Evidence-informed deliberative processes

A practical guide for HTA agencies to enhance legitimate decision-making
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Colophon

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**Copies of this guide can be downloaded from** https://www.radboudumc.nl/revise-h ta

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### Glossary

The definitions provided here are mainly derived from the international HTA glossary, as well as (glossaries of) relevant toolkits.

<table>
<thead>
<tr>
<th>Term</th>
<th>Definition</th>
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<tbody>
<tr>
<td><strong>Cost-effectiveness analysis</strong></td>
<td>An economic evaluation consisting of comparing various options, in which costs are measured in monetary units, then aggregated, and outcomes are expressed in natural (non-monetary) units.</td>
</tr>
<tr>
<td><strong>(Clinical) Effectiveness</strong></td>
<td>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.</td>
</tr>
<tr>
<td><strong>Deliberation</strong></td>
<td>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.</td>
</tr>
<tr>
<td><strong>Evidence-informed deliberative processes</strong></td>
<td>An approach for guiding legitimate decision-making based on deliberation between stakeholders to identify, reflect and learn about the meaning and importance of values, informed by evidence on these values.</td>
</tr>
<tr>
<td><strong>Health technology</strong></td>
<td>An intervention developed to prevent, diagnose or treat medical conditions; promote health; provide rehabilitation; or organize healthcare delivery.</td>
</tr>
<tr>
<td><strong>Health technology assessment</strong></td>
<td>A multidisciplinary process that uses explicit methods to assess the value of using a health technology at different points in its lifecycle. The process is comparative, transparent and involves multiple stakeholders. The purpose is to inform health policy and decision-making to improve patient-relevant outcomes.</td>
</tr>
<tr>
<td><strong>Horizon scanning</strong></td>
<td>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 affect health, health services and/or society.</td>
</tr>
<tr>
<td><strong>Institutionalization</strong></td>
<td>The embedding of certain rules and norms, and associated actions and processes, within a health system.</td>
</tr>
<tr>
<td><strong>Multi-criteria decision analysis</strong></td>
<td>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.</td>
</tr>
<tr>
<td><strong>Priority setting</strong></td>
<td>The assignment of an order of priority based on explicit or implicit criteria for selection of health technologies for assessment.</td>
</tr>
<tr>
<td><strong>Quality-adjusted life year</strong></td>
<td>A unit of outcome of an intervention where gains (or losses) of years of life subsequent to this intervention are adjusted on the basis of the quality of life during those years.</td>
</tr>
<tr>
<td><strong>Safety</strong></td>
<td>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.</td>
</tr>
<tr>
<td><strong>Stakeholder</strong></td>
<td>A person, group or organization that has interest or concern in an organization.</td>
</tr>
<tr>
<td><strong>Stakeholder involvement</strong></td>
<td>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.</td>
</tr>
<tr>
<td><strong>Systematic (literature) review</strong></td>
<td>A synthesis that collates all empirical evidence fitting pre-specified eligibility criteria in order to answer a specific research question.</td>
</tr>
<tr>
<td><strong>Universal health coverage</strong></td>
<td>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.</td>
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</table>
Introduction

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 governments to put universal health coverage (UHC) high on the health agenda. Health technology assessment (HTA) is seen as an important policy tool on the path towards achieving UHC, as it guides governments in their choices on the public funding of health technologies.

Many countries have organized its HTA activities in formal HTA agencies. This guide is intended to support the decision-making process of these agencies, but is also relevant for countries that have not (yet) established such an agency.

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

Why a guide to enhance legitimate decision-making?
Priority setting in health care has long been recognized as an intrinsically complex and value-laden political process that takes place in an environment of diverging social values and interests. Society, including relevant stakeholders, such as patients, providers, insurers, and citizens, has a wide range of social values and interests that result in different perceptions of what makes health technologies valuable. In such pluralist societies, stakeholders may reasonably disagree on what values can be used to guide priority setting.

However, present value frameworks currently employed by HTA agencies around the world do not sufficiently account for this complex reality. These frameworks are typically based on the use of predefined key principles, also labelled ‘substantive’ criteria, which are believed to reflect the most important social values. This has led HTA agencies to use, for example, ‘clinical benefit’, ‘safety’ and ‘cost-effectiveness’ as important decision criteria.

There is broad recognition that such frameworks are ill fitted to take into account the wide range and diversity of stakeholder values and lead to insufficient sets of information. Ethical issues in particular are left unaddressed, thereby compromising the legitimacy of eventual decisions. Legitimacy here refers to the reasonableness, or fairness, of recommendations as perceived by stakeholders, which is an important prerequisite for broad societal support for these recommendations. For example, the decision whether or not to publicly fund expensive drugs for third-line antiretroviral therapy (ART) should include the interests of HIV patients (wanting to receive the best treatment), other patients (whose treatment may be displaced in case ART is funded), or tax payers (wanting to minimize public health expenditure).

The use of Evidence-informed deliberative processes
We propose the use of evidence-informed deliberative processes (EDPs) as an alternative approach for HTA agencies to explicitly address this issue of legitimacy. The aim of EDPs is to develop a shared basis for decision-making among stakeholders. Its use has far reaching consequences for the organization of HTA processes. HTA should be considered as a process in which stakeholders participate in order to identify criteria for the selection of technologies and assessment, to interpret forthcoming evidence, and to deliberate on recommendations and decisions.

Deliberation is defined as “the critical examination of an issue involving the weighing of reasons for and against a course of action”. This guide presents practical guidance on the use of EDPs.

EDPs are used as framework in the guidance on HTA of the World Health Organization (WHO) and employed by health authorities in Indonesia,13 Iran and Kazakhstan. Its principles were used to design the health benefit package in Thailand14 and the Netherlands.15

How to use this guide?
We introduce the conceptual framework of EDPs in the next chapter. The framework involves several steps including ‘installing an advisory committee’, ‘defining decision criteria’, ‘selecting health technologies for HTA’, ‘scoping’, ‘assessment’, ‘appraisal’, and ‘communication and appeal’. In a series of chapters, we provide practical guidance to HTA agencies to conduct each of these steps. In addition, per step, we provide selected examples from HTA agencies around the globe that may serve as inspiration for others16, and we refer to key publications. In Annex 1, we provide a checklist for HTA agencies to determine the level of implementation of EDPs in their country, and identify areas to improve on this. Annex 2 provides a checklist on stakeholder participation.

The remit, scope and purpose of an HTA agency determine which steps are relevant. For example, the selection of health technologies for HTA may only be relevant for HTA agencies who have responsibility for this.

This is the first version of a practical guide on EDPs. It is based on earlier work in this area, existing checklists, relevant publications on good HTA practices, additional literature search via PubMed, and a survey among members of the International Network of Agencies for Health Technology Assessment (INAHTA). We will continue to update this guide.

