
HTA agency	**National Committee for Health Technology Incorporation – CONITEC**	**Haute Autorité de Santé – HAS**	**Institute for Quality and Efficiency in Health Care –IQWiG**	**Health Intervention and Technology Assessment Program HITAP**
Evaluated committee	Plenary	Transparency Committee (TC)	Federal Joint Committee ('Plenum')	Subcommittee for Development of Benefit Package and Service Delivery (SCBP)
Publicly available assessment reports on website	Yes	Yes	Yes	Some reports are publicly accessed in through publications, at the remit of researchers.
Stakeholder involvement in the assessment process	No	Yes, with consultation. External experts may be appointed as rapporteurs, invited to present their reports and answer questions.	Yes, with consultation. Patients and clinicians are consulted.	Yes, with consultation
Independent review of the evidence	Yes, with consultation of external contractors.	Yes, with consultation of experts (from health professional organisations and patient or user associations).	Yes, with consultation. Industry, federations and experts can submit statements on the results HTA report within a three-week time period after publication.	Yes, with consultation. Experts, health practitioners and key stakeholders are invited to comment on HTA report. Final HTA report is subject to external peer review.

Indicator	CANADA	UNITED KINGDOM	SCOTLAND	AUSTRALIA
HTA agency	**Canadian Agency for Drugs and Technologies in Health**	**National Institute for Health and Care Excellence**	**Scottish Medicines Consortium**	**Pharmaceutical Benefits Advisory Committee**
Evaluated committee	Canadian Drug Expert Committee (CDEC)	Technology Appraisals Committee	As above	As above
Publicly available assessment reports on website	Yes	Yes	Yes	Yes
Stakeholder involvement in the assessment process	Yes, with consultation. Patient and clinician group input is summarised in a clinical report.	Yes, with consultation	Yes, with consultation. If the recommendation is negative, the manufacturer can request a Patient and Clinician Engagement (PACE) meeting to gather further information on the added value of a medicine	Yes, with consultation. PBAC can request stakeholder meetings (e.g. with manufacturers, patient groups and medical specialists) to gather additional information.
Independent review of the evidence	Yes, with consultation. After review reports are finalised by CADTH, reports are sent to expert review committees.	Yes, with consultation by Evidence Review Groups or Assessment Groups.	Yes, with consultation of new drugs committee and testimonies from clinical experts.	Yes, with consultation

Step D3 **Appraisal**

This chapter provides guidance on the appraisal of health technologies, with a view on its practical organization and the legitimacy of the decision-making process. The table at the end of this chapter provides an overview on how selected HTA bodies have organised their appraisal process.

What is appraisal?

In the appraisal step, the advisory committee interprets the results of the assessment in a broader perspective and formulates a recommendation to inform decision-makers. This is an intrinsically complex and value-laden task and requires a careful judgement process for two reasons. First, appraisal involves social judgements on the importance of decision criteria, such as weighing the value of a life year gained in very young or old persons. Stakeholders such as health care providers, patients, citizens, payers and decision-makers, have different interests and may judge differently. Second, the assessment step typically results in different types of evidence (from various sources and study designs) involving varying degrees of uncertainty - and an advisory committee needs to judge the relevance of this evidence for the decision under scrutiny.

The core task of the advisory committee is to balance these judgments and to develop a recommendation. EDPs use deliberation as a way to achieve this. Deliberation facilitates the judgement process and aims to create a more coherent and mutual understanding of preferences of recommendations among members (see more on the concept of deliberation in the chapter on evidence-informed deliberative processes').

What is the aim and end product of appraisal?

The overall aim of the appraisal phase is to develop a recommendation on the coverage of a health technology in the benefit package. The objective of this is to maximise understanding and support among involved stakeholders while remaining aware that not all stakeholders necessarily need to agree with the conclusion (see 'How to reach a conclusion').

The end product of the appraisal step follows the mandate of the HTA agency as a whole. Typically, this mandate is to develop a recommendation for the Ministry of Health on the inclusion of a technology in the benefit package. In some situations, however, the HTA agency itself can make decisions – in these instances, the end product of the appraisal step is to develop decisions on the inclusion of a technology.

The recommendations are not necessarily limited to the inclusion or exclusion of technologies from the benefit package. Often, they are defined in terms on conditional coverage (i.e. to only include a technology if certain conditions are met). The conditions could be imposed by the payer (for example, a restriction on the population eligible for the technology), or agreed between the payer and the technology provider as an interim measure during evidence development (for example, managed entry or coverage with evidence development arrangements).

Should appraisal use an explicit framework to trade-off criteria?

Consider an advisory committee that has identified three relevant criteria in the evalua-

Table 3. Example of a performance matrix

Technologies	Effectiveness (quality adjusted life years)	Severity of condition*	Disease of poverty	Age
Antiretroviral treatment in HIV/AIDS	100	✱✱✱✱	✓	15 years and older
Treatment of childhood pneumonia	100	✱✱✱✱	✓	0–14 years
Inpatient care for acute schizophrenia	10	✱✱		15 years and older
Plastering for simple fractures	200	✱		all

* Severity of condition is shown as a four-star scale, with more stars indicating a more severe condition.

tion of a health technology: effectiveness, severity of condition and cost-effectiveness. It now needs to appraise the collected evidence. The technology is very effective and targets a severe condition but is not cost-effective because of its high costs. The advisory committee needs to make a judgment that takes the three criteria into account. If the committee considers effectiveness or severity of condition to be more important than cost-effectiveness, the reimbursement recommendation will be positive. If cost-effectiveness is the most important criterion, the recommendation will be negative. In other words, the central challenge for an advisory committee is to trade off the different decision criteria.

There are different options for how advisory committees can trade off criteria. A starting point is always the performance matrix – this presents the performance of a technology on the generic decision criteria (Table 3). The advisory committee evaluates the performance matrix before formulating a recommendation. They may rely on the criteria included in the performance matrix and, if applicable, include other considerations (i.e. contextual criteria) specific to the technology under scrutiny.

Qualitative analysis

Here, the committee develops its recommendation for a technology by deliberating on its performance regarding explicitly defined criteria (i.e. it makes a qualitative interpretation of the performance matrix). The advantage of qualitative analysis, compared to not using any formal approach, is that criteria are explicitly defined. Important challenges remain related to the cognitive load, which may still be high, and the risk that certain stakeholders dominate the deliberations remains.

Quantitative analysis

This approach is traditionally referred to as multi-criteria decision analysis. It follows several steps: i) the evidence on each criterion in the performance matrix is translated into a score (e.g. between 0 and 100); ii) stakeholders' preferences regarding the relative importance of criteria are measured using criterion weights; iii) scores are multiplied by the relative weight of that criterion; iv) the weighed scores are added together to obtain an overall value for each technology. An example is given in Table 4.

