

INSTITUTIONALIZING HEALTH TECHNOLOGY ASSESSMENT MECHANISMS: A HOW TO GUIDE

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Institutionalizing health technology assessment mechanisms: a how to guide/Melanie Bertram, Gwenaël Dhaene, Tessa Tan-Torres Edejer, editors

ISBN 978-92-4-002066-5 (electronic version)

ISBN 978-92-4-002067-2 (print version)

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Suggested citation. Bertram M, Dhaene G, Tan-Torres Edejer T, editors. Institutionalizing health technology assessment mechanisms: a how to guide. Geneva: World Health Organization; 2021. Licence: CC BY-NC-SA 3.0 IGO.

Cataloguing-in-Publication (CIP) data. CIP data are available at <http://apps.who.int/iris>.

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Acknowledgements

This publication was produced by the Department of Health Systems Governance and Financing of the World Health Organization (WHO) Geneva, Switzerland.

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Acronyms

HTA health technology assessment

M&E monitoring and evaluation

MCDA multi-criteria decision analysis

NICE National Institute for Health and Care Excellence

WHO World Health Organization

Introduction

Health intervention and technology assessment – type approaches to selection of interventions for reimbursement have a long history at the World Health Organization. The first version of the Essential Medicine List was published in 1977, and the first programme on cost-effectiveness in health began in 1998. In 2014 Resolution 67.23 Health Intervention and Technology Assessment for Universal Health Coverage was adopted by the World Health Assembly providing the first global mandate to support countries in developing health intervention and technology assessment mechanisms. This built on previously passed resolutions by the Region of the Americas in 2012 and the South East Asian Region in 2013. In response to the World Health Assembly Resolution, WHO is increasingly supporting countries to develop institutionalised health technology assessment mechanisms to support decision making about which interventions to reimburse for universal health coverage benefit packages.

Now more than ever, it is apparent that systems cannot rely on additional resources to achieve their universal health coverage goals but must generate more health for the resource spent by improving the efficiency of spending. Health technology assessment is one approach to ensuring evidence informed decisions are made, and health funds are used wisely.

This guidance note was prepared for countries that have already made the decision that they require a health technology assessment (HTA) mechanism. It is not intended to convince readers that an HTA is necessary, as it is expected that they have already reached that conclusion. It is designed to be extremely practical, with checklists to support implementation in each chapter. Annotated reading lists are provided for further conceptual understanding.

CHAPTER 1

Establishing a mandate

1 Why conduct health technology assessment (HTA)?

- Why establish a mechanism for HTA?
- What question or problem is to be corrected with an HTA mechanism?

2 What is the purpose of the HTA?

- What functions will the HTA contain?
- Should reimbursement mechanisms be recommendatory or binding?
- Consult legislators (chapter 2) to ensure that the legal framework they are preparing responds to the mandate.

3 Preparing for implementation

- Define a budget for the HTA mechanism.
- Find capacity in the country to carry out HTA functions (chapter 3).
- Consider involving networks and experts with experience in HTA.

4 Planning for the future

- Prepare a long-term strategy.
- Consider extension of the mandate over time.

1.1 Introduction and conceptual framework

This chapter outlines the scope of the document and options for establishing a mandate for an HTA in a country. It is relevant for countries that do not yet have an HTA mechanism or agency.

There are many steps in the value chain between the time at which an intervention or technology enters the market (pharmaceuticals and medical products) or is considered for a policy (regulatory and population health interventions) and when it is prescribed, dispensed and used appropriately (Fig. 1). The focus of this book is on step 4, selection, pricing and reimbursement and, specifically, on the process for institutionalizing an HTA mechanism, in five main steps:

- 1 Establishing a mandate
- 2 Establishing a legal framework
- 3 Establishing institutional arrangements
- 4 Procedural aspects of assessment and appraisal
- 5 Monitoring and evaluation of the HTA mechanism

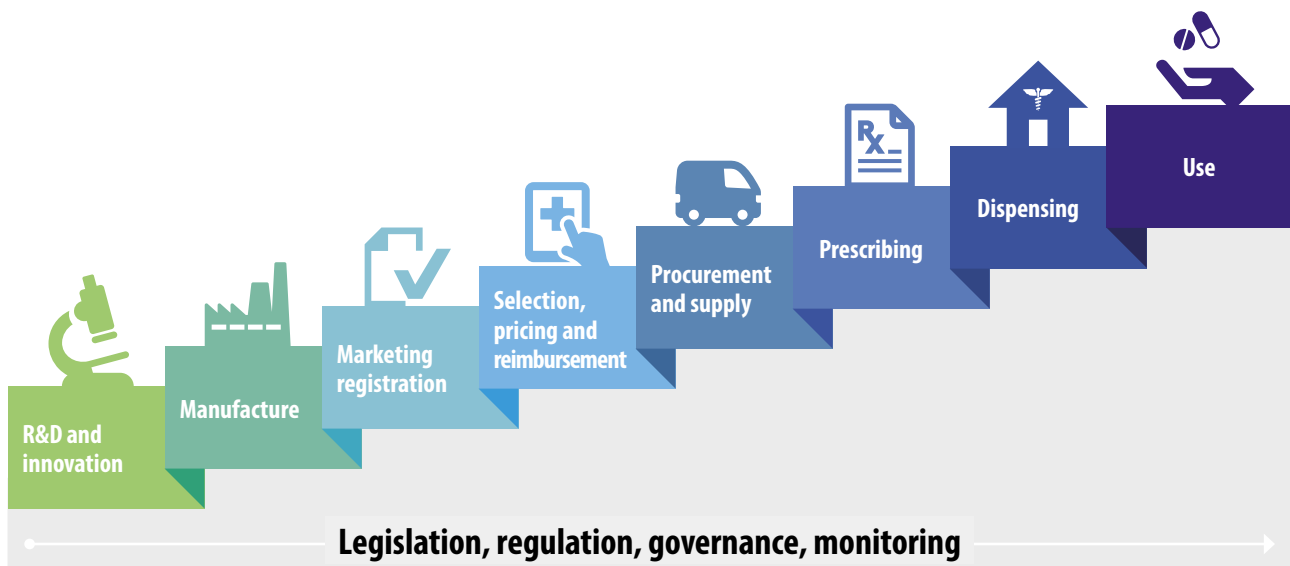


Fig. 1. Value chain for health interventions

1.2 Health technology assessment

HTA is a function performed within ministries of health to support reimbursement decisions. The purpose of this document is to provide advice to countries that are considering using HTA as a tool in the decision-making process but require some practical guidance.

According to WHO, HTA is

the systematic evaluation of properties, effects and/or impacts of health technologies and interventions. It covers both the direct, intended consequences of technologies and interventions and their indirect, unintended consequences. The approach is used to inform policy and decision-making in health care, especially on how best to allocate limited funds to health interventions and technologies. The assessment is conducted by interdisciplinary groups using explicit analytical frameworks, drawing on clinical, epidemiological, health economic and other information and methodologies. It may be applied to interventions, such as including a new medicine into a reimbursement scheme, rolling-out broad public health programmes (such as immunization or screening for cancer), priority setting in health care, identifying health interventions that produce the greatest health gain and offer value for money, setting prices for medicines and other technologies based on their cost-effectiveness, and formulating clinical guidelines.

The WHO definition is necessarily broad, as HTA processes in countries around the world have different mandates, ranging from narrow, specific mandates such as that of the Australian Pharmaceutical Benefits Advisory Committee, which makes recommendations for drug reimbursements, to extremely broad mandates, such as that of the National Institute for Health and Care Excellence (NICE) in the United Kingdom, which recommends interventions for reimbursement and develops clinical guidelines.

WHO considers that HTA is not limited to one type of health intervention or technology but should cover the entire range of interventions that are reimbursed or are considered for reimbursement, including medical devices, medicines, medical services, medical and surgical procedures, assistive devices, diagnostics, screening, vaccination and other health programmed (Fig. 2).



Source: reference 1.

Fig. 2. Technologies and methods considered in HTA

1.3 Importance of health technology assessment

In countries at any income level, the demand for medicines and health products surpasses the available resources. It is therefore critical to ensure that resources are used to purchase interventions and technologies that offer the best value for money while at the same time prioritizing the people who are worse off and protecting the population from any financial risk associated with accessing health care. In low- and middle-income countries, this often takes the form of an essential or guaranteed package of care, which should be prioritized for delivery before more expensive alternative interventions or technologies which may benefit only small subsets of the population.

A formal process for decision-making with regard to reimbursement ensures that choices are made in the same way each time. If they are undertaken within the appropriate legal framework, this can ensure they are not unduly influenced by external factors, such as a conflict of interest. A transparent, robust process that includes all appropriate stakeholders and is communicated effectively also ensures that the population understand why certain decisions were made and have the opportunity to provide their views. This process also provides protection for decision-makers, as recommendations for and against reimbursement are made through a formal process; the decision-maker is no longer considered to be solely responsible for rejection from a reimbursement list, thus avoiding personal conflict.

A logical framework for the inputs, activities, outputs and outcomes of the theory of change behind use of HTA mechanisms is shown in Fig. 3. Essentially, through a transparent, fair HTA mechanism with a strong legal framework, decisions on reimbursement for universal health coverage will be more efficient and lead to better health outcomes.

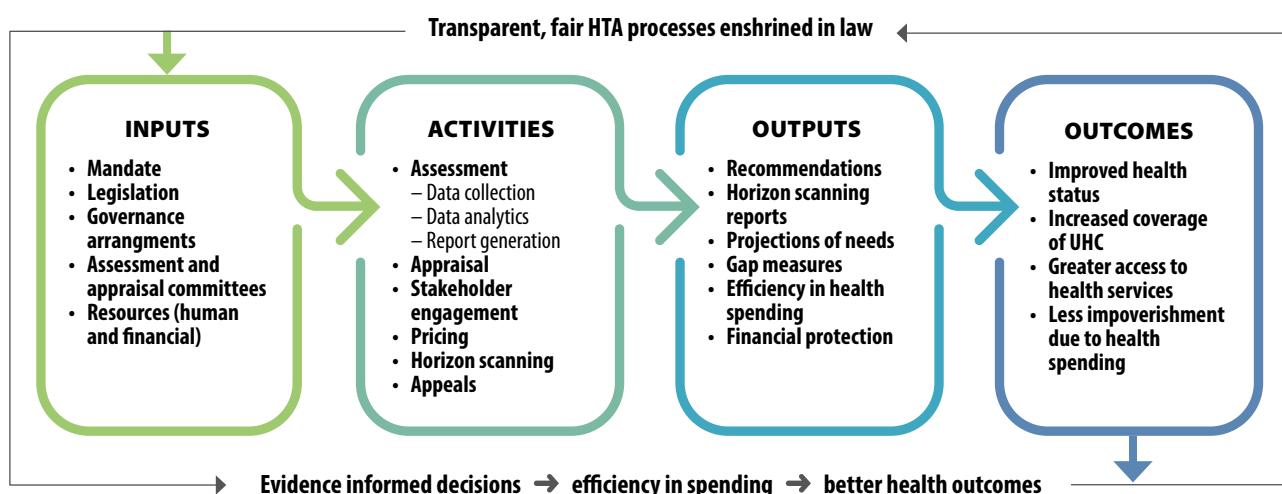


Fig. 3. Logical framework for HTA mechanism

In the absence of a formal priority-setting and decision-making process such as HTA, priorities and funding decisions in countries are often distorted. The consequence is that, if money is spent on the health system without a formal prioritization system, maximal health benefits are not achieved, and the direct result is that lives are lost. This speaks to the need to ensure efficiency in health spending, as concluded by the WHO Working Group on Equity and Universal Health Coverage, in the report “Making Fair Choices on the Path to Universal Health Coverage” (2).

Prioritization of medicines, health products, interventions and technologies using HTA methodologies, often through establishment of a health benefits package, is considered to be critical for facilitating access and ensuring achievable, sustainable, universal health coverage. Many HTA processes are based on transparency and fairness in decision-making, as discussed in chapter 4 on assessment and appraisal.

HTA processes and methods are used in different ways in different countries. The functioning of the mechanism depends on the context and the question to be answered. For example, fragile states may establish a benefits package that can be delivered in the short term with donor financing and with an HTA-type method used in a once-off process to define the package. Countries with emerging institutions are likely to emphasize legal frameworks and institutional arrangements, including the procedural aspects of appraisal.

More advanced systems often focus on increasing capacity for HTA and possibly extending the mandate. At all levels of development of the HTA mechanism, links must be made with actual decisions on resource allocation.

At this stage, it is important to reflect on the first two items on the checklist for this chapter

- Why establish a mechanism for HTA?
- What question or problem is to be corrected with an HTA mechanism?

1.4 Functions of health technology assessment mechanisms

Most of the advice provided to date has stressed analytical **assessment** aspect of HTA, such as reviewing the clinical and economic evidence used to inform decision making, and less attention has been paid to the decision-making (**appraisal**) process, methods, capability and capacity. The overarching legal framework and institutional arrangements required to implement a robust HTA mechanism are often not covered in guidance documents. The present publication covers the entire process of developing an HTA mechanism in a country. It does not include analytical methods for assessment, which are well documented elsewhere.

Fig. 4 shows the range of functions that could be covered by an HTA mechanism, and each possible function is explained below. Those shown in blue, the overarching legal framework and institutional arrangements, are described in this document; however, no explicit recommendation is made about the functions of an HTA mechanism.

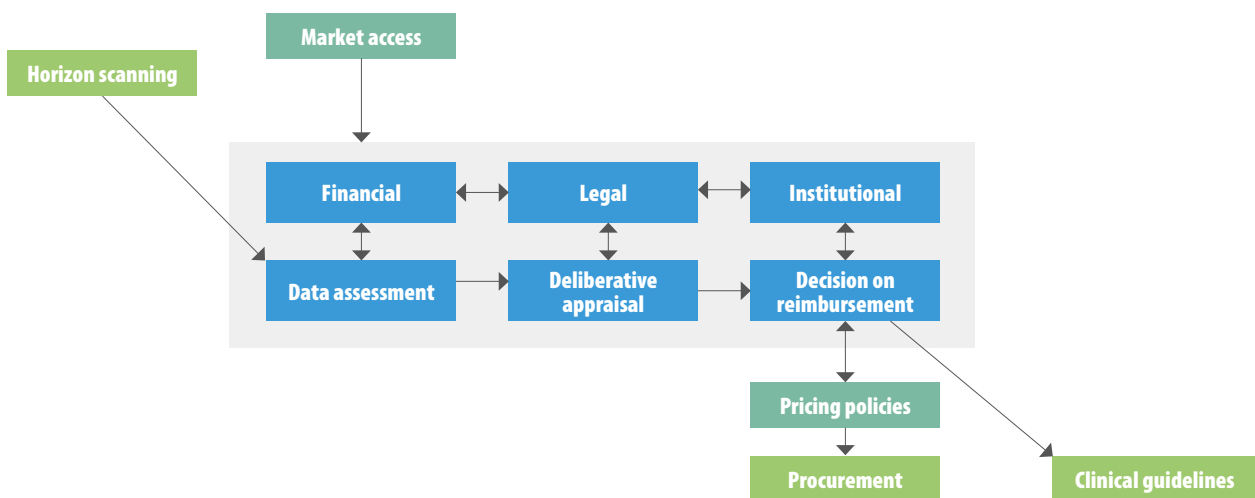


Fig. 4. Activities relevant to accessing health services

Market access: The first step in the access chain is national registration and market authorization. Regulatory authorities require evidence for quality and safety, and clinical data to support the claims made by the sponsor. As there is no requirement to provide evidence of how well the intervention compares with existing clinical practice or whether it represents value for money, registration data alone are often insufficient for decision-making, and further evidence must be requested from the sponsor or modelling. Global efforts are under way to encourage the provision of better evidence at the time of marketing authorization as a basis for informing national decision-making (3). Market authorization is generally undertaken by a separate agency, and it is not recommended that the HTA mechanism attempt to appropriate the mandate of the current market authorization agency in the country. Given the requirement for market authorization before an HTA assessment, it is an important step in the process, and the HTA mechanism should establish a good working relationship with the market authorization body.

HTA assessment and appraisal: In addition to the clinical and economic aspects, other criteria should be taken into account in making decisions (Fig. 5). The criteria used will differ by country and context. In addition, constraints to delivery, such as the structure of the health system, available skills and out-of-pocket payments should also be considered. For established medicines, a robust evidence base will be available that has been reviewed by experts in many jurisdictions, such as medicines on the WHO or national lists of essential medicines. It is not necessary to repeat best-practice technical exercises, such as the assessment process for the Essential Medicines List, although further country- or context-specific evidence on cost or local performance might be reviewed before a decision is made about reimbursement. This process, which is discussed further in chapter 4, is a minimum requirement for an HTA mechanism.