Country support
We carry out projects to support countries in the implementation of EDPs. This may involve workshops, short-term consultancy, or long-term collaboration. Please contact us for more information (contact details are in the colophon).
Evidence-informed deliberative processes

Evidence-informed deliberative processes reflect our vision on how HTA agencies should ideally organize their processes to achieve legitimate decision-making. It is based on the one hand on stakeholder involvement to identify, reflect, and learn about the meaning and importance of each other's interests and values (to foster fair decision-making as reflected in the accountability for reasonableness approach). On the other hand, it is based on rational decision-making through evidence-informed evaluation of identified relevant values (as reflected in multi-criteria decision analysis). Evidence-informed evaluation allows contributions from stakeholders in terms of their experiences and judgments when stronger evidence is unavailable.

Practical steps

We have translated these principles into a set of practical steps, in order to support HTA agencies as much as possible in the development of their processes. We thereby distinguish the decision-making context, and steps A-D in the use of EDPs for HTA agencies (Figure 1). The decision-making context is essential to any conduct and use of HTA, and it is important to consider its key elements to facilitate the implementation of EDPs. In step A, we advise an HTA agency to install an advisory committee, including the organization of stakeholder involvement. In step B, we advise to define decision criteria that reflect the health system values. These steps are undertaken every 3-5 years. In step C, we advice to set up a process for identifying and selecting health technologies for HTA, every 1-3 years.

Steps D1-D4 relate to undertaking HTA on a specific health technology.

While the steps are presented as separate activities and in a linear fashion, in practice there is often iteration between them to ensure useful and relevant HTA. More specifically, we present assessment and appraisal as separate activities. In reality, however, this distinction is arbitrary as both steps require value judgments. We wish to emphasize that assessment of health technologies does not only involve facts, but also value judgments.

The use of EDPs may support HTA agencies in three ways. First, it may improve the quality of their recommendations by taking into account all relevant stakeholder values and by making appropriate trade-offs between them. Second, it may improve the consistency of recommendations by repeatedly considering the same values. Third, it may improve the transparency of recommendations by being explicit on the selection of values and the performance of health technologies with regard to these values. Together, this ultimately improves the legitimacy of their processes.

Figure 1. The EDP framework
Further reading

Evidence-informed deliberative processes

Good practices in HTA

Understanding the context

The use of evidence-informed deliberative processes is strongly dependent on its embedding in the context of an HTA agency. Although this guide does not cover specific guidance on the development of HTA agencies, it is important to consider its key elements for using EDPs. These include the linkage between HTA and policymaking, the level of institutionalisation, and capacity building and networking for HTA (Table 1).

We advice HTA agencies to identify and describe the level to which these elements are present in their country, and to undertake action to optimize these elements where needed. The list of elements can also be used to monitor the progress of HTA development over time. This has, for example, recently been done in China.

Table 1. Key elements to facilitate the implementation of EDPs

<table>
<thead>
<tr>
<th>Existence of linkage between HTA and policy making</th>
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<tbody>
<tr>
<td>A policy statement on the willingness to use HTA in policy and/or practice</td>
</tr>
<tr>
<td>A (formal) mechanism or process to link HTA to policy making (e.g. legislation)</td>
</tr>
<tr>
<td>Allocation of public funding to HTA on an annual basis</td>
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</tbody>
</table>

<table>
<thead>
<tr>
<th>Level of institutionalization</th>
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<tbody>
<tr>
<td>An independent organizational structure and/or institutional set-up for HTA (agency) with a clear remit</td>
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<table>
<thead>
<tr>
<th>Capacity building and networking</th>
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<tbody>
<tr>
<td>Sufficient capacity to carry out HTA, including medical disciplines, public health specialists, epidemiologists, statisticians, psychologists, biomedical engineers and/or economists</td>
</tr>
<tr>
<td>Ability to review international literature (i.e. access to databases), including expertise in searching the internet</td>
</tr>
<tr>
<td>(Domestic) HTA training opportunities (short courses, workshops, Master programmes and PhD training)</td>
</tr>
<tr>
<td>An (international) networking strategy for collaboration between HTA agency(ies) and relevant stakeholders</td>
</tr>
</tbody>
</table>
Further reading

Linking HTA and decision-making


Key elements for the development of HTA

- Strengthening expertise for health technology assessment and priority-setting in Africa. Global Health Action, 10: 11-10

Step A Installing an advisory committee

HTA agencies are recommended to install an advisory committee, which has a central role throughout the HTA process (Steps B-D). Most formal HTA agencies already have such a committee in place, which is also often referred to as appraisal committee or expert committee.

Composition

An advisory committee often consists of several permanent members reflecting the broad public interest, and who take the responsibility of developing recommendations. Permanent members are typically installed for a period of 3-5 years, after which others replace them.

Stakeholder involvement

The role of stakeholders requires special attention. Stakeholders are often categorized as the 7Ps: patients and the public, providers, purchasers, payers, policy makers, product makers (industry), and principal investigators (academia). We define involvement 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, making relevant, transparent, and effective decisions.”

It is known that involvement of stakeholders in the process may improve the relevance of HTA questions, increase the transparency of HTA activities, and enable the adoption of evidence into policy and practice.

Ideally, stakeholders take part of the advisory committee as temporary members, with membership specific to the health technology under assessment. All stakeholders should have equal opportunities to contribute in this committee. However, an advisory committee may include members with different levels of knowledge and expertise, which shape their contributions. For example, experts often express their beliefs based on research and politics, whereas patients are often more focussed on experiences encountered in daily practice. The provision of balanced, factual information is important to reduce differences in knowledge level among members. In addition, members may undergo social pressure to conform to the wishes of the group and by means of doing so settle for less satisfying results. Also, a group decision may be forced or pressed through by one dominant member. The chairperson has an important task to control for these aspects.

Yet, in many countries, meaningful stakeholder participation is non-existent or in its infancies. We advise HTA agencies to organise stakeholder participation, supplemented with other strategies to involve stakeholders where relevant, e.g. through consultation.

Tasks and mandate

The tasks of an advisory committee depend on its mandate. In most HTA agencies, it is involved in the appraisal of health technologies (step D3) – it then acts as a jury, interpreting the collected evidence. In some countries, the committee develops recommendations, e.g., for the Ministry of Health. In other countries it is responsible for taking decisions. However, the advisory committee, as well as other relevant stakeholders, can also be involved in other activities of the HTA process, including the selection of health...
Importance of deliberation
The advisory committee needs to make various judgements throughout the HTA process, for example, in the appraisal of health technologies. This involves both analytical judgements (such as including surrogate endpoints as a source of evidence) and social judgements (such as weighing the value of a life year gained in very young or old persons). Deliberation is a way to facilitate this judgement process, and aims to create a more coherent and mutual understanding amongst members of their preferences regarding decisions. Deliberation is, in principle, governed by mutual sharing of perspectives and respect for diverging views. The chairperson of the committee, or an external facilitator, has an important role here. Throughout the decision-making process, (s)he should identify preferences and underlying beliefs of all committee members, confront others with these and work towards a joint problem definition. (s)He should check continuously if all members still are discussed.