Quantitative analysis reduces the cognitive load of processing several criteria simultaneously, by calculating an overall value and the risk of dominant participants influencing the deliberations. Moreover, the use of explicit criteria scores and weights may improve the consistency and transparency of recommendations. However, there are various challenges to the design of quantitative analysis. A fatal flaw is that it cannot capture opportunity costs as 'costs', and 'cost-effectiveness' should never be included as criteria in the value measurement model (see more detail elsewhere [49]).Typically, applications of quantitative analysis also omit a deliberative component and double count criteria. For these reasons, we do not recommend the use of quantitative analysis in the appraisal phase.

Table 4. Interpretation of performance matrix in quantitative analysis*,**

Technologies	Effectiveness	Severity of condition	Disease of poverty	Age	Overall value
Antiretroviral treatment in HIV/AIDS	50	100	100	0	70
Treatment of childhood pneumonia	50	100	100	100	80
Inpatient care for acute schizophrenia	5	50	0	0	7
Plastering for simple fractures	100	25	0	50	48
Weights	40	10	40	10	

* Quantitative analysis should always be followed by deliberation.

** Preference scores for 'effectiveness' are related to its values, following a linear scale. For 'disease of poverty', if the technology targets a disease of poverty, it scores 100, otherwise it is 0. Preference scores for 'severity of condition' are scaled between 0 and 100 in proportion to their points in the table. Assuming decision-makers prefer to treat young people over old, '0–14 years' receives a score of 100, '15 years and older' a score of 0, and 'all ages', a score of 50. Preference scores are presented here for illustrative purposes only and are arbitrary

Analysis with decision rules

Here, the committee interprets the performance matrix using a set of simple rules. These rules guide them in making trade-offs between criteria, which can be quantitative or qualitative in nature. Some HTA bodies follow this approach, defining the relationships between 'cost-effectiveness' and other criteria. For example, ZIN in the Netherlands first uses the criterion effectiveness as a knock-out criterion. It then appraises the cost-effectiveness of technologies in relation to the severity of the conditions in question (Table 5). Technologies that target mild conditions (i.e. below 0.4 on a burden of disease scale from 0 to 1) should cost less than €20,000 per QALY to receive an initial positive recommendation for reimbursement. Technologies targeting severe and very severe conditions (i.e. between 0.4 and 0.7 and above 0.7) may respectively cost up to €50,000 and €80,000 per QALY. Subsequently, ZIN evaluates in a deliberative process if other criteria affect the initial recommendation and reaches a final

Table 5. Cost-effectiveness threshold in relation to severity of a condition

Severity of condition	Cost-effectiveness threshold (€ per QALY)
From 0.1 to 0.4	Till 20,000
From 0.41 to 0.7	Till 50,000
From 0.71 to 1	Till 80,000

recommendation. In the UK, NICE has issued decision rules on the relationship between 'cost-effectiveness' and other criteria. We advise HTA bodies to consider the use of decision rules.

Irrespective of the specific approach, we advise HTA bodies to always include a deliberative component in its appraisal process. Agencies should report these deliberations and include their argumentation underlying recommendations to ensure the consistency and transparency of recommendations.

How are stakeholders best involved in appraisal?

There are different ways of involving stakeholders in the advisory committee. For further details, see the chapter on evidence informed deliberative processes.

Ideally, stakeholders should *participate* in the advisory committee: i.e. they should actively engage in deliberations and exchange their views based on argumentation and evidence. Their active participation allows HTA bodies to adequately reflect the plurality of social values in their coverage decisions on the technology in question and to improve their technical understanding of the subject matter by tapping into the knowledge and expertise of stakeholders. There are two complementary ways to organise stakeholder participation:

- HTA bodies may choose to include specific stakeholders as *formal members* of their advisory committees. These stakeholders typically represent the general interest of patients and occasionally industry, but do not represent specific interests regarding certain health technologies. These members have voting power.
- Second, HTA bodies may also wish to organise stakeholder participation by inviting specific stakeholders to their meetings. These stakeholders are *not formal members* of the advisory committee and are not granted voting power. These stakeholders typically represent interests or have specific expertise of the health technology deliberated upon.

Alternatively, stakeholders can be consulted (i.e. they are involved in non-deliberative ways such as the provision of verbal comments at meetings or written testimonies prior to meetings). Another option is stakeholder *communication*, in which stakeholders are only informed about processes and/or decisions.

How is deliberation best organised?

The deliberation in the appraisal phase is best organised using a structured process, based on the principles of Nominal Group Technique. See the chapter on 'Installing an advisory committee' for more guidance.

How is evidence best presented in the appraisal step?

An advisory committee needs to interpret all relevant evidence on the selected decision criteria in order to develop its recommendation. This may cause cognitive overload to the committee members especially in the context of the simultaneous appraisals of multiple technologies or drugs. To avoid overload, we recommend the use of 'evidence summary sheets', which summarise all relevant evidence for a certain technology on a single sheet (note that these may resemble a performance matrix, as described above). These summary sheets are then made available to the advisory committee with the full information available in a background report.

The use of colour coding and/or symbols may be helpful in such summary sheets, as it may ease the interpretation of this evidence. For example, in our work in Pakistan we classified cost-effectiveness ratios by reference to thresholds and used colours to indicate if a technology was very cost-effective (green), moderately cost-effective (orange) or not cost-effective (red). We assessed the applicability of this cost-effectiveness evidence to the context of Pakistan and visualised this by using stars (with one to three stars, reflecting low to high applicability respectively).

How much time does the advisory committee need for appraisal?

Ideally, an advisory committee would only develop its recommendation if all issues are fully addressed, for example, on uncertainty around evidence and different perspectives of stakeholders. However, this is often not feasible considering the capacity limitations of advisory committee. In reality, the time spent by advisory committee on a recommendation on a specific technology varies by setting and may range half a day at NICE and ZIN, to some 30 minutes in the context of entire package design as in Pakistan. Spending inadequate time for appraisal may compromise the quality and thereby the legitimacy of forthcoming recommendations.

How can be avoided that an advisory committee says 'yes' to all technologies?

Stakeholders often put pressure on advisory committees to retain existing services in the package and while also including new services. Moreover, in instances where an advisory committee does issue a negative recommendation on a service, there is pressure on policy makers to reject this recommendation and make a positive decision. These dynamics make it politically very difficult to make resource reallocation decisions.

We advise HTA bodies to include two elements in the process to improve on this. Firstly, stakeholders should be involved in the advisory committee to pre-empt their criticism and improve their understanding of the decision-making process – this may possibly enhance their support for the recommendations. Second, and of key importance, the advisory committee should operate as much as possible within an explicit budget constraint. The use of such constraint makes stakeholders, as advisory committee members, co-responsible for reallocation decisions, and makes them aware of the concept of opportunity costs (i.e. no services can be included in the package if other services are not excluded). The potential of this approach is that stakeholders, as advisory committee members, only provide recommendations that fit within the fiscal space.

How can the advisory committee trade off the three dimensions of the UHC cube?