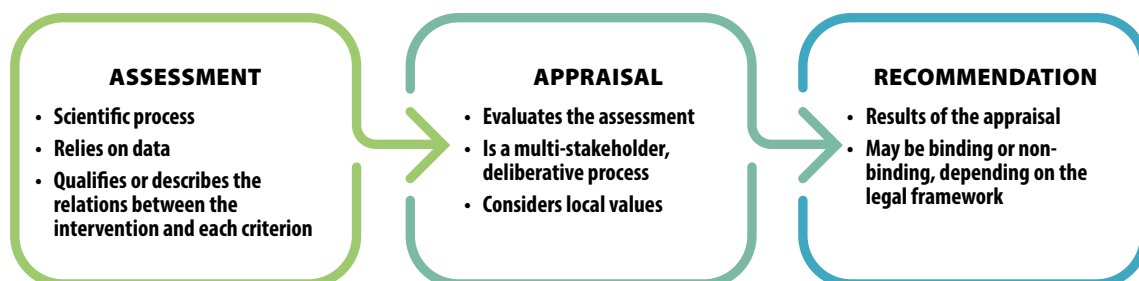


Fig. 5. Relations among assessment, appraisal and recommendation in HTA mechanisms

Recommendations: The recommendations resulting from the HTA mechanism may be recommendatory or mandatory, depending on the mandate given to the HTA mechanism. In Australia, the Pharmaceutical Benefits Advisory Committee recommends which medicines should be subsidized, and a final decision is made by the Government; however, the Government may list medicines only after a positive recommendation from the Advisory Committee. In the United Kingdom, the recommendation of NICE is binding, and the National Health Service England is required to provide access to the medicine within 3 months of a positive recommendation. No best practice model is suggested, but it is crucial to link the recommendations of the HTA process to reimbursement decisions in some way. Chapter 2 describes the legal tools for establishing specific mandates. Countries should not only understand how recommendations are made but should also consider the scope of what the HTA mechanism may advise on, such as whether the agency can recommend an intervention that looks promising but for which there is insufficient evidence for adequate assessment. In this case they may recommend that coverage can begin, with further evidence required for formal listing. Additionally, the agency may be mandated to recommend disinvestment from interventions or technologies that are considered no longer to be of clinical or economic value.

Price policies and procurement: As the WHO definition of HTA suggests, the results of cost-effectiveness analyses undertaken as part of an HTA assessment could form the basis for price negotiations and be part of the scope of practice of the HTA mechanism. Generally, however, medicines are purchased by a separate institution under the public procurement rules of the country; the purchaser is also involved in price negotiations.

Clinical guidelines: Clinical practice guidelines should be available for all interventions and technologies available on the market, regardless of the decision about reimbursement through a government-funded system. In general, clinical practice guidelines are not part of the scope of an HTA mechanism but are overseen by professional clinician groups, although this is not always the case. In the United Kingdom, NICE is responsible for producing clinical practice guidelines for all recommended interventions; however, in Australia, the National Health and Medical Research Council is responsible for validating clinical practice guidelines produced by external groups – not the Pharmaceutical Benefits Advisory Committee which functions as the HTA mechanism. The Cambodian essential health benefits package includes clinical guidelines for each of the 39 interventions included, and the guidelines are produced by the body that developed the package, whereas the Ethiopian essential health benefits package does not include clinical guidelines but refers to those produced in a separate institution. The mandate for the HTA mechanism should therefore be clear regarding clinical practice guidelines. If capacity is limited, clinical practice guidelines may not be the immediate priority.

Horizon scanning: “Horizon scanning” means looking ahead to interventions or technologies that are due to enter the market in the future but may be disruptive due to high cost or because of high demand from citizens. In higher-income settings, this is increasingly part of the mandate of HTA mechanisms; however, it may not be a priority in the initial stages of the mechanism.

At this stage, it is important to reflect on the next items on the checklist for this chapter

- What functions will HTA contain?
- Should reimbursement mechanisms be recommendatory or binding?
- Consult legislators (chapter 2) to ensure that the legal framework they are preparing responds to the mandate.

1.5 Considerations in establishing a health technology assessment mechanism

How much will it cost? The costs involved in establishing a HTA mechanism may seem high, but the benefits of a well-implemented mechanism will improve efficiency of health spending and increase health benefits. For example, in New Zealand, the Pharmaceutical Management Agency reported that growth in pharmaceutical spending has slowed, and has reduced as a proportion of overall health spending since the introduction of HTA in 1993. The savings in the first year were calculated to be NZ\$ 3.1 million, and the cumulative savings for the first 13 years were estimated to be NZ\$ 1032 million (4).

What capacity is required? Often, countries have the capacity to undertake HTA assessment, and chapter 3 on establishing institutional arrangements indicates where such capacity may be found. It may be in academic centres of excellence or departments of the health system currently responsible for decision-making if there is no HTA mechanism. According to best practice, assessment is undertaken independently of those who make decisions in order to minimize any conflicts of interest. Decision-makers may undertake assessments, providing they are done to approved standards and transparency requirements, and any conflicts of interest are managed.

What support is available? A number of networks on HTA exist. WHO hosts the Decide: Health Decision Hub, designed as a meeting place for all HTA networks, academics working on HTA and countries that require advice and support in establishing HTA. The Decide Hub can link national decision-makers to the appropriate providers of technical assistance (WHO or other collaborative partners). Global networks of HTA agencies and individuals working on HTA are:

- Decide: Health Decision Hub – a global network for health decision-making support, hosted by WHO;
- Health Technology Assessment International (HTAi) – a global, non-profit, scientific and professional society for those who produce, use or encounter HTA;
- HTAsiaLink – a network to strengthen collaboration among HTA agencies in Asia;
- International Network of Agencies for Health Technology Assessment (INAHTA) – a network of 50 HTA agencies that support health system decision-making in 31 countries, affecting over 1 billion people;
- European Network for Health Technology Assessment (EUnetHTA) – established to create an effective, sustainable network for HTA across Europe;
- Red de Evaluación de las Tecnologías de la Salud en las Américas (RedETSA) – the health technology assessment network of the Americas;
- International Society for Pharmacoeconomics and Outcomes Research (ISPOR) – a professional society for health economics and outcomes research globally;
- A regional network on HTA for the WHO Eastern Mediterranean Region; and
- EuroScan – the international information network on new or emerging health technologies, appropriate use and re-assessment.

Technical assistance is available from WHO and from academic centres. An expert tool on the Decide Hub web platform at www.decidehealth.world can be used to find a suitable technical assistance provider.

At this stage it is important to reflect on the next items on the checklist for this chapter

- Define a budget for the HTA mechanism.
- Find capacity in the country to carry out HTA functions (chapter 3).
- Consider involving networks and experts with experience in HTA.

1.6 Future of health technology assessment

HTA has grown substantially since its inception. In the early years, HTA was generally used to support decisions to list or de-list pharmaceuticals and devices. The technical rigour and discipline of support to decision-making has since led to new applications of the methods and process, including a broader range of interventions, such as population health approaches. In addition, as stakeholder expertise and experience have grown, many of the methods have become more complex, and links between budget holders and regulatory systems have become more explicit. A burgeoning area of research into deliberative dialogue has also been established (see chapter 4).

HTA mechanisms across the globe now include a range of functions, from horizon scanning to scoping, topic selection, technology assessment and appraisal, supporting decisions on coverage, to price negotiation, guideline development and setting quality standards. A long-term strategy should be developed for the HTA mechanism to encompass the increasing strength of the health system and the fiscal space available for health, which may change the mandate of the HTA mechanism and the complexity of the methods used (Fig. 6). Experience in high-income settings indicates that development of an HTA mechanism requires long-term investment, and countries should be suitably prepared.

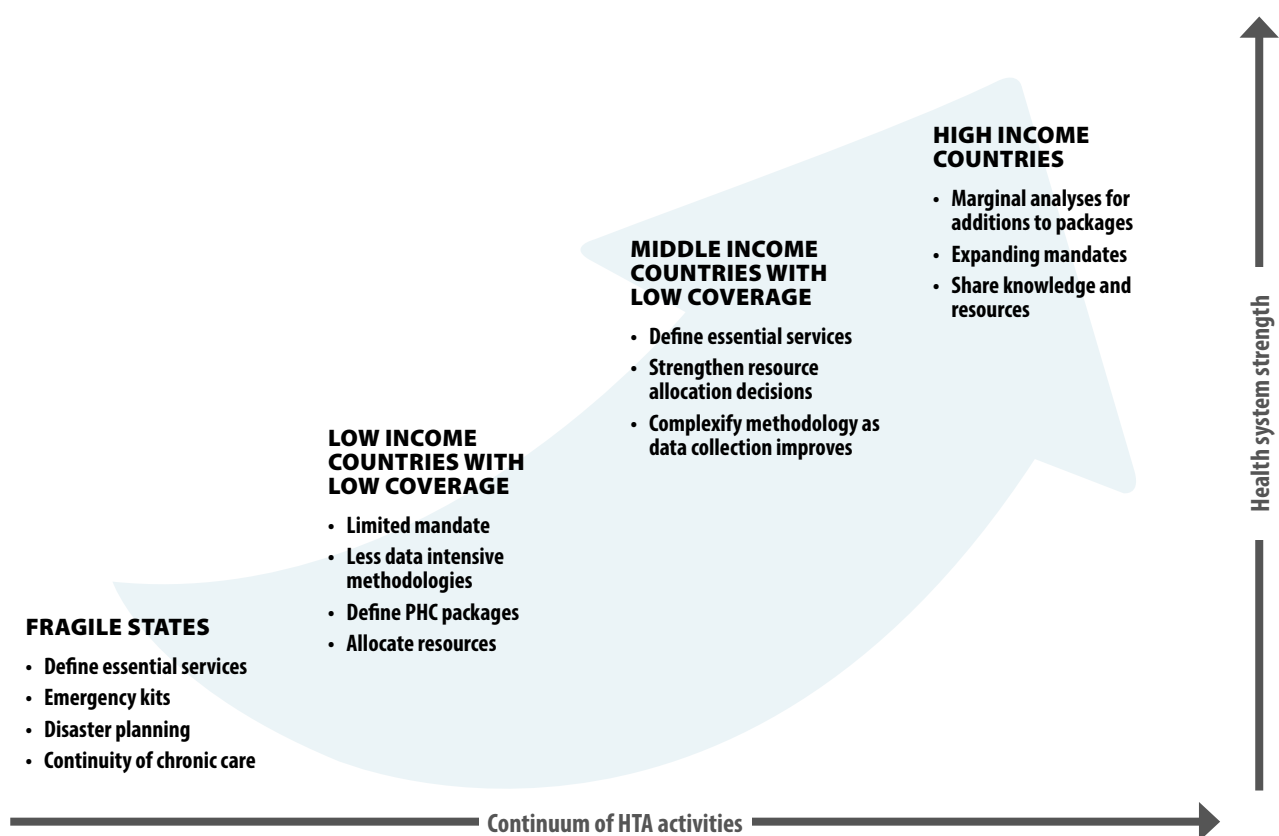


Fig. 6. Extending the mandates of HTA mechanisms as the health system becomes stronger

CHAPTER 2

Legal considerations: reviewing or establishing the legal framework

1 The legal environment

- **Review the legal system to understand how the laws and regulations** form the basis for organization of the health system.
- **Identify where HTA functions or mechanisms fit or may fit** into the legal system, the branches of law involved and the statutes, legislation and regulations that apply.

2 Bolster HTA reform with legal tools

- **Identify or design a legal pathway towards HTA:** Compile a relevant set of legal rules that cover the values and principles that are essential for HTA reform. The set should be comprehensive, e.g. from assessment mandate to procurement rules, and include data confidentiality and prevention and management of conflicts of interest for instance.
- **Craft clear laws and regulations:** Ensure that the scope and provisions of the legal norms are clear to obviate broad interpretation, requalification or avoidable injunctions. Legally binding rules should cover essential areas such as transparency, independence of advice, confidentiality of data, prevention and management of conflicts of interest,
- **Accountability:** Ensure accountability, halting the process in cases of lack of information or planning for effective legal remedies (e.g. for unreasonable delay in decision-making).
- **Take stock of the case law:** Anticipate boundaries, responsibilities recognised by judicial review or the potential practices and decisions likely to be overruled by the Judiciary.
- **Anticipate financial implications of judicial review:** Consider the need for making provision for resources in the event of a judicial decision that affects reimbursement of technologies or remedies to avoid adverse consequences on budgetary planning.
- **Consult relevant stakeholders:** Ensure that all relevant parties are consulted as per legal requirements.

3 Monitor and adjust to new legal standards

- **Avoid “stagnant” law:** Surveillance of discrepancies or outdated norms can maintain an HTA mechanism that is fit for purpose and legally sound, i.e. less susceptible to decisions rescinded by administrative or judicial processes (e.g. introduction of new norms stemming from ratification of international agreements).
- **Set-up a continuous legal review** so that the scope of engagement in the HTA function remains relevant and mirrors legal, policy and societal evolution (e.g. users’ consultation or equity selection criteria).
- **Establish a life-cycle:** A regular, normative life-cycle to ensure systematic monitoring and evaluation (M&E) of the rule of law pertaining to HTA can provide feedback and innovative ideas from stakeholders (e.g. benchmarking studies and reports from HTA networks or introduction of a maturity model to accompany adaptation of the HTA legal framework).

2.1 Context

Growing attention to the use of HTA mechanisms for selecting technologies illustrates the momentum gathered by using data- and evidence-driven processes in health. HTA has been used to support decision-making in many countries for decades; for example, NICE celebrated 20 years of activity in 2019. Decision-makers are now being further encouraged to use HTA processes to accelerate health systems development. HTA makes it possible to harness health with the economic and social values which inspire the technology selection criteria. In this context, decision-makers often overlook an important factor, which proves to be the crux of successful implementation of HTA processes: the role of the law.

The literature on HTA is largely oblivious to legal matters:

Legal analysis can highlight important issues that are relevant when deciding whether a medical technology should be implemented or reimbursed. Literature and studies show that even though the law is an acknowledged part of health technology assessment (HTA), legal issues are rarely considered in practice. One reason for this may be the lack of knowledge about the diversity of legal issues that are relevant for HTA (5).

Lack of knowledge and understanding of the law – particularly public law – often explains such oversight. Health systems and HTA do not, however, operate in a legal vacuum. Understanding legal frameworks is necessary in order to fully comprehend the drivers of decisions in health and the dynamics that underpin policies. This chapter provides operational understanding of the legally binding rules that encompass, organise and define boundaries in HTA processes. Its aim is to help decision-makers to navigate legal concepts that apply to HTA and must be taken into account in either establishing HTA mechanisms or strengthening the effectiveness of those in place.

The chapter therefore takes a four-pronged approach, to:

- help decision-makers grasp the legal environment that rules HTA mechanisms;
- support them in making the best of the rule of law to design HTA mechanisms;
- monitor and adjust the rule of law to preserve the relevance of HTA; and
- highlight elements pertaining to the concept the “right to health”.

Practice in different countries is described, and different legal systems, such as civil and common law systems, are drawn upon to establish useful parallels.

2.2 The legal environment of HTA: Law actually is all around us

There is no such thing as “HTA law”. It is all public law. The binding or “soft” rules that pertain to HTA generally fall within the scope of jurisdiction of the normative area for organizing and regulating health as a public service, i.e. public law. Public law is the branch of law that sets how public authorities interact with individuals and organisations. The HTA process fills the need for set criteria and analyses on which public authorities can base their decisions to authorise the reimbursement of technologies. HTA is therefore consubstantially at the heart of public law, placed under the mandatory rules of public authorities and calling upon all sub-branches of public law.

- Constitutional law, with the constitution as the superior norm, from which stems the hierarchy of legally binding norms and the procedures for setting them, i.e. laws enacted by a parliament or secondary legislation adopted by the executive power (either to implement legislation through or to create rules of law in specific remits devolved to government through decrees for instance). Constitutional law also spans the devolution of powers and jurisdictions over health between a federal government and its states or decentralisation of prerogatives, as is the case in countries as diverse as Australia, Brazil, Germany, Nigeria and Switzerland for instance.
- International public law, which can introduce enforceable rules through ratified bi- or multilateral agreements (e.g. Treaties). This is particularly important in view of the trend to regionalisation in the world, such as the East African Community or the European Union. Directives and Regulations are the major source of law for European Union Member-States, and some of the norms have direct effects (or direct applicability) in domestic law.

- Administrative law, with rules for organising the powers devolved to public authorities, such as ministries or public health agencies in their regulatory capacities, as well as for administrative and/or judicial appeal against decisions, i.e. in access to administrative decisions or protection of confidentiality of data or in decisions grounded statutorily on scientific advice or else facing disqualification by the judiciary.
- Public procurement law, for organisation of the provision of services, goods and works to public authorities according to mandatory principles (such as transparency, fair competition and publicity) and contract models for contracting out HTA to inform decision-makers while offering protection of independence and confidentiality, as is the case in Austria, for instance.
- Tax law, for organisation of the rules of health financing and budget allocation for health. It thus influences the introduction of reimbursable technologies and the rate of reimbursement, often enshrined in laws enacted by a parliament, such as in Belgium.
- Public health law, which can organise the overall duties and prerogatives of departments or agencies to perform HTA processes, such as the Health and Social Care 2012 in the United Kingdom, which provided a new framework for the guidelines drawn up by NICE.
- Social security law, used in planning rules for reimbursement when health insurance funds are associated with the process and even in organising the HTA process, as is the case of Article L161-37 of the Social Security Code in France, which delineates the role and missions of the French Health Authority.
- Criminal law, which can be called upon to disqualify certain conflicts of interest or to apply penalties in the event of collusion between public authorities and drug manufacturers. Criminal law also applies when the personal responsibility of a public official is recognised by a court of law or when penalties are imposed when applications for reimbursement are not made in bona fide.

2.3 Where to find the rule of law

2.3.1 Hierarchy of norms

Health decision-makers interested in HTA must learn to navigate the legal system, starting by situating it in the legal apparatus of the country. A ministry of health with jurisdiction over HTA might wish to determine the influence of public law on all HTA-related activities and the rules that apply. This requires understanding of the hierarchy of norms, which condones a principle of respect by any given legal norm of the norm that is immediately superior to it.

- constitutional rules, at the top of the pyramid, which can include fundamental rights and guidance on societal values;
- primary legislation enacted by a parliament or law-makers, when applicable, which must respect constitutional principles;
- secondary legislation, executive decisions, decrees, by-laws and regulations, which fall under the responsibility of the government for deciding whether to apply the primary legislation (i.e. the acts of parliament) or as part as their own exclusive normative remit; and
- contracts, primarily the contractual relations between public authorities and service providers backed by public law rules.

