A Newcomer's Guide to HTA: A collection of resources for early career professionals

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Preface
The main aim of this collection is to guide newcomers to HTA, concentrating on mentorship and information, as part of an HTAi program to build capacity for HTA within health systems in order to improve health system outcomes through evidence informed approaches to access, appropriateness, availability, and affordability of health care services.

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Development
The collection was firstly proposed by Maoling Wei on behalf of HTAi ECN in 2017 and was initiated by HTAi in 2018. The HTAi Board and HTAi’s Committee for Scientific Development and Capacity Building (SDCB) supported the development of the handbook. The collection is coordinated by the ECN under Maoling Wei. Work on the text was started at the HTAi 2018 Annual Meeting in Vancouver. The draft version was introduced at the HTAi 2019 Annual Meeting in Cologne, and the formal version was introduced for the Virtual Summer Course Frontiers in Health Technology Assessment at Sichuan University, Chengdu, China, on July 5, 2021. A minor modification was by David Hailey and Maoling Wei in the summer of 2022.
HTA 101: Essential Information for Newcomers

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Health technology
A health technology is defined as an intervention developed to prevent, diagnose or treat medical conditions; promote health; provide rehabilitation; or organize healthcare delivery. The intervention can be a test, device, medicine, vaccine, procedure, program or system [http://htaglossary.net/health-technology].

Health technologies can be described in terms of their physical nature, clinical purpose, stage of development or diffusion.

Physical nature:
- Drugs: e.g., aspirin, antibiotics, cancer chemotherapy
- Biologics: e.g., vaccines, blood products, biotechnology-derived substances
- Devices, equipment, supplies: e.g., cardiac pacemaker, MRI scanner, mosquito netting
- Medical and surgical procedures: e.g., acupuncture, bariatric surgery, cesarean section
- Public health programs: e.g., water purification system, vaccination program, smoking prevention program
- Support systems: e.g., clinical laboratory, drug formulary, electronic health record system
- Organizational, delivery, managerial systems: e.g., primary care network, health care payment system

Clinical purpose:
- Prevention
- Screening
- Diagnosis
- Treatment
- Rehabilitation
- Palliation
Stage of development or diffusion:
- Future
- Experimental (laboratory or animal testing)
- Investigational (clinical studies, i.e., in people)
- Established (standard approach)
- Obsolete

**Health Technology Assessment and its Role in Health Care**

**Origins of Technology Assessment**

Technology assessment (TA) arose in the mid-1960s from an appreciation of the critical role of technology in modern society and its potential for unintended, and sometimes harmful, consequences. The term “technology assessment” was introduced in 1965 in the US House of Representatives, with the primary purpose of serving policymaking. Examples of early assessment topics were offshore oil drilling, pesticides, automobile pollution, nuclear power plants, supersonic airplanes, and the artificial heart.

Development of TA in 1960s and 1970s coincided with the introduction of health technologies that prompted widespread interest in matters that transcended their intended health effects. Examples of topics of early HTAs include: multiphasic health screening; in vitro fertilization; predetermination of the sex of children; slowing of aging; modifying human behavior by neurosurgical, electrical or pharmaceutical means; and drug bioequivalence.

**Health Technology Assessment**

HTA can be defined as follows:
- HTA is the systematic evaluation of properties, effects, or other impacts of health care technology.
- The main purpose of HTA is to inform policy making for technology in health care.
- HTA may address the direct and intended consequences of technologies, as well as the indirect and unintended consequences of technologies.
- HTA is conducted by interdisciplinary groups.
- HTA uses explicit analytical frameworks and a variety of methods.
In 2020, a joint task force of INAHTA and HTAi developed a definition of HTA as follows:

“HTA is a multidisciplinary process that uses explicit methods to determine the value of a health technology at different points in its lifecycle. The purpose is to inform decision-making in order to promote an equitable, efficient, and high-quality health system.” (O’Rourke et al. 2020)

HTA is used to inform various functions. It is essential to understand that HTA usually does not generate a policy or decision; instead, it informs policies or decisions made by others. In particular, HTA is used to:

- Advise payers (health authorities, health plans, etc.) about technology reimbursement: coverage, coding, and payment amounts
- Advise/guide clinicians and patients about technology use (e.g., with evidence-based clinical practice guidelines)
- Help managers of hospitals, health care networks, other provider institutions/organizations to make decisions about acquiring or investing in technology
- Support decisions by national and regional public health authorities about conducting population health programs

Also, HTA can be used to:

- Support decisions by health technology companies about technology development and marketing
- Support decisions by investors in the health care sector
- Inform research agencies about evidence gaps, unmet needs

**Related Concepts**

There is a variety of methods, sources of evidence, and concepts that can be related to HTA or interact with HTA. Among these are:

- Outcomes research
- Patient-centered outcomes research
- Real-world evidence
- Comparative effectiveness research
- Systematic review
- Meta-analysis
- Pharmacoeconomics

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1 [http://htaglossary.net/health-technology-assessment](http://htaglossary.net/health-technology-assessment)
Demand for HTA
Multiple factors contribute to the demand for HTA. Among these are:

- Aging populations
- Increases in chronic disease
- Growth of the middle class in developing nations
- Growth in patient/consumer demand for health information
- Ongoing development and marketing of new drugs, biologics, diagnostics, devices, other technology
- Public attention to high-priced technologies (e.g., for cancer care, rare diseases)
- Large variations in health care practice
- Inappropriate use of health care technologies, including over-use, under-use, and improper use
- “Off-label” uses (i.e., not approved by regulatory agency) of drugs, biologics, devices
- Rising health care costs (constraining resources for other important needs)
- Major reforms of national and regional health care systems
- Concerns about social, ethical, legal impacts of health technology

Unintended and Intended Consequences
Consistent with the origins of TA, health technologies can have unintended consequences/effects as well as their intended ones. Unintended consequences can be beneficial or harmful. HTA seeks to anticipate and examine their implications. Some examples of technologies with unintended consequences in some patients are:

- Aspirin: anticoagulation
- Bariatric surgery: cure for diabetes
- Antibiotics: overuse and improper use resulting in multi-drug resistant bacterial strains
- Highly active anti-retroviral therapy (HAART) for HIV/AIDS: increase in high-risk behaviors
- Medical ultrasound: fetal sex selection
- Prostate-specific antigen (PSA) testing: unnecessary invasive testing, therapies, and adverse effects for some men
Inappropriate Use
Inappropriate use of technology can call attention to the need for HTA. It can occur in different ways, including:

- Over-use: used in patients who are not indicated (i.e., for whom there is no evidence of benefit), or used too frequently in those patients
- Under-use: not used in patients who are indicated, or used too infrequently in those patients
- Improper use: although used in patients who are indicated, used incorrectly (e.g., incorrect surgical technique, incorrect drug dosing, incorrect radiation exposure)

Diffusion of Ineffective or Harmful Technologies
The history of health care technology includes many instances in which technologies became widely used with inadequate or even falsified evidence, only to be discovered later to have serious adverse health effects in at least some patients. Among the many examples are:

- Autologous bone marrow transplantation with high-dose chemotherapy for breast cancer
- Antiarrhythmic drugs
- COX-2 (cyclooxygogenase-2) inhibitors for patients at risk for heart disease, stroke, and certain other conditions
- Hormone replacement therapy for healthy menopausal women
- Intermittent positive pressure breathing
- Magnetic resonance imaging (MRI) for low back pain in first 6 weeks
- Oxygen supplementation for premature infants
- Prefrontal lobotomy for mental disturbances
- Prostate specific antigen (PSA) screening for prostate cancer
- Radiation therapy for acne
- Thalidomide for sedation in pregnant women

These and other examples of technologies, including some in current use, call attention to the need to conduct methodologically rigorous HTA in a timely manner.

Properties and Impacts Assessed
What does HTA assess? The main properties and impacts assessed include:

- Technical: conformity with design, performance characteristics, e.g., pharmacodynamics, diagnostic test accuracy
• Safety: judgment of the acceptability of risk (probability and severity of an adverse outcome) associated with using a technology in a given situation
• Efficacy and effectiveness: how well a technology achieves its intended purpose, especially in health outcomes
• Cost and other economic: microeconomic, e.g., cost-effectiveness of particular technologies, cost burden on patients; macroeconomic, e.g., impact on national health care costs, gross domestic product, employment, resource allocation across health care and other industrial sectors
• Ethical, legal, patient and citizen, and political: impacts on or challenges to normative concepts (e.g., valuation of human life, equity); choices about how and when to use technologies; research and the advancement of knowledge; resource allocation

Efficacy vs. Effectiveness
HTA makes an essential distinction between these terms:
• Efficacy: benefit of using a technology for a particular health problem in ideal conditions of use, for example, in a strict protocol of a randomized controlled trial or at a “center of excellence.”
• Effectiveness: Benefit of using a technology for a particular health problem in general or routine conditions of use, for example, in a community hospital.
• Among the main categories of health outcomes that are used to assess efficacy and effectiveness are:
  • Mortality (death rate)
  • Morbidity (disease rate)
  • Adverse health events (e.g., harmful side effects)
  • Quality of life
  • Functional status
  • Patient satisfaction

Biomarkers and Surrogate Endpoints:
A biomarker (or biological marker) is an objectively measured characteristic (such as from a laboratory test or radiological image) that is used as an indicator of normal biological processes, natural history of disease, or effect of a therapy. Examples:
• Blood pressure
• EKG (electrocardiogram)
• Bone density
• Hemoglobin A1c
Biomarkers are not health outcomes (or endpoints). However, when a biomarker is closely associated with a health outcome (or clinical endpoint), and particularly when it is predictive of a health outcome, it can be a surrogate endpoint. Example:
- Decreased blood pressure for decreased risk of stroke
- White spots on an MRI scan for multiple sclerosis lesions

**Patient-Centered Outcomes**
Patient-centered outcomes and patient-reported outcomes are increasingly important in contributing to evidence that informs care decisions by clinicians and patients, and coverage policies of payers.

Patient-centered outcomes are those that patients experience in real-world settings, including: survival, functional status, quality of life, quality of death, symptoms, pain, nausea, psychosocial well-being, health utility (patient-perceived value of particular states of health), and patient satisfaction. Can be assessed at a generic level or a disease/condition-specific level.

Patient-reported outcomes are those that are self-reported by patients or obtained from patients (or reported on their behalf by their caregivers or surrogates) by an interviewer without interpretation or modification of the patient’s response by other people, including clinicians.

Examples of generic patient-centered outcomes include the following (of which there are multiple versions):
- EuroQol (EQ-5D)
- Health Utilities Index
- Nottingham Health Profile
- Quality of Well-Being Scale
- Short Form (12) Health Survey (SF-12), Short Form (36) Health Survey (SF-36)
- Sickness Impact Profile

Examples of disease-specific patient centered outcomes in multiple sclerosis:
- Multiple Sclerosis Quality of Life Inventory (MSQLI)
- Multiple Sclerosis Quality of Life-54 scale (MSQoL-54)
- Functional Assessment of Multiple Sclerosis (FAMS)
- Multiple Sclerosis Impact Scale (MSIS-29)
- Leeds Multiple Sclerosis Quality of Life scale (LMSQoL).
Hybrid or Combination Outcomes
Another category of health outcomes used in HTA is aggregated outcome measure. These are measures of health improvement (or loss) that combine survival and morbidity (including mortality/survival, quality of life, or functional status) into a single unit:

- QALYs: quality-adjusted life years
- DALYs: disability-adjusted life years
- HYE’s: healthy-years equivalents

A strength of these outcomes is that they enable comparisons of the impact of health care or other changes (e.g., environmental or economic) where the outcomes (other than survival) are not the same, e.g., incidence of diabetes, reduction in heart attacks, or prevalence of tobacco use. They are based on somewhat different assumptions and methods (e.g., for determining quality of life and disability).

QALYs are used more often in cost utility analyses to determine ratio of change in cost to QALYs gained from using a particular health care technology. DALYs are used more often in public health to measure population disease burden and impact of health programs on population health.

Some basic principles of QALYs are:

- It is widely accepted that one year of life spent in a good state of health (or function of quality of life) is preferred to one year spent in a poor state of health.
- “Utility” refers to the relative preference (value) that an individual (or society) has for a particular state of health.
- Utility weights are determined using direct methods, e.g., time trade-off or standard gamble, or indirect methods, e.g., SF-36, EQ-5D, Health Utility Index, Quality of Well-Being Scale.*
- The QALY is a unit for measuring outcomes of health care (or other interventions). QALYs combine length of life with quality of life. That is, years of life are adjusted (weighted) by patient/user utility for the quality of life experienced during those years.
- The QALY may be used as the unit of patient/user outcomes in a cost-utility analysis.
HTA Methods

Three main categories of methods used in HTA are: primary data collection, secondary (integrative) methods, and economic analyses:

- Primary data methods involve collection of original data, for example, using experimental designs (e.g., randomized clinical trials) or non-experimental designs such as observational studies (prospective or retrospective). Most HTAs do not involve conducting primary data collection, but they use evidence from available primary data studies.
- Secondary / integrative analyses combine data from existing sources, e.g., systematic reviews, meta-analyses. Combine data from existing sources, e.g., systematic reviews, meta-analyses, modeling. Most HTAs involve conducting one or more of these methods; some HTAs also involve using evidence from available integrative studies.
- Economic analyses involve various techniques of weighing costs and benefits (outcomes or other results). Many HTAs involve one or more of these analyses.