In the context of benefit package design for the progressive realisation of UHC, an advisory committee needs to make decisions that simultaneously take all three axes of the UHC cube into account. The interaction between prioritisation of technologies, their coverage levels and co-payment levels is complicated. We propose the committee can best do this in two stages. In the first stage, they should classify all technologies in low, medium or high priority classes. They can do this on the basis of cost-effectiveness analysis, or by more comprehensive approaches, such as EDPs, as described in this guide. In the second stage they should consider which of the following options they should do first on the path towards UHC: i) should new high priority technologies be introduced? ii) should coverage levels of existing high priority technologies be increased? or iii) should co-payment levels of existing high priority technologies be decreased? The WHO 'Making Fair Choices' report describes five unacceptable trade-offs in doing so, such as 'begin expanding coverage for low- or medium services before there is near-universal coverage for high-priority services.'[50] These trade-offs and choices are to be made in a deliberative process. More details on this approach are available elsewhere,[51] and an example of how this was applied in Pakistan is provided in Annex 3.

How should a decision be reached?

The advisory committee needs to reach a conclusion on its recommendation. For the sake

of legitimacy, this is ideally reached by consensus, but it can also be achieved by majority voting. See the chapter on 'Installing an advisory committee' for more guidance.

How can all argumentation in an advisory committee be best registered?

Advisory committee meetings can be live-streamed or recorded and published online afterwards. Alternatively, meetings can be reported back to stakeholders by means of reporting by providing an explicit description of the decision and the argumentation that has been put forward by the advisory committee to justify it. This should include and reference the argumentation put forward by participating stakeholders and be explicit in how it has been taken into account (or why it was not). Ideally, the person responsible for writing the report (or minutes) should be provided with easy to use standardised templates for noting down argumentation (who, what, when) and reactions to it (who, what, when).

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Countries in the spotlight

Canada CADTH has several specialised advisory committees. CADTH applies a deliberative framework in its appraisal processes. The processes and the decision criteria are described in detail in a publicly available document.

The Canadian Drug Expert Committee (CDEC) is the advisory body for the Common Drug Review (CDR) process. CDEC considers evidence on unmet need, efficacy, effectiveness, safety, cost-effectiveness, budget impact and ethical, legal and social implications. At committee meetings, patients and caregivers' perspectives on the condition under study are also presented. In terms of the process, each CDEC member anonymously votes on whether the pharmaceutical product should be listed (three options: list, list with conditions or do not list). Recommendations are then based on the majority of votes during a meeting. The recommendations and the underlying reasons are made public. Stakeholder involvement is clearly specified and open to the public.

There are separate processes for the other CADTH committees on cancer drugs and non-pharmaceuticals.
More information is available elsewhere.[52]

United Kingdom In its appraisal process, NICE uses a framework of structured deliberation based on social value judgments. The process follows the principles of the Accountability for Reasonableness framework (publicity, relevancy, revisability and enforceability).

Evidence appraisal includes the following criteria: comparator technologies, clinical effectiveness and health-related factors, cost-effectiveness and non-health factors (social value judgments and cost (savings) outside the NHS or non-health gains). The maximum acceptable incremental cost-effectiveness ratio (ICER) is not precisely defined: a range is used instead. Technologies with an ICER less than £20,000 per QALY are usually considered cost-effective and explicit reasons should be provided if they are not recommended. If the ICER is above £20,000 per QALY, it is important that the advisory committee takes into account a number of criteria, such as the degree of uncertainty around the ICER and the innovative nature of the technology. Additional criteria are taken into account for end of life medicines. For highly specialised technologies, a different set of criteria are considered (e.g. nature of the condition, impact of the new technology and the cost to the NHS).

These criteria are considered alongside the statements from consultees (e.g. national groups representing patients/carers, healthcare professionals and commissioning groups, as well as the manufacturer) and commentators (e.g. comparator technology manufacturers), at the first committee meeting. The committee summarises the key evidence and their own view on the evidence and provides a preliminary recommendation (based on consensus) in an 'appraisal consultation document' (or 'evaluation consultation document' for highly specialised technologies). Consultees, commentators and the public may respond. Comments are considered in a second committee meeting, which results in a final recommendation to the NHS.
More information is available elsewhere.[53]

The organization of appraisal by selected HTA bodies

Indicator	BRAZIL	FRANCE	GERMANY	THAILAND
HTA agency	**National Committee for Health Technology Incorporation – CONITEC**	**Haute Autorité de Santé – HAS**	**Institute for Quality and Efficiency in Health Care –IQWiG**	**Health Intervention and Technology Assessment Program HITAP**
Evaluated committee	Plenary	Transparency Committee (TC)	Federal Joint Committee ('Plenum')	Subcommittee for Development of Benefit Package and Service Delivery (SCBP)
Approach to trade-off criteria	Qualitative	Qualitative	Qualitative	Qualitative
Closure mechanism	Consensus	Majority vote. At least 12 voting members need to be present. Chair has casting vote in case of equal division of votes.	Majority vote. The committee passes a resolution if at least seven votes have been cast in its favour.	Consensus
Public record of deliberation	Yes: minutes and video recording are available on website.	Yes: minutes are available on website. Video recording may be placed on website if decided by HAS President.	Yes: minutes and video recording are available on website.	Yes: minutes are available on website.
Frequency of appraisal meetings	Every two weeks	Every two weeks	Every two weeks	Monthly
Duration of meetings	Meetings last two sequential half days.	Not identified	Not identified	Not identified

Indicator	CANADA	UNITED KINGDOM	SCOTLAND	AUSTRALIA
HTA agency	**Canadian Agency for Drugs and Technologies in Health**	**National Institute for Health and Care Excellence**	**Scottish Medicines Consortium**	**Pharmaceutical Benefits Advisory Committee**
Evaluated committee	Canadian Drug Expert Committee (CDEC)	Technology Appraisals Committee	As above	As above
Approach to trade-off criteria	Qualitative	Decision rules ('structured decision-making'). Above an ICER of £20,000 per QALY gained, both qualitative modifiers (e.g. the degree of uncertainty around the ICER) and quantitative modifiers (e.g. end-of-life treatment) can be considered.	Decision rules. Qualitative modifiers of cost-effectiveness (e.g. whether it concerns rare diseases or the absence of other therapeutic options) can be considered.	Decision rules using steps: i) are technologies safe and effective (comparative health gain)?; ii) are they cost-effective? Qualitative modifiers of cost-effectiveness are: rule of rescue/ unmet needs and equity; iii) are there other relevant factors to consider?
Closure mechanism	Majority vote	Consensus, or majority vote if necessary. Before a decision to vote is made, the chair will consider whether continuing the discussion at a subsequent meeting is likely to lead to consensus.	Majority vote	Consensus, or majority vote if necessary.
Public record of deliberation	Yes: minutes are available on website.	Yes: minutes are available on website.	Yes: minutes are available on website.	Yes: minutes are available on website.
Frequency of appraisal meetings	Monthly	Monthly	Monthly	Three times a year, usually in March, July and November.
Duration of meetings	Not identified	10:00 until 17:00, unless otherwise advised.	Not identified	Not identified