Common law systems are relatively distinct, with parliament and the executive power forming essentially a single source of law, as the prime minister and cabinet ministers in charge of implementing policy belong to the majority in parliament and derive secondary legislation power from the lawmakers:

In the United Kingdom, and other common law jurisdictions, the executive and legislature are closely entwined. The Prime Minister and a majority of his or her ministers are Members of Parliament and sit in the House of Commons. The executive is therefore present at the heart of Parliament.... Additionally, Parliament may delegate law-making powers to the Government through powers to draft secondary or delegated legislation. This can liberate Parliament from the need to scrutinise small technical details, while maintaining the safeguard of Parliamentary approval (6).

Without delving into the differences among legal systems, it is worth mentioning that the rule of law tends to be codified in civil law systems. Legal norms are enshrined in statutes and regulations, and every rule and prescribed action or behaviour by administrations, public service and public authorities is described in detail.

One characteristic of many civil law systems is a distinction of the roles and subject matter devolved to the legislative power on the one hand (lawmakers such as parliamentarians) and to the executive power on the other (government, public agencies). In practice, the executive power can adopt:

- stringent rules (such as secondary legislation or by-laws, as well as regulations) stemming from a law to interpret, provide specifications or enforce application thereof; and
- legal norms in areas devolved to government and in which the legislative power has no jurisdiction according to constitutional provisions. This is the case in France (Article 37 of the Constitution) and also in Madagascar (Article 116) and Senegal (Article 65). Overall, it must be concluded from the above that health is generally devolved to legislators, precluding executive decisions to adopt norms other than those in by-laws in application of acts of parliament in the matter of health.

This is an important distinction of which policy-makers in charge of HTA must be cognisant. For instance, if a public health code includes statutory provisions (based on legislation enacted by parliament) as well as by-laws or regulations issued by the government or a public health agency, legal norms should not conflict. Executive decisions must respect the legislation and be prescribed only insofar as those provisions are useful or operationalise the legislative norm.

2.3.2 Influence of International norms

Policy- and decision-makers should not only have a sound understanding of domestic law but are also encouraged to examine the influence and applicability of international law. The direct applicability of international or regional norms in domestic law is a striking illustration of the interactions between norms enacted by national authorities such as a parliament and a government. This is the case throughout the European Union, where European Union Treaties and Regulations are directly applicable in the domestic law of the Member States, while the Directives are equally legally binding but must be transposed by measures to incorporate them into domestic law. European Union law therefore applies directly within Member-States, and violation of European Union legal dispositions and no timely transposition of directives can be challenged in courts of law.

HTA is also susceptible to international laws and regulations that apply to domestic law. European Union Directive 2011/24 on the application of patients' rights in cross-border healthcare in particular can inform decision-makers on the boundaries of their duty in reimbursing medical technologies available to patients covered by a specific benefit package when they are provided care in another Member State of the Union.¹ Article 13 of this Directive specifies that

it is clear that the obligation to reimburse costs of cross-border healthcare should be limited to healthcare to which the insured person is entitled according to the legislation of the Member State of affiliation.

In sum, the portability of rights entails the portability of benefits. This is a crucial consideration in developing an HTA mechanism, to ensure that no unforeseen external factors bias a decision to reimburse a limited number of technologies. According to the European Commission, understanding of patients' rights in the context of the Directive requires knowledge of the Directive and related regulations as well as the case law of the European Court of Justice, which provides specifications. This is an example of the paramount importance of case law or judge-made law in understanding a legal system.

The stringent effect exemplifies progressive alignment of interests and conjoint use of legal instruments at regional level. Similar initiatives are being developed in various regions, particularly the East African Community, the Secretariat of which is engaged in long-term collaboration with the European Commission. West African countries with significant regional migration are also showing interest in this issue.

2.3.3 *Pacta sunt servanda*

The Latin brocard *pacta sunt servanda* means "contracts must be honoured" and is a general principle of international law. It is therefore one of four sources recognised and applied by the International Court of Justice (Article 38b of the Statute). It also applies in all branches of domestic law, including public business law, whereby public authorities contract out the provision of goods, services or works. Purchasing of these commodities often has to abide by specific rules and criteria, which are commonly referred to as "public procurement".

¹ This situation occurs frequently, in view of the free movement of persons enjoyed by European Union citizens since the European Union Treaty 1992 and by workers, as a fundamental principle stemming from the Treaty of Rome 1957.

Procurement of goods or services for the public sector often entails a competitive process, especially when the services are provided at a price, i.e. when public finance management imposes the respect of principles such as “equal treatment, non-discrimination, mutual recognition, proportionality and transparency” (7). Most public procurement codes and statutes in European Union Member-States include similar requirements. The rationale for this framework is partly to ensure cost-effectiveness in purchasing services with public monies, a consideration that echoes the concern of HTA decision-makers. In contracting out the assessment phase of HTA, public health decision-makers entrusted with an HTA mandate ought to understand the scope of their duties as contract award authorities but also as managers of the contract throughout its life-cycle.

Switzerland offers an illustration of the importance of the public finance criterion in application of public procurement rules. In public tendering:

the tender process is intended to make the public purchase more transparent, fair and cheaper. If the institution is private this duty is not obligatory as long as it is not indirectly funded by public money. Example: the Swiss Medical Board (SMB) produces HTA reports in collaboration with private companies. Even though the organization is an association ergo a private law entity, the SMB is obliged to undertake a tender, because of its public funding. Therefore, the SMB's decision to collaborate with an HTA producer must be based on measurable criteria. Furthermore, unsuccessful applicants have the rights to appeal against this decision (5).

Research centres that provide HTA reports are therefore subject to public procurement rules and, more generally, to public law. This is for instance the case for the Ludwig Boltzmann Institute of Health Technology Assessment in Austria, the contribution of which to HTA decision-making falls under public procurement regulations as per the Federal Procurement Act (*Bundesvergabegesetz*).

Members of academia who sub-contract out services for which they receive public funding are also considered under European Union law as under many legal systems as surrogate public authorities. They are therefore under a compulsory requirement to abide by public procurement rules in order to contract services.

The chain of contractual responsibility for abiding by public procurement principles and by public contracting rules is an important factor in the design and implementation of HTA mechanisms for health stewards.

2.4 Who will judge the judges?

2.4.1 Judicial review: the last resort

In many legal systems, appeal of an administrative or executive decision, such as a decision to reimburse health technologies by HTA authorities, must be conducted before an administrative authority before the matter is taken before a court of law. In simple terms, any individual wronged by a decision taken by a public authority can appeal before an administrative committee or commission responsible for deciding whether the decision was legal, legitimate or violated a norm or a legal framework it was deemed to respect. In the latter case, the administrative committee can rescind the decision (in, for instance, Belgium, France, Italy and Luxembourg). Numerous legal systems require completion of an administrative appeal before granting the option to challenge the decision before a court of law.

2.4.2 A specific jurisdiction

Law designed by lawmakers acting as representatives of a national sovereignty is often bent in unexpected directions by the judiciary. Case law, i.e. determination of rules by way of binding precedent based on court decisions, is of particularly importance in public law and hence in a domain applicable to HTA. This is a common trait between Common law systems and Civil law systems the latter being traditionally characterised by codified, written rules stemming from primary and/or secondary legislation. In public law, and particularly in administrative law, the body of rules spawning from court decisions provides the fabric of the public legal framework and is therefore essential to understanding the background of HTA.

One of the idiosyncrasies of administrative justice across a number of countries is the juxtaposition of two separate orders of jurisdiction, whereby special courts and judges are competent for matters involving public authorities, such as the statutory tribunals in the United Kingdom and the administrative tribunals in France (with the Council of State acting as a Supreme Court of judicature in administrative matters). Numerous countries have different orders of jurisdictions, including specialised courts and magistrates, whose remit and jurisdiction encompass every contentious area involving a public authority exercising an executive power. In Europe alone, many countries have special courts to preside over public law matters, including litigation pertaining to HTA and the broader legality of public authorities' actions: Austria, Belgium, Bulgaria, Croatia, Czechia, Finland, France, Germany, Greece, Italy, Lithuania, Luxembourg, the Netherlands, Poland, Portugal, Romania and Sweden.

Despite civil law systems principle *ubi lex non distinguit nec nos distinguere debemus* ("where the law does not distinguish, we should not distinguish"), the judiciary often grants itself the prerogative to interpret statutory texts to create what is referred to in common law as "judge-made law" to designate the capacity of the binding precedent to enrich, add specifications and help implement legal norms.

The role of the judiciary therefore consists of:

- interpreting laws and regulations when their provisions raise doubt and are at the root of litigation. This is the statutory interpretation role of the judiciary.
- assessing the legality of administrative decisions according to their scope of jurisdiction, legal grounds for action and conformity of the decision to laws and regulations in order to decide upon the potential invalidity or error of an administrative decision;
- issuing injunctions, which are court remedies that prohibit a public authority from acting in a certain way or, conversely, ordering the authority to act; and
- granting damages and compensation when a wrong results from a decision of a public authority that is not in accordance with its mandate, for instance.

2.4.3 *Ultra vires*

The capacity of public authorities to exceed their powers is called *ultra vires* and is the starting-point of judicial review of an administrative action. If the act of the public authority respects the boundary of its mandate, the act is valid and deemed *ultra vires*.

Common law systems often require that public authorities have a clear legal basis for their actions, respect the requirements of fair procedure and act in a "rational" manner. Sanctioning the unreasonableness of the administrative action of a government or public agency is therefore *ultra vires*, or excess of power. Civil law in many countries has similar *ultra vires* principles, which enable a judge to re-qualify or quash a decision adopted by a public authority. The countries include Belgium, Colombia, France, Germany, Macedonia, Togo, South Africa and the United Kingdom.

Two British legal commentators noted that

The judicial scrutiny function with regard to the executive is to ensure that any delegated legislation is consistent with the scope of power granted by Parliament and to ensure the legality of government action and the actions of other public bodies. On the application of an individual, judicial review is a procedure through which the courts may question lawfulness of actions by public bodies (8).

The judiciary may decide upon the validity of and compliance with procedural requirements of the act, including reviewing whether the administration disregarded the scope and boundary of the delegation of authority. This would be the case if an authority in charge of HTA decisions disregarded mandatory recommendations from the assessment phase. It is the judge's prerogative to stave off the consequences of unfounded or ill-founded administrative decisions. An illustration of such judicial power is a decision of a federal judge in Washington DC, USA, who ruled in December 2018 that the plan of the Department of Health to substantially reduce hospitals' reimbursement programmes because of a change in method was adopted in excess of the authority of the Department (9).

2.4.4 Creation of law through litigation: case law or judge-made law

Lord Phillips of Worth Maltravers, President of the United Kingdom Supreme Court, once said that:

the citizen must be able to challenge the legitimacy of executive action before an independent judiciary. Because it is the executive that exercises the power of the State and because it is the executive, in one form or another, that is the most frequent litigator in the courts, it is from executive pressure or influence that judges require particularly to be protected (10).

The judiciary is in essence entrusted with a duty to assess and control the action of the administration but also to offer a legal bulwark to protect people's rights. In the course of performance of this duty, courts of law enjoy the privilege of autonomy from undue influence, which enables the judiciary to create binding rules even in the absence of statutory provisions. Swiss Federal Court case law, for instance, demands that the Swiss Social Insurance Authorities, who usually follow "expert opinions", base their decisions on such informed opinion. This is no longer a matter of good practice but a stringent requirement based on the binding force of precedent and is a rule of law designed by the courts of law.

Such review by courts of law may also entail risks because of the potential uncertainty of decisions and the often unfathomable financial consequences, such as when reimbursement of medicines is not included in a benefit package as a result of the HTA process decided by judicial review. This risk is frequently associated with right-based approaches (11). Through praetorian law, i.e. legally binding rules based on the force of precedent, courts of law may override administrative HTA decisions that may have been taken on the basis of selection criteria such as sound management of public finance, value for money or cost-effectiveness.

2.4.5 Checklist milestone

At this stage, health policy-makers should understand the critical idiosyncrasies of their legal environment. In particular, they should be able to understand the legal environment

- **Review the legal system to understand which laws and regulations** found and organise the health system.
- **Determine where HTA functions or mechanisms fit or may fit** in the legal system, the branches of law involved and the statutes, legislation and regulations that apply.
- **Map the public authorities and institutions involved in HTA** and the devolution of powers, responsibilities, roles and decision-making processes.
- **Analyse gaps and bottlenecks:** Identify legal norms that should be updated, aligned, modified or amended to meet the objectives of HTA reform.
- **Review legal risks:** Identify areas in which HTA reforms to develop strategies to tackle risks may face legal difficulties, such as *ultra vires* (judicial review of an administrative decision that is beyond the authority's power or scope of work), conflict of norms (for instance between international and domestic law) or contract management issues.
- **Draw up an action plan to address the risks:** Amend existing legislation, add regulations, or simply contract management training and acquisition of new skills.

Once decision-makers thoroughly understand the idiosyncrasies of their legal system, the working of the rule of law and normative processes, the next steps will involve use of legal instruments to optimise HTA processes or institutions.

2.5 All you need is Law: Strengthening HTA reforms with legal tools

In 2018, the European Public Health Alliance described HTA as

primarily about improving the quality of healthcare, more than cost-containment. It offers solid evidence for policy-makers, operates as a gate keeper to ensure that new medicines can show added benefits, and most importantly, as a spur for genuine public health needs-driven innovation. HTA is not about rationing medicines, but about rationalizing public spending (12).

While this statement may appear subjective and perhaps not comprehensive, as HTA provides a process for selection and reimbursement of health technologies and not only medicines, the rationale for HTA is clearly sketched out: support to decision-making about the selection of technologies based on need and optimal use of limited public resources.

Through statutory rules that apply specifically to the HTA process and to broader public law, health decision-makers should strive for clarity, particularly in the following areas, summarised as the 7Cs of HTA Clarity:

- Clarity of process and selection criteria,
- Clarity of principles and values,
- Clarity of mandate,
- Clarity of responsibilities,
- Clarity of laws and regulations in the field of HTA,
- Clarity of the interactions among stakeholders and
- Clarity of the financial consequences of decisions.

The legal aspects of these areas are discussed hereunder to draw decision-makers' attention to their importance for an effective HTA process.

2.5.1 Clear process and selection criteria

No HTA template is appropriate for every country context. The legal, financial, institutional, political and social backdrop of each country determines the political economy of HTA, and health decision-makers should be wary of ready-made solutions, which often result in imbalances and do not meet their needs. While the cost-effectiveness of technologies is a prime consideration (and one recognised by most constitutional courts as a legitimate criterion for a decision to reimburse care), other criteria can be included in the decision process, such as ethical considerations or social values deemed to be essential.

Laws and regulations often specify the duty of applying such criteria as a basis for public decisions and also of informing stakeholders about the rules, either for them to apply the rules (for instance by an industry in charge of the assessment of their technology) or to inform other stakeholders about the value of the criteria, which they may challenge, leading to administrative or judicial review.

2.5.2 Clear principles and values

The wide array of public law rules, which often go beyond the scope of HTA, enshrine principles by which public authorities must abide in decision-making. The principles may apply to different levels of the hierarchy of norms. Consequently, they may have constitutional value or the value of a general principle of law, in which case lawmakers and regulators have to abide by them, or they may have legal or regulatory value and apply to authorities who are subject to the binding force of laws and regulations; therefore, a law could also quash the principles and override them, providing less protection.

The principles include accountability, evidence-based decision, impartiality, transparency and traceability, which are cross-cutting, as they tend to apply to all public activities.

- They hold the authority in charge of a decision accountable before ad-hoc administrative committees or the judiciary for respecting the basic criteria of decisions.
- They provide a framework for public procurement and therefore apply when an HTA assessment stage is outsourced for instance.
- They apply to public decisions, entailing that public data and information pertaining to a decision may be disclosed, when the information is not classified.
- They impose a duty on public authorities to ensure a legal basis for their decisions, even in the case of discretionary powers that do not preclude impartiality.
- They require legal checks to ensure that the evidence is unbiased and irrefragable, particularly in countries such as Australia or Switzerland, where industry is responsible for assessing its own products and applying for reimbursement. Protection of evidence is a crucial function of the HTA process.

2.5.3 Clear mandate and responsibilities

The scope of work should be delineated precisely by statutory rules, whether the HTA process is entrusted to a specific public authority, such as the National Authority for Assessment and Accreditation in Health Care in Tunisia, the National Health Authority in France or NICE in the United Kingdom, or devolved to the ministry of health, such as the Malaysian Health Technology Assessment Section. All HTA bodies in the European Union have several roles in addition to the HTA process, including quality standards, clinical guidelines, health care promotion, horizon scanning, registries, education and scientific advice (e.g. 13).

The prevention and management of conflicts of interest must be specified in statutory provisions, including a declaration of absence of conflicts of interest, the legal responsibility of post holders or the investigation that the administration may have to conduct to prevent any conflict. It might be difficult to circumvent undue influence when the political agenda interferes with scientific evidence, such as granting 100% reimbursement of technologies for certain pathologies to confer a distinct advantage to a category of voters, regardless of the criteria set for the HTA process.

2.5.4 Clear laws and regulations

Clear norms must be drafted to ensure that administrative decisions are not quashed by judicial review or are re-qualified because of imprecise legal drafting. Statutory interpretation by courts of law is proportional to the clarity of a legal provision. The quality of legal drafting can have a considerable effect on the health budget when a legal norm is the basis for entitlement to access to care and obligatory reimbursement by public authorities. Potential budgetary and financial consequences should be considered in assessing the robustness of the legal framework for HTA.

Health decision-makers could therefore be encouraged to design a legal pathway to HTA by harnessing relevant legal rules covering the values and principles that are considered important for HTA reform. The pathway should be comprehensive, from assessment mandate to procurement rules, and include data confidentiality and the prevention and management of conflicts of interest. Health decision-makers should consider case law and anticipate boundaries, responsibilities recognised by judicial review and practices and decisions that are likely to be overruled by the judiciary.