Primary Data Methods

When HTAs examine evidence from primary data studies, they consider attributes associated with stronger or weaker evidence for determining the causal effect of a technology on outcomes (e.g., efficacy or effectiveness and safety). In general:

- Prospective studies > retrospective studies
- Interventional studies > observational studies
- Controlled studies > uncontrolled studies

Using QALYs to capture changes in length of life (survival) and quality of life (e.g., utility for state of health) resulting from new or additional treatment.
• Studies with contemporaneous control groups > studies with historical control groups
• Studies with randomized assignment of patients to treatment and control groups > studies with non-random assignment.
• Large studies (with enough patients to detect true treatment effects) > small studies
• Blinded studies (patients, providers do not know which intervention is being used) > unblinded studies

Primary data methods include experimental and non-experimental designs. Main types of experimental designs include:
  • Randomized controlled trial (RCT)
  • Randomized cross-over trial
  • N-of-1-trial
  • Group randomized trial
  • Non-randomized controlled trial*
  • Pragmatic trials (randomized or non-randomized)

*A controlled trial in which participants are assigned to treatment and control groups using a method other than randomization, yet intended to form similar groups. Sometimes known as a “quasi-experimental” design.

The diverse types of non-experimental designs include:
  • Prospective cohort
  • Retrospective cohort
  • Case-control
  • Cross-sectional
  • Interrupted time series with comparison
  • Non-concurrent cohort
  • Interrupted time series without comparison
  • Before-and-after
  • Time series
  • Case Series
  • Case study
Evidence Hierarchies
HTA organizations (and others) use various evidence hierarchies or frameworks to rate the quality of individual studies and bodies of evidence (groups of studies). Most of these are based on, or start with, principles of stronger vs. weaker evidence for establishing causal effects of a technology, as noted above. Different types of evidence questions call for different evidence hierarchies. For example, evidence hierarchies for questions about disease prevalence, diagnostic accuracy, or detecting rare adverse events will differ from evidence hierarchies for efficacy/effectiveness of treatments. A basic example of an evidence hierarchy for therapies is:

- Systematic reviews and meta-analyses of RCTs
- Randomized controlled trials (RCTs)
- Non-randomized controlled trials
- Prospective observational studies
- Retrospective observational studies
- Expert opinion

There are many versions of such hierarchies, including some with more extensive levels/breakdowns.

A weakness of such hierarchies is that, while they tend to reflect internal validity of a cause-and-effect relationship between a technology and one or more outcomes (e.g., mortality, morbidity), they do not generally reflect external validity (generalizability) of the evidence to more diverse patients and care settings.

Secondary or Integrative Methods
The main categories of secondary methods, which combine or integrate data from primary data studies, include:

- Systematic literature review
- Meta-analysis
- Modeling (e.g., decision trees, Markov models)
- Group judgment (“consensus development”)
- Unstructured literature review
- Expert opinion
The set of methods shown above is not arranged in a hierarchy. However, the general trend in HTA is to rely on systematic reviews, meta-analyses, and modeling. Unstructured literature reviews are not considered good practice in HTA, because they are especially subject to biases that can diminish the credibility of HTA findings. Although expert perspectives are an important part of certain aspects of HTA, the use of expert opinion as the main method for conducting HTA is not considered good practice in HTA.

Systematic Literature Review
A systematic literature review (or systematic review) is a form of structured literature review that addresses one or more evidence questions that are formulated to be answered by analysis of evidence. Systematic reviews involve:

- development of evidence questions (or “key questions”) that are intended to capture the body of evidence to be assessed
- an objective means of searching the literature, typically using prospectively designed automated searches of bibliographic databases (usually peer-reviewed literature and selected other sources, including “grey literature” as appropriate for an assessment topic)
- applying predetermined inclusion and exclusion criteria to this literature derived from the search
- critically appraising the relevant included literature
- extraction and synthesis of data and information from relevant body of evidence base to formulate answers to the evidence questions
- A systematic review may include one or more meta-analyses.

Systematic reviews differ from unstructured literature reviews in multiple ways. Most systematic reviews start with focused evidence questions, have predefined literature search and inclusion/exclusion criteria to identify relevant evidence, include a detailed description of the methods used, are quantitative (accounting for the numbers of articles retrieved from the initial searches and numbers of articles excluded consistent with predefined criteria), and are reproducible. In contrast, a traditional or unstructured literature review may have broad or poorly defined methods section, be less likely to be quantitative, and not be reproducible.
Most systematic reviews use a format for defining a set of evidence questions and related parameters that define the evidence base for the HTA. A commonly used approach is:

**PICOTS**
- **P**: Patient, population, or problem of interest
- **I**: Intervention or exposure
- **C**: Comparator (basis of comparison, e.g., standard of care, control group)
- **O**: Outcomes (primary or secondary endpoints, e.g., mortality, morbidity, quality of life)
- **T**: Timing (duration of intervention or follow-up period of data collection, if applicable)
- **S**: Setting (location of delivery of intervention, e.g., inpatient, outpatient, home)

For example, an evidence question about treatment for hypertension could be expressed in the following PICOTS format:
- **Population**: males and females age 55-75 years with mild hypertension
  - diastolic blood pressure 85-99 mm Hg
  - systolic blood pressure 130-159 mm Hg
  - no other serious health problems
- **Intervention**: standardized, moderate exercise program
- **Comparator**: usual physical routine and diet
- **Outcomes**: changes in:
  - general and abdominal obesity
  - systolic blood pressure
  - diastolic blood pressure
  - aerobic fitness
- **Timing**: 6-24 months follow-up
- **Setting**: outpatient (clinics, physician offices)
Meta-Analysis

Meta-analysis refers to statistical procedures for combining results from different studies. This combination may produce a stronger conclusion than can be provided by any single study. Meta-analysis is generally most appropriate when there are not definitive studies on a topic (e.g., when their sample sizes are too small) and non-definitive studies are in some disagreement (e.g., when their treatment effects are contradictory or show large variance). The purposes of meta-analysis include:

- Encourage systematic organization of evidence
- Increase statistical power for primary end points and general applicability of findings
- Resolve uncertainty when reports disagree
- Provide quantitative estimate of effect (e.g., effect size or odds ratio)
- Answer questions not posed at the start of individual trials
- Identify needs and planning for major trials

Certainly, meta-analyses can be very useful in synthesizing evidence from multiple sources. However, like other forms of analysis, they can have weaknesses. These may include: publication bias of the primary studies comprising the meta-analysis, biased selection of available relevant studies, poor quality of the primary studies, unexplainable heterogeneity across the studies (differences in, e.g., study populations, delivery/dosage of the interventions or comparators, or how outcomes were measured), and biased interpretation of findings by the authors.

In recent years, there has been increased interest in applying meta-analysis in circumstances in which there are insufficient direct (“head-to-head”) comparisons of technologies. Network meta-analysis is a type of meta-analysis in which multiple (three or more) alternative technologies are compared when there are limited or no available “head-to-head” trials of those technologies. This enables integration of data from available direct trials and from indirect comparisons across trials based on a common comparator, which could be a placebo or standard care. Network meta-analysis is also known as “multiple-treatment” or “mixed-treatment comparisons meta-analysis.”
Modeling

In HTA, modeling refers to analytical techniques for simulating (representing) real processes involving decisions and their outcomes. For example, in determining which technology or regimen option is more effective or cost-effective for a particular patient population, modeling can account for the uncertainties (probabilities) that each option or decision will result in particular outcomes (e.g., health states), and/or the value (e.g., patient utility, cost-effectiveness) associated with each outcome.

Among the main types of modeling used in HTA are: Markov modeling; decision tree; multi-criteria decision analysis; Monte Carlo simulation; and various simulations of disease processes, health care interventions, and health care systems. For example, Markov modeling represents changes from one state of health to another, such as different stages of disease and death. This type of modeling is useful for representing patient or population experience when: health states change over time, some or all of the health states may recur, and there are known probabilities of transition across health states. Markov modeling assumes that each patient is in one of a set of mutually exclusive and exhaustive health states for a given period of time, there is a set of allowable (i.e., non-zero) probabilities of moving from one health state to another (including remaining in the same state from one period to another), and patient utilities and costs can be assigned to each health state.

Shown below is a general hypothetical example of a Markov modeling in which there are four possible health states: normal, asymptomatic disease, progressive disease, and death. Transition probabilities are shown for one-year periods. In this example, a baseline population of 100,000 is followed for two years (i.e., year 0 to year 2) as is progresses through the transition probabilities. A model such as this could be made more complex, such as if the transition probabilities were to differ for patient subgroups (e.g., by age, sex, or comorbidities).

**Markov Modeling Disease States and Transition Probabilities (Annual, Year 0 to Year 2)**

![Diagram showing Markov modeling with transition probabilities and population sizes for Year 0, Year 1, and Year 2.](image-url)
Economic Analyses

HTA can use various types of economic analyses. Each of these has different purposes. Any particular HTA may include one or more of these. The main types include:

- **Cost effectiveness analysis (CEA):** costs weighed against outcomes focused on a single natural unit, e.g., deaths, heart attacks, lung cancer cases
- **Cost utility analysis (CUA):** form of CEA, outcomes aggregated into a unit of utility, e.g., quality-adjusted life-years (QALYs)
- **Cost benefit analysis (CBA):** costs weighed against outcomes aggregated into monetary units. Cost benefit analysis depends on assigning monetary values to the costs of interventions as well as the outcomes.

A basic example of a cost effectiveness calculation, to yield an ICER in the form of a cost per life-saved, is shown below.

\[
\text{CE Ratio} = \frac{\text{Cost}_B - \text{Cost}_A}{\text{Effect}_B - \text{Effect}_A}
\]

For example:

<table>
<thead>
<tr>
<th></th>
<th>Total $ per 100 pts</th>
<th>Lives saved (LS) per 100 pts</th>
<th>Average CE Ratio</th>
<th>Incremental CE Ratio (ICER)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug A</td>
<td>1,000</td>
<td>8</td>
<td>$125/LS</td>
<td>-</td>
</tr>
<tr>
<td>Drug B</td>
<td>1,000,000</td>
<td>10</td>
<td>$100,000/LS</td>
<td>$499,500/LS</td>
</tr>
</tbody>
</table>

A basic cost utility analysis, which is a form of cost effectiveness analysis that uses a measure of patient utility such as a quality-adjusted life-year (QALY) is shown below.

\[
\text{CU Ratio} = \frac{\text{Cost}_Y - \text{Cost}_X}{\text{QALY}_Y - \text{QALY}_X}
\]

For example:

<table>
<thead>
<tr>
<th></th>
<th>Total $ per patient</th>
<th>Life years per patient</th>
<th>Utility*</th>
<th>QALYs</th>
<th>Cost/QALY gained</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug X</td>
<td>5,000</td>
<td>5.0</td>
<td>0.5</td>
<td>2.5</td>
<td>-</td>
</tr>
<tr>
<td>Drug Y</td>
<td>250,000</td>
<td>6.0</td>
<td>0.8</td>
<td>4.8</td>
<td>$107,000</td>
</tr>
</tbody>
</table>

*Patient utility for remaining life years, ranging from 0=death to 1.0=perfect health
A useful concept for understanding the concept of cost-effectiveness is the cost effectiveness plane. This can be understood in four quadrants, which depend on the differences in costs and the differences in outcomes between a technology and its comparator, as shown below. The CEP plane demonstrates that any interventions that fall in either quadrant II or IV do not need ICERs to be calculated as they are either more effective and less costly (II), or more expensive and less effective (IV). Interventions falling in quadrant II are typically accepted, while those falling in quadrant IV are typically rejected. Since interventions in quadrant I are more effective but more costly, and those in quadrant III are less effective but less costly, ICERs need to be calculated and compared.

HTA has increasingly included budget impact analysis in recent years, particularly to provide a distinctive perspective of “affordability” to complement cost effectiveness analysis in support of decision making. Indeed, allocating resources efficiently (e.g., maximizing cost-effectiveness) may not be consistent with affordability, i.e., remaining within a particular budget.

**Cost Measurement**

Some key attributes to examine quality of cost measurement are: perspective for measuring cost; direct costs (health care and non-health care); indirect costs (e.g., loss of productivity); data capture method; time horizon of analysis; discounting of costs and outcomes over time.

Cost Utility Analysis Using Cost per QALY

QALYs are often used in cost-utility analysis for the purposes of optimizing allocation of health care spending to maximize QALYs gained, and thereby maximize social welfare. Cost per QALY gained, i.e., the marginal (additional or incremental) cost required to gain 1.0 QALY by using a technology (a type of ICER), is one way to quantify the value to society of using that technology instead of the standard of care or other alternative. Because the QALY incorporates length of life and quality of life but is not specific to any particular disease state or condition, it enables cost-utility comparisons across a wide spectrum of health care interventions.

Certain cost-per-QALY-gained levels have been cited as formal or informal decision “thresholds” for coverage of new interventions (e.g., the equivalent of approximately US$50,000, or US$100,000, or US$150,000 per QALY in the wealthy nations). There is increasing interest in using such thresholds in low- and middle-income countries, where the thresholds are adjusted downward for national wealth, sometimes as a function of gross domestic product (GDP) per capita. Comparisons of the cost per QALY gained from various health care interventions in widespread use can be revealing about how efficient health care systems are in allocating their resources.