Structured decision-making process
An advisory committee requires a structured decision-making process to take into account the above-mentioned aspects in e.g. the question, or the appraisal of health technologies. We advice to use a systematic process that allows all committee members to express their preferences and considerations. This can be achieved by starting to ask all committee members individually to express their preferences and considerations. In a subsequent group phase, all individual contributions can be mentioned elements in a publicly available document.

An advisory committee needs to reach a conclusion on its recommendation or decision. This can be achieved by majority voting but is, for the sake of legitimacy, ideally 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 is in the overall best interest of society.’ For reasons of transparency and legitimacy, it is important to clearly describe the above-mentioned elements in a publicly available document.

Further reading
Stakeholder involvement

Public and patient involvement in HTA

Country examples

Brazil
The remit of the National Committee for Health Technology Incorporation (CONITEC), created by law, is to recommend the Minister of Health about inclusion or disinvestment of health technologies regarding the Brazilian Public Health System (SUS). CONITEC represents 13 stakeholders, including different secretariats of the Ministry of Health, as well as representatives of state and municipality health secretaries, regulatory agencies, national health council, and health professionals. The HTA appraisal process is becoming more transparent, due to the introduction of a mandatory public consultation (for scientific-technical contributions and for patients or caretakers), and an optional public hearing as part of the process. Further information is available elsewhere.

United Kingdom
The National Institute for Health and Care Excellence (NICE) is the HTA agency for England. The technology appraisal committee that operates independently of NICE comprises of 20-25 members from diverse backgrounds (health professionals working for the National Health Service (NHS), academia, industry) and also includes patients and carers. Members are recruited through advertisement, and are, once selected by NICE, appointed for a term of three years. Names can be found on the NICE website. Its role is to appraise the evidence gathered in the assessment phase, and to provide NICE with a recommendation on whether and how the (new) health technology should be used within the NHS. Further information is available elsewhere.

Canada
The Canadian Agency for Drugs and Technologies in Health (CADTH) is an example of an HTA agency that has several specialized appraisal committees. CDEC is the advisory body for the Common Drug Review (CDR) process, and consists of fourteen members. Two of them are public members, the rest are experts in disease management and evaluation of pharmaceutical products such as physicians, economists, and pharmacists. Recommendations are made to each participating federal, provincial, and territorial publicly funded drug plans, regarding the listings on their formularies. The main responsibility regarding HTA is to support CADTH in selecting and developing tools that optimize prescribing and pharmaceutical products use.

pERC is the advisory body for the pan-Canadian Oncology Drug Review (pCODR) process and provides funding recommendations by assessing the clinical evidence and cost-effectiveness of cancer drugs for the provinces. The committee consists of 13-17 members, including oncologist, physicians, health economists, a haematologist and patient representatives.
Step B Defining decision criteria

In this step, the advisory committee selects relevant criteria for the assessment and subsequent appraisal of health technologies. These criteria should reflect the broad goals of the country’s health system (often defined 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 should first identify these broad goals and values, and then specify them into a list of decision-making criteria. We hereby distinguish two types of criteria.

Generic decision criteria
First, HTA agencies often use a number of explicit criteria for the evaluation of every technology. We label these criteria as ‘generic criteria’, as they are of generic relevance across technologies and should be used consistently.

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

- Cost-effectiveness as a criterion is important because it maximizes the health benefits in the population, given the budget. Ignoring cost-effectiveness and spending the budget on cost-ineffective technologies would have substantial opportunity costs in terms of healthy life years foregone.
- Equity, or priority to the worse-off, is important because it captures the value of fairness, which calls for providing technologies on the basis of need and reducing unfair inequalities. The worse-off can be defined as: a) those with least health (or the most severe conditions) without the technology, or b) the poorest or otherwise disadvantaged (gender, area of living, or marginalized groups). Since the most cost-effective services do not always benefit the worse-off, reimbursement decision may sometimes consider whether health benefits for the worse-off could be assigned extra value. In practice, this will imply that some technologies that are not considered cost-effective may still be reimbursed because they promote a fairer distribution of health and access to healthcare. The best way to trade-off criteria is discussed in step D3.
- Financial risk protection may also be considered important because some technologies may cause substantial out-of-pocket expenditure 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. Again, how to make these trade-offs is discussed in step D3.

In addition, still other decision criteria may be found 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’).

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 e.g., “responsibility for own health” for technologies targeting behaviour-related diseases such as smoking, or “impact on caregivers” for technologies related to home-based care. Since these contextual criteria can be many, we advise HTA agencies to develop a checklist of all potentially relevant criteria (possibly based on past evaluations or existing lists of decision-criteria). The advisory committee should systematically use this checklist in every appraisal to verify if all possibly relevant criteria are considered, to safeguard the consistency of evaluations across technologies.

Process of criteria selection
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, and not all advisory committee members may have the capacity to perform this without further training. For this reason, HTA agencies may choose to organise a workshop that contributes to the development of a good shared understanding of the relevant health system values and how (generic and contextual) decision criteria can be specified.

In the workshop, committee members are asked to develop recommendations for a series of health technologies and requested, for every technology, to list the criteria they find relevant. They may first do so individually, and then embark on a group discussion – this task will 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. In a next step, these decision criteria need to be operationalized, i.e. need to be meaningfully defined so that it is clear for committee members and the public what is meant by a specific decision criterion.

Stakeholder involvement
The HTA agency should subject their final list of specified decision-criteria to public scrutiny by means of a democratic process. If stakeholders are not involved, HTA agencies risk compromising the legitimacy of their specified criteria and any forthcoming recommendations. For public and patient involvement in HTA, we refer to step A (Installing an advisory committee).

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


Examples of decision criteria used in policy making


Examples of decision criteria based on surveys


Country examples

The Netherlands In 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 agency (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 proved difficult to be understood. The interpretation of the criterion necessity was for example used differently in coverage decisions, as testified for example 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 the recognition by ZIN of the need to operationalise the necessity criterion, as well as a public debate regarding the reimbursement of expensive drugs (for rare diseases) and about how to address social values in coverage decision-making.

Further information is available elsewhere.