Indicator	BRAZIL	FRANCE	GERMANY	THAILAND
Stakeholder involvement beyond committee membership	Yes, with consultation. External specialists may be invited to meetings. Patients may register to make statements at the Plenary.	Yes, with consultation. Experts may present and answer questions during the committee meetings, but will not attend deliberations and voting.	Yes, participation without voting. Five patients and two representatives appointed by the Conference of Health Ministers of the German states have a discussion and petition rights on all agenda items. There are representatives of several associations with participation rights on specific topics.	Yes, participation without voting.
Opportunity for stakeholders to comment on draft recommendations following committee meetings	Yes: via public consultation within 20 days.	Yes: for manufacturer only.	Yes: for payer, patients organisations, physicians and hospital representatives.	Not identified
Training of stakeholders	Yes: committee members are trained in HTA. Specific forms are also available for public hearings: for technical-scientific contributions and for contributions with reports of experience or opinion of stakeholders.	Not identified	Not identified	Not identified. HITAP and other organisations provide HTA training to stakeholders.

Indicator	CANADA	UNITED KINGDOM	SCOTLAND	AUSTRALIA
Stakeholder involvement beyond committee membership	Yes, with consultation of external experts, patients and caregivers.	Yes, with consultation. Clinical experts and patients can be consulted to present their views.	Yes, with consultation.	Yes, with consultation.
Opportunity for stakeholders to comment on draft recommendations following committee meetings	Yes: all draft recommendations are posted on the website for stakeholder feedback.	Yes: consultees and commentators can comment if the advisory committee does not recommend use of the technology.	No: draft recommendations are published online after meeting, but there is no opportunity to comment.	Not identified
Training of stakeholders	Not identified, although there is a dedicated patient engagement team to coordinate and assist with patient group input.	Yes: the PIP is the team at NICE that supports and develops public involvement across NICE's work programme. A PIP public involvement adviser is assigned to each appraisal and supports patient and carer consultee organisations, their representatives and individual patients or carers throughout the appraisal.	Not identified. There is guidance on website and dedicated Public Involvement staff.	Not identified

Step E **Communication and appeal**

Communication and appeal are important features that enhance the legitimacy of decision-making by making the decision and underlying argumentation public, while the conditions of revision and enforcement establish responsiveness and accountability. The table at the end of this chapter provides an overview on how selected HTA bodies have organised communication and appeal

How should the outcome of the deliberation of the advisory committee be communicated?

The communication should allow any interested person to understand:

- What the decision was and what options or alternatives were considered;
- What facts were used and what the reasons were for taking into account certain criteria, or for excluding other criteria;
- Who was involved in making the decision as a member of the advisory committee and/or as stakeholder in the process;
- Who has written the decision;
- If and how the decision will feed into the policy process and relate to decision-making, either on macro (national), regional or local level. For example, how will the decision be translated into resource allocation via budgets, fiscal transfers, payment, reimbursement, product procurement etc.? Who will be responsible for this?

The communication needs to be well coordinated, systematic and well planned. This means that the responsible health authorities – typically the Ministry of Health – should strive to ensure that reimbursement decisions are communicated to all relevant stakeholders, using a variety of channels. This may include the use of official documents (e.g. official journals), websites of relevant organisations (at least from Ministry of Health itself), policy briefs, newsletters and news items on popular media to address the broader public. HTA bodies should liaise with the responsible health authorities to establish a protocol for communication of reimbursement decisions.

How should a formal mechanism for reviewing decisions and addressing disagreements be organised?

'Appeal' refers to the need for a mechanism that gives stakeholders the possibility to apply for a revision of a decision, or by providing (new) arguments or evidence and receive a reasoned response. Bodies should establish a protocol for appeal, such as the requirements on new evidence and clear revision rules.

It is important that the protocols for communication and appeal are explicitly documented and publicly available for reasons of transparency and legitimacy.

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Countries in the spotlight

United Kingdom An advisory committee of NICE summarises key evidence, their argumentation and their preliminary recommendation in 'appraisal consultation documents' (ACD), or 'evaluation consultation documents' (ECD) for highly specialised technologies. Consultees, commentators and the public may respond to ACDs. Comments are considered in a second committee meeting. The final recommendation to the NHS is described in the 'final appraisal determination' (FAD). An appeal can be lodged by any of the appraisal consultees. Once the FAD is issued, an appeal needs to be made within 15 working days. An appeal can only be made if: i) NICE has either failed to act fairly or exceeded its powers when making the assessment that preceded the recommendation, or ii) when the recommendation is unreasonable in light of the evidence submitted to NICE.

The Vice Chair of the Committee will decide if an oral or written appeal hearing will be held. The aim is to hold hearings within eight weeks of the end of the appeal period for oral hearing and ten weeks for written submissions. A panel is installed to hear the appeal and consist of persons approved by the Secretary of State for Health and Social Care. Each appeal panel consists of five members (a NHS representative, patient representative, representative of life sciences industry and a non-executive director of NICE), four of whom are independent of NICE. An external member chairs the panel; the chair should either be a patient/carer; engaged in the provision of NHS health care or experienced representative of patients/carers. An overview of health technologies for which appeals have been issued and an overview of the complete appeal process can be found on the NICE website.
Further information is available elsewhere.[54]

Scotland The HTA body, Scottish Medicines Consortium (SMC), has an appeal mechanism in place. Manufacturers of which the advice for a product was 'not recommended' can resubmit their applications when there is new evidence available and/or can request an independent review. SMC communicates their advice on new medicines via detailed advice documents (DADs) on their website and a press release each month. To increase the transparency and the public understanding of SMC decisions, the SMC has started to produce a 'Decision Explained' factsheet for each SMC appraisal. These factsheets are written for a lay-audience. They provide information about each medicine, indication, SMC decision and reason for the decision, along with signposting for further information and support. The 'Decision Explained' factsheet is published alongside the full guidance for each medicine and circulated to patient groups with an interest in the medicine.
Further information is available elsewhere.[55]

The organization of communication and appeal by selected HTA bodies

Indicator	BRAZIL	FRANCE	GERMANY	THAILAND
HTA agency	**National Committee for Health Technology Incorporation – CONITEC**	**Haute Autorité de Santé – HAS**	**Institute for Quality and Efficiency in Health Care –IQWiG**	**Health Intervention and Technology Assessment Program HITAP**
Evaluated committee	Plenary	Transparency Committee (TC)	Federal Joint Committee ('Plenum')	Subcommittee for Development of Benefit Package and Service Delivery (SCBP)
Communication strategy to inform stakeholders	No, only published on website.	Yes: sent to government, sponsor and published on website.	No, published on website.	Yes: adjusted to target audience (i.e. public, health professionals, researchers and patients). Recommendations are published in journals, magazines or other media. They are also sent to discussion groups and distribution lists.
Appeal mechanism	Yes: appeals to the secretariat's decision should be made within ten days starting from the date of publication in the official magazine. If appeals are accepted, there are hearings with the public.	Yes: industry may provide written comments or request a hearing within ten days of receipt of the draft recommendation.	Yes	Yes