Evidence Sources for HTA

The field of HTA has made great advances in methods for systematically searching literature to assemble the evidence base for conducting HTA. In planning for what types of literature should be searched, some considerations might be whether the following should be included any particular languages; grey literature; and data sets, registries, or information about ongoing clinical trials.

Depending on the purpose, scope, and target audience of an HTA, the potential sources of evidence and related information for an HTA could include any of: bibliographic databases, study registries, scanning reference lists, queries to authors, searching for grey literature and so on. Among the bibliographic databases used most often in HTA are:

- PubMed (including MEDLINE)
- EmBase
Horizon Scanning
In HTA, horizon scanning refers to ongoing tracking of multiple information sources to identify potential topics for HTA and provide input for setting priorities. Horizon scanning reports can provide rapidly completed, brief descriptions of new/emerging technologies and their potential impacts. While horizon scanning usually is used to identify new technologies that eventually may be topics for assessment, it can also involve identifying technologies that may be outmoded or superseded by newer ones. Horizon scanning can be used to:

- Identify technologies that have potentially major implications for health care
- Manage adoption and use of new technologies
- Identify areas of technological change
- Identify inappropriately used (including under- and over-used) technologies
- Enable health care providers, payers to plan for, adapt to technological change
- Plan data collection to monitor adoption, use, and impacts

Priority setting in HTA
Given the many technologies that could be assessed in HTA, it is necessary to set priorities. Horizon scanning can help to identify candidate technologies for assessment. Most HTA programs establish criteria and processes for selecting technologies for assessment. Examples of such criteria include:

- High individual burden of morbidity/mortality
- Large number of patients affected
- High individual or population cost of disease
- High unit or aggregate cost of technology
- Substantial variations in practice
- Unexpected adverse event reports
- Available findings not well disseminated or adopted by practitioners
- Sufficient research findings available upon which to base assessment
- Recent or impending regulatory approval
- HTA findings likely to have impact on clinical practice or coverage policy
- Public or political interest/pressure
**Bibliographic sources and suggested readings**


Introduction
The research protocol is an essential part of a research project. A research protocol defines how the assessment will be conducted. The preparation and writing of the research protocol starts after the topic is selected, a policy question is defined and the assessment team is put together. In the following paragraphs, the reader can learn more about the purpose of an HTA protocol and the components which are part of an HTA research protocol.

The purpose of an HTA protocol
The HTA protocol is a full description of the (planned) research and production of the assessment report in the field of HTA. In many cases, the research protocol is also called project plan, whereas the actual research done and the results are documented in an assessment report. The protocol serves as a reference to members of the assessment team to ensure that everyone adheres to the process of creation, the methods and the provisional timeline outlined. The assessment team can be made accountable to the protocol depending on the obligation to follow the project plan, which can vary from HTA institution to HTA institution depending on the purpose of the report.

In most cases, the protocol and the final assessment report are based on templates with common elements and items. These templates vary between countries and institutions as they develop the templates independently with their specific requirements and policy question in mind, which is due to the different health care systems. The EUnetHTA HTA project, for example, is a first pan-European effort to develop common templates for joint assessments.

The use of templates guarantees that the form, structure and content of each research protocol look the same. This standardisation of the protocol helps an efficiency gain in the production and, more important, a fixed and verifiable quality standard. An HTA protocol answers to the following questions (Perleth et al. 2014):

- which aspects and domains should be considered in the assessment
- which information sources should be searched for each chosen domain
- which methods will be used for the assessment of the information sources
- how will the evidence be analysed and summarised

1 The policy question addresses the following aspects: the initiator of the HTA report, the institution which commissioned the assessment, the reason for the timing, the decision to be informed, the recipient/end-user of the report. In many cases, the elements of the policy questions are recurrent and framed into a statutory health context, e.g. horizon scanning, reimbursement decisions.

2 www.eunethta.eu
Writing the research protocol

The writing of the research protocol by the assessment team happens after the formulation of the policy question and the establishment of the assessment team and ends with the publication of the protocol. In some cases, the protocol needs approval by executive bodies within the agency or a consultation of stakeholders (e.g. patients, service users) may follow. The assessment team is composed of the authoring team and the dedicated reviewers who check the project plan.

If, during the development of the protocol the research questions have altered, this needs to be agreed with the commissioning body before the work starts. Modifications to the protocol may arise from a clearer understanding of the research question. Any modification to the protocol should be clearly documented and justified in the assessment report and in a protocol addendum.

The writing of the protocol itself can be separated in different schematic steps: kick-off, scoping, writing the protocol by the authoring team, review of the protocol by dedicated reviewers and other stakeholders (e.g. patients, external experts), revision, approval and publication, sometimes complemented by a consultation of stakeholders. All steps follow a predefined time plan.
Figure 1. Example of a process of writing a research protocol

1. Kick-off with assessment team
2. Collection of background information
3. Determination of research questions (e.g., PICO)
4. Stakeholder input (e.g., patients, external experts)
5. Writing the research plan
6. Research Protocol (1st draft)
   - Key sections of a research protocol:
     - Research questions
     - Search strategy
     - Study selection
     - Data extraction
     - Quality assessment
     - Data synthesis
     - Conflict of interests
7. Review of the protocol by independent reviewers
8. Revision
9. Final research protocol
10. Publication and public consultation, if applicable
Problem description and background information

An HTA protocol starts in a first section with the problem description and the presentation of the background information about the health problem at stake and the technology. Based on this information, further research questions crystallise. Both steps, which are described here consecutively, actually happen at the same time. The background information can address the following aspects for example:

- Characteristics of health problem and illness
- Epidemiology, prevalence
- Alternative treatments
- Current medical standard of therapy/ diagnosis
- Description of the technology (status of the technology)

Table 1. Background information about the health problem and patient population (Perleth 2014)

<table>
<thead>
<tr>
<th>Question</th>
<th>Example</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Health problem</strong></td>
<td>Illness</td>
</tr>
<tr>
<td></td>
<td>Condition</td>
</tr>
<tr>
<td><strong>How does the illness work?</strong></td>
<td>Aetiology, pathology</td>
</tr>
<tr>
<td><strong>How does the illness develop and progress?</strong></td>
<td>Presentation of symptoms, stage of illness and progression</td>
</tr>
<tr>
<td><strong>Which are the consequences?</strong></td>
<td>Disabilities, symptoms, health-related quality of life, death</td>
</tr>
<tr>
<td><strong>Alternative ways of treatment</strong></td>
<td>Drug</td>
</tr>
<tr>
<td></td>
<td>Operation</td>
</tr>
<tr>
<td></td>
<td>Current standard of intervention</td>
</tr>
<tr>
<td><strong>Population (epidemiology and burden of disease)</strong></td>
<td>Patients</td>
</tr>
<tr>
<td></td>
<td>Healthy individuals</td>
</tr>
<tr>
<td><strong>How many persons are affected?</strong></td>
<td>Incidence and prevalence</td>
</tr>
<tr>
<td><strong>Who is affected?</strong></td>
<td>Age</td>
</tr>
<tr>
<td></td>
<td>Sex</td>
</tr>
<tr>
<td></td>
<td>Risk factors</td>
</tr>
<tr>
<td></td>
<td>Socio-economic factors</td>
</tr>
</tbody>
</table>
In order to generate background information, authors can conduct preliminary scientific literature searches for orientation. These searches are not comparable to systematic searches aimed at providing the answers to the research questions.

**Definition of the research question**

Based on the background information, the aim of this step in the HTA assessment is to identify important aspects and domains for the assessment in order to specify the research questions and scope of the assessment. Another term for the preparation of the assessment (collecting background information, defining research questions is therefore ‘scoping’). If the HTA assessment is depicted in phases, the definition of the research question takes place in the scoping phase of the assessment, followed by the development of the project plan (see figure 1), whereas the phases are overlapping.

The following nine domains can be subject to an assessment including the aspects covered by the background information:

- Health problem, epidemiology, prevalence, alternative treatments, Current medical standard of therapy/ diagnosis
- Characteristics of the technology
- Safety
- Effectiveness,
- Cost-effectiveness,
- Ethical aspects
- Patient and social aspects,
- Legal aspects and
- Organizational aspects

<table>
<thead>
<tr>
<th>Question</th>
<th>Aspects</th>
</tr>
</thead>
<tbody>
<tr>
<td>How does the technology work?</td>
<td>Drug or medical device, technical characteristics</td>
</tr>
<tr>
<td>What are the prerequisites of an application?</td>
<td>Prerequisite for application, qualifications, maintenance</td>
</tr>
<tr>
<td>What is the status of the technology?</td>
<td>Marketing Degree of use Fields of application Current regulations Manufacturers Benefits and costs</td>
</tr>
</tbody>
</table>

Table 2. Background information about the health problem and patient population (Perleth 2014)
Moreover, the research question should identify, inter alia, the intervention to be assessed, the comparators to be considered, the outcome criteria according to which the benefits of the technology will be measured, and the patient population to which the intervention and its comparators are applied. The so-called PICO model summarizes all these aspects and is a strategy for formulating questions and search strategies. PICO stands for four different potential components of a research question:

**PICO**
- **P**: Patient, population, or problem (e.g. age, sex, illness)
- **I**: Intervention, (= pharmaceutical, diagnostic or therapeutic procedure)
- **C**: Comparison (= comparator, e.g. current medical standard in treating the illness)
- **O**: Outcome (= patient-relevant endpoints, e.g. survival)

The classic PICO question may be extended by:
- **S**: Study design (= study type, e.g. randomised controlled trials, non-randomised controlled trials)

The PICO schema determines which studies are going to be considered in the data analysis: the study selection follows the criteria agreed on by the research questions. Therefore, in practice, other criteria can also be decisive in selecting studies (e.g. further requirements to be met by the study, setting of a study, language, type of publication, and period of publication). In the course of an assessment, it can always become apparent that the criteria need to be adapted which is then justified in the assessment report.

Research questions should be clearly formulated, answerable, restricted in quantity, consider patient-relevant endpoints and relevant comparators. The selection of domains and the PICO question(s) determine the following research plan and strategy.

**Involvement of external stakeholders in the scoping phase**
When drawing up the research question, the assessment team can ask for the expertise of external stakeholders: When determining the outcomes to be analyzed, it can be helpful to involve patients who are living with the condition in question, in order to ensure that the outcomes are important and relevant from a patient’s point of view.

3 https://linkeddata.cochrane.org/pico-ontology
Moreover, they may have information on the disease and treatment process, which is not accessible to the assessors through the evaluation of clinical studies. If the patient is not able to communicate, as a result of the illness or because of being a child, a caregivers’ perspective may be useful in such cases.

The way of involving patients in the scoping phase can take various forms: e.g. questionnaire, (telephone) interviews. Patients can be consulted regarding the following aspects:
- their disease/condition and their unmet needs,
- currently available treatments,
- expectations with respect to new treatments (e.g. fewer side effects),
- identification of subgroups and possible effect modifiers,
- quality of life issues,
- target treatment population and risks of off-label use

By involving external experts, the HTA assessors gain an overview in the medical context, in which the pharmaceutical or the medical device will be applied. A consultation of external experts at this moment in the assessment process, allows identifying relevant issues at an early stage, which need to be considered in the assessment of the technology. During the scoping phase, the following topics may be included in a questionnaire to be distributed to the experts:
- illness and effects of the illness
- aims of therapy
- patient in daily care
- therapy options (drug and non-drug)
- therapeutic need beyond the existing therapy options
- status of the current medical standard

There are other ways for involving external experts in the assessment: they accompany the assessment process and are at the disposal of the assessment team if it has questions regarding the condition and related aspects.

There are examples of involvement of manufacturers in HTA processes (e.g. France, EUnetHTA). By the so-called ‘fact check” manufacturers can check if the data they submitted are displayed correctly. The involvement of manufacturers in assessment is a contentious issue and would demand a chapter of its own. The main point is the strict requirement of guaranteeing the independence and objectivity of the assessment team and the HTA report.
Research plan to answer the research questions
After having outlined the background information, the scope and PICO, the description of the search strategy follows in a second section. This part presents the selected information sources and search strategies for the assessment.

A following section explains the procedure for study selection (2-stage procedure or 3-stage procedure with four-eyes-check) and the procedure for data extraction. This goes into further detail, for example the protocol could specify whether authors of primary studies be contacted to provide missing or additional data.

The protocol describes which methods of data analysis and synthesis will be applied (e.g. risk of bias assessment, meta-analysis, sensitivity analysis, analysis of subgroups and effect modifier). As analyses will depend on what data are available, and because it is difficult to anticipate all of the statistical issues that may arise, it can be difficult to pre-specify full details of the planned synthesis. Therefore, the need of adapting the protocol may occur. Any change in the protocol has to be reasoned and documented with strict demands on quality and transparency.

The project plan ends with a list of literature collected during the preliminary literature search for generating the background information and determining the research questions.

The HTA protocol also contains a section on the time-plan for the assessment and the conflicts of interest of the involved parties to the assessment. As the study selection and data extraction and review of the protocol are commonly executed by several researchers, a procedure on how to solve disagreements between the involved parties needs to be described in the protocol.

Review of the research protocol as quality check
The reviewers are part of the assessment team and are selected when it is appointed at the beginning of the project. The reviewers should work independently from the authoring team.