Sweden The Health and Medical Services Act describes the goals of the Swedish health system, and the so-called ‘ethical platform’ has translated these into principles to guide national and local health decisions. The principles include human dignity (all individuals have equal value), which precedes the principle of needs and solidarity (resources should be primarily allocated to 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 they are obliged to consider these ethical principles. However, the use of these principles is not always transparent in practice: although the government made clear that severe diseases and significant impairments in the quality of life should be prioritized, even at a higher cost for society, it is not clear how high those costs may be. In addition, the New Therapies Council develops recommendations to 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.
Step C Selecting health technologies for HTA

Because of the rapid development of health technologies on the one hand, and budget constraints on the other hand, countries increasingly feel the need to conduct HTA. However, HTA agencies only have limited capacity, and they need to make important choices which health technologies to assess given their budget. The advisory committee has a central role in the identification and selection of technologies for HTA, and should establish a work programme every 1-3 years. This selection process is different for reimbursement and disinvestment decisions.

Selection of health technologies in need for HTA for reimbursement decisions

This selection process involves three steps.

In the first step, HTA agencies or related organisations should employ horizon scanning to systematically identify new and emerging health technologies likely to have a significant impact on health care. Organizations, which can be HTA agencies, can scan and monitor various health information sources to identify promising technologies not yet widely used in their health system. Horizon scanning is frequently done in high-income countries, and increasingly 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 2-3 years time horizon for scanning health technologies. Alternatively, HTA agencies can also invite stakeholders to nominate technologies for HTA (e.g. in Thailand).

In the second step, the advisory committee should use the information from the horizon scanning and should ask the question ‘which of the identified health technologies are most important from a societal perspective?’ Importance should be interpreted here as the impact that a health technology has on society. To answer this question, we advice the committee to employ criteria that reflect societal impact such as potential population health benefits; potential budget impact and potential 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 be in need for HTA.

Finally, the advisory committee should ask the question ‘which of these health technologies need to be prioritised for HTA?’ In other words, is the required evidence to make a decision already available, or is more evidence collection needed regarding 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) in combination with a large target population. This step results in a list of technologies with potential high impact 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 or may not be in need for HTA.

Finally, the advisory committee should ask the question ‘which of the identified health technologies need to be prioritised for HTA?’ In other words, is the required evidence to make a decision already available, or is more evidence collection needed regarding 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).

Selection of health technologies in need for HTA for disinvestment decisions

The selection process for disinvestment decisions also involves three steps.

In the first step, HTA agencies 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 on the basis of 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. Alternatively, HTA agencies may consider the whole benefit package as the basis for the next steps.

In the second step, the advisory committee 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 advice the committee to employ criteria that reflect societal impact such as budget impact, or potential (potential) population health benefit (which combines clinical effectiveness and size of target population).
Further reading

**Horizon scanning**


**Selection of health technologies in need of HTA**

- The Galician Health Technology Assessment Agency (avalia-), a member of the Spanish horizon scanning (GENTEC) network, has developed the PriTEC prioritization tool for prioritizing health technologies for assessment. The tool is available from: http://priectools.es/index.php?idiom=es

**Criteria for priority setting of health technologies for HTA**


**Country examples**

**Brazil** In Brazil, the National Committee for Health Technology Incorporation (CONITEC) is involved in horizon scanning. The objective is to predict which technologies have the potential to impact on health care in the Public Health System of Brazil. The sources for the identification of new technologies include clinical trial databases as well as commercial pharmaceutical databases, websites on registrations and licensing, scientific and grey literature.

CONITEC prioritizes health technologies for HTA that can be introduced at affordable cost for the health system, but also have a favourable impact on clinical practice, on service organization and on the social and ethical aspects related to their use. CONITEC produces so-called Alerts (concise information on a single technology in 6-8 pages) and Briefs (deeper analysis of a theme, consisting of 20-40 pages).

The Brazilian Ministry of Health sees horizon scanning also as a potential tool for proactively identifying health technologies for re-assessment in the context of disinvestment, and for the HTA process in general, by avoiding the evaluation of a health technology that could be replaced in the short-term. CONITEC has been supported by members of EuroScan, an international network on horizon scanning/early warning systems, making use of the EuroScan Toolkit.

Further information is available elsewhere.20

**United Kingdom** The National Institute for Health Research (NIHR) Innovation Observatory (IO), an independent research team based at Newcastle University in the UK, is contracted by NIHR to provide timely information on new and emerging health technologies with a potential significant impact on patients or the provision of health services in the near future. The identification process includes two approaches: a) routine identification regardless of clinical specialty, and b) in-depth scanning and review of market pipelines for diseases / technologies. These reviews are requested by the government, NIHR or identified as priority by the NIHR IO itself. Members of the public can also suggest topics. The NIHR IO identifies mainly pharmaceuticals and cell therapies, followed by diagnostics and imaging, devices and biotechnology. The identification of emerging health technologies starts up to three years before market authorization, with the aim to collect information 24-30 months prior to the launch of a health technology. For new pharmaceuticals and advanced therapies, they aim to provide an assessment report 20 months prior to expected market authorization; and use 15 months as the timeline for new indications of products that are currently licensed. The NIHR IO uses advanced data systems that scan open and confidential data sources to inform pipeline analyses. These are supplemented by interactions with companies to verify and confirm. Data sources include trial registers, as well as secondary sources such as scientific literature, regulatory agencies (including Food and Drug Administration (FDA), European Medicines Agency (EMA), Medicines and Healthcare products Regulatory Agency (MHRA)), clinical experts,
Step D1 Scoping

Scoping concerns the explicit definition of the research questions of the HTA. It 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. HTA agencies are often responsible for scoping, but policy makers, Ministry of Health, external committees, in consultation with relevant stakeholders and/or experts, can also do this.

We advise the advisory committee 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 which are the relevant Outcome measures. The TICO format describes which Technology is being evaluated, for which Indications (in terms target disease, population, and intended use), which Comparator is used, and which are the relevant Outcome measures. In the context of complex health technologies, a more flexible approach may be required.

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, i.e. those that are specific to the technology under evaluation. Scoping these criteria is ideally done in consultation with relevant stakeholders, e.g. patients, informal carers, and health professionals.

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

Further reading


patients/patient organizations, media, and tertiary sources (other horizon scanning organizations/trade data). The filtration is mainly based on period to licensing, the priority areas set by the government (appropriateness for the NHS) and whether or not the health technology is cancer-related. The identified health technologies are reported as filtration notes, drug briefings, MedTech alerts or Intelligence notes. 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 prioritization, gain consultation on an innovation (by companies as well as horizon scanning organizations), or educate members on a topic.

NICE undertakes additional filtration and conducts the actual prioritization to select health technologies for HTA asking the NIHR IO to provide technology briefs. The selection for conducting a technology brief (for pharmaceuticals) as input to the scoping of a full HTA by NICE is determined 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 added value of national guidance. The technology brief prepared by NIHR IO staff is sent to industry and one or two clinical experts for review. A technology brief includes the following information: target group, information about the technology, patient group, patient pathway, efficacy and safety and estimated costs and impact.