2.5.5 Consultation with stakeholders

Consultation with stakeholders such as representatives of users' groups, health insurance funds and health professional bodies is often mandatory by laws and regulations in the field of HTA. The results of a survey by the European Commission in 2017 offer insight into the huge appetite of health services users for greater engagement with health authorities:

the survey showed that most individuals (95%) believe that information on whether a new health technology works better, equally well or worse than a health technology already available in their country should be easily accessible to doctors to enable an informed decision when prescribing the treatment of their patients. Respondents consider that if easily available to doctors, HTA can help them to accurately inform their patients about the benefits of the new treatments compared to the current standard... In the same way, most respondents (84%) consider that information on whether a new health technology works better, equally well or worse than a health technology already available in your country should be easily accessible to patients and patients' representatives (14).

The legal decision pathway for HTA should be based on clear understanding of the broader legal framework in order to achieve the objectives set out to health authorities. This is also the competence of health decision-makers to ensure that the law evolves.

2.5.6 Checklist milestone

Once the legal framework is fully understood, legal tools ought to be used to strengthen the HTA process

- **Draw a legal pathway to HTA:** Use relevant legal rules covering the values and principles that are considered the basis for HTA reform. The pathway should be comprehensive, from the assessment mandate to procurement rules, and should include data confidentiality and the prevention and management of conflicts of interest.
- **Craft laws and regulations:** Ensure that the scope and provisions of the legal norms are clear to avoid broad interpretation, re-qualification or avoidable injunctions. Legally binding rules should cover all essential areas, such as transparency, independence of advice, confidentiality of data, prevention and management of conflicts of interest, accountability and pausing in the absence of information or an effective legal remedy (e.g. for an unreasonable delay in decision-making)
- **Take stock of the case law:** Anticipate boundaries, responsibilities recognised by judicial review and practices and decisions likely to be overruled by the judiciary
- **Anticipate the financial implications of judicial review:** Consider providing resources in the event of a judicial decision that affects reimbursement of technologies or granting a remedy to avoid adverse consequences on budgetary planning.
- **Consult relevant stakeholders:** Ensure that all relevant parties are consulted as per legal requirements.

2.6 Across the life-cycle of the Law: monitoring and adjustment (M&A)

Monitoring and adjustment of the legal framework for the HTA process can prove instrumental to its relevance over time. Obsolescence, conflict of norms and failure to incorporate new statutory rules into the HTA process could prove tangible risks and end up hamper its efficiency or lead to judicial review. Non-exhaustive recommendations are presented hereunder to ensure that laws and regulations for HTA mechanisms are both relevant and effective.

2.6.1 Avoiding “stagnant” law

Surveillance of discrepant or outdated norms can maintain an HTA mechanism that is fit for purpose and legally sound, as it will be less susceptible to decisions rescinded by administrative or judicial processes. The obsolescence of legal norms for the HTA process could have detrimental consequences, such as conflict among norms or the inapplicability of norms that have been superseded by new statutory rules that are not aligned with the original legal framework for HTA. Legal rules pertaining to HTA may appear in various documents, such as Acts and decrees. For example, the French Code of Public Health specifies the rules for listing authorised reimbursed technologies approved by the National Health Authority (15), while the Social Security Code delineates the rules for pricing and reimbursement of authorised health technologies in the HTA process (16). The combination of rules in two different acts of parliament must therefore be understood for certain essential steps in the HTA process.

Particular attention should be paid to innovations in relation to:

- the HTA process, such as the introduction of new criteria or formulations springing from the policy agenda (e.g. the increasing importance of equity and health-for-all in policies to extend coverage and improve the quality of accessible technologies and the growing demand of users to be consulted in health-related matters);
- legal instruments, such as new procurement and contracting models to improve the effectiveness of services delivered to the commissioning authority (e.g. payment on performance or delegation of powers to carry out a public service activity or to develop an information technology-intensive information system); and
- new institutional arrangements that may affect the organisation of HTA programmes, such as recent legislative recognition of international cooperation in the mission statement of the French Health Authority, opening new opportunities for sharing good practices, gaining expertise and favouring cross-fertilization.

2.6.2 Continuous review

To reduce the risks of obsolescence and inadequacy, decision-makers in HTA processes could establish an observatory of laws and regulations. Routine “horizon scanning” could be planned of pertinent branches of law to identify public law rules (as HTA is a public service activity), constitutional rules or rules resulting from ratification of international agreements that are relevant to HTA. Regular review of case law and precedents will indicate trends in statutory interpretation of the rule of law. Precedents and judicial reviews pertinent to HTA could result in a management assessment of any discrepancies between the design and aim of the HTA process and its interpretation by the judiciary and could indicate behavioural changes or practices recommended by courts of law (e.g. in the prevention and management of conflicts of interest).

An observatory could empower decision-makers to anticipate the potential impacts on HTA processes of legal, policy and societal evolution (including gender equity, consultation of users, equity or seemingly opposing demands for better data protection and for increasing transparency of public activities). Epidemiological trends in the burden of diseases should alert decision-makers to the budgetary consequences of political decisions. For instance, 100% reimbursement of treatment for noncommunicable diseases could have drastic consequences on the benefit package and the availability of financial resources for health. Areas that would require legal scrutiny are those in which changes would have drastic consequences for the HTA process, such as rights-based approaches and introduction of the right to access public data.

Such an observatory could strengthen the capacity of HTA stewards to suggest new statutory rules or amendments to existing laws and regulations. It would require continuous dialogue with lawmakers, the government and political authorities. It might therefore be recommended that a regular normative system be designed to ensure systematic M&E of the rule of law pertaining to HTA to gather feedback and innovative ideas from stakeholders. This could include benchmarking studies, reports from HTA networks or introduction of a maturity model to accompany adaptation of the HTA legal framework. This recommendation could be implemented with the support of international networks that offer such expertise and share excellence and good practices.¹

2.6.3 The era of the “right to health”: societal debate and trends

In a crowded space where competing voices strive to shape health priorities, the “right to health” seems to find a particular echo. This right is sometimes stated to be a “universal human right”, a very flimsy concept in law. However it is often defined in domestic law and can have constitutional value. This entails that lawmakers such as parliaments as well as regulatory authorities have a duty to act in accordance with the recognition of these rights.

A disambiguation must be operated: there is no right to health that can guarantee health for every one. No public authority could possibly guarantee good health to every citizen or individual in its jurisdiction. The criteria would be too difficult to define for a start. The term “right to health” is in reality shorthand for recognition of a constitutional value granted to individuals: access to a reasonable set of public services, such as healthcare or suitable water and sanitation. It could also include prevention of exposure to harmful products or a duty to extend care to protection of the environment and reduce exposure to pollution.

The right to health not infinite, unlimited or universal. It usually stems from a legal norm or constitutional value and can be summarised as the right to access care. The Constitution of Italy offers a good illustration of this blurred line with Article 32 proclaiming that “The Republic safeguards health as a fundamental right of the individual and in the collective interest and guarantees free medical care to the indigent” (17), although the philosophical principle does not necessarily match its pragmatically limited application. Other constitutions have similar clauses, including the Constitution of South Africa (Article 27), which proclaims the right to access health care; Article 31 of the Moroccan Constitution; Article 9 of the Chilean Constitution; and Article 19 of the Constitution of Madagascar. The preamble to the French Constitution states that “protection of health” is the duty of the Nation, i.e. the public authorities (18), and this was incorporated into the Constitutional norms in a 1971 judgement of the Constitutional Court.

¹ For example, the European Network for Health Technology Assessment, the International Network of Agencies for Health Technology Assessment and the International Decision Support Initiative. Details of the most important stakeholders can be found on the platform of the WHO Decide – Health Decision Hub www.decidehealth.world

Common law in the United Kingdom and other countries may differ, in view of the principle of parliamentary sovereignty combined with the fact that they do not necessarily have a single written constitutional document encompassing all supra-legislative norms. Nevertheless, ratification of legally binding international instruments that include provisions pertaining to the right to access to health care have a significant influence on domestic law of these countries.¹ Parliament as a lawmaker or the public administration as a regulator is therefore obliged to respect this norm and act accordingly. In these countries, the right to health is the duty of public authorities to define, organise and facilitate reasonable access to care for those in need. This is an important consideration for policy- and decision-makers in analysing the legal context, in four areas.

- HTA and priority-setting may result in the selection of some technologies and exclusion of access to others. This does not contradict the constitutional “right to health”, which is usually a seminal legal norm for public authorities to organise a public health service.
- Constitutional protection of health does not preclude limitation of the services and technologies that are accessible and/or reimbursed. For example, the German Constitutional Court on 5 March 2017, decided that no one has the right to obtain from the health care services reimbursement of a technology that is not authorised or included in the benefit package.
- Constitutional courts are usually cognisant of the importance of the “scientific opinions” that form the basis for public decisions to authorise reimbursement of technologies and tend to recognise a limitation to the right of judges to override the opinions of informed experts.
- The right to access health care services is also necessarily limited by the legal duty of public authorities to ensure good governance and management of public finances.²

Health decision-makers often face a challenge in justifying the grounds on which technologies are made available and reimbursed. Many constitutional courts have decided that the constitutional “right to health” does not constitute per se the right to access technologies that have not been cleared for market access or the right to claim reimbursement for accessible technologies that are not included in the benefit package. There is no guarantee that:

- exceptions may be made by constitutional judges on other grounds, such as the combination of fundamental rights, e.g. the right to life and the right to access care;
- constitutions may be revised to ensure more stringent universal rights, at the risk of unbalancing the financing of the health system; or
- courts of law may interpret norms or create law that override HTA decisions.

The matter is not simple. Stanley de Smith, a famous British lawyer, summed up this ontological difficulty as follows (19):

if one is asked what legal principles a public authority is obliged to observe when exercising a specific discretionary power, one's answer may often have to be hedged about by words like “probably” and “perhaps”. The state of the law is elusive and fluid.

2.6.4 Checklist milestone

Monitoring and
adjusting to new
legal trends

- **Avoid stagnant law:** Surveillance of discrepancies or outdated norms can maintain an HTA mechanism that is fit for purpose and legally sound, i.e. less susceptible to decisions rescinded by administrative or judicial processes, such as the introduction of new norms due to ratification of international agreements.
- **Ensure continuous review,** so that the scope of work in the HTA function remains relevant and mirrors legal, policy and societal evolution (e.g. consulting users and equitable selection criteria).
- **Establish a regular normative system** to ensure systematic M&E of the rule of law pertaining to HTA, to gather feedback and innovative ideas from stakeholders, such as benchmarking studies, reports from HTA networks or introduction of a maturity model for adaptation of the HTA legal framework.

¹ For instance, ratification by the United Kingdom of the International Covenant on Economic, Social and Cultural Rights and the European Convention on Human Rights.

² E.g. Article 14 of the French Declaration of Human Rights 1789.

2.7 Annotated bibliography (see also list of references at the end of the document)

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CHAPTER 3

Establishing institutional and governance arrangements

1 Design an institutional arrangement

- Evaluate HTA mechanism models to determine the most appropriate one for your country.
- Determine the appropriate location of the HTA mechanism.
- Identify the public authorities that are or should make decisions on reimbursement.
- Map all stakeholders relevant to HTA.

2 Build institutional capacity

- Analyse gaps in existing capacity and the expertise available and compare with those required.
- Establish a capacity development action plan to ensure:
 - understanding of assessment models,
 - commissioning of assessment exercises,
 - the situation of the appraisal committee,
 - prevention and management of conflicts of interest during assessment and appraisal,
 - public procurement and contract management and
 - understanding of the rule of law and legal duties.

3 Conduct risk assessment

- Identify potential institutional gaps, including the availability and quality of data, resources, political commitment or networking support.
- Draw up an action plan to address the risks.

4 Establish governance and an operational structure

- Establish a governance structure that covers all functions.
- Devolve authority through adequate laws and regulations.
- Plan appropriate staff, accountability mechanisms, financial resources and procedures.
- Operationalize institutional arrangements.
- Monitor and review, as appropriate, to ensure the consistency of institutional performance against the objectives.

3.1 Introduction

This chapter describes implementation of the mandate and legal framework described in the first two chapters. It describes development of institutional arrangements to support the mandate established for the HTA process. As there are strong links between institutional strengthening, development of a legal framework and assessment and appraisal, readers are strongly advised to consider all the chapters together.

This chapter addresses establishing an HTA mechanism, which is an organized entity or institutional arrangement for performing HTA tasks and delivering HTA results in a country. The HTA mechanisms considered in this document are those involved in informing reimbursement decision making.

The models of HTA mechanisms include stand-alone agencies, bodies, committees or secretariats that operate at national, regional or local (including facility) levels. Some of these mechanisms include technical assessments and the production of technical reports, while appraisal and recommendations for decisions on reimbursement are done elsewhere; others include arrangements for transforming technical reports into policy recommendations for decision-makers to consider in making decisions about reimbursement. This chapter describes good practices in setting up HTA mechanisms and the organized entities that conduct HTA. This includes a discussion of different types of institutional arrangement, considerations in choosing a model and the steps in setting up an HTA mechanism. The chapter then describes pitfalls of institutionalization and practical steps for implementing the chosen institutional arrangement. Although the chapter is addressed to government HTA agencies, it is also relevant for countries that have not yet established such an agency.

3.2 Designing an institutional arrangement

HTA models vary from small committees to strong virtual networks, technical hubs in academia coordinated by a small central secretariat to agencies that perform all HTA activities in-house. Different models may co-exist. The model of institutional arrangements for an organized HTA mechanism is chosen after three key decisions: (1) on where the appraisal process is situated, in an existing institution or as a new, independent function; (2) whether assessment is to be undertaken within the institution or by commissioning an external entity to which either industry submits dossiers or expert groups provide information; and (3) the degree of authority of the HTA function, which should have been decided when designing the mandate and legal framework but may be reviewed at this stage.

In a review of 11 appraisal committees in seven countries (Australia, Canada, Germany, the Netherlands, Switzerland, the United Kingdom and the USA), the Ludwig Boltzmann Institute for Health Technology Assessment (20) found that the panels consisted of 9–39 participants, and all were multi-disciplinary, including patient groups and in some instances industry. All the panels published the names, affiliations and statements of conflicts of interests of their members online, representing best practice. Some panels operated independently of the assessment process (for example, in Switzerland, where dossiers are submitted by an industry representative), while others managed both assessment and appraisal (such as NICE in the United Kingdom, which commissions assessments and manages their appraisal). In general, two best practices have been described: separation of assessment and appraisal in order to protect the data science components from undue influence; and explicit management of conflicts of interest in the appraisal committee.

An HTA entity can be a knowledge repository, in which international assessments and guidelines are compared and curated and recommendations are given on their application to the particular country context. In this case, the agency should have the capacity to appraise existing assessments and guidelines rather than perform assessments. Local contextual assessments can be made in-house by staff assigned to or employed by the organized entity, or they can be outsourced entirely to expert groups, academia or research bodies. When full analyses are outsourced, the HTA entity must be able to understand and appraise the quality of the commissioned analyses. Except for processes such as the Health Intervention and Technology Assessment Program in Thailand, in which all analyses are conducted internally, contract management is increasingly important, to ensure that assessments are delivered on time and in the appropriate format.

Decisions about each of these points should be based on:

- the affordability of each option for the country and the funding available;
- the human resources required and available;
- an appraisal of national HTA capacity and where it is located; and
- potential options for locating the HTA mechanism, if appropriate.

The last could be an independent public agency or a function spread across the Ministry of Health, or an arm's length agency tied to the Ministry for instance, depending on the country's legal and institutional practices.

Once there is motivation for establishing an HTA mechanism and an opportunity for doing so, the participants in the process should be identified. Most countries have a certain level of HTA-related activity, and HTA mechanisms are not established *de novo*. A situation analysis should be conducted of experience in HTA to determine the existing expertise, the resources available for an institutionalized HTA mechanism, the additional capacity to be built and who should be involved in capacity-building. This step forms the basis for a decision on which model of HTA mechanism is most suitable for the country. For example, a model of external commissioning or a mixed model is suitable for a country with HTA capacity in research agencies.

The public authorities responsible for making decisions about reimbursements (e.g. ministry of health, health insurance, other purchasing authorities) should be identified, perhaps through a review of the legal framework (see chapter 2). Any existing link between HTA activities and decision-making on reimbursement should be identified at this to determine whether such links already exist and could be formalized or need to be initiated. As mentioned in chapter 2, updating of the laws and regulations that govern the process should take the mandate of the HTA process into consideration. The institutional arrangements, such as separation of the assessment and appraisal processes and management of conflicts of interest, should be the same whether the recommendation from the HTA process is advisory or binding; however, the mechanism for communication to and feedback from policy-makers may differ. Decision-makers should be identified from the beginning and sensitized about the HTA process to ensure that they do not consider that their mandate is being superseded, which could lead to lack of engagement.

The review also serves the purpose of identifying, sensitizing and training key actors in the health system from the onset, which is critical to the success of implementation. The people to be involved in the process should not be limited to those engaged in assessments *per se* but could include:

- entities that generate evidence for HTA, which could be an organized HTA mechanism but also national and international bodies that generate data and research evidence in the health system: Multiple, diverse sources of data can be used for context-specific HTA, and those who produce and own these data should be included in a review of HTA.
- decision-makers or users of HTA: Many bodies have the power and mandate to translate HTAs into policy decisions. In the context of HTA for reimbursement, it is the bodies responsible for deciding what is reimbursed in publicly funded service provision, including the ministry of health, health insurance agencies or other actors to which these powers have been devolved.
- stakeholders directly affected by HTA results and the related decisions: These people are mainly patients, whose access to care and services depend on HTA results and decisions, but also funders of health interventions and programmes and pharmaceutical and other private companies whose commercial interests are affected by HTA decisions.