The review of the research protocol ideally is based on a set of review questions:
- the readability and completeness of the document,
- the consistency of terminology,
- the adherence to applicable guidelines,
- the check of information retrieval strategy,
• the consistency of inclusion and exclusion criteria for study selection,
• the check if the process of stakeholder involvement (patients, external experts) is described appropriately,
• the check of the timelines (feasible, correct).

Finally, external experts may also review the research protocol at the same time as the reviewers or after them.

**Publication of the HTA protocol and consultation**
The research protocol is published in the internet. In this way, the assessment team can be held accountable for what it has planned to do in relation to what it reports in the final report. Since assessments conducted by public bodies have an impact on budget decisions and affect a wide range of stakeholders, a publication of the research protocol answers to calls for transparency. Moreover, a public consultation of the protocol might be considered. External stakeholders may make helpful statements on the research protocol, for consideration by the assessment team.
Bibliographic sources and suggested readings

Perleth et al. 2014. The Perleth 2014 reference is in German hand had not been translated unfortunately. We will check for English references.

www.eunethta.eu.

https://linkeddata.cochrane.org/pico-ontology
Ethical Evaluation in HTA

Authors: Kenneth Bond, Georges-Auguste Legault, Sylvia Nabukenya, Wija Oortwijn, Dario Sacchini, the HTAi Ethics Interest Group
**Introduction**

Fundamentally, a health technology assessment (HTA) aims to help inform the overall question “what is good in health (including social and long-term) care?” Ethics analysis aims to provide a systematic reflection about what is of importance to those developing, using, and affected by a particular health technology and provides guidance about the course of action that best reflects this importance. Ethical issues are present in the assessment of every health technology. Nevertheless, the extent to which these issues are explicitly identified and discussed may vary and will depend on the decision that is being informed by the HTA, the expertise available, and the willingness to make ethics an explicit part of the discussion of the appropriateness of the health technology being assessed.

Our objective in this chapter is not to provide an introduction about how to conduct ethics analysis in HTA. We believe working examples of how ethics analysis proceeds are illustrated well in the many references contained here, and highly selective examples of partial analyses may be misleading and counterproductive. Instead, we aim to provide a brief description of the nature and importance of ethics analysis in HTA, the approaches that have been proposed and used, how approaches may differ depending on the type of health technology being examined, and provide some examples of recent ethics analysis. We end the chapter with a list of books and journal articles for those wishing to delve more deeply into this fundamental topic in HTA.

**What is ethics analysis in HTA and why is it needed?**

Ethics is at the core of the HTA decision-making process and an inherent part of HTA. Recognizing this, early and more recent definitions explicitly include ethical issues within the scope of assessment (Autti-Rämö 2007; INAHTA 2019; WHO 2019). Decision-making consistently involves facts and values because determining the best thing to do within a specific context involves value judgments that are made on the factual analysis of this context. Substantiated value judgments (Legault et al. 2018) allow for more transparency in the final conclusion. Since the fundamental goal of the HTA process and its reports is to guide the decision-makers, an ethical consideration of the implied value judgments in the final recommendation or decision should be communicated in the final report and therefore become an active part of the HTA process.

Given the implicit nature of value judgments in the decisions made during the HTA process, eliciting these implicit value judgments is the first approach proposed for integrating ethics into HTA.
Eliciting value judgments is a reflexive process by which reasonable grounds of the evaluation that guide the decision-making process are clarified. Such a reflexive approach on the decision-making process in HTA provides an opportunity for HTA producers to take into consideration the different ethical aspects involved in their activities. The HTA producer’s awareness of the value judgment implied in their process can influence and provide grounds to the choices made throughout the HTA process. One approach to gaining this awareness is the Socratic approach proposed by Hofmann (2015). In the Socratic approach, relevant moral questions guide the identification and elucidation of some of the most fundamental value judgements. An example would be to ask, in the context of the evaluation of bariatric surgery for morbid obesity, whether this health technology challenges our conception of the treatment of disease, for example, by modifying healthy organs in order to reduce disease symptoms.

A new drug, a new medical device or a new health intervention has many impacts. Notwithstanding their importance, clinical effectiveness, safety and cost-effectiveness represent only three impacts among others. Not only is the clinical state of the patient impacted, but so is their quality of life in his or her social setting. Sometimes the introduction of new health technologies have a major impact on health professionals’ practices and their organizational settings as well as their procedures. Ethical analysis often regroups the analysis of such impacts as proposed by Busse and colleagues (2002) as psychological, social and ethical impacts. Other reports gather them under the heading of “contextual considerations” or “ethical, legal, and social considerations.” Usually the information gathered under these headings list different ethical issues related to what is assessed. When a systematic analysis of the ethical dimensions involved is carried out in a way similar to that done for clinical effectiveness, safety or cost-effectiveness, this is another way of integrating ethics into HTA. This type of analysis requires an ethical framework that structures the analysis. Different ethical approaches have been proposed and used in HTA reports.

**What approaches for ethical analysis have been used?**

In the literature, the following approaches have been identified for addressing ethical issues in HTA: principlism, casuistry, coherence analysis, wide reflexive equilibrium, axiology, social shaping of technology, constructive technology and the triangular method (Assasi 2014). These approaches, and their applicability, which are described in more detail elsewhere (Assasi 2014; Hofmann 2014), can be divided into norm-based and value-based approaches. In norm-based approaches, the facts that will be correlated to a moral norm must be made explicit in order to identify conformity or non-conformity with the norm.
In value-based approaches, the impacts of the health technology (e.g. harms, benefits, social-cultural issues) will be subject to ethical evaluation (Patenaude 2017; Assasi 2014).

To guide practices for integrating ethical analysis in HTA, the proposed framework of Assasi et al. (2016) can be useful. The framework consists of three building blocks:

1. A flowchart that provides an overview of the steps to be taken for conducting ethical analysis in HTA.
2. A more detailed description of the different steps, based on earlier work:
   - define the objectives and scope of the evaluation
   - perform stakeholder analysis
   - assess organizational capacities
   - frame ethical evaluation questions
   - perform ethical analysis
   - deliberate with experts and stakeholders
   - knowledge exchange/translation to decision-making
3. An overview of commonly used tools for ethical analysis in HTA.

Three main approaches to ethical analysis are commonly found in HTA reports. The first identifies, without further analysis, the ethical issues that can be raised by the introduction of a health technology. The four principles of bioethics, as articulated and made popular as principlism by Beauchamp and Childress (1994), are often used to report the ethical issues related to beneficence and non-maleficence such as quality of life, personal preferences, value of social or economic impacts for the patient, autonomy such as free and informed consent, and justice such as equity in the delivery of treatment.

The second approach not only identifies the ethical issues at stake, but also describes the social debates surrounding them, such as in the guide of the Haute Autoritééen Santé (2016) which weights the relative force of the arguments presented for each of them.

A third kind of approach goes a step further and evaluates the issues by using a specific ethical or moral approach such as casuistry, coherence analysis, wide reflective equilibrium and the ‘triangular model’ based on a human-centred approach (EUnetHTA 2014). As one might suspect from this brief overview, there is no universal approach for integrating ethics in HTA and there is a lack of consensus on a practical method for conducting ethical analysis (Bellemare 2018).
Moreover, the choice of a particular approach is not value-free; it must be justified by a moral point of view (Patenaude 2017).

**What different approaches might be taken, for example, for single technologies, for complex technologies, for innovative technologies?**

As the diversity of approaches for addressing ethical issues in HTA, there are several approaches for carrying out a full HTA. The European network for Health Technology Assessment (EUnetHTA) represents one important initiative to offer a common framework for addressing the different HTA domains, also including the ethical one (Kristensen 2009). The HTA Core Model® (HTA Core Model) is a standardized synthesis of available methods intended to address all relevant considerations in the HTA process. The basic idea of the HTA Core Model is to structure the contents of an HTA into pieces of information. The HTA Core Model does not purport to solve the philosophical debate, but to offer a tool for identifying, explicating and organizing ethical issues for HTA organizations, irrespective of their resources (material, time and knowledge). It has three elements: a question-based approach that covers issues essential for ethical analysis within HTA; a brief explanation of methods that can be used to approach the issues; and a discussion on the integration of ethical analysis into the process of HTA (Saarni 2008; Sacchini 2016).

There exists no explicit HTA Core Model application for complex health technologies, such as disease management or public health programs. The Collaborative Project INTEGRATE-HTA (integrated health technology assessment for evaluating complex technologies) aimed to developed concepts and methods for HTA to enable a patient-centred, integrated assessment of the effectiveness, and the economic, social, cultural, legal, and ethical issues of complex health technologies that takes context and implementation into account. INTEGRATE-HTA is explicitly addressing the complexity of health technologies. It represents an attempt to overcome two possible limitations of the HTA Core Model. On the one hand, it addresses the risk of separation between the more technical or quantifiable (e.g. effectiveness, safety, economic) and non-technical (e.g. organizational, ethical, legal and social) domains. On the other hand, INTEGRATE-HTA more explicitly focuses on the process, including the public-patient involvement in scoping questions for the development of HTA as well as the actual decision-making process.

More specifically, the INTEGRATE-HTA project developed guidance on how to assess the effectiveness and economic, social, cultural, legal, and ethical issues of
complex health technologies; to elicit patient preferences and patient-specific moderators of treatment; to include context, setting, and implementation in the assessment of complex health technologies; to choose adequate qualitative evidence synthesis methods; and on how to integrate all these issues to a patient-centred, comprehensive assessment of complex health technologies. The guidance was applied in a case study on palliative care (Bakke Lysdahl 2016; Gerhardus 2016; Van Hoorn 2016) and showed that the key concepts and methods could provide a helpful structure for the integrated assessment and analysis of a complex health technology and for reporting clearly what had been done and why. Some experiences utilizing the INTEGRATE-HTA framework have been reported. (Bond 2017) The development of frameworks for the analysis of ethical issues in newer and challenging areas of HTA, such as in disinvestment, is nascent, but progressing.

Examples of how ethics analysis has been done (by different HTA agencies or ethicists)

The previous sections outlined a number of different approaches that have been used in ethical analyses of HTA and these approaches have been employed in practice by different HTA agencies to analyze ethical issues raised by health technologies. The Swedish Council on Health Technology Assessment (SBU) developed a context specific framework that adapted principles of the Swedish ethical platform for healthcare priority-setting (Heintz 2015). This framework relied on four aspects to consider when developing a framework for ethical analysis in HTA: (1) The use of checklists at specific stages of the HTA process; (2) The use of standpoints to provide guidance for assessment of ethical aspects such as equity, autonomy, and privacy based on laws, regulations and case rulings of agencies or courts. This also involved the use of the “need and solidarity principle” which focuses on health care resources being given to those in greater need and implies that not everyone can have their needs met in part or at all; (3) Questions to consider on how health technology is funded in particular contexts and its implications on the organization’s interests, the specific role of the organization that will adopt the framework which introduces the cost-effectiveness principle that involves choosing between different interventions an individual needs to strive for a reasonable relationship between costs and effects, measured in terms of improved health and quality of life; (4) The availability of ethics experts to assess the ethical aspects with respect to the ethical theory and argumentation. The framework also outlines the effects of the intervention on health, its compatibility with ethical norms, structural factors with ethical implications and the long-term ethical consequences.
For example, the report identified specific social aspects about the technology arising from patient interactions that are not necessarily related to clinical ethics.

While bariatric surgery has shown positive results in weight reduction in the past several years (Buchwald 2004), it is associated with high costs and a rise in health care expenditure. In addition, there is inadequate information on the psychological consequences (Pratt 2009), and this inadequacy has raised questions about the patients’ autonomy such as the validity of informed consent (Madan 2007) as well as the symbolic value of the surgery and its effect on distribution of healthcare. The analysis also identified challenges to human integrity or dignity, the social conception of an individual, and the relationship between physician and patient.

The Canadian Agency for Drugs and Technologies in Health (CADTH) produced an ethical analysis of DNA mismatch repair deficiency (dMMR) testing for Lynch syndrome diagnosis among patients with colorectal cancer. This test can help to optimize chemotherapy treatment in colorectal cancer patients and diagnoses Lynch syndrome at an early stage for treatment. The ethics analysis examined issues identified in the literature as well as those identified by an analysis of the other sections of the HTA report and raised and discussed issues regarding ten “core values” that are challenged by the use of dMMR testing: patient autonomy (informed consent for both tumour and germline testing, maintaining confidentiality, etc.), maximizing benefits and minimizing harms to patients, others (burden and anxiety of testing, harm of undiagnosed or late-diagnosed cancer, etc.) and to populations, the duty to warn, distributing benefits and burdens fairly, providing excellence in health care, consistency, relational considerations, and stewarding scarce health care resources.

**Current Methodological Issues in Ethics Analysis in HTA:**
Two areas that are in the early stages of development are competencies for ethics analysis and quality assessment of ethical analysis. As these topics develop, they will have an important influence on how and when ethics analysis is conducted.

Skepticism about the existence of ethical expertise and the difficulty of finding relevant experts have been advanced as reasons for why ethics analysis has not been successfully integrated into HTA (Hofmann 2014; ten Have 2004). Ethicists and philosophers are normally not trained in evidence-based-medicine, HTA, or health policy decision-making processes, while HTA experts are rarely trained in ethics (Hofmann 2015).
The questions of who possesses the relevant expertise for conducting ethics analysis in HTA and which qualifications are necessary are important and are the subject of discussions in the international ethics in HTA community (Hofmann 2015). Some HTA researchers have proposed that conducting these analyses demands a reasonable amount of knowledge of ethical theories and principles and at a minimum, competent researchers in this area should be familiar with approaches in clinical ethics and moral philosophy (Bond 2014). Regardless, philosophical expertise is only one aspect of competency for conducting sound ethics analysis in an HTA.