Further information is available elsewhere.25

**Germany** The Federal Joint Committee (G-BA) initiates the selection of a topic for a HTA report on pharmaceuticals. The research question is specified by the Institute for Quality and Efficiency in Health Care (IQWiG) and the G-BA. After this, a project group conducting the HTA formulates the final scientific research question and the outcomes for the project in agreement with IQWiG and potential external experts.

In addition, IQWiG can initiate assessments for non-pharmaceutical products. Since 2016, the public is invited to suggest topics for HTA via a website. To select relevant topics, IQWiG uses a stepwise procedure. A committee, representing the consumer and patient perspective, preselect up to 15 topics; the final selection of a maximum of five topics per year is done by IQWiG. The selected topics are addressed by external researchers using scientific literature searches. The results and a commentary of IQWiG are published via the website “Topic check medicine”. Further information is available elsewhere.25

**Thailand** The Thai HTA agency HITAP invites representatives of relevant stakeholders, such as policy-makers, health professionals, academics, patient associations, industry, civil society and lay citizens, to annually suggest topics for assessment. Scoping and prioritization of HTA takes place in the form of a panel including representatives from health professionals, academics, patients, and civil society that make use of criteria: size of the affected population, severity of problems, effectiveness of interventions, variation in practice, economic impact on household expenditure, and ethical and social implications.

Further information is available elsewhere.25

Evidence-informed deliberative processes
**Step D2 Assessment**

The assessment of health technologies includes various activities: systematic evidence collection on the selected decision criteria; synthesizing the evidence including an analysis of its quality; independent review of the evidence, and reporting the findings and implications.

We advise HTA agencies to establish a protocol on this process. An independent party such as an academic organization ideally does the collection and provision of evidence. In some countries it is also done by the HTA agency itself, or by the manufacturers of the health technology. The synthesis of evidence, including an analysis of its quality, can best be carried out by the HTA agency. The HTA agency subsequently issues an evidence report, which includes standardized 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.

In this guide we present assessment and appraisal as separate activities. We do this for practical reasons. In reality, however, this distinction is arbitrary as both steps require value judgments. We wish to emphasize that assessment of health technologies does not only involve facts, but also value judgments.

For reasons of transparency and legitimacy, we recommend HTA agencies to clearly describe the following assessment activities in a publicly available protocol:

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

This guide does not provide detailed methodological guidance on the various activities, as these already exist elsewhere. In the ‘Further reading’ section, we refer to guidelines on data collection and assessing the quality of evidence; guidance on reporting the evidence; and how to transfer evidence reports from one setting to another.
Evidence-informed deliberative processes

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

Guidelines for assessing the quality of evidence


Guidance on reporting the evidence


Guidance on adapting evidence reports to other settings


Country examples

Argentina HTAs are conducted by several universities and private organizations 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 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 could 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 also developed a tool to inform the “evidence-to-decision making” process to be applied in HTA, consisting of quality of the evidence (using GRADE), magnitude of net benefit (considering both benefits and adverse effects), and economic and organizational aspects (cost-effectiveness or expected budgetary impact).

Further information is available elsewhere.

Australia In Australia, the Pharmaceutical Benefits Advisory Committee (PBAC) is responsible for preparing recommendations for reimbursement of medicines. The applicant (industry) submits an assessment dossier, which is then reviewed by an independent assessment group. The submission dossier and the review are sent to the Economic Subcommittee (ESC) of the PBAC, which advises PBAC on the clinical and economic aspects of the HTA.

Further information is available elsewhere.

Canada CADTH has a very strict and thorough scientific method of reviewing available evidence. For each piece of evidence (e.g. studies, trials), CADTH documents in detail why it was or was not considered in the assessment process. CADTH formally asks feedback from different stakeholder groups (including health care professionals, patients, manufacturers, associations, and other interested parties) on projects and draft reports. In addition, processes are in place for patient groups to provide input on pharmaceutical products that are reviewed through the CDR and pCODR.

Further information is available elsewhere.
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. Stakeholders such as health care providers, patients, citizens, funders and decision-makers, have different interests and may reasonably disagree on which decision criteria are most important in the development of a recommendation.

Consider e.g. an advisory committee that has identified three relevant criteria in the evaluation 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 taking into account the three criteria. In case the committee considers effectiveness or severity of condition to be more important than cost-effectiveness in this example, the reimbursement recommendation is positive. In case cost-effectiveness is considered as the overriding criterion, the recommendation is negative. In other words, the central challenge for an advisory committee is to trade-off the different decision criteria.

This chapter provides guidance on how to make these trade-offs through means of multi-criteria decision analysis (MCDA). It is based on our recent consensus statement on the use of MCDA for HTA agencies.7 We use a broad definition of 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’.8 This definition also includes the decision-making approaches used by HTA agencies such as NICE in the UK and ZIN in the Netherlands, which are typically referred to as structured deliberation.

Principles of MCDA

Any MCDA starts with defining the decision problem and selecting the criteria that reflect relevant values. These steps are similar to steps in the use of EDPs (respectively B and D1), and are therefore not described in this chapter. The third step concerns the construction of the performance matrix. The performance matrix is a central element and, when applied to HTA, typically includes a set of generic criteria that are relevant to many technologies. The performance matrix presents an assessment of each technology against each of these criteria using descriptive information. An example can be found in Figure 2.

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. contextualised criteria) specific to the technology under scrutiny. We distinguish ‘qualitative MCDA’, ‘quantitative MCDA’ and ‘MCDA with decision rules’, depending on the way the committee interprets the performance matrix.

Qualitative MCDA

In qualitative MCDA, the committee develops its recommendation on a health technology by deliberating on its performance regarding explicitly defined criteria, i.e. it makes a qualitative interpretation of the performance matrix. (Figure 2) Qualitative MCDA is used to
Evidence-informed deliberative processes

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develop the health benefit package in Thailand.

The distinctive feature of qualitative MCDA that makes it different from intuitive prioritisation (without any specific method), is that it uses explicit criteria, including the technologies’ performance on these criteria. This has three advantages. First, the use of explicit criteria improves the quality of recommendations as it fosters in-depth consideration of the criteria, including the available evidence, and it provides structure to deliberative discussions of the committee. Yet, important challenges remain related to the cognitive load which may still be excessive; and the risk that certain stakeholders dominate the deliberations. These aspects are also communicated to the public, it further enhances the transparency of technologies waiting for appraisal, and insufficient HTA capacity for more detailed evaluation of other technologies. Third, when these aspects are also communicated to the public, it further enhances the transparency of recommendations. These benefits of using explicit weights are especially relevant for HTA agencies in certain contexts. If an agency operates in a country with limited tradition of transparency and accountability in public decision-making, this may raise trust in its decision-making. Also, the use of quantitative MCDA can be instrumental if an agency operates in a country with a backlog of technologies waiting for appraisal, and insufficient HTA capacity for more detailed evaluations.

