

Key principles of HTA or What is Meant by HTA?

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Introduction

- Who are we?
- Why are we here?

Session overview

Learning objective:

- for the participants to be able to explain what is meant by Health Technology Assessment (HTA) and be aware of the key principles of how to conduct an HTA

Session overview

What is HTA?

- Definition; purpose; history; use

How is HTA carried out?

- Topic selection; research question; stakeholder engagement; evidence gathering; economic evaluation; organisational issues; ethical, legal, and social considerations; findings.

Wrap up

- What does HTA do; and what does it not do.

What is HTA?

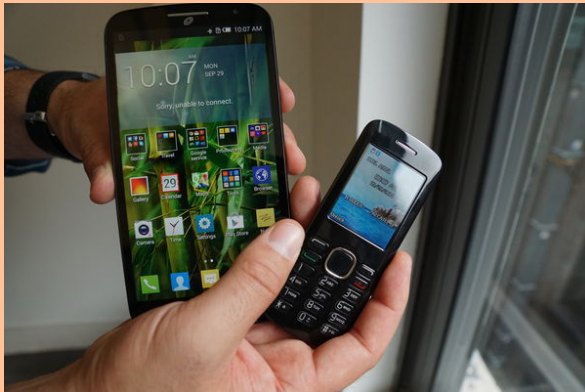
Definition of HTA

HTA is “the systematic evaluation of the properties and effects of a health technology, addressing the direct and intended effects of this technology, as well as its indirect and unintended consequences, and aimed mainly at informed decision making regarding health technologies.” (NIHR)

Phablets

Intended benefits

Great screen resolution



Enables additional functionality



Unintended consequences

Hard to use as a phone



No longer fits in your pocket



Purpose of HTA

“The main purpose of HTA is to inform policymaking for technology in health care, where policymaking is used in the broad sense to include decisions made at, e.g., the individual or patient level, the level of the health care provider or institution, or at the regional, national and international levels.” (HTA 101)

Brief history of HTA

- 1960s: Growing appreciation that technologies could have harmful effects in addition to their intended benefits
- 1970s: Office of Technology Assessment established in the USA. Growth in use of technology assessment.
- 1980s: Beginnings of health technology assessment (HTA) as a formal discipline
- 1990s: Introduction of HTA programmes to support reimbursement decisions
- 2000s: The European Commission and Council of Ministers targeted HTA as 'a political priority'


Use of HTA internationally

HTA is used for decision making by:

- Regulatory agencies (whether or not to allow marketing of a technology)
- Payers (whether or not and how much to pay for a technology)
- Providers (to support decisions about acquisition and management)
- Clinicians and patients (to identify most appropriate use of interventions)
- Government health department officials (evaluating public health programmes)
- Health care technology companies (identifying product development options)

How is HTA carried out?

Identifying topics

- The method of picking topics for assessment depends on the country and type of technology. For example, many countries require HTA for pharmaceuticals to qualify for reimbursement.
- Potential cost, perceived impact on patient outcomes, burden of disease are quoted as the main criteria for selecting health technologies for HTA.
- HTA usually used for new technologies rather than established or widely used technologies.
- Topics should be selected according to defined prioritisation criteria. 

Transparency

- The findings of a HTA can have major implications for patients, clinicians, service providers, manufacturers, etc
- HTA is intended to be accessible, inclusive, transparent and unbiased. ★
- To achieve transparency, the methods and data used, and how the HTA was influenced, must be clearly reported.
- Conflicts of interest of individuals involved in the process must be documented.


Specifying the research question

- The HTA process should ideally include development of a detailed scoping document with involvement of stakeholders in clearly defining the questions to be addressed by the HTA and its link to decision-making regarding the use of the technology.
- The research question must be relevant to the decision makers' problem. ★
- There must be clear definition of what is to be covered in the HTA.

Specifying the research question

- Intervention (what are we looking at?)
- Comparators (what alternatives are available?)
- Target population (who will be treated with the intervention?)
- Outcomes (how will we measure benefits and harms?)
- Time horizon (over what time period will benefits and harms be measured?)
- Perspective (who is paying for the intervention?)

Choice of comparators


What is the comparator and have all the reasonable alternatives been considered? 

- Something mediocre can look good when compared to something lousy. One comparator should be current routine practice.
- Not including good viable alternatives can make something look good when much better alternatives are available.