An analysis of stakeholders and an adequate strategy to engage them in each step of establishing HTA is key to success.

3.3 Building institutional capacity

HTA is by nature a multi-disciplinary process, as many skills are required for appropriately assessing and appraising technologies (Table 1). In developing a national HTA process, all the necessary skills should be identified or should be developed when they do not exist. Existing capacity might be found in ministries or departments of health, national statistics offices, academic units and other institutions that produce health data, such as a health insurance commission or nongovernmental organizations.

The available data should also be mapped, by determining who holds data on current health service use, how they can be accessed, whether there is a local price database and demographic and epidemiological data. As these data are necessary for local assessments, their availability will influence the institutional arrangement for HTA in the country.

Table 1. Non-exhaustive list of types of staff involved in the HTA process

Staff type	Function
Physicians, nurses	Development and understanding of clinical practice guidelines and implementation of interventions
Biomedical engineers	Use and effects of medical products, including development of clinical practice guidelines and contribution to budgetary requirements (purchasing, maintenance, repairs, life cycle)
Pharmacists	Use and effects of medicines
Health facility managers	Feasibility and constraints to implementation, commitment
Epidemiologists	Balance of benefits and harms, calculation of size of health impact
Health economists	Calculation of cost-effectiveness and budget impact
Legal experts	Judicial appeal (litigation); commissioning of assessments, contract negotiation; management of conflicts of interest (and prevention in case of advisory services)
Ethicists	Application of ethical criteria
Patients and civil society organizations	Public engagement and stakeholder involvement
Communication officers	Communication of results in a transparent manner to ensure that the population is aware of and understands their benefits and rights

Once the existing staff has been mapped, a plan can be made for capacity development. Although it will depend on the model chosen for HTA, capacity should be built for the entire HTA process. The capacities illustrated in Fig. 7 might have to be developed further to support HTA processes. Technical assistance in capacity development is available from many of the global HTA networks, or a technical assistance partner could be found through the Decide Health Decision Hub. (See chapter 1.)

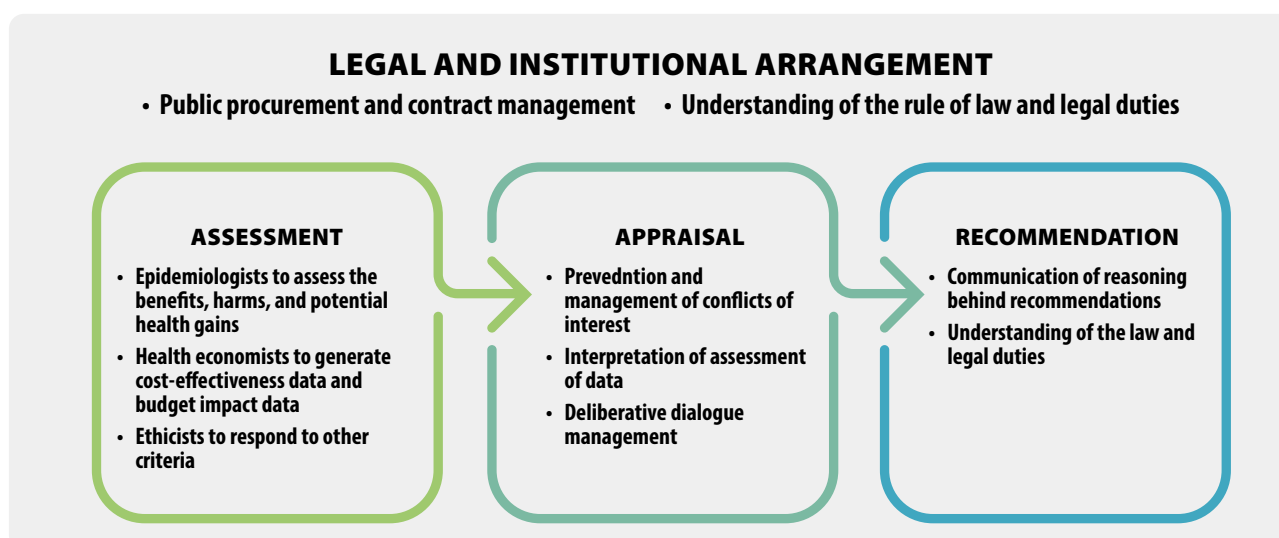


Fig. 7. Capacity that should be developed during institutionalization of reimbursement

3.4 Conducting risk assessment

The risks associated with drivers that create barriers to or facilitate establishment of HTA mechanisms in a country (Table 2) must be carefully considered.

Table 2. Framework for risk assessment

Driver	Risks
Availability and quality of data	Poor assessment reports lead to poor decision-making and undermine confidence in the value of HTA.
Cultural aspects	Resistance from stakeholders to changing the model of decision-making, perception that HTA will be used for rationing and cost control
Financial support	An unsustainable system and poor-quality work undermine confidence in and the credibility and legitimacy of the process.
Health system context	<p>Difficulty in translating evidence from international HTA to the local context.</p> <p>Use of HTA recommendations at all levels of care, in public and private health care, in public health programmed and in curative services, can complicate the HTA approach.</p> <p>When the purchasing system is fragmented, some purchasers and not others may consider HTA, creating differences in access.</p> <p>Highly decentralized systems may require adaptation of HTA institutional arrangements to subnational settings.</p>
Political support	The absence of political support may compromise funding and acceptance, make it difficult to systematize the process, reduce the likelihood that recommendations will be used and increase the likelihood that they will be less influential than they should in decision-making.
Stakeholder understanding and acceptance	Resistance of stakeholders to implementation of the recommendations, capture or distortion of the process, misrepresentation of the motives, pressure and lobbying by specific groups.
Networking	<p>Networking increases the likelihood of use of good practice and of improving the quality of HTA. The absence of networking increases the risk of isolation from the HTA community, duplication of effort and repeating mistakes.</p> <p>The disadvantages of networking include the commitment of time, potential reaction to an external time frame and process and influence towards an operating model that is not tailored to the country.</p>

Other risks include the following.

- Unrealistic expectations about the size and mandate of the organization: It is better to start with small arrangements, according to country capacity and available funding, followed by sustainable growth. Establishment of a large agency may be costly and require much capacity.
- Regardless of the institutional structure, conflicts of interest, data and confidentiality must be managed transparently. Countries often disregard these aspects if the organized entities are small, integrated into other agencies or constitute virtual networks. They may begin to consider these aspects only when they establish larger, independent agencies, when it may be too late.
- Unclear boundaries between the technical function of generating HTA evidence, the function of formulating recommendations and final decision-making. Safeguards must be in place to avoid overlaps of functions and conflicts of interest. Specifically, the institutional arrangement that separates assessment from appraisal and separation of the production of scientific data from conflicts of interest must be extremely clear from the outset.
- Priorities that change according to political priorities and pressures or changes of governments. The HTA process must have sufficient independence from changes of government and sufficient protection from political influence to avoid this risk.

An action plan for identifying and managing risks, the probability of risk, the level of risk and strategies for avoidance should be drawn up. For example, unrealistic expectations of the HTA mechanism could be identified as a serious risk with a high level of probability due to the political environment. The risk management strategy could include a communication strategy to sensitize decision-makers and other stakeholders about the time required to develop an HTA mechanism, the number of staff required and their capacity to perform at the level expected by stakeholders. The communication strategy could lower expectations on the basis of the staff and data available.

3.5 Establishing governance and an operational structure

Once a situation analysis has been conducted and existing capacity has been mapped, the staff capacity necessary for different HTA mechanisms should be considered in order to develop the institutional model. Table 3 lists examples of the budget required per HTA, which depends on several factors, including locally available resources for the assessment process (e.g. labour and office space) and the type of assessment process chosen.

Table 3. Required HTA capacity and costs in various countries

Country	Entity	No. of staff	Time required to produce an HTA	Cost per HTA (2011 US\$)	Operating budget (2011 US\$)
Australia	Pharmaceutical Benefits Advisory Committee	18 members; > 40 support staff (in Ministry of Health); 5 contracted external evaluation groups	Dossier assessment, 8–9 weeks	~ 60 000	15 million (0.01% statutory health insurance operating budget)
Australia	Medical Services Advisory Committee	4 executives and 20 additional staff with expertise in clinical medicine, health economics and consumer matters	13 months (12–13 evaluations conducted per year)	~ 250 000	
Australia	Prostheses List Advisory Committee	16 staff; independent board of members with expertise in clinical practice, health insurance, consumer health, health economics, health policy, private hospitals and the medical device industry	Not available; list updated semiannually	Not available	
Brazil	Department of Science and Technology, Commission on Technology Incorporation	30 total	Quick review, 3 months; primary studies, 1–2 years	15 000–150 000	Not known
Colombia		Total staff 63, comprising expert commissioners and 20 technicians with expertise in clinical medicine, economy, public policy, statistics, actuarial sciences	3–4 months	~ 6000–10 000; 250 000/clinical practice guideline	Not known
Germany	Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen	122 (in 2011)	Full reports, 18 months; rapid reports, 4–6 months; dossier assessment, 3 months	65 000–650 000	19 million (0.01% statutory health insurance operating budget)
Poland	Agency for Health Technology Assessment in Poland	~ 55 staff	Full HTA report generally 2–3 months	28 000–43 000	3.8 million (0.018% of completely separate national Health Fund budget)
Thailand	Health Intervention and Technology Assessment Programme	50 staff (39 researchers and 11 administrative staff)	9–12 months	17 000, not including dissemination	1 million
United Kingdom	NICE	500 staff	7–14 months	320 000–400 000/clinical practice guideline; from 90 000 for review of manufacturers' submission to 230 000 for de novo systematic review and decision model	~ 90 million (0.06% of National Health System annual budget)
Uruguay	National Resources Fund	Most studies contracted out; 6 institutes and about 60 experts produce most assessments upon request.	3 months	3000–15 000	Not known

Source: adapted from reference 21.

This table demonstrates that even a low input of HTA processes can provide advice on many reimbursement decisions every year. A stable budget is necessary to develop a sustainable HTA mechanism. The available data indicate that less than 1% and probably about 0.01% of the health budget is required for a functional system. The budget may be an allocated line item in the health budget, or, if the model of industry submission is preferred, a fee should be charged to the company for each submission to cover the cost of dossier assessment.

In Australia, dossiers for the assessment of pharmaceuticals are submitted to the Pharmaceutical Benefits Advisory Committee and contracted external evaluation groups assess the dossiers. This system costs the Australian Government approximately US\$ 15 million per year in 2011. This indicates that, at a cost of approximately US\$ 60 000 per HTA assessment, more than 200 HTA assessments were conducted per year. In Thailand, all assessments are undertaken internally by the Health Intervention and Technology Assessment Programme at a cost of approximately US\$ 17 000 each. The Programme had an operating budget of approximately US\$ 1 million in 2011, including for assessment processes, capacity development and communications. In 2017, the Programme reported that 150 assessments had been completed in the first 8 years, indicating that approximately 20 assessments can be conducted each year.

Different assessment models may have different effects on financing requirements and the capacity of the system to issue HTA reports. If capacity and financing are limited but a large number of assessments are foreseen, the complex assessment process can be circumvented by borrowing data from other sources while capacity is being developed. For example, in the Romanian HTA system, the criteria for assessment include HTA decisions from France, Germany and the United Kingdom, the number of European Union countries that offer reimbursement, a development of a local real-world data study and a local budget impact assessment. This process enabled Romania to review 200 medicines in 1 year with a lower budget outlay than that in Australia.

3.6 Annotated bibliography

- Priority-setting in health. Building institutions for smarter public spending. A report of the Center for Global Development's Priority-setting Institutions for Global Health Working Group. Washington DC; 2012:50-1 (https://www.cgdev.org/sites/default/files/1426240_file_priority_setting_global_health_FINAL_0.pdf, accessed April 2020). This book contains evidence for the role of priority-setting institutions. In particular, chapter 6 describes institutionalization of HTA mechanisms, possible obstacles and mitigation strategies. As referenced within this chapter, table 3 is drawn from this publication, and the full explanation should be referred to by users of this guide.
- Patera N, Wild C. Assessment – APPRAISAL – Decision: (Good) Practice examples and recommendations. Decision Support Document 72. Vienna: Austrian Institute for Health Technology Assessment GmbH; 2014 (<http://eprints.hta.lbg.ac.at/1036/#>, accessed April 2020).

This paper provides 11 examples of appraisal panels for HTA, their composition and mandate. They provide recommendations on how to appropriately put together and engage with an appraisal panel. In particular section 4 on recommendations for appraisal panels should be reviewed.

CHAPTER 4

Processes and evidence required for assessment and appraisal

1 Establish an appraisal committee

- Identify and involve all relevant stakeholders. Consider reviewing the situation assessment outlined in chapter 3.
- Define the HTA process and what the HTA mechanism is intended to do. Review chapter 1 for the mandate and chapter 2 for the legal framework for defining the responsibilities of the HTA mechanism and ensure that the procedures are aligned with the mandate.

2 Scoping

- Select decision-making criteria for assessment and appraisal; involve the appropriate stakeholders to ensure that local values are represented in the decision-making process.
- Nominate interventions for assessment and appraisal, or request nominations from those with the authority to do so.

3 Assessment

- Identify how each criterion is to be reported.
- Develop reference cases for each criterion to ensure consistency.
- Assess interventions against the criteria identified in the scoping phase.

4 Appraisal

- Prepare the terms of reference of the appraisal committee, including processes for managing conflicts of interest.
- Discuss the relative importance of the selected criteria.
- Collect additional data when relevant for the discussion.
- Develop recommendations.

5 Communication and appeal

- Decide on a process for making the decision and the underlying argumentation publicly available.
- Ensure that appeal mechanisms are in place (see chapter 2 on legal frameworks).

4.1 Introduction

This chapter addresses the assessment and appraisal of health interventions within HTA mechanisms, with a strong focus on legitimacy, indicating that the process should be evidence-based and fair and perceived to be so by all stakeholders. The chapter does not provide a prescriptive list of criteria or methods for decision-making but describes the selection of criteria in a fair, transparent manner. Although this chapter addresses government HTA agencies, it is also relevant for countries that have not yet established such an agency.

4.2 Conceptual framework

The goal of HTA is to support health authorities in making well-reasoned, legitimate decisions about reimbursement (1,22,23) “legitimacy” referring to the fairness of recommendations or decisions and their perception as such by stakeholders. This is an important prerequisite for broad societal support for recommendations or decisions that have significant impact on the population’s health and well-being. It also implies that evidence must be used when relevant. Legitimate decision-making thus requires approaches that are both fair and evidence-based.

HTA agencies can organize decision-making processes in different ways, which may affect the legitimacy of the decisions made (1,24). In general, many HTA agencies around the world could improve their decision-making processes and thereby enhance their legitimacy.

4.2.1 Fair processes

Recommendations or decisions may be perceived as fair because they result from a clear, transparent process or because they also represent widely held moral values, such as reducing inequity or maximizing well-being. Even if there is agreement on the importance of the values, however, there may be debate about how many and which should dictate each given decision. It would therefore appear to be easier to agree on a process for making decisions and hold that the decisions are legitimate if the recommendations result from a fair decision-making process. This approach was proposed by Daniels and Sabin in their framework for “accountability for reasonableness” (A4R) for fair decision-making (22,25). They defined a fair process as a deliberation that meets four conditions: (i) publicity (the decisions and the justifications for those decisions must be transparent and publicly available); (ii) relevance (all relevant stakeholders should be given the chance to provide arguments that contain reasons and principles that are accepted as relevant by all); (iii) “revisability” (a mechanism should be in place for stakeholders to appeal against decisions, propose revisions and receive a reasoned response); and (iv) enforcement (a process in place to ensure that the above conditions are met). The A4R framework is used increasingly to support reimbursement decisions, e.g. in Australia, Canada, the Netherlands, New Zealand, Norway, Sweden and England and Wales (United Kingdom) (26). While the framework has the advantage that it can be implemented without prior agreement on the specific values for guiding decisions, that may also be seen as a disadvantage, as deliberations will be very broad if the values that guide decisions have not been identified previously and the criteria have not been established to operationalize them (27).

4.2.2 Substantive criteria

To address the disadvantage of an exclusively procedural approach, specific criteria have been developed for decision-making (e.g. cost-effectiveness or priority to people who are worse off). These so-called “substantive” criteria provide guidance and useful input to the process (see step 2.2 below). Multi-criteria decision analysis (MCDA) is an increasingly important framework for systematic, quantitative or qualitative assessment of the impact of these criteria on decisions. MCDA has been used to in guide coverage decisions in Colombia, Italy and Thailand (28). In 2016, the ISPOR Emerging Good Practices Task Force issued guidelines on best practice for MCDA in supporting health care decision-making (29). The Task Force confirmed that MCDA might be applied to decisions informed by HTA but that some methodological challenges should be addressed before it could be implemented. MCDA lacks a deliberative component and does not indicate how criteria carrying moral weight should be balanced against each other when making decisions to ensure that those decisions are fair.

4.2.3 Frameworks for fair process and substantive criteria

A number of frameworks have been proposed that provide guidance to countries on decision-making for moving towards universal health coverage and for making reimbursements (23,24,30–33). This chapter describes one such framework, “evidence-informed deliberative processes”, which integrates fair process (i.e. A4R) and substantive criteria (or “values”) to enhance the legitimacy of decisions. Evidence-informed deliberative processes are based on both early, continued stakeholder consultation to understand the importance of relevant social values and also on structured, rational decision-making through evidence-informed evaluation of the identified values.

4.3 Five steps in organizing processes

The conceptual framework has important implications for how HTA agencies should ideally organize their processes (23,34). The framework has five steps:

- 1 Set up an appraisal committee.
- 2 Scope interventions.
- 3 Assess interventions.
- 4 Use results of the appraisal to make recommendations.
- 5 Communication and appeal

This framework should not be considered a blueprint for HTA agencies but rather as an aspirational goal towards which agencies can take incremental steps.