Quantitative MCDA

In quantitative MCDA, the evidence on each criterion in the performance matrix is translated into a score, e.g. between 0 and 100. Thereafter, stakeholders’ preferences regarding the relative importance of criteria are measured using criterion weights. Then, a so-called value measurement model is used, which typically multiplies scores by the relative weight of that criterion, to sum the weighted scores and obtain an overall value for each technology. Technologies are ranked on the basis of these overall values (an example is provided in Figure 3) and uncertainty analysis is performed to understand the level of robustness of the results. Finally, the committee deliberates on the rank ordering of technologies, allowing a flexible interpretation of the results. This means that its members can put forward and discuss (aspects of) criteria that were not (fully) captured in the performance matrix, e.g. a complex consideration such as ‘own responsibility’. This step may lead to changes in the rank ordering of technologies.

Quantitative MCDA has several benefits in comparison to qualitative MCDA. First, the use of a value measurement model reduces the cognitive load of processing several criteria simultaneously by calculating an overall value score, and the risk of dominant participants influencing the deliberations. These aspects may further contribute to the quality of recommendations. Second, the use of criteria scores and weights may further improve the consistency of recommendations, if these scores and weights are also used for the evaluation of other technologies. Third, when these aspects are also communicated to the public, it further enhances the transparency of recommendations.

These benefits of using explicit weights are especially relevant for HTA agencies in certain contexts. If an agency operates in a country with limited tradition of transparency and accountability in public decision-making, this may raise trust in its decision-making. Also, the use of quantitative MCDA can be instrumental if an agency operates in a country with a backlog of technologies waiting for appraisal, and insufficient HTA capacity for more detailed evaluations.

There are also various limitations to quantitative MCDA, which may compromise the quality of recommendations: i), only few of these studies include a deliberative component; ii) studies typically assume that criteria are not interdependent which does not always hold; iii) the approach cannot capture opportunity costs, even though it tries to achieve this by including cost or cost-effectiveness as criteria in the value measurement model; iv) many studies involve double counting of one or more criteria; v) many studies do not use preference-based techniques for eliciting scores and weights, and do not provide descriptions of performance ranges. Details on these limitations are described elsewhere.
quantitative MCDA to improve decision-making. They should also be aware that quantitative MCDA, but should be aware of the design challenges when interpreting the results.

We advise HTA agencies to be explicit about their appraisal process, i.e. how they trade-off decision criteria and develop their recommendations. Agencies can, at a minimum, undertake qualitative MCDA. The use of explicit criteria has several advantages compared to intuitive prioritisation. They may also consider the use of quantified MCDA, but should be aware of the design challenges when interpreting the results. They should also be aware that quantitative MCDA does not capture opportunity costs and may thus lead to a suboptimal allocation of resources. Alternatively, HTA agencies may consider the use of MCDA with decision rules. This approach has the same potential as quantitative MCDA to improve decision-making, but, depending on the included number of criteria, may rely more on deliberation. It also avoids certain challenges in study design and can capture opportunity costs.

Irrespective of the specific approach, we advise HTA agencies to always include a deliberative component in its appraisal process. Agencies should report these deliberations, including the argumentation underlying a recommendation, to ensure the consistency and transparency of recommendations. Finally, HTA agencies should ensure that its appraisal process is legitimate and reflects societal preferences. The debate in the UK on a proposed ‘value-based assessment’ framework demonstrates this issue.44

Further reading

- There are generic patient submission templates as input for both the assessment and appraisal of health technologies. Available at: https://htai.org/inter-est-groups/pcgi/resources/for-patients-and-patient-groups/

Country examples

Canada CADTH has several specialized appraisal committees. CADTH applies a deliberative framework in its appraisal processes, and the processes, as well as the decision criteria are described in detail in a publicly available document.

CDEC is the advisory body for the Common Drug Review (CDR) process and includes 14 members. CDEC considers evidence on unmet needs, treatment outcomes and expectations, and economic evidence, including cost-effectiveness. In case the committee considers a technology to target a condition with unmet need, it accepts a higher cost-effectiveness ratio. At the committee meeting, 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). The recommendation will be based on the majority of votes during a meeting. The recommendations and the underlying reasons are made public.

The HTERP (the advisory body for non-pharmaceuticals) includes six core members. In addition, specialists will be appointed on a per-project basis to provide subject matter expertise on specific topics. Core members include individuals with qualifications in evidence-based medicine and/or critical appraisal, including a chair, an ethicist, a health economist, a health care practitioner, and one public member who represents the broad public interest. The HTERP uses a multi-criteria framework that considers the strength and quality of available clinical evidence; the strength and quality of available economic information; current practices and resource utilization patterns; and other factors including, but not limited to, patient input and practical, ethical, environmental, and psychosocial considerations. More information is available elsewhere.46
In its appraisal process, NICE is using 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 NHS or non-health gains). The maximum acceptable incremental cost-effectiveness ratio (ICER) is not precisely defined, and instead a range is used. Technologies with an ICER less than £20,000 per QALY are usually considered to be 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 appraisal 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 will be taken into account for end of life medicines. For highly specialized technologies, a different set of criteria will be considered (e.g. nature of the condition, impact of the new technology and cost to the NHS and Personal Social Services).

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 appraisal committee meeting. The appraisal committee summarizes the key evidence and their own view on the evidence, and provides a preliminary recommendation (based on consensus) in a so called ‘appraisal consultation document’ (or ‘evaluation consultation document’ for highly specialized technologies). Consultees, commentators and the public may respond. Comments are considered in a second appraisal committee meeting, which results in the final recommendation to the NHS.

More information is available elsewhere.\footnote{46}

Communication and appeal are important features that enhance the legitimacy of decision-making.

Communication refers to the publication of reimbursement decisions, including the argumentation that has been put forward by the advisory committee to justify these decisions. This may include the reasons for taking into account certain criteria, but also the reasons for excluding other criteria. The responsible health authorities - typically the Ministry of Health – should make efforts to ensure that reimbursement decisions are communicated to all relevant stakeholders, possibly using a variety of channels. This may include the use of policy briefs, newsletters but also news items on popular media. HTA agencies should liaise with the responsible health authorities to establish a protocol for communication of reimbursement decisions.

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

For reasons of transparency and legitimacy, it is important that the protocols for communication and appeal are publicly available.