Indicator	CANADA	UNITED KINGDOM	SCOTLAND	AUSTRALIA
HTA agency	**Canadian Agency for Drugs and Technologies in Health**	**National Institute for Health and Care Excellence**	**Scottish Medicines Consortium**	**Pharmaceutical Benefits Advisory Committee**
Evaluated committee	Canadian Drug Expert Committee (CDEC)	Technology Appraisals Committee	As above	As above
Communication strategy to inform stakeholders	Yes: all communications for drug review programmes are consolidated into a single email newsletter issued once per week.	Yes: the communications lead is responsible for circulating and communicating the guidance to appropriate groups within the NHS in England, to patients and the public. NICE also publishes a lay-version for patients and carers (known as 'information for the public').	No, once a decision is made, it is shared in confidence with NHS boards and the pharmaceutical company four weeks before it is published to ensure that Area Drug and Therapeutics Committees (ADTCs) can take steps to prepare for the introduction of the new medicine in health boards.	Yes
Appeal mechanism	Yes	Yes: all consultees have the opportunity to consider an appeal against the final appraisal determination and have the opportunity to report factual errors.	Yes: industry may request a meeting following publication of a not recommended advice for a full or resubmission.	Yes

Step F **Monitoring and evaluation**

HTA bodies are advised to carefully monitor and evaluate their processes and impact over time. This chapter provides practical support on how monitoring and evaluation (M&E) can best be organised.

What is M&E?

M&E concerns the process of systematically collecting data over time on a set of pre-defined indicators and, subsequently, using this data to judge if objectives are being achieved in line with expectations or if measures for improvement are required.

Why is M&E important?

Every HTA body has its own unique context in which EDPs are implemented. Data collected as part of M&E efforts ideally informs the HTA body about any shortcomings in terms of how their processes are being implemented and/or its overall impact and why this may be so. This enables the HTA body to be responsive to new insights and correct for potential shortcomings in a timely and proactive manner by implementing measures for improvement. Over time, this can enhance the legitimacy of the process by ensuring the body's continued responsiveness and accountability.

How should M&E be organised?

Defining the aims

HTA bodies are advised to focus their M&E efforts on two main aims. Firstly, M&E should provide information as to whether HTA processes are being implemented in line with expectations. Secondly, M&E should provide information on the overall impact of the HTA activities, understood in terms of tangible outputs, benefits and outcomes during or after EDP implementation and more long-term impacts.

Developing a theory of change

HTA bodies are advised to start by developing a theory of change that provides a comprehensive description of how and why a desired change is expected to happen following the implementation of EDPs in their context (Table 6). The theory of change should explain how the implementation of an EDP is expected to achieve desired impacts, in terms of used inputs (A) and activities (B), tangible outputs (C), outcomes (D) and more long-term impacts (E).

Defining and selecting indicators

Based on the theory of change, a set of indicators can be derived (Table 7). In this guide we provide a checklist (Annex 1) that describes the most important elements or indicators for each step of applying EDPs, which may serve as inspiration. When selecting indicators for use in M&E, it is important to keep in mind if a monitoring system is already in place for certain indicators or if a new set of indicators is required, as well as the likely costs of establishing the system required, capacity needs and whether findings are likely to be actionable.

Stakeholder involvement

The HTA body should ensure that a M&E plan is operational and described in a publicly available document and subject it to scrutiny by stakeholders. If stakeholders are not involved, HTA bodies risk compromising the legitimacy of their M&E and related recommendations.

Table 6. An example of a theory of change for EDP implementation

EDP implementation		Intended results		
A. Input *Resources used*	**B. Activities** *How the HTA process uses resources to deliver planned products and fulfil its mission*	**C. Output** *Tangible products*	**D. Outcome** *Benefits during or after implementation*	**E. Impact** *Long term benefits*
• Local expertise in HTA, data collection and analysis, health systems strengthening and clinical experience • Political will to initiate EDP implementation • Funding, time, convening capacity	• Establishing and implementing EDPs steps A–F with stakeholders • Development of publicly available documents describing steps A–F • Capacity building and provision of technical support and strengthening the capacity of decision makers and institutions	• Transparent and publicly available HTA report(s) and recommendations and/or decisions, detailing the used argumentation • Publicly available documents describing steps A–F	*Intermediate outcomes:* • Local ownership of outputs • Improved stakeholder understanding, satisfaction and acceptance of the HTA process *Final outcomes:* • Strengthened capacity and expertise in using EDPs • Increased political commitment • Implementation of decisions • Positive externalities	• Better health outcomes, improved equity and financial risk protection • Improved decision-making quality • Institutionalisation of HTA processes

Table 7. An example of M&E questions and indicators for EDP implementation*

EDP implementation		Intended results		
A. Input *Resources used*	**B. Activities** *How the HTA process uses resources to deliver planned products and fulfil its mission*	**C. Output** *Tangible products*	**D. Outcome** *Benefits during or after implementation*	**E. Impact** *Long term benefits*
Example M&E questions				
• What contextual opportunities and constraints are there?	• How appropriate and relevant are the used methodologies, tools and processes for meeting the objectives?	• What outputs are produced? • What is the quality and relevance of outputs? • Do outputs meet stakeholders' demands?	• How have outputs been used? • How well received are public outputs? • How do stakeholders view the effects and impact? • Are stakeholders satisfied with the used methodologies, tools and processes?	• To what extent have long-term results improved? • To what extent have decisions been implemented?
Example indicators				
• Government policies, regulations and practices that promote HTA/EDPs.	• Percentage of stakeholders that view the activities as relevant. • Percentage of stakeholders that view the activities as appropriate.	• Type and number of outputs produced. • Percentage of stakeholders that perceive outputs are of good quality. • Percentage of stakeholders that perceive outputs as relevant.	• Type and number of times stakeholders make references to outputs. • Number of people that are capable to support EDPs, per step. • The percentage of stakeholders that understand EDPs. • Percentage of stakeholders that are satisfied with EDPs.	• National equity indices • Burden of disease indicators • Percentage of people that have effective financial risk protection. • Number and nature of documented cases of shifts in policy and policy thinking. • Amount and percentage of public spending on the health sector.

* The table reports examples of M&E questions and indicators developed in the context of using EDPs for UHC benefit package design in Pakistan. HTA bodies are advised to develop their own theory of change and adjust the table according to local needs.