One recent attempt to articulate and clarify the relevant dimensions of competency for conducting ethics analysis in HTA describes three main domains: knowledge, skills, and attitudes (Sacchini and Refolo 2018). Knowledge covers both the basic knowledge of typical HTA content, comprising an understanding of scientific concepts in epidemiology and health economics as well as a general understanding of the theories and approaches of moral philosophy (e.g.: utilitarianism, casuistry, egalitarianism) and common bioethical issues (e.g.: self-determination, privacy, autonomy, informed consent, etc.). Advanced knowledge includes an understanding of specific debates about ethics in HTA, methods of ethics for HTA, and health law and policy.

The domain of skills includes abilities in ethical assessment (clearly articulating ethical concerns and distinguishing them from overlapping issues, identifying relevant beliefs and values, and gathering relevant information), HTA process (communicating and collaborating effectively, identifying and resolving potential conflicts of interest, and identifying who needs to be involved in consultations), and interpersonal skills (facilitating communication, educating others about the existence of the ethical domain, and eliciting the moral views of various stakeholders). The attitudes deemed important include integrity, prudence, courage, open-mindedness, and respect for multiculturalism. Defining and agreeing on the core competencies required for good quality ethics analysis remains to be done.

Quality assessment of ethics analysis is still nascent and there do not currently exist any broadly accepted tools or checklists for conducting this quality assessment as there are for assessing the strength of primary empirical studies, systematic reviews, and economic evaluations (Scott et al 2014). The development of a quality assessment tool for ethics analysis is thought to be desirable for a number of reasons.
It would increase the transparency and readability of ethics analyses; it could help to assess the transferability of ethics analyses across jurisdictions; and it would provide a common vocabulary and means of structuring communication among members of an HTA team.

Members of the HTAi Interest Group on Ethics in HTA have published two articles (Scott et al 2014; Scott et al 2016) describing a tool they have developed for quality analysis that provides guidance on assessing both the content of the argument of an analysis as well as the applicability of the results and potential sources of bias. The Q-SEA (Quality Standards for Ethics Analysis) checklist divides the quality criteria into those bearing on internal or process features of the analysis and external or output features. The process domain includes ratings of the reporting and conduct of the research question, the literature search, description of the perspective, and the ethics framework employed. The output domain aims to rate the reporting and conduct of completeness of the analysis, identified sources of bias, policy implications, conceptual clarification, and identification of conflicting values (Scott 2016). While the Q-SEA tool has good face validity, it has yet to be formally validated and its feasibility and utility as a quality assessment tool for ethics analysis within HTA remain to be assessed.

Concluding Remarks
HTA is a moral enterprise and making explicit the many value judgments involved in designing, conducting, and interpreting the results of an HTA is essential to decision-makers to make reasonable, fair, and sound decisions at multiple levels (clinical, institutional, and ministerial). The newcomer to HTA is an area with challenging issues, partially unresolved, but which is essential to consider. At least identify ethical issues relevant to an HTA, if necessary with referral to others for resolution. While many methods exist for analyzing ethical issues in HTA, there are more methods than actual analyses and HTA agencies face a number of conceptual and operational challenges in advancing this important work. Articulating who can do this work and what it looks like to do this work well is an important next step in facilitating the conduct and uptake of ethics analysis.
Bibliographic sources and suggested readings


Suggested books


Additional journal articles not cited within the text


HTA in Hospitals

Author: Americo Cicchetti
Introduction
The use of HTA as a part of the decision-making process at national, regional, and international levels has evolved considerably over the past 40 years. However, a large number of good-value innovative Health Technologies (HTs) never reach clinical practice while, in many instances, others with no significant added value actually do. This situation can create an ineffective and inefficient resource allocation having impact on the health of the populations and equitable access to healthcare in public healthcare systems facing financial pressures.

A way to overcome this problem is by approaching HTA at hospital level, notably because hospitals are the main entry level for innovative HTs. The adoption of the HTA logic to support managerial as well as clinical decision making at hospital level has been labelled “hospital based health technology assessment” (HB-HTA). HB-HTA can be considered as one possible approach to enhance the use of HTA for managerial decision making in hospitals and other healthcare organizations (HCOs) and to improve the use of evidence, complemented with local information, to inform clinical practice in the “real world”.

Traditionally, HCOs are mainly considered as HTA users. More precisely, hospitals, as well as other HCOs, are seen as places where clinicians and other healthcare professionals use – or should use – HTA to support their decision making processes. Recently, HCOs’ role as HTA producers is becoming increasingly crucial. In fact, ad hoc units are being established with the special purpose to produce customized HTA reports (mini-HTAs) and enhance decision making processes, by applying HTA principles and methods.
Moreover we have assisted to an evolution of the landscape in HTA where national and regional HTA bodies are networking with hospitals to produce and use HTA at different levels.

Thanks to the recent EU funded research project AdHoPHTA (Adopting HTA in European Hospitals) we have a full range of evidence regarding the applications of HTA at hospital level. The project has also produced pragmatic tools that can be ready-to-use such as the mini-HTA template and the AdHopHTA Database. The vast majority of results of the AdhopHTA project have been summarized in a “Handbook”.

The AdHopHTA project has also produced pragmatic outputs: Guiding principles for good practices in HB-HTA and related toolkit; the AdHopHTA Mini-HTA Template; the mini-HTA quality checklist; A database of HB-HTA reports (AdHopHTA Database).
Thanks to this work we have now diffused knowledge about potential applications of HTA at hospital level and a set of tools publicly available that are fostering the diffusion of HTA at hospital level. This chapter is dedicated to clarifying what we should intend for HB-HTA, which are the motivations for the diffusion of HTA in hospitals and how it has been applied worldwide. The chapter is also providing basic information about procedures and tools of HB-HTA and its role within the whole HTA ecosystem.

**Definition of hospital based HTA**

HB-HTA consists of the implementation of principles, methods and tools of health technology assessment at hospital level. The contextualization of HTA to a specific hospital brings into the assessment process the consideration of its unique characteristics, such as the choice of an available comparator and the specific organizational structure of the hospital. Hospital based HTA can be performed by a team of professionals working at the hospital, or by an external team of professionals that is generating structured evidence to support decision making at hospital level.

**Definition**

Hospital-based Health Technology Assessment (HB-HTA) means performing HTA activities tailored to the hospital context to inform managerial decisions on different types of health technologies. It includes the processes and methods used to produce HTA reports in and for hospitals.

Definition developed by the partners of the AdHopHTA project (AdHopHTA Handbook; Sampietro-Colom et al, 2015)

**HB-HTA: a tool for healthcare management**

Different motivations can explain the diffusion of the adoption of HTA methods and tools at hospital level. Taking an “HTA” perspective, HB-HTA can be seen as a solution to support the implementation of national-level HTAs' recommendation in clinical practice (Cicchetti et al. 2008). The problem of “diffusion” of HTAs and their impact is one of the major issues, still to be solved by HTA agencies (Jacob & McGregor, 1997). An HTA report tailored for the specificity of the organizational context could help the implementation of a recommendation (McGregor and Brophy, 2005). On the other hand, HB-HTA can be considered as a solution for healthcare managers facing the major challenge to decide effectively about the adoption of medical technologies in a challenging context. The role of HTs is crucial for success (or failure) of any healthcare organization.
Hospitals are more and more acting under budget constraints and economic pressures as a result of the tendency present in many healthcare systems to foster micro-economic efficiency to reach financial equilibrium at system level. Actually, a sustainable and successful healthcare organization should be based on a perfect combination of profound professional competencies and valuable health technologies.

Health technologies, in fact, are crucial for clinical effectiveness. On the other hand, new technologies usually increase the costs of care creating pressures for financial sustainability. In addition, the adoption of new health technologies often needs relevant capital investments. Nevertheless, it is not possible to imagine any organizational development and evolution without investing money in new technologies. Managerial decisions regarding investments in new technologies, even when are “evidence based”, can be perceived “discretionary” and have the potential for producing organizational conflicts among professionals. As a result, decisions about medical technologies are usually considered very critical.

As a consequence, hospitals need contextualized assistance on how to make sound investment decisions on innovations, that should be tailored to their specific contexts (e.g. organization of care), focused on the HTs of their strategic interest (financial growth, focusing healthcare services, etc.) and adjusted to their timing (faster answers). Hospital managers are looking for advanced managerial tools, based on economic-rational approaches but also acceptable in medical contexts.

HB-HTA, in general, provides a positive framework that seems to face the need for “rationality” asked by managers about hospital’s technologies related decisions. Thanks to HTA, such critical decisions can be based on criteria that reflect values and visions of all stakeholders inside the system: safety, efficacy, cost-effectiveness are important dimensions for patients, clinicians and managers. HB-HTA also gives the opportunity to healthcare managers to take into consideration the interaction between technology and the organizational texture (e.g. need for new workforce, modification of work processes, infrastructural changes, knowledge creation, etc.). HTA principles, methods and tools seem to fit with the needs of “rational” decision making at this level and it makes HB-HTA an effective tool for management.
**Forms of hospital based HTA**

Examples on the use of HTA at hospital level have been reported since the 1980s when CEDIT had been established within the Assistance Publique Hôpitaux de Paris. We have learnt that HB-HTA can be performed with varying organizational complexity. It can be a unit with permanent full-time HTA professionals or a network of clinicians dedicated part-time, but planned and assigned regularly to assessment duties.

There is no “one-size fits all” model to look at when setting up an HB-HTA unit. The way an HB-HTA unit is framed, organized and run depends on the characteristics of both the context and values of the hospital, and is influenced by the culture of the professionals working in the specific healthcare system.

In the past, only a few surveys have tried to capture these experiences in different geographical contexts. The first worldwide survey was carried out by the HB-HTA Interest Sub Group of HTAi in 2008 (Cicchetti et al, 2008). This survey, identified four types of HTA activities carried out in hospitals based on their focus of action and organizational complexity, classified as follows: ambassador model (clinicians recognized as ‘opinion leaders’ play the role of ambassadors of the HTA “message” inside the hospital), mini-HTA (clinicians carry out the assessment process filling a check-list), internal committee (a group of clinicians who perform reviews of evidence to provide a recommendation on a HT), and HTA unit (formal organizational structure based on specialized HTA personnel in hospital). This survey was useful to depict the variety of organizational solutions and ways of application of HTA around the world. Nevertheless, the survey was not able to fully capture the characteristics of the processes, the quality of the products or the impact of each units’ activity.

The most recent results regarding HB-HTA organizational models come from AdhopHTA (Sampietro-Colom et al. 2015). On the base of the analysis of 7 in-depth case studies it was possible to create a taxonomy of models of HB-HTA functions based on two different relevant variables: the level of interaction with nation-regional level HTA bodies and the level of organizational structuration and maturity of the function:

- Independent group; these units operate within the hospital as an “independent group” that provides support for management decisions in a fairly informal way;

1 List of Case Studies. Hospital Clinic de Barcelona (HCB/FCRB); Ankara Numune Training and Research Hospital (ANH); University Hospital of Lausanne (CHUV); Hospital District of Helsinki and Uusimaa (HUS); Odense University Hospital (OUH); Policlinico universitario “A. Gemelli”, Università Cattolica del Sacro Cuore (UCSC); Auckland City Hospital (ACH), Auckland District Health Board, New Zealand.
• Integrated-essential HB-HTA unit; these are units of small size, with a limited number of staff members, but which are able to involve many other actors and “allies” in their activities;
• Stand-alone HB-HTA units; units with usually highly formalized and specialized procedures, acting internally within hospitals and not strongly influenced by the national or regional HTA organizations (currently the most frequent model in Europe).
• Integrated-specialized HB-HTA units; the functions of the HB-HTA unit are influenced by formal collaboration with the national or regional HTA agency.

In general, the involvement of HB-HTA units in the technology adoption process is considered advisable and the HTA-based recommendations are closely followed by hospital decision-makers.

These four groups should be considered as ideal-types, recognizing that none of them is able to capture the real complexity of units belonging to one of the four options. Nevertheless, the model communicates, at least, the richness of available solutions to run an HTA program/initiative within a hospital. The model can also describe a sort of organizational life cycle for HB-HTA programs/initiatives. During start-up, units are typically informal and less connected with the external environment (Independent groups). People work part-time, on a voluntary basis without strong formal endorsement from management and with informal procedures. The presence or absence of national/regional HTA bodies acting as hub of an HTA network, influences the evolution of the unit toward an integrated or a stand-alone solution. Evolution towards a more mature HB-HTA program/initiative is characterized by increasing levels of formalization and structuration in the processes and the progressive alignment between strategies and goals pursued by the HTA program/initiative and hospital-level strategies. In this evolution, the HB-HTA program/initiative gains internal and external legitimation until it is fully recognized as a key actor for the hospital’s development strategies and is also considered as a partner at the national-regional level.