Further reading
- Drummond M, Schwartz J, Jonsson B et al. Key principles for the improved conduct of health technology assessments for resource allocation decisions. International Journal of Technology Assessment in Health Care, 2008; 24(3), 244-258.
United Kingdom

The Appraisal Consultation Document (ACD) is a document published by NICE that contains key evidence, their argumentation, and their preliminary recommendation. Consultees, commentators, and the public may respond to the ACD. Comments are considered at a second Appraisal 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 consultation parties. The FAD is issued, an appeal needs to be made within 15 working days. An appeal can only be made if (i) NICE has failed to act fairly, or exceeded its powers when making the assessment that preceded the recommendation, or if (ii) when the recommendation is unreasonable in the light of the evidence submitted to NICE. The manufacturer is not allowed to appeal when disagreeing with the recommendation.

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 8 weeks of the end of the appeal period for oral hearings and 10 weeks for written submissions. A panel consists of five persons approved by the Secretary of State for Health and Social Care. Each appeal panel consists of persons approved by NICE, who are independent of NICE, and persons approved by the Secretary of State for Health and Social Care, or experienced representatives of patient care, or experienced representatives of patients, or patient representatives.

An overview of health technologies for which an appeal was issued, as well as an overview of the complete appeal process, can be found on the NICE website. Further information is available elsewhere.

Scotland

The HTA agency, Scottish Medicines Consortium (SMC), has two mechanisms for appeal in place. Manufacturers of which the advice for a product was ‘not recommended’ might resubmit their application when there is new evidence available, and/or might request for an independent review. SMC communicates their advice on new medicines via the Detailed Advice Documents (DADs) on the website and a press release each month. To increase the transparency of SMC decisions, SMC has started to produce a ‘Decision Explained’ factsheet for each SMC appraisal. These factsheets are written for a lay audience, and provide information about each medicine, indication, SMC decision, and reasoning for the decision. They also provide 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.

Annex 1 Checklist on EDP implementation

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

Evidence-informed deliberative processes

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Evidence-informed deliberative processes
<table>
<thead>
<tr>
<th>Step element</th>
<th>Description</th>
<th>Level of implementation/area for improvement</th>
</tr>
</thead>
<tbody>
<tr>
<td>Existence of linkage between HTA and policy making</td>
<td>A policy statement on the willingness to use HTA in policy and/or practice</td>
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<tr>
<td></td>
<td>A (formal) mechanism or process to link HTA to policy making (e.g. legislation)</td>
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<td></td>
<td>Allocation of public funding to HTA on an annual basis</td>
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<tr>
<td>Level of institutionalization</td>
<td>An independent organizational structure and/or institutional set-up for HTA (HTA agency) with a clear remit</td>
<td></td>
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<tr>
<td>Context</td>
<td>Sufficient capacity to carry out HTA, including medical disciplines, public health specialists, epidemiologists, statisticians, psychologists, biomedical engineers and/or economists</td>
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<tr>
<td>Capacity building and networking</td>
<td>Ability to review international literature (i.e. access to databases), including expertise in searching the internet</td>
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<td></td>
<td>(Domestic) HTA training opportunities (short courses, workshops, Master programmes and PhD training)</td>
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<td></td>
<td>An (inter)national networking strategy for collaboration between HTA agency(ies) and relevant stakeholders</td>
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<tr>
<td>Every 3-5 years</td>
<td>Existence of an advisory committee for appraisal/HTA decision-making</td>
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<tr>
<td>Step A: Installing an advisory committee</td>
<td>The composition, terms, and selection of members</td>
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<td></td>
<td>The roles and responsibilities of the committee and its members</td>
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<tr>
<td></td>
<td>The approach followed by the committee</td>
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<td></td>
<td>The approach followed to ensure meaningful stakeholder involvement in the HTA process</td>
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<tr>
<td>Step B: Defining decision criteria</td>
<td>Existence of a list of specified decision-criteria</td>
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<tr>
<td></td>
<td>The criteria to be used for decision-making</td>
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<td></td>
<td>The methods used, i.e. how are criteria derived from health system values</td>
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<td></td>
<td>The process for defining these criteria</td>
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<td></td>
<td>The approach followed to ensure meaningful stakeholder participation</td>
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<tr>
<td>Every 1-3 years</td>
<td>Existence of a selection procedure for health technologies for HTA</td>
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<tr>
<td>Step C: Selecting health technologies for HTA</td>
<td>Existence of a horizon scanning system</td>
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<td></td>
<td>The process of identification and selection of health technologies (i.e. methods, procedures, criteria)</td>
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<td></td>
<td>The approach followed to ensure meaningful stakeholder participation</td>
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<td></td>
<td>The roles and responsibilities of stakeholders involved</td>
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<tr>
<td>Every health technology</td>
<td>Existence of a scoping procedure</td>
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<tr>
<td>Step D1: Scoping</td>
<td>The process of scoping (i.e. methods, procedures, criteria)</td>
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<td></td>
<td>The approach followed to ensure meaningful stakeholder participation</td>
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<td></td>
<td>The roles and responsibilities of stakeholders involved</td>
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</tr>
</tbody>
</table>
Step D2: Assessment

- Existence of an assessment protocol
- Existence of a tool/template for reporting and summarizing the (quality of the) evidence per relevant aspect as part of assessment
- Publicly available document describing: The assessment protocol in terms of evidence collection, analysis and reporting
- The approach followed to ensure stakeholder consultation to review the plausibility of evidence reports

Step D3: Appraisal

- Existence of explicit appraisal process
- The process of appraisal (i.e. methods, procedures, deliberation)
- Publicly available document describing: The approach followed to ensure meaningful stakeholder participation
- The roles and responsibilities of stakeholders involved in the process

Step D4: Communication and appeal

- Existence of a protocol for communication and appeal
- The mechanism(s) for the communication of decisions and the underlying reasons to all relevant stakeholders
- Publicly available document describing: The mechanism(s) for appeal, how to propose revisions, and to receive a reasoned response

Annex 2 Checklist on stakeholder participation

We advise HTA agencies to use a checklist on stakeholder participation. This checklist can assist them in the practical organization of meaningful stakeholder participation throughout EDPs and in particular during the appraisal step (D3) (Table 2).50

How to use the checklist?
HTA agencies can use the checklist to revise for possible shortcomings 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 would constitute ‘right answers’ to individual questions in the checklist. In some contexts it may e.g. be reasonable to reimburse travel expenses for the sake of accessibility, while in other cases this may be judged irrelevant or inappropriate. Nevertheless, HTA agencies are advised to inform their specific choices by evidence if available – or to learn from other countries’ experiences. Finally, HTA agencies should take incremental steps by prioritizing specific efforts according to local needs and affordances.
Table 2: A Checklist for Stakeholder Participation