Further reading

Theory of change

- Norheim, OF. Disease Control Priorities Third Edition Is Published: A Theory of Change Is Needed for Translating Evidence to Health Policy. Int J Health Policy Manag 2018 Sep 1;7(9):771-777.
- Isaranuwatchai W, Li R, Glassman A, Teerawattananon Y, Culyer AJ, Chalkidou. Disease Control Priorities Third Edition: Time to Put a Theory of Change Into Practice Comment on "Disease Control Priorities Third Edition Is Published: A Theory of Change Is Needed for Translating Evidence to Health Policy". International Journal of Health Policy and Management, 2019, 8(2), 132–135.

M&E practical guidance

- Pasanen T, Shaxson L (2016) 'How to design a monitoring and evaluation framework for a policy research project'. A Methods Lab publication. London: Overseas Development Institute.
- Fretheim A, Oxman AD, Lavis JN, Lewin S. SUPPORT Tools for Evidence-informed Policymaking in health 18: Planning monitoring and evaluation of policies. Health Research Policy and Systems 2009, 7(Suppl 1):S18.
- McMasterUniversity. Public and Patient Engagement Evaluation Tool (PPEET). Available at https://ppe.mcmaster.ca/our-products/public-patient-engagement-evaluation-tool.

M&E example questions and indicators

- Culyer AJ, Lomas J. Deliberative processes and evidence-informed decision-making in health care: do they work and how might we know? Evidence and Policy, Vol. 2, No. 3, 08.2006, p. 357-371.
- Barasa EW, Molyneux S, English M, Cleary S. Setting Healthcare Priorities at the Macro and Meso Levels: A Framework for Evaluation. Int J Health Policy Manag 2015, 4(11), 719–732.
- Kapiriri L, Martin DK. Successful Priority Setting in Low and Middle Income Countries: A Framework for Evaluation. Health Care Anal (2010) 18:129–147.
- Kapiriri L. International validation of quality indicators for evaluating priority setting in low income countries: process and key lessons. BMC Health Services Research (2017) 17:418.
- Sibbald SL, Gibson JL, Singer PA, Upshur R, Martin DK. Evaluating priority setting success in healthcare: a pilot study. BMC Health Services Research 2010, 10:131.
- Waithaka D, Tsofa B, Barasa E. Evaluating healthcare priority setting at the meso level: A thematic review of empirical literature. Wellcome Open Research 2018, 3:2.

Countries in the spotlight

Scotland The Public Involvement Network (PIN) Advisory Group assists the Scottish Medicines Consortium in continuously evaluating how the SMC involves patients and the public and informs SMC of best practices in public involvement work. The PIN Advisory Group meets three times a year. Membership includes three patient group partners, each of whom has active contact with patients and carers and previous has experience with submitting to the SMC. One representative is nominated through each of the following umbrella organisations: Scottish Cancer Coalition, Alliance and Genetic Alliance UK. The group also includes all SMC public partners and some members of the SMC team, including an Area Drug and Therapeutics Committee representative and a clinical expert SMC Committee member.
Further information is available elsewhere.[56]

The organization of monitoring and evaluation by selected HTA bodies

Indicator	BRAZIL	FRANCE	GERMANY	THAILAND
HTA agency	**National Committee for Health Technology Incorporation – CONITEC**	**Haute Autorité de Santé – HAS**	**Institute for Quality and Efficiency in Health Care –IQWiG**	**Health Intervention and Technology Assessment Program HITAP**
Evaluated committee	Plenary	Transparency Committee (TC)	Federal Joint Committee ('Plenum')	Subcommittee for Development of Benefit Package and Service Delivery (SCBP)
Mechanism for monitoring and evaluation	Yes, at the request of MoH or related organisations.	Yes, if requested by stakeholders, ministry of health or HAS. Otherwise, every five years.	Yes	Yes
Stakeholder involvement in monitoring and evaluation	Not identified	Yes, with consultation	Yes, with consultation	Not identified

Indicator	CANADA	UNITED KINGDOM	SCOTLAND	AUSTRALIA
HTA agency	**Canadian Agency for Drugs and Technologies in Health**	**National Institute for Health and Care Excellence**	**Scottish Medicines Consortium**	**Pharmaceutical Benefits Advisory Committee**
Evaluated committee	Canadian Drug Expert Committee (CDEC)	Technology Appraisals Committee	As above	As above
Mechanism for monitoring and evaluation	Not identified	Yes	Not identified	Yes: specific medicines are monitored (e.g. through post-market reviews of Pharmaceutical Benefits Scheme Subsidised Medicines).
Stakeholder involvement in monitoring and evaluation	Not identified	Yes	Yes, with participation. The Public Involvement Network (PIN) is made up of patient and carer groups who have submitted evidence to SMC. PIN also has a core advisory group which works with SMC to continuously improve how we involve patients, carers and members of the public in our work.	Not identified

Annex 1 **Checklist on EDP implementation**

This checklist describes the important elements for each step of implementing EDPs. We advise HTA bodies to assess their HTA context and processes on the basis of this checklist, by assessing the extent to which these elements are in place. The checklist can be used to monitor progress over time or as a tool to identify areas for improvement.

Table 1: Checklist for Stakeholder Participation

Step	Element		Level of implementation (- / - + / +)
Context	Institutionalisation design	Legal and regulatory provisions stating the level of independence of the HTA body	
	Policy context	A policy statement on the willingness to use HTA in policy and/or practice	
		A (formal) mechanism or process to link HTA to policy making (e.g. legislation)	
		Involvement of stakeholders in HTA activities and/or decision-making process	
		Allocation of public funding to HTA on an annual basis	
	Capacity building and networking	Sufficient capacity to carry out HTA, including ability to review international literature	
		Access to international databases of scientific articles	
		Availability of HTA training opportunities	
		Availability of (inter)national networking strategy	

Step	Element		Level of implementation (- / - + / +)
Step A: Installing an advisory committee		Existence of an advisory committee for appraisal/HTA decision-making	
	Publicly available document describing:	The composition, terms and selection of members	
		The roles and responsibilities of the committee and its members	
		The approach followed by the committee	
		The approach followed to ensure meaningful stakeholder involvement in the HTA process	
Step B: Defining decision criteria		Existence of a list of specified decision-criteria	
		The criteria to be used for decision-making	
	Publicly available document describing:	Methods used (i.e. how are criteria derived from health system values)	
		Process for defining these criteria	
		Approach followed to ensure meaningful stakeholder participation	
Step C: Selecting health technologies for hta		Existence of a selection procedure for health technologies for hta	
		Existence of a horizon scanning system	
	Publicly available document describing:	Process of identification and selection of health technologies (i.e. methods, procedures and criteria)	
		Approach followed to ensure meaningful stakeholder participation	
		The roles and responsibilities of stakeholders involved	
Step D1: Scoping		Existence of a scoping procedure	
	Publicly available document describing:	The process of scoping (i.e. methods, procedures and criteria)	
		Approach followed to ensure meaningful stakeholder participation	
		Roles and responsibilities of stakeholders involved	