Choice of comparators is important



Perspective

- Some countries advocate a societal perspective when conducting HTA. That is, all costs and benefits accruing to society are considered in the assessment.
- Other countries use the perspective of the State health and social services.
- Other countries restrict themselves to only the costs and benefits accruing to the publicly-funded health service.
- A societal perspective has been advocated to optimise efficiency and avoid distorted policy making. 
- A full societal perspective requires extensive data collection, particularly in terms of indirect costs and also impacts on those other than the people receiving the intervention (e.g., carers) which can be very difficult to gather..

Systematic review

- The purpose of a systematic review is to sum up the best available research on a specific question. This is done by synthesizing the results of several studies.
- “A systematic review uses transparent procedures to find, evaluate and synthesize the results of relevant research. Procedures are explicitly defined in advance, in order to ensure that the exercise is transparent and can be replicated. This practice is also designed to minimize bias.” (Campbell Collaboration)
- A HTA should incorporate a broad range of evidence and outcomes. 

Systematic review

- Planning: refine the review question, define inclusion and exclusion criteria, identify range of sources
- Search: implement the planned search and adjust to ensure good coverage. Search within found texts to find any missed references. Check published, grey literature and unpublished studies.
- Review: eliminate irrelevant results. Potentially relevant studies should be reviewed by two people based on the agreed criteria.
- Synthesis: relevant studies must be quality assessed and data extracted. Pooled results may be calculated where data meet standards.

Clinical effectiveness and safety

- The outputs of the systematic review should provide the best level of evidence regarding the clinical effectiveness and safety of the intervention and comparators.
- The evidence should be applicable to the context of the HTA (i.e., same population, same disease, same setting, etc).
- The evidence is often for a 'similar' population – the important thing is that there is confidence that the findings apply to the target population of the HTA.

Measuring outcomes

The choice of outcomes is critical as it will dictate whether or not we can compare across disease areas and previous HTAs within the same disease area.

The choice of endpoints used in a study or comparison will be influenced by the purpose for which they are measured. For example, if the primary purpose of a technology is to improve survival, then mortality will be the relevant endpoint. If, however, a technology is designed to improve mobility, then functional status may be a more appropriate endpoint.

Measuring outcomes

Patient-reported outcomes (PROs)

- A whole range of measurement types usually referring to self-reported patient health status focussing on how the patient functions or feels in relation to a health condition and its treatment.
- Examples include EQ-5D and SF-36.

Clinical endpoints

- An aspect of a patient's clinical or health status that is measured to assess the efficacy or harm of a treatment relative to the best available alternative.
- Examples include mortality and stroke.

Measuring outcomes

Surrogate endpoints

- An objectively measured endpoint that is expected to predict clinical benefit or harm based on epidemiologic, pathophysiologic, therapeutic and other scientific evidence.
- An example is blood pressure as a surrogate endpoint for cardiovascular mortality.

Composite endpoints

- Combine multiple single events into one endpoint showing the overall and clinically relevant treatment effect.
- For example, mortality, myocardial infarction and stroke in patients with hypertension.

Measuring outcomes

Adverse events

- Unintended effects that may be harmful.
- An example is toxicity-related side effects due to external beam radiotherapy.
- Adverse events tend to be rare events with most studies underpowered to detect statistically significant differences.

Measuring outcomes

- Some countries express a preference for outcomes to be expressed as utilities so that an analysis can be based on Quality Adjusted Life Years (QALYs).
- QALYs are intended to capture both quantity and quality of life. Thus life saving interventions can be compared with life improving interventions.
- Without using QALYs, interventions for different disease areas may be difficult or impossible to fairly compare (e.g., reduction in cholesterol vs. Prevention of amputation).
- Data on quality of life can be collected in different ways and may not be applicable to similar patient populations in other countries or settings.
- Quality of life data can be difficult to find.


Epidemiology

- Epidemiology: literally meaning "the study of what is upon the people"
- The epidemiology relating to the disease and patient population in the jurisdiction needs to be clearly understood and described
- Different countries may have different epidemiology (disease courses, prevalence/incidence rates, different survival, etc) which will impact on the applicability of study estimates of clinical effectiveness