<table>
<thead>
<tr>
<th>Identification of potentially adversely affected stakeholders</th>
<th>Level of implementation (area(s) for improvement ( - / - + / +))</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Are efforts made to identify those who experience a health loss as a result of a negative decision?</td>
<td></td>
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<tr>
<td>2. Are efforts made to identify those who experience a health loss as a result of a positive decision?</td>
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<tr>
<td>3. Are efforts made to identify those who are responsible for communicating the decision?</td>
<td></td>
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<tr>
<td>4. Are efforts made to identify those who are responsible for implementing the decision?</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Comprehensive stakeholder inclusion</th>
<th>Level of implementation (area(s) for improvement ( - / - + / +))</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Are all relevant stakeholders informed about the possibility and procedures of participation?</td>
<td></td>
</tr>
<tr>
<td>2. Is participation organized in a way that effectively and efficiently facilitates the inclusion of stakeholders?</td>
<td></td>
</tr>
<tr>
<td>3. Are efforts made to include all relevant, especially difficult-to-reach, stakeholders?</td>
<td></td>
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<tr>
<td>4. Can stakeholders participate in the identification and selection of health technologies for HTA?</td>
<td></td>
</tr>
<tr>
<td>5. Can stakeholders participate in the scoping of relevant questions for evaluation?</td>
<td></td>
</tr>
<tr>
<td>6. Can stakeholders participate in the development of recommendations (assessment and appraisal)?</td>
<td></td>
</tr>
<tr>
<td>7. Can stakeholders participate in the evaluation of decisions?</td>
<td></td>
</tr>
<tr>
<td>8. Are alternative non-participatory strategies used for inclusion of stakeholders’ values?</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Meaningful stakeholder participation</th>
<th>Level of implementation (area(s) for improvement ( - / - + / +))</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Are stakeholders fully and in time informed about the available evidence?</td>
<td></td>
</tr>
<tr>
<td>2. Is argumentation and evidence presented in a way that is understandable to all relevant stakeholders?</td>
<td></td>
</tr>
<tr>
<td>3. Can stakeholders freely voice their perspectives (i.e., no stakeholder is allowed to dominate a discussion or activity)?</td>
<td></td>
</tr>
<tr>
<td>4. Are stakeholder perspectives addressed in respectful and courteous ways?</td>
<td></td>
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<tr>
<td>5. Do stakeholders have sufficient time to provide input?</td>
<td></td>
</tr>
<tr>
<td>6. Are stakeholder perspectives equally accounted for in the deliberation?</td>
<td></td>
</tr>
<tr>
<td>7. Is it clear to all stakeholders involved how their input is going to be considered, scrutinized and put to use?</td>
<td></td>
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<tr>
<td>8. Can stakeholders actively interact in the deliberation?</td>
<td></td>
</tr>
<tr>
<td>9. Is further evidence collection considered when judged relevant and feasible?</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Transparent communication of recommendations and/or decisions</th>
<th>Level of implementation (area(s) for improvement ( - / - + / +))</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Is information provided on the underlying argumentation and process to come to a recommendation and/or decision?</td>
<td></td>
</tr>
<tr>
<td>2. Is input from stakeholders documented and addressed explicitly?</td>
<td></td>
</tr>
<tr>
<td>3. Are recommendations and/or decisions clearly communicated?</td>
<td></td>
</tr>
<tr>
<td>4. Are stakeholders informed in time on the recommendation and/or decision?</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Appeal and evaluation</th>
<th>Level of implementation (area(s) for improvement ( - / - + / +))</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Can stakeholders easily make an appeal on the underlying argumentation or process?</td>
<td></td>
</tr>
<tr>
<td>2. Are appeals documented and publicly accessible?</td>
<td></td>
</tr>
<tr>
<td>3. Are appeals handled consistently and is justification provided in an understandable way?</td>
<td></td>
</tr>
<tr>
<td>4. Are mechanisms in place to revise decisions or the process based on appeals?</td>
<td></td>
</tr>
</tbody>
</table>
Evidence-informed deliberative processes


3 Mainly from members of the International Network of Agencies for Health Technology Assessment (INAHTA)


6 Website of NICE: https://www.nice.org.uk/process/pmg20/chapter/introduction; https://www.nice.org.uk/process/pmg20/chapter/evidence


14 Thokala P, Devlin N, Marsh K et al. Multi criteria decision analysis to support HTA agencies – benefits, limitations and the way forward. Submitted for publication, 2019

15 Baltussen R, Thokala P, Marsh K et al. Multi criteria decision analysis to support HTA agencies – benefits, limitations and the way forward. Submitted for publication, 2019

16 Mainly from members of the International Network of Agencies for Health Technology Assessment (INAHTA)


27 Topic selection at NICE: https://www.nice.org.uk/about/what-we-do/our-programmes/topic-selection


3015(17)32772-9/fulltext


34 Website of PRAC: https://prac.pbs.gov.au/;


37 Baltussen R, Devlin N, Marsh K et al. Multi criteria decision analysis to support HTA agencies – benefits, limitations and the way forward. Submitted for publication, 2019

38 Thokala P, Devlin N, Marsh K et al. Multi criteria decision analysis to support HTA agencies – benefits, limitations and the way forward. Submitted for publication, 2019


Evidence-informed deliberative processes
effective use of NHS resources will specifically take account of the following factors: The degree of certainty around the ICER( ), the innovative nature of the technology( ), the technology meets the criteria for special consideration as a ‘life-extending treatment at the end of life’ ( ), and aspects that relate to non-health objectives of the NHS’. Source: National Institute for Health and Care Excellence. Guide to the methods of technology appraisal. London 2013.

43 In its highly specialised technology program for very rare diseases, NICE raises the threshold to £100,000-£300,000 per QALY gained. This increased threshold reflects the fact that NICE assigns a quantitative weight to the treatment of these diseases. Source: National Institute for Clinical Excellence. Highly specialised technologies guidance. https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-highly-specialised-technologies-guidance.


Procedure and Submission Guidelines for the CADTH Common Drug Review https://cadth.ca/sites/default/files/cdr/process/Procedure_and_Guidelines_for_CADTH_CDR.pdf


Evidence-informed deliberative processes (EDPs) are a value framework for HTA agencies to support the development of reimbursement decisions. It considers HTA as a value-laden process, and not just as the production of evidence on effectiveness or cost-effectiveness. This guide provides practical support for HTA agencies on how to organise their processes, with an emphasis on enhancing legitimate decision-making.

EDPs involve seven steps: installing an advisory committee including stakeholder involvement, identifying decision criteria, selecting health technologies for HTA, scoping, assessment, appraisal and communication & appeal. For each of these steps, this guide provides examples of HTA agencies around the globe and key publications for further reference.