Step	Element		Level of implementation (- / - + / +)
Step D2: Assessment		Existence of an assessment protocol	
		Existence of a tool/template for reporting and summarising the (quality of the) evidence per relevant aspect as part of assessment	
	Publicly available document describing:	Assessment protocol in terms of evidence collection, analysis and reporting	
		Approach followed to ensure stakeholder consultation to review the plausibility of evidence reports	
Step D3: Appraisal		Existence of explicit appraisal process	
	Publicly available document describing:	Process of appraisal (i.e. methods, procedures and deliberation)	
		Approach followed to ensure meaningful stakeholder participation	
		The roles and responsibilities of stakeholders involved in the process	
Step E: Communication and appeal		Existence of a protocol for communication and appeal	
	Publicly available document describing:	The mechanism(s) for the communication of decisions and the underlying reasons to all relevant stakeholders	
		The mechanism(s) for appeal, how to propose revisions and to receive a reasoned response	
Step F: Monitoring and Evaluation		Existence of a protocol for monitoring and evaluation	
	Publicly available document describing:	Selected indicators, their definitions and operationalisations	
		Process to ensure responsiveness to new insights and accountability for any shortcomings that are revealed by the M&E	

Annex 2 **Checklist on stakeholder participation**

We advise HTA bodies to use a checklist on stakeholder participation. This checklist can assist them in the practical organisation of meaningful stakeholder participation throughout the EDPs and, in particular, during the appraisal step (D3).[57]

How should the checklist be used?

HTA bodies can use the checklist to evaluate their current stakeholder participation approach and to identify possible limitations of current processes and install mechanisms for improvement. The checklist is not meant to be all-encompassing or exhaustive. Rather, it is meant to cover key concerns and invoke reflection by health authorities on the most relevant and actionable choices they make. Answers to questions are context-specific and there is no decisive evidence on what constitutes 'right answers' to individual questions in the checklist. In some contexts, it may be reasonable to reimburse travel expenses for the sake of accessibility, for example, while in other cases this may be irrelevant or inappropriate. Nevertheless, HTA bodies are advised to inform their specific choices by evidence if available – or to learn from other countries' experiences. Finally, HTA bodies should take incremental steps by prioritising specific efforts according to local needs and affordances.

Table 1: Checklist for Stakeholder Participation

	Level of implementation (- / - + / +)
Identification of potentially adversely affected stakeholders	
1. Are efforts being made to identify those who experience a health loss as a result of a negative decision?	
2. Are efforts being made to identify those who experience a health loss as a result of a positive decision?	
3. Are efforts being made to identify those who are responsible for c ommunicating the decision?	
4. Are efforts being made to identify those who are responsible for implementing the decision?	
Comprehensive stakeholder inclusion	
1. Are all relevant stakeholders informed about the possibilities and procedures of participation?	
2. Is participation organised in a way that effectively and efficiently facilitates the inclusion of stakeholders?	
3. Are efforts being made to include all relevant, especially difficult-to-reach, stakeholders?	
4. Can stakeholders participate in the identification and selection of health technologies for HTA?	
5. Can stakeholders participate in the scoping of relevant questions for evaluation?	
6. Can stakeholders participate in the development of recommendations (assessment and appraisal)?	
7. Can stakeholders participate in the evaluation of decisions?	
8. Are alternative non-participatory strategies being used for the inclusion of stakeholders' values?	
Meaningful stakeholder participation	
1. Are stakeholders informed fully and in time about the available evidence?	
2. Is argumentation and evidence presented in a way that is understandable to all relevant stakeholders?	
3. Can stakeholders freely voice their perspectives (i.e. no stakeholder is allowed to dominate a discussion or activity)?	
4. Are stakeholder perspectives addressed in respectful and courteous ways?	
5. Do stakeholders have sufficient time to provide input?	
6. Are stakeholder perspectives equally accounted for in the deliberation?	
7. Is it clear to all stakeholders involved how their input is going to be c onsidered, scrutinised and put to use?	
8. Can stakeholders actively interact in the deliberation?	
9. Is further evidence collection considered when judged relevant and feasible?	
Transparent communication of recommendations and/or decisions	
1. Is information provided about the underlying argumentation and process used to come to a recommendation and/or decision?	
2. Is input from stakeholders being documented and explicitly addressed?	
3. Are recommendations and/or decisions clearly communicated?	
4. Are stakeholders informed in time about recommendations and/or decisions?	
Appeal and evaluation	
1. Can stakeholders easily make an appeal on the underlying argumentation or process?	
2. Are appeals documented and publicly accessible?	
3. Are appeals handled consistently and is justification provided in an understandable way?	
4. Are mechanisms in place to revise decisions the processes based on appeals?	

Annex 3 **The DCP Pakistan project**

The Disease Control Priorities 3 (DCP3) project provides long-term support to countries with the development and implementation of their UHC benefit packages (UHC-BP). DCP3 responds to the increasing need of LMICs for technical guidance and support in benefit package design and in accelerating progress towards UHC. Pakistan is one of the first countries globally to implement the project.

This annex reports on the priority setting process used in the development of the UHC-BP in Pakistan during 2019–2020, employing EDPs.

Institutional context

The priority setting process was implemented by the Health Planning, System Strengthening and Information Analysis Unit (HPSIU) of the Ministry of National Health Services Regulations and Coordination (MNHSRC), referred to as the UHC-BP secretariat. Partners in the project included the Community Health Sciences Department of Aga Khan University (AKU) and Health Services Academy (HSA), London School of Hygiene and Tropical Medicine (LSHTM), World Health Organisation (WHO) and Radboud university medical center (Radboudumc).

Operationalisation of the EDP framework

We operationalised the six steps of the EDP framework (Figure 1) for implementation in Pakistan during two separate workshops at Radboudumc, in the Netherlands, with participants from all project partners (October 2019 and February 2020). The focus in the present project is to implement steps A–D; steps E–F are to be implemented in a subsequent stage. All procedures, templates and instructions were pilot tested. Training courses for facilitators were organised prior to implementation at the UHC-BP workshops in Islamabad, Pakistan (November 2019 and February 2020).

Figure 1: The six steps of the EDP framework as adapted to the context of the DCP-3 Pakistan project

The rationale of using EDPs is to improve the decision process of the development of the UHC-BP in Pakistan in terms of its quality (by taking into account all relevant stakeholder values, supported by evidence and making appropriate trade-offs between them), consistency (by repeatedly considering the same values) and transparency (by being explicit on the selection of values and the performance of services on these values). With all of these aspects combined, the legitimacy of decisions may ultimately be improved.

Step A: The installation of advisory committees

Supported by DCP partners, the UHC-BP secretariat designed a governance structure for the UHC-BP, based on three connected stages of deliberation around several specific priorities.