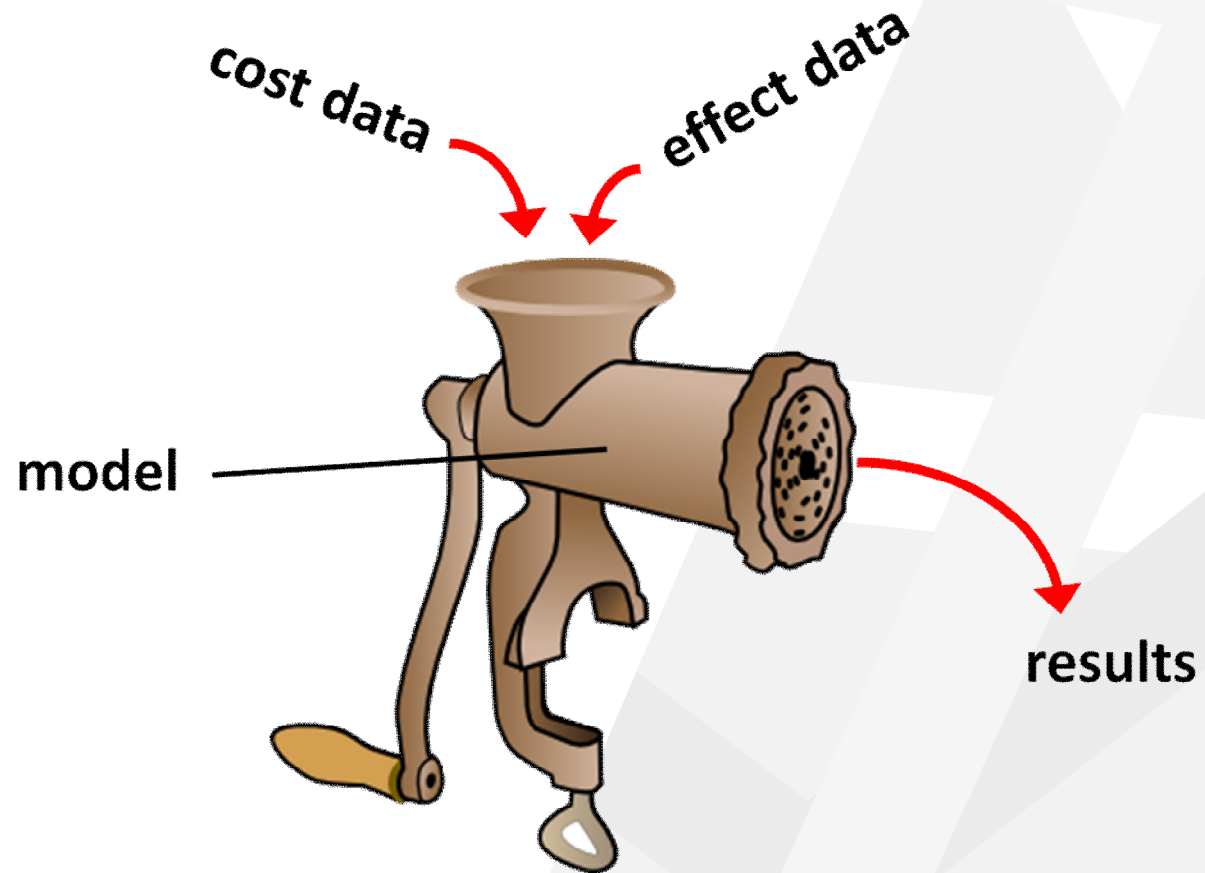
Data gathering

- HTA is an evidence-based activity, but it is also multi-disciplinary – it requires data on many fronts.
- A HTA should ideally be based on all available evidence.
- Hence attempts should be made to gather all of the available evidence. ★
- All data should be assessed in terms of quality and quantity.
- By viewing all of the evidence and taking quality into account, the intention is to produce the least-biased assessment possible.


Economic evaluation

- Data on costs, benefits, harms, resources must all be combined to generate a summary statistic that describes the costs and benefits associated with the technology and comparators.
- Data and evidence must be analysed using appropriate and widely accepted methodology to ensure that findings can be considered accurate. 
- The summary statistic should ideally be comparable across technologies, diseases, and settings. This is usually translated into the cost per QALY (quality adjusted life year).