Table 1-1 reports a summary of 30 case studies presented by the Sampietro-Colom and Martin in 2017 grouped using the model introduced before.
Table 1-1. Thirty hospital based HTA units world wide

<table>
<thead>
<tr>
<th>Acronym</th>
<th>Name of the hospital</th>
<th>Country</th>
<th>Name of the unit</th>
<th>Establishment (Year)</th>
<th>Model</th>
</tr>
</thead>
<tbody>
<tr>
<td>ARHP</td>
<td>Parnes University hospital</td>
<td>France</td>
<td>CEDHT</td>
<td>1982</td>
<td>Stand alone</td>
</tr>
<tr>
<td>Hospital Galran</td>
<td>Pediatric Hospital Galran</td>
<td>Argentina</td>
<td>HTA Committee</td>
<td>2001</td>
<td>Stand alone</td>
</tr>
<tr>
<td>ICSC</td>
<td>Policlinico Gemelli</td>
<td>Italy</td>
<td>HTA and Innovation Unit</td>
<td>2001</td>
<td>Stand alone</td>
</tr>
<tr>
<td>CHUM</td>
<td>Centre Hospitalier de l’Université de Montréal</td>
<td>Canada</td>
<td>HTA Unit</td>
<td>2005</td>
<td>Stand alone</td>
</tr>
<tr>
<td>CHU</td>
<td>CHU de Québec - Université Laval</td>
<td>Canada</td>
<td>HbHTA Unit</td>
<td>2006</td>
<td>Stand alone</td>
</tr>
<tr>
<td>EPD</td>
<td>University of Pennsylvania</td>
<td>USA</td>
<td>Penn Medicine Center for Evidence-Based Practice (CEBP)</td>
<td>2009</td>
<td>Stand alone</td>
</tr>
<tr>
<td>Sick Kids</td>
<td>The Hospital for Sick Children - Peter Gilgan Centre for Research and Learning</td>
<td>Canada</td>
<td>Technology Assessment at Sick Kids (TASK)</td>
<td>2007</td>
<td>Stand alone</td>
</tr>
<tr>
<td>HCB</td>
<td>Hospital Clinic Bamberg</td>
<td>Spain</td>
<td>HbHTA Unit</td>
<td>2009</td>
<td>Stand alone</td>
</tr>
<tr>
<td>NurseCare Hospital (NAN)</td>
<td>Alexane Nuturn Training and Research Hospital</td>
<td>Turkey</td>
<td>AHTA.b</td>
<td>2012</td>
<td>Stand alone</td>
</tr>
<tr>
<td>Hospital El Cruce</td>
<td>Hospital El Cruce</td>
<td>Argentina</td>
<td>Assessment Committee (MOs, equipment, procedures) Pharmacy and Therapeutics Committee (Drugs)</td>
<td>2016</td>
<td>Stand alone</td>
</tr>
<tr>
<td>KP</td>
<td>Kaiser Permanente</td>
<td>USA</td>
<td>Interregional New Technologies Committee (INTC)</td>
<td>1980</td>
<td>Stand alone</td>
</tr>
<tr>
<td>China (IBS Unit Overview)</td>
<td>Overview Very few HTA units</td>
<td>China</td>
<td>General description of IBS-HTA Unit</td>
<td>NA</td>
<td>Stand alone</td>
</tr>
<tr>
<td>Resnitz Univ Hospital</td>
<td>Resnitz University hospital</td>
<td>Finland</td>
<td>HTA Group coordinated</td>
<td>2001</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>CHU</td>
<td>Odense University Hospital</td>
<td>Denmark</td>
<td>HTA Unit</td>
<td>2002</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>CHU</td>
<td>Centre hospitalier universitaire de Sherbrooke</td>
<td>Canada</td>
<td>Health Technology Assessment (HTA) Unit</td>
<td>2004</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>Sahlgrenska</td>
<td>Sahlgrenska University hospital</td>
<td>Sweden</td>
<td>HTA-Centrum</td>
<td>2007</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>Eastern Health Alliance</td>
<td>Changi General Hospital</td>
<td>Singapore</td>
<td>Health Services Research (HSR) department</td>
<td>2014</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>ESNM</td>
<td>Surgery Strategic Clinical Network of Alberta Health Services (ARCh)</td>
<td>Canada</td>
<td>Evidence Decision Support Program (EDSP)</td>
<td>1997</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>ESNM</td>
<td>Surgery Strategic Clinical Network of Alberta Health Services (ARCh)</td>
<td>Canada</td>
<td>Evidence Decision Support Program (EDSP)</td>
<td>1997</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>Liselund (CHU)</td>
<td>University Hospital Cadizan</td>
<td>Switzerland</td>
<td>HTA Unit</td>
<td>2002</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>GMJAR</td>
<td>Charlotte Maxeke Johannesburg Academic Hospital</td>
<td>South Africa</td>
<td>Charlotte Maxeke Research Consortium (CMFRC) Unit</td>
<td>2002</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>PRVNI</td>
<td>Virgen del Rosario/Virgen de la Victoria Hospitals</td>
<td>Spain</td>
<td>Joint Commission for HTA</td>
<td>2002</td>
<td>Integrated or specialized</td>
</tr>
<tr>
<td>Radboud Hop</td>
<td>Radboud University Medical Center</td>
<td>The Netherlands</td>
<td>HTA Unit</td>
<td>1993</td>
<td>Independent group</td>
</tr>
<tr>
<td>McGill University Health Centre (MUHC)</td>
<td>La Montagne General Hospital (LGH), Montreal General Hospital (MGH), Montreal Children’s Hospital (MCH), Montreal Neurological Hospital (MNH) Royal Victoria Hospital (RVH)</td>
<td>Canada</td>
<td>Technology Assessment Unit (TAU)</td>
<td>2001</td>
<td>Independent group</td>
</tr>
<tr>
<td>Mon Health</td>
<td>Monash Health</td>
<td>Australia</td>
<td>New Technology and Clinical Practice Committee</td>
<td>2002</td>
<td>Independent group</td>
</tr>
<tr>
<td>Auckland DHB</td>
<td>Auckland City Hospital</td>
<td>New Zealand</td>
<td>HTA-HTA Committee - Northern Region Clinical Practice Committee (VRCPC)</td>
<td>2005</td>
<td>Independent group</td>
</tr>
<tr>
<td>NATS-ING</td>
<td>Instituto Nacional de Cardiologia</td>
<td>Brazil</td>
<td>HTA unit</td>
<td>2009</td>
<td>Independent group</td>
</tr>
<tr>
<td>Conceição Hospital Group</td>
<td>Conceição Hospital Group</td>
<td>Brazil</td>
<td>HTA unit</td>
<td>2012</td>
<td>Independent group</td>
</tr>
<tr>
<td>HSJD</td>
<td>Hospital Sant Joan de Déu</td>
<td>Spain</td>
<td>HTA Committee</td>
<td></td>
<td>Independent group</td>
</tr>
<tr>
<td>Geneva Univ Hospital (MUG)</td>
<td>Geneva University Hospital</td>
<td>Switzerland</td>
<td>Commission for new technologies plus Specific Committees for drugs and devices</td>
<td>not available</td>
<td>Independent group</td>
</tr>
<tr>
<td>Faurecia (KRVV)</td>
<td>North Vaudois hospital</td>
<td>Switzerland</td>
<td>Drug committee for drugs, Biomedical Engineering for equipment and devices</td>
<td>NA</td>
<td>Independent group</td>
</tr>
</tbody>
</table>

Source: (Cicchetti et al., 2017)
**Hospital Based HTA: mission, structure, products and processes**

Table 1-2 is structured to summarize major characteristics of HB-HTA units/functions based on observed trends in many case studies around the world. The use of HTA in and for hospitals is intended to (1) support managerial decision making or (2) just to assess the potential impact of the introduction of a medical technology in the clinical practice. In the first case the HTA process produces direct impact on managerial decisions and is often integrated within procurement procedures of medical technologies. Alternatively, the assessment can only produce a recommendation for clinical practice and, in general, is not mandatory.

In both cases HTA is playing a strategic role for hospital management and for this reason is typically performed by units or individuals reporting hierarchically or in staff to a top management team (CEO, CMO). Alternatively, this activity is depending hierarchically or functionally by quality directorates or research and innovation departments.

HB-HTA is typically a multidisciplinary process that involves clinicians, pharmacists, clinical engineers and health economists. The variety of competencies often includes nurses, sociologists and bioethicists. The number and the variety of professionals involved in HB-HTA depends on different factors such as the dimension of the hospital, its institutional orientation (teaching or non-teaching), and the phase of development of the HTA unit (start-up phase, mature).

The evidence generated by 34 case studies within the AdHopHTA project as well 30 case studies collected by Sampietro-Colom and Martin (2017), provide a full picture of the variety of procedures and products produced by hospital HTA bodies.

**Table 1-2. Characteristics of hospital based HTA units/functions**

<table>
<thead>
<tr>
<th>Characteristics of HB-HTA</th>
<th>Micro-trends in organization and performance of HB-HTA units</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mission (how it is defined by the HB-HTA unit)</td>
<td>Managerial support for decision-making (in this case, the hospital management body is committed to taking the results of the assessment into account in its decision-making process) Assessing health technologies (in this case, there is no formal commitment to integrate the assessment results in the final decision-making process)</td>
</tr>
<tr>
<td>Characteristics of HB-HTA</td>
<td>Micro-trends in organization and performance of HB-HTA units</td>
</tr>
<tr>
<td>--------------------------</td>
<td>-------------------------------------------------------------</td>
</tr>
</tbody>
</table>
| Position in the organizational structure of the hospital | CMO (Chief Medical Officer) (most)  
CEO  
Quality and Research Directorate  
Research and Innovation Directorate |
| Funding source (public) | External (e.g., competitive grants, contract with other organizations) – *most cases*  
Internal (from hospital budget) (in most cases there is little funding support from the hospital budget) |
| Role of HB-HTA in the decision-making | Advisory – *most cases*  
Mandatory |
| Role after the assessment | None – *most cases*  
Procurement (acquisition) phase – *few cases*  
Implementation of recommendation – *few cases* |
| Background of professionals in the unit | Clinicians, health economists, public health – *most cases*  
The same as a) plus nurses, bioengineers, and other allied health professionals |
| Careers opportunities | Formal (specific plans for development) – *none*  
Informal (e.g., ad-hoc conferences, courses, etc.) – *most cases* |
| Staff dedication in the HB-HTA unit | Part time – *most cases*  
Full time |
| Dissemination of the activities performed by HB-HTA unit | Internal (clinical rounds, word of mouth, information sends to clinical departments, broadcast email, presentation at the hospital board meeting)  
External (media, national journals, newsletters, websites, courses, events and conferences) |
| Prioritization of health technologies for assessment | Based on specific criteria – *few cases*  
First-in-first assessed – *most cases* |
| Types of health technologies assessed (in order of frequency) | Medical devices  
Medical equipment  
Diagnostic tests  
Procedures (clinical and organizational) and drugs |
<table>
<thead>
<tr>
<th>Characteristics of HB-HTA</th>
<th>Micro-trends in organization and performance of HB-HTA units</th>
</tr>
</thead>
<tbody>
<tr>
<td>Performance of the assessment</td>
<td>By professionals in the HB-HTA unit involving closely clinicians and hospital managers</td>
</tr>
<tr>
<td></td>
<td>Shared between clinicians (e.g., literature review) and the HB-HTA unit (e.g., economic analysis + supervision of work by clinicians)</td>
</tr>
<tr>
<td></td>
<td>By clinicians supported and supervised by the HTA unit</td>
</tr>
<tr>
<td>Scope</td>
<td>PICO (patient, intervention, comparator, outcome) – all cases</td>
</tr>
<tr>
<td></td>
<td>Type Comparator: gold standard and technology available at hospital</td>
</tr>
<tr>
<td>Recommendations included</td>
<td>Yes – most cases</td>
</tr>
<tr>
<td></td>
<td>No, just results (e.g., clinical or economic) of the assessment are presented.</td>
</tr>
<tr>
<td>Role of HB-HTA in the decision-making</td>
<td>Advisory – always</td>
</tr>
<tr>
<td></td>
<td>Mandatory – never</td>
</tr>
<tr>
<td>Impact of the recommendations on the final decision</td>
<td>High – most cases</td>
</tr>
<tr>
<td></td>
<td>Low</td>
</tr>
<tr>
<td>Assurance of transparency during the assessment</td>
<td>Internal reviews – often</td>
</tr>
<tr>
<td></td>
<td>Step-by-step, explicit (e.g., published or shown to clinician)</td>
</tr>
<tr>
<td></td>
<td>External review – less frequent</td>
</tr>
<tr>
<td>System/approaches to assure independence of assessment</td>
<td>Informal – most</td>
</tr>
<tr>
<td></td>
<td>Systematic</td>
</tr>
<tr>
<td>Dissemination of the HB-HTA product/assessment</td>
<td>Internal (e.g., Intranet-database: complete assessment, abstracts or summaries of the assessment) – most cases</td>
</tr>
<tr>
<td></td>
<td>External (e.g., database open to other hospitals) – few cases</td>
</tr>
<tr>
<td>Measurement of impact of HB-HTA unit</td>
<td>None – most cases</td>
</tr>
<tr>
<td></td>
<td>Non-systematic – few cases</td>
</tr>
<tr>
<td></td>
<td>Systematic – never</td>
</tr>
</tbody>
</table>

Different organizational procedures should be designed in order to produce specific outputs. Two main outputs of the work carried out by HB-HTA functions can be outlined:

- **Mini-HTA Reports**, designed to support managerial and clinical decision-making regarding the adoption of a specific technology in a hospital;
- **Broad technical analysis** to support multi-year investment plans mainly related to large biomedical equipment.