The first stage involved four technical working groups (TWGs) for specific disease areas: reproductive maternal neonatal child and adolescent health, non-communicable diseases, communicable diseases and health services access. These TWGs already were already in place to advise the Ministry on different areas and represent relevant stakeholders. TWGs were tasked with reviewing the technical aspects of the services for potential inclusion and broadly allocating services into priority categories, with each TWG covering between 28–51 services. The second stage involved the set-up of a National Advisory Committee (NAC), whose mandate is to interpret the recommendations of the TWGs. The NAC had 90 members, including stakeholders representing societal interests, development partners and provincial representation, and one representative from each of the TWGs, in order not to be dominated by any specific disease/service area interest. The third stage involved initiating a high-level Steering Committee (SC) responsible for reviewing the NAC recommendations and approving or revising them. Terms of reference were drafted and adopted for each entity in the structure. Conflict of interest forms were designed and used for TWG and NAC members. The governance structure was endorsed by the SC.

Step B: Defining decision criteria

The Ministry conducted a survey on decision criteria to develop consensus on the importance and definition of criteria for the prioritisation of services for use by TWG and NAC members. It was sent electronically to all TWG and NAC members invited for the November meeting, and 52 members responded (response rate 52%). The following criteria were selected: effectiveness, health gain for money spent, avoidable burden of disease, equity, financial risk protection, budget impact, socio-economic impact and feasibility.

Step C: Selecting services
The Ministry, together with the provincial department of health and key stakeholders, compared the current scope of Essential Health Services in Pakistan against the services covered by the DCP3 Essential UHC package (EUHC) (a model benefit package for UHC which LMICs are recommended to consider for the development of their own health benefit packages). Participants concluded that 169 out of 219 (77%) recommended that EUHC services should be assessed for inclusion in the UHC-BP, while others may be included at a later stage. None of the services that were currently provided in Pakistan were omitted at this stage. Thereafter, the identified DCP3 services were further defined in terms of process and resource use by the UHC-BP secretariat, and reviewed by TWG members before and during the UHC-BP workshops.

Step D1: Assessment
In the EDP framework, step D1 is usually 'Scoping'. However, this was not part of the project in Pakistan and therefore omitted here. The UHC-BP secretariat collected evidence on three criteria: cost-effectiveness, budget impact and avoidable burden of disease. No evidence was collected for other criteria selected by TWG members, and these were assessed during the appraisal stage using expert judgments only.

Step D2: Appraisal
The appraisal step involved the complex trade-off across the three UHC dimensions and was split into two sub-steps.

Appraisal sub-step i – division of health services into priority categories
The first sub-step involved the division of the 169 services into categories of 'high priority', 'medium priority' and 'low priority', reflecting the relative value of services for the health system in Pakistan and its importance for implementation. To this end, the TWGs interpreted the results of the assessment stage and deliberated in two meetings in November 2019 (on community and primary care services, involving 130 TWG members) and in February 2020 (on first and tertiary level hospital care, involving 74 TWG members).

Each meeting started with an introduction of the process, followed by the actual TWG deliberations to prioritise health services. Each TWG was allocated a trained facilitator, who received instructions to follow a stepwise deliberative process. A rapporteur recorded the arguments that participants put forward and their votes in a 'rapporteur notebook'. TWG participants received an argumentation notebook to record their own votes and argumentation. The evidence collected for each of the health services in relation to three of seven criteria was summarised in 'evidence sheets'; a 'criteria explanation sheet' was also produced with definitions of each of the criteria, phrased for laypeople. Immediately after the TWG stage, rapporteurs were asked to populate a 'health service reporting sheet' for each service and summary presentation slide. The NAC subsequently reviewed TWG recommendations and amended these where necessary.

Appraisal sub-step ii – making choices among high priority services
Subsequently, the NAC had the complex task to further prioritise the list of high priority services within the available fiscal space, taking into account coverage and co-payment levels, and considering complementary investments in the health system. To inform these decisions, the UHC-BP secretariat prepared evidence on various packages with alternative assumptions on fiscal space, coverage levels and co-payment levels; and taking into account the appropriate time horizon of NAC recommendations (0–10 years). Some packages also represented specific trade-offs (e.g. explicitly prioritising high priority community health services). The NAC developed recommendations for preferred packages and these were presented to the SC for their approval upon consultation with the IAG.

The evidence-informed priority setting process in Pakistan was part of the Disease Control Priorities Project : Translation of DCP3 in priority countries which was a collaboration between the London School of Hygiene and Tropical Medicine, Pakistan's Ministry of National Health Services Regulation and Coordination, and Radboud MC, funded by the Bill & Melinda Gates Foundation". This work is being published as: Baltussen R, Jansen M, Akhtar S, Bijlmakers L, Vassal A, Zaidi R, Siddiqui S, Alwan A et al. The use of evi-dence-informed deliberative processes for designing the universal health coverage benefit package in Pakistan.

Notes

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HTA-informed decision-making processes. International Journal of Technology Assessment in Health Care, 2020'; 36(2): 87-95. doi:10.1017/S0266462320000094

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9 https://www.who.int/healthsystems/universal_health_coverage/en/

10 Daniels N. Accountability for reasonableness. BMJ 2000;321:1300–1

11 Glassman A GU, Smith PC. What's In, What's Out: Designing Benefits for Universal Health Coverage. 2017. Published by: Brookings Institution Press, Center for Global Development, Washington DC, United States

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14 The HTA Toolkit of the international Decision Support Initiative (iDSI), 2018. Available from http://www.idsihealth.org/HTATOOLKIT/

15 Castro H, Suharlim, Kumar R. Moving LMICs toward Self-Reliance: A Roadmap for Systematic Priority Setting for Resource Allocation.

16 Throughout the guide, the term 'country' also reflects 'regions', while the term 'HTA body' also reflects formal HTA units.

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22 World Health Organization, Bertram, Melanie, Dhaene, Gwenaël & Tan-Torres Edejer, Tessa. (2021). Institutionalizing health technology assessment mechanisms: a how to guide. World Health Organization. https://apps.who.int/iris/handle/10665/340722.

23 Contemporary HTA is mainly focused on assessing the properties of health technologies. The criteria traditionally include: level of clinical benefit, safety, quality of the evidence, and in some cases a measure of the incremental cost-effectiveness. Recently, also indirect or unintended outcomes (e.g. potential benefits and harms for patients and their caregivers), as well as ethical, social-cultural, legal, and organisational issues are considered.

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Countries around the world are increasingly rethinking their health benefit packages as a way to achieve universal health coverage. They rely largely on criteria like ‘effectiveness’ and ‘cost-effectiveness’, but lack guidance on how to capture a broader range of relevant values and stakeholders’ perspectives.

This guide on evidence-informed deliberative processes (EDPs) fills in that gap. It provides a practical stepwise approach for HTA bodies to improve the legitimacy of their decision-making process. It brings together relevant theories and best practices from HTA bodies around the world.

EDPs involve several steps where stakeholder involvement plays a key role: installing an advisory committee, defining decision criteria, selecting health technologies for hta, scoping, assessment, appraisal, communication and appeal, and monitoring and evaluation. The guide is organised by providing guidance on 10–15 essential questions in each of these steps.

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