The economic model



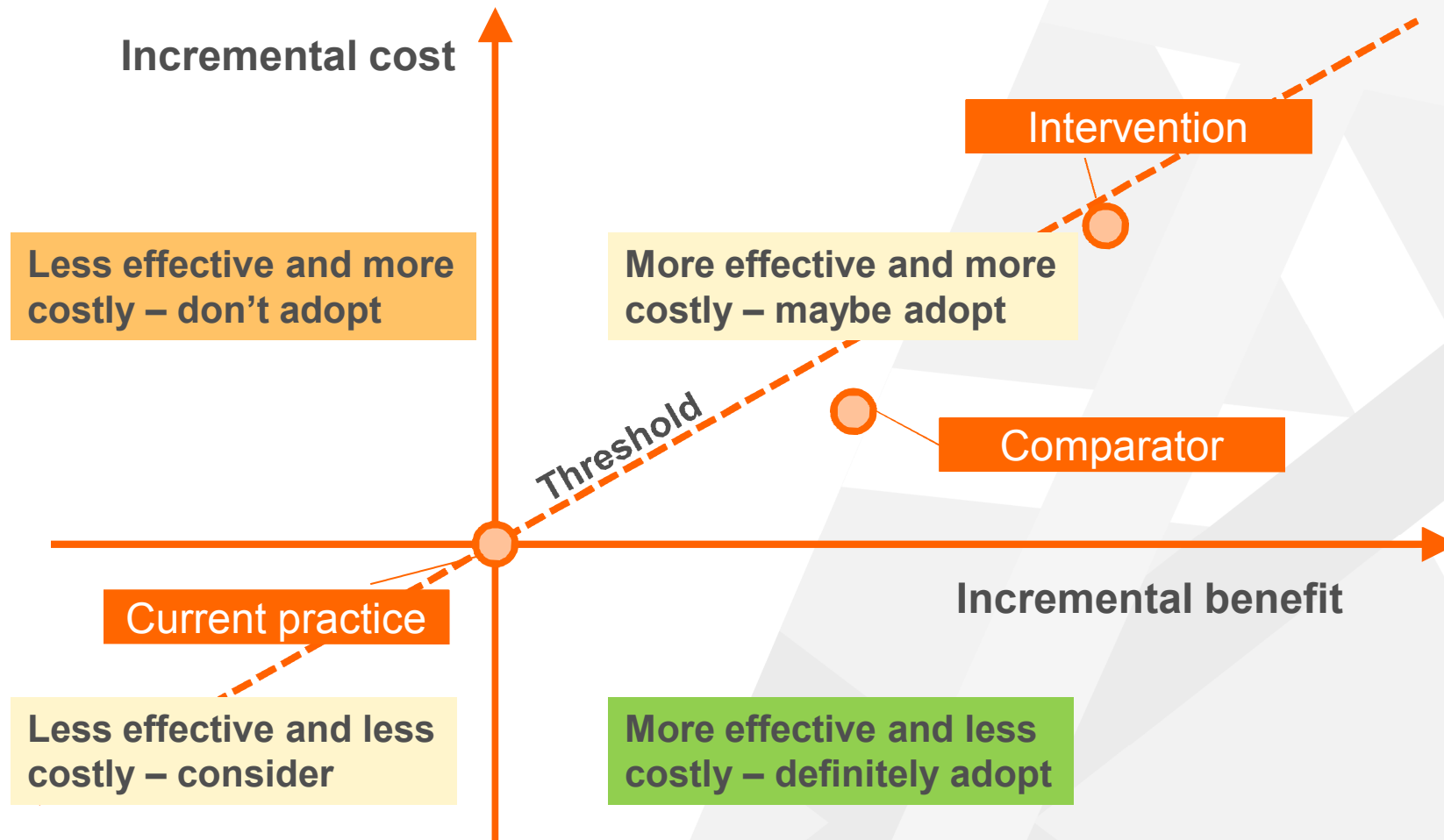
Economic evaluation

- HTA acknowledges that little is known with certainty.
- Cochrane review of 'vaccines for preventing influenza in healthy adults': "at least 40 people would need vaccination to avoid one influenza-like illness case (95% confidence interval (CI) 26 to 128)."
- HTA takes this uncertainty into account so that the decision maker can see  how clear the case for a particular course of actions is.

Economic evaluation

- The results are often summarised as a cost per unit benefit, also called the incremental cost-effectiveness ratio.
- This allows the intervention and the comparators to be evaluated on an equivalent scale.
- The incremental cost-effectiveness ratio is often evaluated in relation to a willingness-to-pay threshold.

Economic evaluation



Economic evaluation

- A HTA will ordinarily also include what is called a budget impact analysis.
- The cost-effectiveness indicates the cost-per-patient in a manner that can be quite abstract, particularly if it based on a cohort which is of a very different size to the true target population, and if they are followed over a very long time horizon.
- The budget impact enumerates the total cost per annum and addresses the question of affordability.

Economic evaluation

- Sensitivity analysis is an essential component of any economic evaluation.
- An economic evaluation is often based on numerous assumptions or on potentially biased data. It is critical to test whether these assumptions have a major impact on the estimated cost-effectiveness.
- What might happen in best or worst case scenarios, etc (e.g., what if the clinical effectiveness is at the high/low end of what the studies estimated?)

Organisational issues


- The resource requirements for delivery of the intervention must be accurately documented.
- The technology may not be deliverable within the existing healthcare system (e.g. due to staff shortages in particular disciplines).
- A HTA may investigate alternative methods of using existing resources to most efficiently deliver health services.