HTA agencies should involve relevant stakeholders throughout the HTA process. As a first step, agencies are advised to establish an appraisal committee with permanent members who endorse the broad public interest and are responsible for developing recommendations through a deliberative process. Temporary members can be included to represent specific stakeholders, including their interests and expertise, with their appointment dependent on the recommendation under scrutiny. The appraisal committee is essential to the HTA process and is involved in all subsequent steps.

“Stakeholders” are defined as the people or organizations that might be affected by a decision on reimbursement of an intervention. Key stakeholders may include representatives of the ministries of health and of finance, the national health insurance agency, health professionals, civil society organizations and patient and carer groups. All relevant stakeholders must be included. Their participation should be politically mandated and institutionalized; otherwise, they risk being ignored or they may ignore the HTA process (see chapters 2 and 3). Stakeholder involvement in HTA processes serves to identify the full range of relevant societal values in relation to a particular recommendation, assure collection of relevant evidence on these values and improve understanding of the values of other stakeholders.

No appropriate stakeholders may be identified for some decisions, because it cannot be determined beforehand who will be directly affected by an intervention (e.g. preventive interventions). In general, the committee must always in its deliberation consider the widest possible array of arguments, even if they are not put forward by a member of the committee. A diverse committee facilitates this aspect of the process.

Although the HTA process should meet the conditions of A4R, the process also requires specification of, for example, how often decisions will be revised. Moreover, the condition of publicity can be met in various ways, such as making a summary of decisions and the rationale supporting them publicly accessible by posting them on a website or allowing the public to participate in some discussions. The box below provides examples of inclusion of stakeholders in appraisal committees in two countries.

In the **Philippines**, the appraisal committee comprises members with various backgrounds, including public health (epidemiologist, biostatistician–statistician, economist–health economist and public health professional); clinical science (medical doctor, nurse, pharmacist, health professional organization); bioethics and sociology (sociologist, ethicist); legal, engineering and information science (biomedical and clinical engineer, lawyer, librarian, information specialist) and consumers (representatives of civil society and patients).

In **Tunisia**, the reimbursement committee is chaired by the Director-General of Social Security and includes physicians from the Ministry of Social Affairs, representatives from the Ministry of Health (Director-General of pharmacy and medicine) and representatives of the National Insurance Fund. A new HTA-based decision-making mechanism will shortly be in place, as proposed in a public–private dialogue. A new appraisal committee for decisions on pricing and reimbursement will be established, with stakeholders from the ministries of Social Affairs and of Health, the national insurance company, the Ministry of Commerce, the National Authority for Assessment and Accreditation in Health Care, academics, statisticians, health economists, public health professionals and potentially other stakeholders.

Now would be a good time to reflect on the following steps on the checklist

- Identify and involve all relevant stakeholders. Consider reviewing the situation assessment outlined in chapter 3.
- Define the HTA process and what the HTA mechanism is intended to do. Review chapter 1 for the mandate and chapter 2 for the legal framework for defining the responsibilities of the HTA mechanism and ensure that the procedures are aligned with the mandate.

2 Step 2. Scoping

Agencies are advised to introduce “scoping”, which is systematic exploration of relevant aspects of a specific problem area from the perspectives of, for instance, patients, informal carers and health professionals. The scoping phase should result in a clearly defined policy question of direct significance to decision-makers. To form the question and facilitate the process, the PICO (patient or population problem; intervention; comparison or control; and outcome) framework can be used. Important

elements of scoping include nomination of interventions for assessment and the choice of relevant values to be considered.

Nomination of interventions

In the scoping phase, the HTA agency coordinates the nomination of interventions for assessment. Countries select interventions for an HTA according to local values and considerations; however, the set process ensures that all interventions are treated equally. In Chile and Colombia, the ministries of health set priorities for interventions during strategic planning, which guides selection of interventions for the HTA mechanism. In Thailand, seven groups of stakeholders (health professionals, academics, patients, civil society, policy-makers, the health care industry and citizens) may nominate a maximum of three interventions for consideration at one point in time during the year, and from all nominations, 10 interventions are selected for analysis. The initial decision is based on six prioritization criteria: the size of the affected population, the severity of the problem, the effectiveness of the intervention, variation in practice, the impact on household expenditure and ethical and social implications (35), with a scoring system for each criterion (36). The minutes of the consultation for topic selection are distributed to all stakeholders. In many high-income countries, such as Australia, Canada, Norway and the United Kingdom and increasingly in countries such as Brazil and the Republic of Korea where the HTA mechanisms are still developing, national horizon scanning is undertaken, usually by HTA agencies, to identify new and emerging technologies, which are then filtered and selected for evaluation by nomination (37). The filtering is often done according to disease burden, potential health benefits in comparison with current standards and potential side-effects.

The criteria applied in nominating interventions are often (38):

- whether the intervention adds to or replaces current treatment options, and, if it replaces current care, how the previous technology is used and if it is reimbursed;
- health benefits for both patient and the population;
- regulatory considerations;
- pricing and reimbursement;
- factors that affect appropriate dissemination (e.g. acceptance by health care systems and society);
- utilization of other health technologies; for example, introduction of faster, cheaper next-generation sequencing (molecular profiling) might increase the use of targeted medicines in cancer; prevention of Alzheimer disease could reduce the need for care homes for dementia patients);
- unit cost or budget impact; and
- ethical and legal considerations.

While ethical considerations are presented separately on this list, ethical judgement should be central to balancing all aspects in the nomination of interventions.

Further important considerations are inclusiveness and transparency. On the one hand, when the ministry of health or a horizon-scanning unit selects interventions, the public, patients and industry stakeholders are not involved, which can reduce confidence in the process. On the other hand, although Thailand's system is considered to be extremely inclusive, the nomination process creates a heavy work load for the HTA mechanism. These opposing weaknesses should be balanced. Transparency can be increased by publishing an overview of topic selection, as is done by NICE in the United Kingdom since January 2015 (39).

Choice of decision-making criteria

In the scoping phase, the appraisal committee may deliberate and agree on meaningful, relevant questions for the assessment and subsequent appraisal of interventions and relevant evidence and values. This enables a timely collection of all evidence considered to be relevant. Countries must decide whether to define a set of criteria that are considered relevant for all interventions and are to be used consistently in the deliberation or to use a purely procedural approach, in which criteria are not defined beforehand. This section describes use of defined criteria, as is the process used for most countries with an institutionalised HTA mechanism.

Almost all countries with institutionalized HTA use at least three common substantive criteria: the quality of the evidence, effectiveness and cost consequences (usually cost-effectiveness). These are based on the widely recognized goal of improving or maximizing population health by the use of interventions that are proven to be effective and cost-effective. Use of a broader set of criteria is, however, increasingly being recommended, especially in the context of priority-setting and reimbursement decisions for universal health coverage (31,40,41), because global organizations and many countries in their national policy documents have formally committed themselves to the global goal of universal health coverage. This goal has three guiding criteria: equitable access, fair distribution and financial risk protection (2,42).

Table 4 lists a number of examples. In the United Kingdom, NICE identifies the most cost-effective services through open, accountable HTA, while also taking social value into consideration, as recommended by their Citizen's Council. Priorities are recognized in clinical practice guidelines and reimbursement rules. In Thailand, the Health Intervention and Technology Assessment Programme appraises health technologies and public health programmes by cost-effectiveness and budget impact (21). Ethiopia identified an essential health service package in 2005 with six criteria: cost-effectiveness, affordability, equity, necessity, human resource capacity and accessibility. In Germany and the USA, comparative effectiveness analysis is used widely but only to assess the quality of evidence and effectiveness. In these two countries, cost-effectiveness analysis is not used for reimbursement decisions (45,46).

Table 4. Criteria used for decisions on reimbursement in 11 countries

Country	Decision Criteria
France	Severity of disease and impact on morbidity and mortality Clinical efficacy, effectiveness and safety of the medicine Reason for use of a drug (preventive, curative or symptomatic) Therapeutic strategy in respect of alternatives Impact on public health (burden of disease, community health, relevance of clinical trial results)
Germany	Additional benefit over a relevant comparator with regard to: health status, survival, duration of disease, quality of life, risk Additional benefit, decided by joint Federal committee
Italy	Therapeutic characteristics (including relative value with standard of care) Disease-specific criteria (severity of illness, size of target population, medical needs) Results of clinical trials Risk–benefit studies (comparison with existing therapies) Cost–effectiveness analyses (often provided by manufacturers) Cost in comparison with other interventions Production methods and costs
Norway (48)	Health gain Resource use Severity of disease
Spain	Absolute therapeutic value of the product with respect to the severity, duration and consequences of the condition, a clinical need, therapeutic and social value Degree of innovation Price in comparison with that of alternatives Budget impact
Sweden	Cost–effectiveness (cost–utility) from a social perspective Marginal benefit over alternative treatments Severity of the disease Unmet need for a new drug Social criteria: vulnerability of patient groups, impact on equity and ethical dimensions
England, Ireland and Wales	Appropriateness and relevance in comparison with other technologies Clinical effectiveness, risks and health-related factors Cost-effectiveness (cost, quality-adjusted life years) Non-health factors (considered socially valuable)
Scotland (Scottish Medical Consortium)	Clinical effectiveness and risks Cost-effectiveness (cost, quality-adjusted life years) Budget impact
Philippines	Effectiveness Cost-effectiveness Household financial impact Magnitude and severity with regard to equity
Thailand	Cost-effectiveness Budget impact
Tunisia	Clinical benefit in comparison with standard of care and transferability of clinical trials results Potential risks Budgetary impact Cost–effectiveness

Source: reference 47.

Several frameworks are used to identify criteria for priority-setting, and none is applicable in all cases (40). Nevertheless, *impartiality* is a fundamental guiding principle in all systems. All interventions should be selected for reimbursement by the same comprehensive criteria (49). The HTA mechanism contributes crucially to this goal, and HTA agencies set relevant, socially accepted criteria for their setting.

The WHO consultative group on equity and universal health coverage has proposed three criteria for priority-setting for universal health coverage: cost-effectiveness, priority to the worse off and financial risk protection (2). These general principles are widely accepted and recommended as core criteria for use in HTA for reimbursement decisions in most countries.

Basing reimbursement decisions on the cost-effectiveness of interventions is important because it will improve the health of populations.¹ This criterion operationalizes the value of improving the health of a population as much as possible with any budget. All things being considered, failure to improve health as much as possible would have substantial opportunity costs in terms of healthy life years foregone (50). The quality of the evidence of effectiveness and the degree of effectiveness must be assessed before a cost-effectiveness analysis can be performed. These concerns are captured under cost-effectiveness criterion (51–54).

Giving priority to the worse off is important because it captures the value of fairness, which calls for interventions to be provided according to need in order to reduce inequality (55). The worse off can be defined as: a) those with least health (or the most severe and large individual disease burden) without the intervention, or b) the poorest or otherwise disadvantaged (gender, area of living, or marginalized groups (2). As the most cost-effective services do not always benefit those who are worse off, a decision on reimbursement might include consideration of assigning extra value to health benefits for these groups. In practice, this implies that some interventions that are not considered to be cost-effective might still be reimbursed because they promote fairer distribution of health and access to health care (56–58). The opportunity cost of giving priority to the worse off in terms of lost health for the better off must be considered. Evidence on equity impact or who are the worse off may be hard to find, but a small and growing literature is now available (for references, see 57).

Financial risk protection is an important criterion because some health services require substantial out-of-pocket payment, which can impoverish people (2,59). Two measures of “effective purchase of financial risk protection” that are often used are catastrophic health expenditures averted and poverty cases averted (60,61). The health benefits of less cost-effective services that provide high financial protection (at an acceptable cost) could be assigned extra value, so that some interventions that are not considered cost-effective may still be reimbursed because they provide substantial financial risk protection (59,62). There is little evidence on effective purchase of protection from financial risk, but some studies are available (60).

All the criteria must be further specified and balanced for the country context, and other criteria may be locally relevant (29,63,64). HTA agencies can draw up a checklist of all potentially relevant criteria that are appropriate in their setting according to national values, citizens’ preferences and national policies, laws and regulations and use these criteria to collect evidence.

Now would be a good time to reflect on the next steps on the checklist

- Select decision-making criteria for assessment and appraisal; involve the appropriate stakeholders to ensure that local values are represented in decisions.
- Nominate interventions for assessment and appraisal, or request nominations from those with the authority to do so.

¹ It has been argued that the burden of disease (e.g. disability-adjusted life years lost) associated with a given condition or risk factor should be an independent criterion. In our view, the underlying concern is captured by cost-effectiveness. If an intervention can avert many disability-adjusted life years at an acceptable cost, it is by definition cost-effective.

Step 3. Assessment

In the assessment phase, agencies are advised to collect evidence in accordance with the values and criteria identified and the relevant questions (PICO) to be addressed. The glossary of Health Technology Assessment International¹ defines assessment as

A scientific process used to describe and analyse the properties of a health technology – its safety, efficacy, feasibility and indications for use, cost and cost-effectiveness, as well as social, economic and ethical consequences.

An HTA agency may consider including other aspects, depending on the context and characteristics of implementation and patients for the intervention. A standard reporting format could be used to synthesize the evidence, such as that used by the Canadian Agency for Drugs and Technologies in Health to prepare reports of economic evaluations. To increase the plausibility of a report, experts and other stakeholders could be consulted.

Below, we review sources of information for the three criteria often used in HTA reports – effect size, cost-effectiveness and budget impact; similar considerations should be made for all the criteria used in HTA. Consideration should also be given to who should generate the evidence for an assessment: employees of the HTA agency, external academics or other partners, or pharmaceutical companies, and, in the last case, who should be responsible for an independent review.

Evidence for the effectiveness of an intervention can be derived from the global literature and does not usually have to be replicated in each setting. WHO considers evidence of safety and efficacy in adding drugs to the Model List of Essential Medicines and through the Guideline Review Committee. The Cochrane Collaboration publishes high-quality systematic reviews of evidence. When developing protocols for the HTA mechanism, acceptable levels of evidence – such as high quality meta-analyses, systematic reviews of randomised controlled trials, or randomised controlled trials with a very low risk of bias for pharmaceuticals – should be established. Different types of evidence may be suitable for different types of intervention; for example, it may not be feasible to conduct randomized controlled trials for legislative interventions, and alternative evidence will have to be collected.

To ensure consistency in evidence for cost-effectiveness, a guideline or reference case could be used. The case may be developed locally, as in Thailand, or an international reference case can be used (65–68) with adaptations to the local context. The results may be influenced by many factors, such as the costing perspective, the prices used, the analytical timeframe and discount rates. Consistency must be maintained for these factors to ensure comparable cost-effectiveness ratios for different interventions. Although cost-effectiveness ratios in different settings have general consistencies, some aspects of the analysis are not transferrable, as local prices and delivery mechanisms and the burden of disease influence the cost-effectiveness ratio. An international model will require some adaptation to a local setting.

The data collected should be peer-reviewed independently of those who conducted the analysis, by professionals who have no conflicts of interest. For example, in Australia, academic centres are contracted to review data submitted by pharmaceutical companies to the Pharmaceutical Benefits Advisory Committee. In Brazil, the HTA agency can request additional research and evidence from external parties, although there is no formal procedure for inclusion of other stakeholders in submitting or reviewing evidence in the assessment phase.

Now would be
a good time to
review the
next steps on
the checklist

- Identify how each criterion is to be reported.
- Develop reference cases for each criterion to ensure consistency.
- Assess interventions on the basis of criteria identified in the scoping phase.

¹ The HTAi Glossary is available online at <http://htaglossary.net/HomePage>

4 Step 4. Appraisal

In this step, members of the appraisal committee (if established) scrutinize and interpret the evidence and other information collected in the assessment phase. The linkage between the assessment and appraisal steps should therefore be considered. It is, for example, remarkable that the assessment and appraisal phases do not appear to be aligned in some countries, although it is the remit of a single organization to be responsible for the link between both phases (e.g. the National Health Authority

in France, the Pharmaceutical Benefits Advisory Committee in Australia). An HTA body may not intrinsically consider themselves responsible for comprehensiveness for several reasons. In Australia, for example, the HTA body does not perform HTAs but relies on information provided by manufacturers and HTA contractors. The appraisal process should be transparent and explicit and preferably described in a publicly available document in order to be fair to stakeholders.

HTA agencies should use the evidence collected to recommend whether an intervention should be included in the benefits package and provide argumentation for the role of each value or criterion in making the recommendation. In order to develop recommendations on the ranking of services into priority classes, the appraisal committee should make balanced judgements about the importance of the criteria and how the interventions meet them. Various strategies can be used to make such judgements, such as “structured deliberation”, which can be used to distinguish between quantifiable and non-quantifiable criteria. An example of the former is “cost-effectiveness”, and an example of the latter is “responsibility for one’s own health”, i.e. the extent to which an individual can be held accountable for a disease (which is difficult to quantify). Agencies can define quantifiable trade-offs for the quantifiable criteria, such as between “cost-effectiveness” and “severity of disease”, in which the maximum allowed cost-effectiveness ratio may be lower for more severe diseases (58,69). This will result in an initial recommendation to reimburse an intervention or not. HTA agencies should then deliberate on all the remaining criteria, which might alter the initial recommendation. We recommend that agencies report how each criterion affects the initial judgement (neutral, positive or negative), with supporting arguments. A checklist of potentially relevant criteria can foster a systematic appraisal. HTA agencies need not establish quantitative trade-offs in the appraisal phase, they may instead consider all trade-offs in a deliberative process.