The evidence shows how it is crucial to ensure that HTA reports are “fit-for-purpose” and meet the needs and expectations of end-users. In the case of HB-HTA, these users are hospital decision-makers. Hospital decision-makers require information on the clinical effectiveness, budget impact, safety, organizational and strategic aspects of the technologies they consider for adoption (Kidholm et al., 2016). This information can be provided at different levels of comprehensiveness. Even more important is the correct timing in relation to the subsequent decision. Moreover, ensuring the quality of information is crucial; AdhopHTA has produced a quality checklist for HB-HTA reports and this was applied to assess HTA documents produced by hospitals participating to the consortium. Among the main observations of the analysis are the following:

- There is no one type of HB-HTA report. The reports range from almost full HTA reports to simpler checklists of questions without a deep level of detail;
- The overall quality of the reports evaluated is moderate, leaving room for improvement;
- The higher the quality score of an HB-HTA report, the greater the volume and amount of staff-effort required to produce it.

What is relevant to point out is that the domains of the assessment for HB-HTA products are significantly overlapping with those of traditional HTA with some differences but their relative relevance is different. In fact, doing a mini-HTA “in” or “for” a hospital, is not just re-doing an HTA in a specific context.

Figure 1-1 is providing explanation of this issue. If we consider the 9 domains of the typical EUNetHTA Core model it is clear that some of them are more relevant when the assessment is performed in a hospital. Moreover in the specific hospital context organizational and strategic issues became relevant. In fact, the assessment should take into consideration which is the specific mission of the hospital in the wider healthcare system and its strategic orientation in this scenario. A research hospital, for instance, that is willing to provide excellent care in its context will be keener to adopt innovative medical technologies than a community hospital.
All these considerations were relevant in order to identify the AdhopHTA mini-HTA Template that provides a model to collect the information needed to carry out the assessment of a new technology in a hospital setting. The template is structured as a list of questions (32) grouped in 5 sections to be answered for a comprehensive collection of information to support managerial decision-making regarding the adoption of a specific technology.¹

**Figure 1-1. HTA and HB-HTA domains**

<table>
<thead>
<tr>
<th>Domain</th>
<th>HTA Core model</th>
<th>HB-HTA Core model</th>
</tr>
</thead>
<tbody>
<tr>
<td>D1: Health problem and current use</td>
<td>✔ relevant</td>
<td>✔✔✔ most important</td>
</tr>
<tr>
<td>D2: Description and technical characteristics</td>
<td>✔ relevant</td>
<td>✔ relevant</td>
</tr>
<tr>
<td>D3: Clinical effectiveness</td>
<td>✔ relevant</td>
<td>✔✔✔ most important</td>
</tr>
<tr>
<td>D4: Safety aspects</td>
<td>✔ relevant</td>
<td>✔✔✔ most important</td>
</tr>
<tr>
<td>D5: Costs and economic evaluation</td>
<td>✔ relevant</td>
<td>✔ relevant</td>
</tr>
<tr>
<td>D5.1 Societal point of view</td>
<td>✔ relevant</td>
<td>✔✔✔ most important</td>
</tr>
<tr>
<td>D5.2 Hospital point of view</td>
<td>✔ relevant</td>
<td>✔ relevant</td>
</tr>
<tr>
<td>D6: Ethical aspects</td>
<td>✔ relevant</td>
<td>✔ relevant</td>
</tr>
<tr>
<td>D7: Organizational aspects</td>
<td>✔ relevant</td>
<td>✔✔✔ most important</td>
</tr>
<tr>
<td>D8: Social aspects</td>
<td>✔ relevant</td>
<td>✔ relevant</td>
</tr>
<tr>
<td>D9: Legal aspects</td>
<td>✔ relevant</td>
<td>✔ relevant</td>
</tr>
<tr>
<td>D10: Political and strategic aspects</td>
<td>✔ relevant</td>
<td>✔✔✔ most important</td>
</tr>
<tr>
<td>D10.1 Political aspects</td>
<td>✔ relevant</td>
<td>✔ relevant</td>
</tr>
<tr>
<td>D10.2 Strategic aspects</td>
<td>✔ relevant</td>
<td>✔ relevant</td>
</tr>
</tbody>
</table>

Source: Sampietro-Colom et al. 2015 (AdHopHTA Handbook).

¹ Structure of the AdHopHTA Mini-HTA Template. Question 1: Summary; Question 2-7: Basic information; Question 8-13: General methodological aspects & reporting; Question 14-27: Results within domains; Question 28-32: Discussion, conclusion and recommendations (Source: www.adhophta.eu).
Another relevant output of the AdhopHTA project was the Guiding principles for good practices in HB-HTA that is completed by a toolkit. The Guiding Principles have been defined to guarantee good practices for those hospitals that want to start to carry out or use HTA as well as for those that want to improve their current work on HB-HTA (Sampietro-Colom et al., 2016). The 15 guiding principles for good practices in HB-HTA are grouped into 4 dimensions: the assessment process; the frame of the unit (in particular its leadership, strategy and partnerships); the resources needed by the unit; and the impact of the unit’s work.

**Assessing Hospital Based HTA**

There exists a wide literature regarding the application of HTA logic at the hospital level. The HTAi’s Worldwide survey (Cicchetti et al, 2008), case studies in AdhopHTA (Sampietro-Colom et al., 2015) and those collected in the book edited by Laura Sampietro – Colom and Janet Martin in 2017 (Sampietro-Colom and Martin, 2017) has created a significant evidence useful to identify key success factors and potential barriers for the development of HB-HTA functions (Cicchetti et al., 2017).

**Key success factors**

- Competence and training: The presence of well-trained and motivated people in HTA and easy access to scientific journals and other informational resources, are key factors for a successful experience in HB-HTA.
- Transparency and rigour: Transparency and rigour of the assessment process is considered as one of the major success factors for a HTA hospital based unit.
- Legislative framework: Legitimation of HB HTA unit by law, where it is present (e.g. Quebec), is considered a facilitator.
- Multi-disciplinary Team: Diversity in the cultural and professional background of units’ personnel is considered a key success factor for many hospitals around the world.
- Top Management commitment: A clear and formal endorsement from top management team is key to ensure genuine collaboration from clinical departments and other hospitals’ units. These conditions are considered fundamental to increase the acceptability of recommendations produced by HB-HTA programs/initiatives.
- Clinicians’ (users) involvement: Many of the units highlighted the importance of active end-user involvement (clinicians, nurses) in the assessment process. Continuous education programs that incorporate an evidence-based medicine approach for clinical decision-making, is key to promote the diffusion of a positive cultural attitude towards HTA.
Research-Management collaboration: University collaboration, especially in the case of academic medical centres, raises the opportunity to have access to specific and broad competencies that may be lacking within the available HB-HTA unit staff and resources. This can ensure greater robustness, and increase acceptability of recommendations.

Clear role of the HB-HTA unit and explicit methodology: Clarity of the role played by the HTA initiative/program within the hospital organizational processes (e.g. in procurement process) and the existence of a formalised methodology for the assessment is important in order to reduce internal conflicts and improve impact of recommendations.

Timeliness: Having timely HB-HTA reports is highly appreciated by many hospital clinical staff, managers and clinicians.

Patient involvement: Only in two cases, direct and systematic involvement of patients and consumer representatives in the HTA process was reported, and in both cases it was considered as a key factor for success.

Stakeholder and industry interaction: In a few cases, engagement with industry and other business stakeholders was reported as a way to gain extra financing for clinical activities and to find competencies that are not usually available in the hospital.

What has not worked

Cultural barriers: One of the most common problems reported by HB-HTA units is the presence of cultural barriers. For some hospitals, especially in countries where the HTA is not well established at a national level, lack of a widespread HTA culture across the country, is considered an important barrier. The lack of physician and managerial awareness and training in HTA are suggested as contributors to this cultural barrier.

Political interests: In those countries where political power has most influence on hospital decision making, the use and the impact of HTA is more difficult.

Informational barriers: Lack of available global and contextual information for performing specific assessment of hospital technologies remains an important barrier. This is specially the case for relevant cost data and real world data, which are essential for performing a useful contextualized HB-HTA.

Systematic stakeholder inputs: Input from patients and other stakeholders beyond the usual hospital-based multidisciplinary decision-makers is rare and many hospitals and HB-HTA units indicated a willingness to invest in developing broader stakeholder engagement in the future.
Limited focus on HTs assessed: Hospitals seem to be mainly devoted to assessing new technologies in their adoption phase; whereas reassessment of pre-existing technologies already in use in the hospital setting are less commonly assessed. Some of the latter may represent better opportunities for assessment due to their lack of effectiveness and inefficiencies relative to better alternatives; therefore, pre-existing technologies should be routinely considered for disinvestment. In particular, efforts provided by clinicians in contributing to the detection of technologies and clinical procedures to be withdrawn, remains insufficient in many HB-HTA programs/initiatives to date. A more structured and proactive disinvestment process may produce financial savings for hospitals and other areas of the healthcare system.

Lack of resources: Lack of resources to maintain the required HB-HTA activities is reported as a problem in half of the cases. This lack seems to affect productivity more than the quality of the assessments, specifically reducing the potential impact of the HTA program/initiative due to lack of resourced capacity to produce information within the most window of opportunity for decision-makers.

Inter-organizational coordination: The need for more effective coordination among multiple HTA programs and initiatives in the same country or region is reported in some cases. This coordination is expected to contribute to economies of scale and better use of limited resources for HTA.

Monitoring: Since the primary focus of most HTA programs/initiatives is on the adoption phase of newer technologies, a lack of attention and processes is reported for monitoring the real impact of the technologies after their introduction. There also remains a general lack of efforts to understand the impact of the assessments on overall indicators of hospital performance (productivity, effectiveness, outcomes, efficiency).

Internal use variability: The use of HTA in the same hospital may differ from department to department, and the resources available to assess all technologies varies considerably; therefore, hospital decision-making is not uniformly based on HB-HTA recommendations. This may cause concern for inequities and differences in thresholds of rigor for decision-making across departments.

This long list of positive and negative issue provides a sort of guideline that can help in the phase of establishing new units.
**Hospitals in the HTA Ecosystem**

The application of HTA principles and tools at hospital level is evolving and is more and more diffused worldwide. The value beyond the application of HTA to support decision making regarding health technology in hospitals and other healthcare organizations has been recognized thanks to a wider and wider range of evidence that has been produced during the last 20 years. It is now clear that HB-HTA is a different application of traditional HTA logics that can contribute to manage the introduction of new technologies within healthcare systems.

**Table 1-3. Most frequently seen differences between standard HTA carried out at national or regional level and at hospitals.**