Ethical, legal, and societal issues

We are not solely interested in costs and direct health effects. We also want to consider other aspects that might have an important bearing on the decision:

- ethical issues – provision of the technology may discriminate between different population subgroups or compromise civil rights
- legal aspects – the technology may give rise to legal challenges that could have implications for liability

Stakeholder engagement


- Identify parties who might be affected by the decision (patients, clinicians, providers, payer, etc).
- Involve stakeholders in the process either continually (through participation),  or at selected times (through consultation), or by a mix of approaches.
- Stakeholders should be able to provide meaningful input into the process.

Summary of findings

- HTA is the business of writing reports.
- Findings must be clearly and unambiguously reported. ★
- HTA as a discipline favours transparency, so everything tends to go into the report.
- The report needs to address a number of audiences: the decision maker, patients, clinicians, healthcare providers. Everyone who will be affected by the decision should be able to access the information.
- Writing HTA reports is a challenge. Reading them also a challenge.

Group task



Generalisability

- It must be clear that the findings of the HTA are accurate for the target population defined at the outset. 
- The generalisability must be considered in terms of a variety of factors, including:
 - Patient population
 - Disease spectrum
 - Patient behaviour
 - Service delivery
 - Geography
 - Clinical practice

Timeliness

- A HTA will be of little value if it reports long after a decision has been made.
- Equally, a HTA will be of limited value if it is produced before adequate evidence is available.
- HTA is a resource-intensive exercise that takes time to report, but it must be completed within a reasonable time-frame. ★
- Methods of rapid review have been developed to support faster turn-around, but it depends on the research-question whether it is a reasonable option.

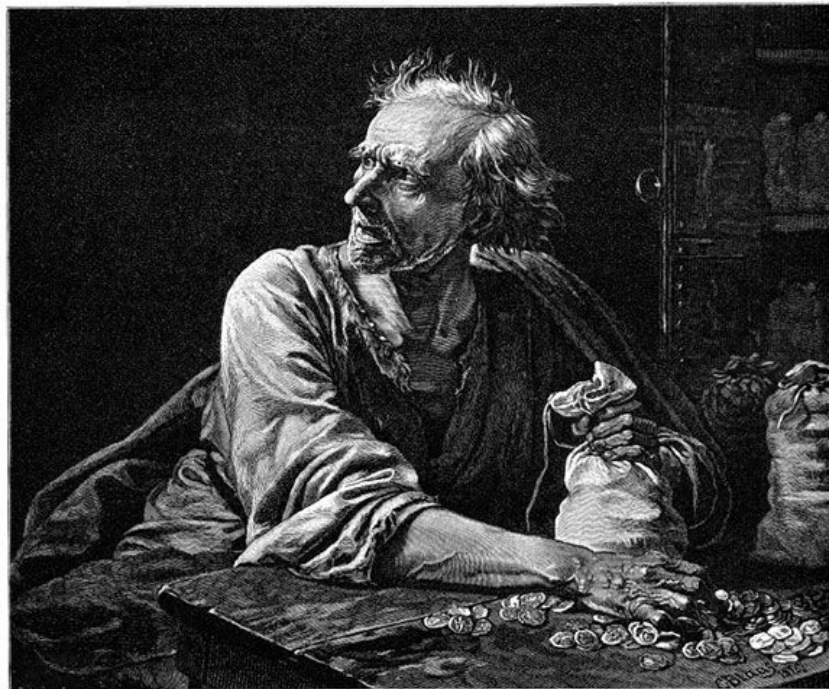
Implementation

- For HTA to be of use, it must support the decision making process.
- Implementation of HTA findings should be monitored. A decision maker may  not necessarily follow the advice of a HTA (for legitimate reasons). However, it must be discerned if this is a failure on the part of the HTA.
- The link between the findings of a HTA and the decision making process  should be transparent. If the decision maker acts contrary to the advice, then it should be clear why.
- In many jurisdictions there is a separation between the HTA agency and the decision maker, hence transparency of decision making is not in the control of the agency.

Wrap-up

Myths about HTA

- That it is just a cost-containment tool



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Myths about HTA

- That it puts a price on a human life



Questions answered by a HTA

- Can the intervention work?
- Does the intervention work?
- Is it good value for money?
- Is it affordable?
- Are there other reasons why we should or shouldn't adopt it?

Question not answered by a HTA

- What should the decision maker do?
 - HTA is a decision support tool, it does not make the decision and other considerations may impact on decisions.

Key principles

HTA tries to be:

- Relevant
- Transparent
- Unbiased
- Systematic
- Evidence-based
- Comprehensive

Thank you

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