Several European HTA agencies have set good examples of the use of structured deliberation in practice. The National Health Care Institute in the Netherlands applies a decision rule that relates “cost-effectiveness” to “severity of disease”. Thus, the cost-effectiveness threshold is €80 000 per quality-adjusted life year for an intervention against a medical condition with a severity greater than 0.71 on a scale from 0 to 1 and less than €80 000 for less severe conditions (70); subsequently, other considerations that may affect the initial recommendation are included through a framework of structured deliberation. In the United Kingdom, NICE uses a similar approach, which it refers to as “structured decision-making”, in which cost-effectiveness is traded-off quantitatively with the criteria “end of life” and “very rare disease”, which are explicitly operationalized for this purpose. NICE also allows additional considerations to affect the overall recommendation (71). A similar practice is used in Norway (48).



Review the next steps on the checklist

- Prepare terms of reference for the appraisal committee, including processes for managing conflicts of interest.
- Discuss the relative importance of selected criteria and how the appraisal committee should consider and interpret the information.
- Collect additional data when relevant for the discussion.
- Make recommendations..

5 Step 5. Communication and appeal

In the fifth step, HTA agencies should publish their argumentation, introduce options for appeal (for example, to request reconsideration of the evidence) and organize M&E after implementation of their recommendations. In general, HTA agencies should subject their decision-making criteria and related processes to public scrutiny.

We strongly recommend that the appeal process has a strong legal basis. Decisions should be reviewed regularly, on the understanding that the data underlying a recommendation change with changes in costs and epidemiology. An interval after which an intervention can be re-nominated should be proposed. As an example, in the Philippines, appeals are allowed in written form with supporting documents. In France, pharmaceutical products listed for reimbursement in community pharmacies are re-assessed every 5 years, and pharmaceutical products are re-assessed at any time if significant new information becomes available.

Consider the final two steps on the checklist

- Decide on a process for making the decision and underlying argumentation publicly available.
- Ensure an appeal mechanism (see chapter 2).

4.4 Discussion

This chapter described the ideal organization of processes for making recommendations in HTA agencies (23,34). They have a duty to ensure ethical standards in their processes, including promotion of democratic values and strengthening of democratic governance. While agencies should strive to reach the goal of legitimate reimbursement decisions, they may do so in incremental steps.

A review of HTA practices around the world (24) showed that the conditions for revision and enforcement are not yet fully met, even in countries with explicit HTA processes. Also, the extent of stakeholder involvement throughout the HTA process (part of the principle of relevance) differs among countries. The steps that can be taken depend on the local context, including the degree of HTA institutionalization, health system factors (e.g. governance, legislation), the availability of resources, cultural factors (e.g. trust in certain stakeholders), level of education and tradition of use of evidence in decision-making. In adapting this guidance to local practice, countries are strongly encouraged to learn from each other, e.g. by participating in regional and international HTA networks and societies, such as Decide, EUnetHTA, HTAsiaLink, REDETSa, INATHA and HTAi (see section 1.6).

4.5 Annotated bibliography

- WHO Consultative Group on Equity and Universal Health Coverage. Making fair choices on the path to UHC. Geneva: World Health organization; 2014 (https://www.who.int/choice/documents/making_fair_choices/en/, accessed April 2020).

This book outlines fair priority-setting processes. It identifies three criteria for identifying high priority interventions: cost-effectiveness, financial risk protection and priority for those who are worse off. The book sets out processes for achieving fairness and legitimacy, with explanations of the theoretical basis.

- Guide to the processes of technology appraisal. London: National Institute for Health and Care Excellence; 2018 (<https://www.nice.org.uk/Media/Default/About/what-we-do/NICE-guidance/NICE-technology-appraisals/technology-appraisal-processes-guide-apr-2018.pdf>, accessed April 2020).

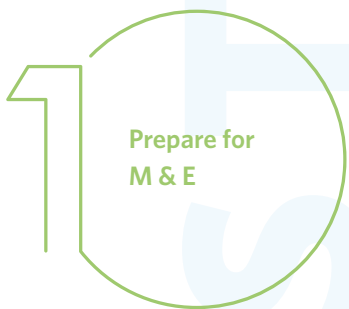
This monograph provides an example of guidance for a national HTA process. It recommends that countries select their process for nomination of interventions, criteria for assessment and methods and appraisal processes in a standardized, methodological approach. This document could be used as a framework by other countries.

- Terwindt F, Rajan D, Soucat A, editors. Priority-setting for national health policies, strategies and plans. Chapter 4 in: Schmets G, Rajan D, Kadandale S, editors. Strategizing national health in the 21st century: a handbook. Geneva: World Health Organization; 2016 (<https://www.who.int/healthsystems/publications/nhpsp-handbook-ch4/en/>, accessed April 2020).

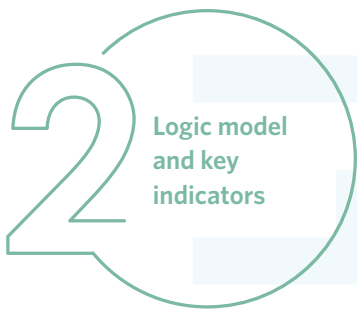
Priority-setting determines the strategic directions of the national health plan. Led by citizens who are the principals and decision-makers, priority-setting is a shared responsibility between the ministry of health (MoH) and the entire health stakeholder community. This chapter elaborates various criteria and approaches for priority-setting. It closes with some specificities of the priority-setting exercise in particular contexts such as the decentralized and highly centralized setting, fragile states, and an aid-dependent environment.

CHAPTER 5

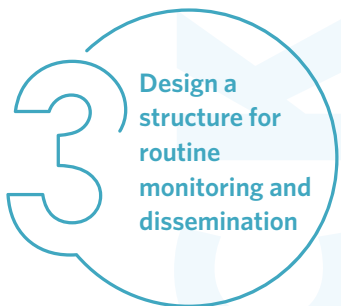
Monitoring and evaluation



- Review the mandate of the HTA mechanism and identify stakeholders.



- Determine the logic model (if not already available).
- Select key indicators in the logic model to monitor annual and multi-year targets. Consider indicators of the HTA mechanism, the quantity and technical quality of HTA reports and procedural aspects of HTA.



- Conduct a landscape analysis of who currently collects information for the indicators.
- Provide missing indicators revealed in the landscape analysis.



- Decide the frequency of data collection, analysis and dissemination, and plan the work cycle for the year.
- Consider the capacity requirements, specifying who will collect, analysis and disseminate data.



- Plan or contract early on for an evaluation at 3, 5 or more years, and collect baseline data.
- Assess the quality of the M&E system and modify it as necessary.

5.1 Summary

This chapter outlines what indicators to monitor, how stakeholders can be involved, and how to set up a system for collecting and using the information to assess and accelerate development of the health technology mechanism.

Many policy champions and stakeholder groups are involved in an HTA mechanism. Although they may all agree in principle that an evidence-informed, transparent, fair process is required as a basis for decisions on reimbursement, stakeholders may differ in what they value, e.g. individual patient access to technology versus total system efficiency, rapid market access versus rigorous evidence of effectiveness. The concerns of major stakeholders must be made explicit and taken into consideration in designing the monitoring and evaluation (M&E) system of the HTA mechanism.

Ideally, stakeholders are involved from development of a logic model of how the HTA mechanism is expected to have an impact, to selection of the indicators of progress, to the preferred modes of communication by type of audience. At the least, feedback from stakeholders should be solicited on the proposed indicators for monitoring and on the objectives of an evaluation.

Monitoring is routine and is closely linked to business planning and implementation. It goes beyond ensuring delivery in an annual plan, however, to monitoring multi-year progress in three areas: the quantity and quality of HTA reports and other outputs, the extent of adherence of HTA procedures to best practice principles and the growth of the HTA mechanism towards preferred attributes.

Evaluation, whether formative or summative, implies additional work within and/or outside the HTA mechanism. It is recommended that evaluation be planned in advance and that a baseline evaluation be performed to allow comparative analyses in the next 3 or more years.

There is general agreement on the standards for HTAs and the best practices for the procedural aspects. There is also growing consensus on the preferred attributes of HTA mechanisms. Many attributes, standards and best practices are not achieved in a single step, and an indication of absence or presence of a parameter is not sensitive to change. Progress can be indicated more objectively by setting potential milestones towards a preferred attribute or standard over several years and by seeking consensus on current status from different stakeholders.

Both quantitative and qualitative approaches to M&E are useful, but comprehensive, consistent documentation is necessary to demonstrate change over the years.

The results of M&E and the actions taken in response must be communicated regularly and transparently, with attention to the modes of communication to different audiences (policy champions and stakeholders). This will ensure public accountability and can provide evidence for increasing value for money of the HTA mechanism.

5.2 Introduction

The first paper on HTA appeared bibliometrically in 1978 (72). The number of reports on HTA then increased rapidly in the late 1980s, followed by an increasing number of HTA mechanisms in countries, which led to the establishment of the International Network for Health Technology Assessment in 1993 (73). This brief overview on monitoring of the impact of HTA reports and HTA mechanisms, and on determining the drivers of success or failure of HTA mechanisms can inform the design of M&E systems.

5.2.1 Impact of health technology assessment

A systematic review of publications between 2000 and 2013 of the influence of HTA and guidelines resulted in 43 studies (74); and an updated study in 2016 yielded similar findings (75). Most of the studies were conducted in high-income countries. Only six were on national HTA programmes, and the rest were on individual HTA reports. The most common approach to determining the influence of HTA was a review of policies or decisions after publication of HTA reports and their recommendations, indicating the impact on policy. Some studies were based on analysis of administrative clinical data to show the impact on practice, while others were based on surveys of or interviews with decision-makers on the usefulness of HTA reports, to indicate the impact on awareness.

One study provided the estimated annual savings achieved with HTA. Some of the studies were planned as part of HTA programme management, as either routine monitoring or a planned evaluation. The authors of the review concluded that there was good evidence of the influence of HTA in most of the studies and a mix of influences in others. The authors expressed concern about the appropriate timing of the evaluations and the difficulty in attributing impacts on policy and on practice.

“There is a progression of possible influence from the decision maker level with increased knowledge and awareness, to decision maker level change in policy, to changes in healthcare delivery, up to changes in patient outcomes. With each increase in level, the control over which the HTA producers can exert an influence decreases and the number of factors influencing decisions on a health technology increases.”

Many of the methods and approaches mentioned above are still used in M&E, but the trend is towards using several methods together, including quantitative methods, to answer questions beyond “Did it have an impact?” to indicate the extent of the impact. An evaluation can thus determine both health impact (with service coverage as a proxy) and, increasingly, the impact on financial risk protection. Impact can be attributed to the HTA report by identification of the “counterfactual”, and then determining the difference between the current situation after the intervention has been recommended and implemented, and the counterfactual with no intervention implemented.

The considerations in the M&E of a single HTA report are broader than those in an M&E for an HTA mechanism or programme. In the M&E of the outputs (e.g. HTA reports) of an HTA mechanism, the objective is to demonstrate consistency (or lack thereof) in the impact of all HTA reports released and not just that of a single HTA report. The trend is also towards greater inclusiveness in reviewing the impact of HTA reports or mechanisms beyond a decision on reimbursement or coverage. For example, M&E of the HTA mechanism could include indicators that measure increasing HTA research capacity.

Drivers of success and best practice principles can also inform the design of M&E of HTA mechanisms. They can explain how and why the HTA mechanism is having an impact or not and can indicate gaps. The following checklist of indicators of progress in HTA development was drawn up after a survey of HTA status in Asia (76):

- formal link between the HTA unit and policy-makers;
- full-time group of HTA researchers;
- use of HTA results in policy implementation;
- availability of HTA process guidelines;
- availability of HTA method guidelines;
- appointment of an HTA focal point agency;
- collaboration with local stakeholders in conducting HTA research;
- domestic HTA training;
- allocation of annual budget for HTA activities by the government; and
- policy statement on willingness to use HTA in policy decision-making.

This checklist could be converted into a more nuanced assessment by using an ordinal grade rather than a “yes or no” response. Surveys of the maturity of HTA mechanisms or “implementation roadmaps” have benchmarked best practices or attributes similar to or in addition to this checklist and have provided ordinal rankings. Such surveys have been conducted in eastern Europe, Latin America and other high- and middle-income countries. Some countries have taken the extra step of questioning whether the best practice principles or attributes are equally valuable or whether some are more important or should be weighted differently in different contexts.

5.2.2 General concepts of monitoring and evaluation

Evidence-based practice includes understanding the impact of a policy and working to improve it during implementation. HTA policy champions and stakeholders must follow the progress (or lack thereof) of the HTA mechanism through M&E. M&E involves embedding a system for routine collection and use of data as efficiently as possible in the HTA mechanism. The objectives and the general approaches in M&E will be familiar to HTA practitioners and anyone with a background in evidence-based medicine: define what matters, measure it, and evaluate it. This chapter shows that measurement of the “effectiveness” (monitoring) or the “impact” (evaluation) of a policy or agency is much more complex and uncertain than ascertaining the effect of a drug; however, M&E ensures that information is available on the HTA mechanism for continuous improvement and increased impact.

Monitoring is a continuous multi-year activity, in which information about an intervention (in this case, the HTA function within the health system) is used to indicate the progress being made towards the intended targets. The most visible tasks in monitoring are systematic collection of data and consistent documentation in a specified format. Monitoring is also closely linked to business planning, in which annual objectives and targets are set and inputs and activities defined, with a risk assessment. Monitoring, however, continues beyond 1 year, to track progress over the years with indicators that show increasing scale, scope or depth every year.

Evaluation is a rigorous, science-based analysis of information about programme activities, characteristics, outcomes and impact that determines the merit or worth of a specific programme or intervention (77).

Evaluations therefore include attribution of impact or analysis of various counterfactuals based on logic models. Evaluation may be conducted by independent or external assessors and may be done at some phase of the programme, e.g. in the middle (formative, to identify gaps) or at the final or stabilization phase of a programme (summation of value in relation to the stated objectives). For the purposes of this guidance on institutionalizing an HTA mechanism, the focus is on formative evaluation, which addresses implementation gaps. However, it is also important to create a summative plan at the start for evaluation at 5, 10 or 15 years. This will establish a baseline and ensure prospective collection of data, which can demonstrate changes in key indicators and increase the plausibility of attributing any change in key indicators to the HTA mechanism and its associated outputs and procedures.

5.2.3 Motivation for monitoring and evaluation and importance of context

M&E does not consist only of “reporting” achievements in documents or annual reports but is a key tool for holding the HTA mechanism accountable. In practice, the most important question in M&E of nascent HTA mechanisms is linked to the motivation for creating the mechanism. For example, if the country’s HTA mechanism was created largely in response to concerns expressed by decision-makers about uncontrolled escalation of health expenditure or about sustainability, M&E would be expected to track some measures of health expenditure once HTA recommendations are being implemented. Likewise, if the motivation was slow uptake of new drugs and other interventions claimed to be clinically effective by patient groups, the timeliness of HTA reports and associated reimbursement decisions would be monitored, with subsequent diffusion of selected interventions and equitable population access to treatments (and, ultimately, evaluation of some health outcomes).

The local context when NICE was established is described in the box below.

In 1997, the incoming government in the UK were concerned about the care of National Health Service (NHS) patients. There was “post-code lottery” restricting the availability of expensive new medicines and there was also documented variation in quality of care. The government also had financial constraints and only limited resources could be provided to improve the NHS. It then set up the National Institute for Clinical Excellence with “the role of advising the National Health Service on use of individual or groups of similar pharmaceuticals and devices (in technology appraisals) and to develop clinical guidelines so that health care professionals could provide National Health Service patients with the highest attainable quality of care. In both forms of guidance, however, the new Institute was expected to take account of both clinical and cost-effectiveness (78).”

Thus, equity, quality and more value for money were the main concerns that led to the establishment of NICE, and the production of guidelines and technology appraisals were seen as part of the response to those concerns.

With understanding of the local context, a logic model can be drawn up that provides clear conceptual understanding of the objectives of the HTA mechanism, the performance targets, the necessary inputs and how they can be translated into activities that meet the performance targets, and whether achievement of the performance targets and outputs would lead to greater equity, quality and value for money. Other considerations for monitoring might be external factors that could facilitate reaching the targets and any potential unintended effects. All these aspects would inform a coherent, effective design of M&E.

5.3 Practical guidance

5.3.1 Review of mandate and identification of stakeholders

The design of M&E starts with a review of the mandate and the stated objectives of the HTA mechanism. This can be contained in the legal and/or administrative documents establishing the HTA mechanism or functions. As described in chapter 1, the mandate of an HTA mechanism can be limited or wide, covering several activities. Examination of the mandate, functions and outputs can answer the question of “impact on whom or what?” and identify the stakeholders or groups that will be affected to varying degrees by fulfilment of the mandate(79). For example, if the mandate of the HTA mechanism is to provide HTA reports as a basis for decisions on reimbursement, then the financing agency, potential beneficiaries or patients, health care professionals, and the pharmaceutical industry would be the stakeholders.

5.3.2 Establishment of a logic model and priorities for monitoring and evaluation

Once the mandate is set, a logic model can be established, from inputs to outputs to expected impact on identified stakeholders. Inputs can range from mandate, governance, networks memberships, financial and human resources. Processes include management including contracting HTAs, communications, consultations of stakeholders, appeals. Outputs would be the reports, whether rapid or full or adaptations, or if the HTA mechanism has an expanded mandate, outputs would include guidelines, horizon scans, etc. The impact could be from awareness raising to influence on policy, health delivery and to final outcomes on health and financial protection (80).

To better inform the logic model, the figure can include an entire health system or determinants of success other than outputs. If, for example, only a decision on reimbursement is made, and there are no guidelines, training, supervision or support systems to assist providers in offering the intervention, it is unlikely that the intervention will change practice patterns and affect health outcomes. It is worthwhile identifying the health system variables that positively or negatively affect practice patterns after a reimbursement decision is made and include them in the design of M&E, or at least include a note on the assumptions used in determining health outcomes.

Gerhardus & Dintios (81) reviewed methods for assessing the impact of HTA and included a question on factors that enhance or hinder the impact of HTA. They divided the factors into two: one intrinsic and one extrinsic to the development of HTA (including the timeliness of HTA reports). The extrinsic factors that enhance the impact of HTA were listed as: a felt need for cost control in the health sector and a “culture” of considering evidence-based information in a health system. Among the hindering factors were “a high degree of influence by partisan groups, a substantial leeway for making decisions at the operational level, a lack of competence in interpreting HTA reports under the decision-makers, rapidly changing political situations, changes of personnel in the HTA providing agency, and the absence of a central institution that gathers information.” Indicators of extrinsic enhancing or hindering factors considered to be important and relevant can be included in the design of M&E.

5.3.3 Selection of indicators for monitoring annual and multi-year targets

M&E indicators are selected in the logic model and taking into account the users and target audience of the information. Each stakeholder’s concerns are identified, and, if relevant and feasible, indicators are identified to monitor their concerns. For example, the timeliness of HTA reports (e.g. number of days from market approval to completed HTA report) would be a concern to all major stakeholders, from the head of the HTA mechanism, who has to show good performance, to those making reimbursement decisions, to health professionals and their patients who wish to use the technology, to the manufacturers who will market the technology. A study of HTA agencies in Europe showed that on the average, it took about two to three months to complete a report on pharmaceutical, including the time needed to review. An HTA on medical devices took longer, about three to 6 months (82). Progress would manifest in terms of shorter turn-around periods for the dissemination of the reports without a decrease in quality.

Ideally, the indicators should cover three general areas: the HTA mechanism, the procedural aspects of HTA and the outputs or HTA reports. The indicators may be quantitative and/or qualitative.

Monitoring progress of the HTA mechanism

The first monitoring activity is to review the annual or multi-year business plans of the HTA mechanism to identify targets or deliverables in terms of the inputs and outputs and to monitor them through the years. The NICE business plan for 2000–2001, at the inception of the agency said that they would deliver 26 sets of guidance based on their technology appraisals, review 13 clinical guidelines, commission 10 new guidelines of which 7 will be completed (83).

The number of technology appraisals could serve as an indicator, as progress could be demonstrated by an increasing number (or quality) of technology appraisals every year, within the limit of the available resources.

As this was the first year of NICE, several of the activities were not repeated (e.g. new documents as evidence for issuing revised guidance to manufacturers and sponsors for the technology appraisal programme) and are therefore not indicators. Such activities and production of documents to guide the selection of topics, development of methods with partners, dissemination mechanisms and other activities, will lead to further institutionalization of the mechanism. This is a 1-year plan with short-term targets. Multi-year, more strategic implementation plans can also be drawn up, which will necessarily be less specific.

The attributes of mature HTA mechanisms and best practice principles can be consulted to determine how inputs, processes, structures and one-time outputs can be combined or interact to increase the scope, quantity and quality of the outputs and procedures. Sample implementation road maps may also be referred to. An implementation road map with some key attributes and best practice principles that was applied in countries in central and eastern Europe (84) and subsequently in Latin America (85) is shown below. A points system was introduced in another variant of this roadmap and applied to high- and middle-income countries but includes additional domains, such as horizon scanning and dissemination (86). Examples of some parameters being assessed are shown below:

HTA funding

Financing critical appraisal of technology assessments reports or submissions (single choice)

- No funding
- Predominantly private funding (e.g. submission fees) by manufacturers
- Predominantly public funding

Legislation on HTA

Legislation on the role of HTA process and recommendations in decision-making (single choice)

- No formal role of HTA in decision-making
- Predominantly international HTA evidence taken into account in decision-making
- International and also local HTA evidence taken into account in decision-making
- Local HTA evidence mandatory in decision-making

Quality and transparency of HTA implementation

Quality elements of HTA implementation (several choices)

- None of the quality elements below are applied
- Published methodological guidelines for HTA and economic evaluation
- Regular follow-up research on HTA recommendations
- Checklist to conduct formal appraisal of HTA reports or submissions exists but not available to the public
- Published checklist applied to formal appraisal of HTA reports or submissions

Use of local data

Requirement to use local data in technology assessment (single choice)

- No mandate to use local data
- Mandate to use local data for certain categories but no requirement to assess the transferability of international evidence
- Mandate to use local data for certain categories requirement to assess the transferability of international evidence

A review of the different implementation roadmaps would allow critical selection of domains and adjustment of the grading according to what is important and relevant in the local context, as was done in some Latin American countries (87). A roadmap could then be designed for one's own HTA mechanism, setting a target year for attaining a progressively higher grade in each domain.

Monitoring procedural aspects of HTA

The attributes of the procedural aspects of HTA could also be monitored. Integrated HTA provides a three-point ordinal ranking (“yes, to some extent, no or very limited”) of attributes of the procedural aspects to assess the legitimacy and fairness of the process (24). Integrate-HTA proposes a basis for selecting indicators on inclusiveness of the process with regard to stakeholders at different steps of the HTA, the transparency of the decision making process (whether open to the public and/or adequate documentation), the availability of an appeals process, etc. This is particularly useful when a complex intervention is being evaluated (88) or when there are different perspectives or outcomes that may be weighed differently using various criteria (89).

Monitoring HTA reports

The International Network for Health Technology Assessment has published a checklist for assessing the quality of an individual HTA report (90) in several languages. The Network has also issued a simple self-reporting form for assessment of short-term (within 6 months of publication of the HTA report) impact (91), classified by levels of influence and impact.

The Malaysian Health Technology Assessment Section recently completed a short assessment of the impact of 121 reports made between 1997 and 2018, using variations of the short form. Their report is reproduced in Annex 1, and the results are summarized in Table 5.

Table 5. Indications and levels of impact of 121 HTA reports and mini-HTA reports in Malaysia

Impact or influence	Proportion (%)
Indication of impact	
Recommendations accepted	90.9
Used as reference	86.8
Linked to change in procedure or practice	47.1
Incorporated into policy or administrative document	38.8
Level of impact	
Major influence on decision	48.8
Informed decision	36.4
Some consideration by decision-makers	14.9

5.3.4 Establishment of a structure for a monitoring and evaluation system, including dissemination of results

Once the logic map, domains and indicators (including targets) are decided, the M&E system can be established for the HTA mechanism, HTA reports and procedural aspects. The first step should be to undertake a quick landscape analysis or a scan of the M&E systems that may be collecting the indicators. These might be the monitoring systems of academic institutions, which might have the same conceptual approach, such as committees for essential medicines lists or national drug formularies. Then, the frequency of data collection, reporting and dissemination should be decided, and the cost of consistent, timely implementation should be calculated. Quantitative data could be obtained from administrative records; however, regular surveys and interviews of stakeholders should be conducted for implementation roadmaps with indicators that require qualitative judgement.

All indicators and data should be interpreted and communicated to the relevant authorities and users and the necessary action taken. The uncertainty of quantitative data should be presented if possible, and the plausibility of qualitative data or judgement should be assessed. A standard format including a narrative report of the results and the actions taken is then prepared, with different versions for different stakeholders if necessary. A dissemination and communication platform should be set up for the communication and dissemination of the monitoring and evaluation reports; social media can provide an important additional platform. A major consideration in choosing a platform for dissemination of the M&E report of an HTA agency is that it promotes inclusive country accountability processes for the press, patient groups, civil society, ministries of health and parliamentary health and budget committees.

Once the indicators for the M&E system, the frequency of reporting, the monitoring tools to be used, and the platforms for dissemination to different stakeholders have been chosen, a work plan should be prepared, to:

- decide on the capacity requirements, who to involve in data collection, reporting, dissemination and use (which might require different skills and different times);
- plan the annual cycle of collecting, analysing, reporting and disseminating data and taking action; and
- change monitoring indicators over time, if necessary, with experience in use of the indicators, as some might have to be reduced or nuanced, and others might be ineffective or unreliable and be eliminated.

5.3.5 Periodic evaluations

Periodic evaluations should be scheduled and programmed into the business plans of the HTA mechanism. Both policy-makers and HTA mechanism staff should have realistic expectations about the questions to be answered by the evaluation. As mentioned previously, the initial evaluation might be formative, reviewing the functioning of the HTA mechanism and identifying gaps and opportunities. A formative evaluation is based on most of the data produced by routine monitoring but may require additional surveys of stakeholders about the credibility of the institution and the usefulness of its outputs. It may be commissioned to an external group.

WHO conducted formative evaluations (92) of the technology appraisal and clinical guidelines work programmes of NICE in 2003 (93) and 2005 (94), respectively. The early achievements of NICE in terms of transparency, inclusiveness and technical rigour were praised, and recommendations were made to further enhance its operations. Since 2012, NICE has been reviewed every 3 years.

Summative evaluation can be planned after 5, 10 and 15 years for a broad range of impact indicators. A multi-method approach is frequently used, as more resources may be available for such studies, and this approach was used for the 10-year assessment of the National Institute for Health Research HTA programme in the United Kingdom (95). The authors used the “payback” framework, with purposive sampling of 12 high-impact studies. Although the 12 case studies were acknowledged as not generalizable to the entire portfolio, they illustrate the range and nature of the impact of the HTA programme.

A quantitative approach requires specification of a counterfactual and use of modelling techniques to track changes in health outcomes. Such an approach is useful for justifying the value for money of the HTA mechanism. To demonstrate a change in health outcomes, it would necessarily require analysis of specific HTA reports. A report in 2015 (96) showed the expected and the actual (with consideration of the time lag) health outcomes associated with maternal and child health vouchers in Myanmar and in the human papillomavirus programme in Thailand.

The quantitative approach can also be extended to determine the economic benefits of HTA. The economic benefits could include those to the market (direct cost savings to the health care system, benefits to the economy of a healthy workforce and commercial development) and non-market benefits (intrinsic value of health gains to society). In these examples in which gains were quantified in terms of health outcomes and the economy, the main difficulty was attributing the gains to HTA reports (97).

In a study on the prevention of cervical cancer in Thailand in 2007 (21), it was calculated that cost savings of 0.02% of total health expenditure would cover the operating costs of the Health Intervention and Technology Assessment Programme that year. A study of the National Institute of Health Research in the United Kingdom showed that 12% of the potential net benefit of implementing the findings of a sample of 10 HTA studies would cover the total cost of the HTA programme between 1993 and 2012 (98).

5.4 Review of a monitoring and evaluation system

An adaptation of the desired key attributes of an M&E platform (77) that could be used for self-assessment is shown below. It recognizes that an M&E system must be assessed and reviewed periodically, as feedback from users and stakeholders might show that some indicators are not useful and others might have to be expanded as the mandate of the HTA mechanism widens.

Attribute 1: Context of M&E

- The multi-year strategic or business plan of the HTA mechanism acknowledges its public accountability and specifies a sound M&E component in its work plan.

Attribute 2: Institutional capacity

- The roles and responsibilities for M&E are clearly defined in the HTA mechanism, and it is an intrinsic part of the HTA mechanism.
- Capacity-strengthening in M&E is addressed. The HTA mechanism and its associated network include systematic identification of gaps in skills and competence and a learning to address them.

Attribute 3: M&E

- M&E is based on a logic model, including core indicators and targets.
- Data sources and modes of data collection, analysis and reporting are specified and publicly available, including the calendar year cycle that shows the data flow.
- A data dissemination platform addresses different stakeholders.
- Prospective evaluation is planned and implemented.

Attribute 4: Review and action

- The results of M&E are regularly reported and discussed in a forum, in which excellent work is acknowledged and corrective measures planned.

5.5 Annotated bibliography

Monitoring

- NICE Business Plan 2000-2001 (<https://www.nice.org.uk/Media/Default/About/Who-we-are/Corporate-publications/Corporate-and-business-plans/NICE-business-plan-2000-2001.pdf>).

This is actually a business or work plan for the early years of NICE. It is included here as an example of how NICE laid its foundations in order to support its work programme. This is best read together with the monitoring of key deliverables as documented in <https://www.nice.org.uk/Media/Default/About/Who-we-are/Corporate-publications/Annual-reports/NICE-Annual-Report-2000-01.pdf>

- Oortwijn W, Broos P, Vondeling H, Banta D, Todorova L. Mapping of Health Technology Assessment in Selected Countries in International Journal of Technology Assessment in Health Care, October 2013. DOI: 10.1017/S0266462313000469.

This document pulls together different criteria for the HTA institution and the HTA process itself. It is intended to map the progress of health technology agencies, using indicators for each of the criteria. Most of the self-assessment responses for the criteria are ordinal in nature and thus can be easier used for monitoring of progress vis-à-vis a criteria with yea/no responses. Another document shows almost the same criteria applied to Latin American countries but with a very strong recommendation to contextualize the criteria to the local setting (Pichon-Rivière A, Soto N, Augustovski F, García Martí S, Sampietro-Colom L. Health technology for decision making in Latin American countries: good practice principles. Int J Technol Assess Health Care. 2018;34(3):241-7)

Evaluation

- Wanke M, Juzwishin D, Thornley R, Chan L. HTA Initiative #16: An Exploratory Review of Evaluations of Health Technology Assessment Agencies. Alberta Heritage Foundation for Medical Research, 2006. Canada. <http://www.assembly.ab.ca/lao/library/egovdocs/2006/alhfm/153507.pdf>

This monograph develops a generic framework for evaluation of HTA agencies, informed by a review of literature and of evaluations of 16 INAHTA member agencies. It discusses the intent, timing and target audience of the evaluation, the dimensions being evaluated, and methods used in the evaluations. In particular, it presents the dimensions for evaluation within a logic model.

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ANNEX:

Monitoring and evaluation of HTA programme in Malaysia

| *By Dr Junainah Bintu Sabirin, MOH and team (June, 2019)*

Background

Health Technology Assessment (HTA) evaluates the properties and effects of health technology and provides information to support all healthcare decisions at local, regional, national and international level. (1) The Malaysian Health Technology Assessment Section (MaHTAS) endeavours to provide information on the safety, efficacy, effectiveness and economic impact of health technologies to support healthcare decision making especially for Ministry of Health facilities. Effectiveness of Health technology Assessment (HTA) programme will depend on its influence, the extent to which information provided has had an effect on decision makers and in what ways. (2) Influence/impact of HTA reports is a guide to the effectiveness of a programme. This information is useful as a key indicator of output and performance of HTA agencies in quality assurance processes, in reporting to funders of HTA programmed, and in contributing to global indications of HTA achievements. (2,3) Hence, measurement of HTA impact/influence has been routinely conducted by MaHTAS.

Method

MaHTAS produced HTA reports, Technology Review (mini-HTA) reports as well as rapid review, with three types of report recommendations; i) recommended, ii) not recommended, iii) recommended for research. Once the reports were endorsed by the HTA-CPG Council meeting, the requestors of the report will be informed on MaHTAS intention that impact evaluation will be done at least six months following endorsement. The impact evaluation was done using self-administered Evaluation Form (adopted from International Network Agencies for Health Technology Assessment (INAHTA)-Framework for reporting of impact). The evaluation form differs according to reports recommendations. Follow-up on the report outcome is done via emails, letters or telephone calls to the requestors. A report is considered to have an impact/influence if any indication is present, whereas level of impact on decision is classified at four levels accordingly.

Result

A total of 121 HTA/mini-HTA reports regardless of types of recommendation (64 HTA reports from 1997 to 2018; and 57 mini-HTA reports from 2015 to 2018) were evaluated. All the reports have shown indication of impact/influence. The most common indications of impact/influence and the levels of impact/influence are illustrated in Table 1. The reports were mainly used for provision of services, initiation of programmed, procurement and clinical practice as shown in Table 2.

Discussion and conclusion

Overall, the framework for assessing the HTA impact/influence seemed feasible and useful particularly in assessing initial and immediate indication of impact of the technology. However, engagement and longer term follow-up with clients are still needed to obtain and evaluate the actual impact on clinical practice. Although from our experience to date, we managed to get good responses, close engagement and follow-up with the requestors are undoubtedly crucial in the implementation. Additionally, other information sources such as policy and administrative documents were also sought to ascertain the impact depending on the technology. A continuous impact/influence monitoring mechanism should be built within the HTA programme.

Table 1. Indications and level of impact of HRA and mini-HTA reports in Malaysia

Parameters of impact/influence (n=121)	Proportion (%)
Indication of impact	
HTA/mini-HTA recommendations accepted	90.9%
Use as reference material	86.8%
Link to change in procedure/practice	47.1%
Incorporate into policy/administrative document	38.8%
Level of impact	
Major influence on decision	48.8%
Informed decision	36.4%
Some considerations of HTA/mini-HTA by policy/decision maker	14.9%

Table 2. Impact of HRA and mini-HTA reports and decision making

Type of impact/influence	Examples
Initiation of programmes	National Thalassaemia Prevention and Control Programme
Provision of services	Management of Haemophilia Enzyme Replacement Therapy for metabolic diseases Continuous Intrathecal Baclofen Infusion for severe spasticity and dystonia Screening for congenital hypothyroidism School scoliosis screening programme HPV DNA based screening for cervical cancer Prostate cancer screening (for high risk group) IFOBT for colorectal cancer screening
Clinical practice	Intraocular lens implantation hydrophilic acrylic versus hydrophobic acrylic Bronchial thermoplasty Insulin analogue
Procurement	Endobronchial ultrasound Exhaled nitric oxide measurement using NIOX or NIOX MINO for bronchial asthma Transcranial direct current stimulation for stroke rehabilitation Automated auditory brainstem response (AABR) and otoacoustic emission (OAE) device in universal newborn hearing screening
Pricing	Tyrosine Kinase Inhibitors as first line treatment for advanced non-small cell lung cancer

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