<table>
<thead>
<tr>
<th>Characteristics</th>
<th>National or regional agency</th>
<th>Hospital</th>
</tr>
</thead>
<tbody>
<tr>
<td>The Assessment process</td>
<td>Drugs, Medical equipment, Medical devices, Diagnostic tests</td>
<td>Drugs*, Medical equipment, Medical devices, Diagnostic tests, Organizational technologies</td>
</tr>
<tr>
<td>Type of technologies assessed</td>
<td>The comparator is the &quot;gold standard&quot; or the technology most used in the country</td>
<td>The comparator is normally the technology that is being used in the hospital (current standard practice)</td>
</tr>
<tr>
<td>Scope of HTA</td>
<td>Description of HT and technical characteristics, Health problem and current use of the HT,</td>
<td>Health problem and current use of the HT, Clinical effectiveness, Safety aspects, Organizational aspects, Political and strategic aspects, Cost and economic evaluation (hospital point of view)</td>
</tr>
<tr>
<td>Most frequently required information (criteria)</td>
<td>Health problem and current use of the HT, Clinical effectiveness, Safety aspects, Ethical, organizational, social and legal aspects, Cost and economic evaluation (societal and hospital point of view)</td>
<td></td>
</tr>
<tr>
<td>Perspective of the health economic assessment section</td>
<td>Cost-effectiveness with a societal perspective and using average costs</td>
<td>Differential cost analysis process, budget impact analysis, cost-effectiveness using hospital perspective or payer of hospital services (i.e., actual costs for hospital)</td>
</tr>
<tr>
<td>Characteristics</td>
<td>National or regional agency</td>
<td>Hospital</td>
</tr>
<tr>
<td>---------------------------------------------</td>
<td>---------------------------------------------------------------------------------------------</td>
<td>---------------------------------------------------------------------------</td>
</tr>
<tr>
<td>Primary target audience of the assessment</td>
<td>Policy-makers, healthcare payers</td>
<td>Hospital and clinical managers</td>
</tr>
<tr>
<td>Type of decision which HTA assessment is going to support</td>
<td>Payment, coverage, reimbursement</td>
<td>Acquisition/investment, strategic alliances, collaborative public-private research, disinvestment</td>
</tr>
<tr>
<td>Relevant stakeholders involved</td>
<td>Healthcare payers, representatives of clinicians, patients</td>
<td>Clinician asking for the HT, manager, nurses, bioengineers, planners</td>
</tr>
<tr>
<td>Follow-up process</td>
<td>Hardly ever</td>
<td>Seldom</td>
</tr>
<tr>
<td>HTA report</td>
<td>Full HTA review, more frequently rapid reviews</td>
<td>Hospital HTA (e.g., using mini-HTA, rapid review, full HTA review)</td>
</tr>
<tr>
<td>Timescale of assessment</td>
<td>12-24 months</td>
<td>1-6 months (average = 3)</td>
</tr>
<tr>
<td>Performance of the assessment</td>
<td>Most frequently: Scientists at national or regional HTA agency University scientists commissioned for the purpose</td>
<td>Most frequently: Scientists at HB-HTA unit Clinicians trained in HTA assisted by scientists at HB-HTA unit Scientists at national or regional HTA agency working for the hospital Clinicians trained in HTA assisted by university scientists</td>
</tr>
<tr>
<td>Initiators of the assessment</td>
<td>Policy makers, healthcare payers</td>
<td>Clinicians</td>
</tr>
<tr>
<td>Leadership &amp; Strategy &amp; Partnerships</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Leaders</td>
<td>Civil servants or contracted by the national or regional agency with different levels of experience and training</td>
<td>Fully or partly dedicated professionals contracted by the hospital, mostly trained in HTA and with long experience</td>
</tr>
<tr>
<td>Characteristics</td>
<td>National or regional agency</td>
<td>Hospital</td>
</tr>
<tr>
<td>-----------------------------------------------------</td>
<td>-------------------------------------------------------------------------------------------</td>
<td>--------------------------------------------------------------------------</td>
</tr>
<tr>
<td>Leadership &amp; Strategy &amp; Partnerships</td>
<td>Providing high-quality evidence to inform decision-making by national health services</td>
<td>Managerial support to decision-making, assessing health technologies for clinical practice</td>
</tr>
<tr>
<td>Mission, vision and values</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Priority setting of HTs to evaluate</td>
<td>Established by policy makers or healthcare payers at national (ministry of health) or regional level</td>
<td>Established by clinical leaders and hospital managers</td>
</tr>
<tr>
<td>Partnerships and networks</td>
<td>Formal partners of established networks from national or regional HTA agencies and international organizations</td>
<td>Informal contacts between hospitals at local, regional, national and/or international level</td>
</tr>
<tr>
<td>Resources</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Financing</td>
<td>Mainly by government (national or regional)</td>
<td>Mainly by external sources (e.g., competitive grants, contracts with other organizations) Rarely by internal sources (from the hospital’s budget)</td>
</tr>
<tr>
<td>Profiles and skills (more frequent)</td>
<td>Medical doctors Epidemiologists Economists, statisticians Social workers, ethicists</td>
<td>Medical doctors Epidemiologists, public health specialists Economists</td>
</tr>
<tr>
<td>Characteristics</td>
<td>National or regional agency</td>
<td>Hospital</td>
</tr>
<tr>
<td>---------------------------------</td>
<td>---------------------------------------------------------------------------------------------</td>
<td>---------------------------------------------------------------------------</td>
</tr>
<tr>
<td>Capacity of local adaptation</td>
<td>Limited (high degree of adaptation to local needs required)</td>
<td>Frequently total</td>
</tr>
<tr>
<td>Impact measurement (benefits / outcomes to end-users)</td>
<td>Usually end-point outcomes (health &amp; social impact); requires significant funds</td>
<td>Usually intermediate outcomes (e.g., satisfaction with the HB-HTA unit and its assessments, net present savings or avoided loss from adopting/not adopting HTs). Impact measurement for specific, recommended HTs</td>
</tr>
<tr>
<td></td>
<td>Occasionally difficult to prove direct cause-effect relationship</td>
<td>Costly and difficult to prove direct cause-effect relationship</td>
</tr>
<tr>
<td>Customers’ results</td>
<td>Level of use and adoption of the recommendations</td>
<td>Level of use and adoption by hospital managers and clinicians (usefulness in decision-making, satisfaction with HB-HTA function)</td>
</tr>
<tr>
<td>Impact on society</td>
<td>Difficult to assess</td>
<td>Difficult to assess</td>
</tr>
</tbody>
</table>


*In EU countries there are pharmacy committees in hospitals responsible for analysing drugs to be introduced into the hospital; hospitals usually focus on other technologies, although this may vary from country to country.

$ If needed by the type of HT being assessed.
HB-HTA can provide an enormous contribution to deliver valuable medicine in healthcare systems worldwide, increasing the appropriateness in resource use and making (public and private) systems sustainable. At the moment HB-HTA seems able to produce “local value”: this has been confirmed within the AdHopHTA project as well as in a recent comparative analysis of 30 HTA hospital functions around the world (Cicchetti et al, 2017).

In order to translate the “local value” in a greater global impact, HB-HTA functions should be interacting and integrating along the HTA “supply chain”, contributing to international, national, regional and provincial HTA efforts, offering unique knowledge about the impact of healthcare technologies on “local contexts”. The role of HB-HTA functions, in this manner, could evolve assuming two different “missions”: on one hand hospital HTA functions could continue to operate as they mainly did up to now, integrating globally produced reviews of clinical research and HTAs with local evidence, experiences and resources and data to provide intelligent support to managerial and clinical decisions. On the other hand, they should be asked to share locally produced evidence with international, national and regional HTA bodies (and networks such as EuNetHTA) and regulatory bodies (such as EMA or CADTH) supporting adaptive / progressive drug licensing models that are under experiment worldwide or complementing the task that National/Regional HTA agencies are performing around medical devices.

In this fashion HB-HTA functions could assume a clear positioning and role within the global “HTA eco-system”, needed to fully manage the life cycle of health technologies. This should be done by extracting the maximum value from the tremendous economic effort that countries worldwide are providing to face the future challenges of healthcare systems.
**Bibliographic sources and suggested readings**


Patient and Social Engagement in HTA

Authors: Janney Wale, Maria Sharmila Alina de Sousa, Li-Ying Huang
**Background**
Patients and their carers are present throughout the continuum of an individual’s health care. They see the full process of care, and through a different lens than healthcare providers. This means that the absence of engagement with patients and carers, as well as with those who provide healthcare access, can deprive health care of a rich source of information on the experience of living with a disease or health condition, its management, and treatment. Because patients, carers and families, and the public are generally not as well organized and resourced as other stakeholder groups, their interests and needs have not always been captured or integrated into policy development, healthcare services and health care, and research activities.

In this chapter we define social engagement as the participation of consumers, patients and their families, carers, legal representatives, patient advocates, citizens, and the broader public in healthcare policy and HTA.

Table 1 summarizes the reasoning behind why social engagement is important in health policy decision-making related to the technologies available within our healthcare systems (Wale et al 2017).

**Table 1. Why incorporating health system user healthcare experiences into HTAs is important**

<table>
<thead>
<tr>
<th>1. Patient’s Rights Perspective</th>
</tr>
</thead>
<tbody>
<tr>
<td>• WHO’s Alma-Ata Declaration (1978) – “...people have the right and duty to participate individually and collectively in the planning and delivery of their health care”</td>
</tr>
<tr>
<td>• WHO resolution on health intervention and HTA in support of universal health coverage</td>
</tr>
<tr>
<td>2. Need for Value Determination</td>
</tr>
<tr>
<td>• Value determinations and value judgements needed beyond cost effectiveness, such as patient and social value</td>
</tr>
<tr>
<td>3. Evidentiary Contributions</td>
</tr>
<tr>
<td>• Citizens, consumers, patients and their families, carers, legal representatives as well as advocates provide the ‘lived experience’ that reflects benefits (and harms) that may be broader than the outcomes reflected in trials or traditional quality of life data</td>
</tr>
<tr>
<td>4. Methodological Perspective</td>
</tr>
<tr>
<td>• New approaches for obtaining timely evidence, such as managed entry schemes</td>
</tr>
<tr>
<td>• Social input on design of and relevant endpoints for clinical studies including patient relevant outcomes</td>
</tr>
</tbody>
</table>

An example of why social engagement should be a part of HTAs is provided by the Ontario Health Technology Advisory Committee from Ontario, Canada (OHTAC 2015). The health technology advisory committee strengthened social engagement in its evidence review process to foster transparency, awareness, legitimacy, acceptability and trust in the final recommendations from their HTA process.

**Different levels of social engagement**

At the lowest level of social engagement, the public may simply be informed about HTA recommendations, if they know where to access this information. The next level is consultation, to be invited to comment on recommendations of an HTA committee. Greater levels of engagement are by participating in the processes for decision making, providing input, and having a greater say in the decisions through collaboration. Empowerment would be where all social actors who have an interest in specific health technologies make the final decisions together (IAP2). See Table 2.

**Table 2. Levels of engagement, as adapted from the IAP2 public participation spectrum**

<table>
<thead>
<tr>
<th>SOCIAL PARTICIPATION GOAL</th>
<th>INFORM</th>
<th>CONSULT</th>
<th>PARTICIPATE</th>
<th>COLLABORATE</th>
<th>EMPOWER</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient and Social Engagement in HTA</td>
<td>To provide society with balanced and objective information to assist them in understanding the problem, alternatives, opportunities and/or solutions.</td>
<td>To obtain social feedback on analysis, alternatives and/or decisions.</td>
<td>To work directly with society throughout the process to ensure that social concerns and aspirations are consistently understood and considered.</td>
<td>To partner with society in each aspect of the decision including the development of alternatives and the identification of the preferred solution.</td>
<td>To place final decision making in the hands of society.</td>
</tr>
<tr>
<td>PROMISE TO SOCIETY</td>
<td>We will keep you informed.</td>
<td>We will keep you informed, listen to and acknowledge concerns and aspirations, and provide feedback on how social input influenced the decision. We will seek your feedback on drafts and proposals.</td>
<td>We will work with you to ensure that your concerns and aspirations are directly reflected in the alternatives developed and provide feedback on how social input influenced the decision.</td>
<td>We will work together with you to formulate solutions and incorporate your advice and recommendations into the decisions to the maximum extent possible.</td>
<td>We will implement what you decide.</td>
</tr>
</tbody>
</table>
HTA to inform universal health coverage

As countries strive to deliver universal health coverage (UHC), the process of deciding which health technologies and healthcare interventions to invest in has become increasingly important, and HTA is proposed by the World Health Organization (WHO) (WHO 2014-media release). WHO has developed a handbook to support social participation for UHC (WHO Handbook).

Countries face complex choices in deciding how to direct their finite health budgets to meeting the priority health needs of their populations, and in selecting from the vast array of technologies and interventions that are available. HTA is a multidisciplinary process to evaluate the social, economic, organizational and ethical aspects of any health intervention or technology and is directed at reaching a fair and efficient outcome. The HTA approach uses a systematic process to evaluate the properties, effects, and impacts of health technologies or interventions. It can be applied to medical devices, medicines, vaccines, procedures, health services, and public health interventions. The International Network of Agencies for Health Technology Assessment (INAHTA) recognizes patient involvement in the process of HTA as important and valuable (INAHTA Statement).

In 2015, the WHO conducted a survey of member countries on their HTA capabilities (whether formally recognised as HTA or not) and the aspects of HTA that the countries assessed. Ministries of health or national health insurance bodies were the main initiators of HTAs. This survey has been used to inform the work of the WHO in promoting universal health coverage (WHO 2015 Survey). From the survey of 111 countries, WHO developed country profiles (WHO country profiles) where 'social participation' in HTA activities was one of the fields collected. A lack of qualified human resources appeared to be the main barrier to producing and using HTAs. HTAs looked principally at safety and clinical effectiveness (benefit/risk) for all types of health technologies, followed by economic and budgetary considerations. Acceptability to healthcare providers and society, ethics, equity or feasibility were less often considered. About half of the countries did not provide the opportunity for society to comment on the recommendations of HTA reports. Yet not accommodating social acceptance of technologies can lead to greater non-adherence to technologies. Among the countries that completed the WHO survey, those that included social participation were Australia, Canada, England and Scotland, Germany, Brazil, Colombia, India, Kazakhstan, Poland, Portugal, Romania, Thailand, Turkey. Others such as Ghana, Kenya, Vietnam did not have a national authority for HTA in 2015.
Social engagement can be seen as an added cost, and processes and timelines may have to change to accommodate such engagement. Similarly, objective ‘hard’ outcomes such as deaths, number of cardiovascular events are much easier for HTA reviewers to work with than are the more subjective, less well-defined patient relevant outcomes such as quality of life (QoL) measures. Patient-reported outcomes are not easy to measure or compare across diseases, and are much more context specific. That is, the place or setting of health care and circumstances may influence the findings, meaning that data from one country or region may not be transferable to another target country or region. How direct patient input into HTAs is incorporated into HTA decision making is more difficult again (Wale and Sullivan 2019). Potential barriers for patient involvement have been identified through review of the literature. From the HTA body or payer perspective these include limited willingness to involve patients, need for confidentiality and possible conflicts of interest of patient organisations, finding the ‘right’ patient representative, lack of resources to support patient involvement, and needing guidance on how to involve patients. From the patient perspective barriers include the lack of understanding of the processes, the data and evidence required to inform input, resources to be effectively involved, and guidance on how to represent a patient community (Dimitrova 2022).

**Seeking patient input, an example**

The European Network for Health Technology Assessment (EUnetHTA) addressed patient input and its quality in the work of the network by requesting “…facts, information and summaries of experiences that give a concise, accurate and balanced overview of a range of patients’ and care-givers/carers’ perspectives/views” (EUnetHTA website; Elvsaaas et al 2021). Different stages of disease and disease severity were specified, with a particular focus on symptoms. The stated source of information (such as web survey, helpline analysis, social networking, focus group, patients’ records, interviews, one-to-one conversations with those who have experience of an intervention, patient stories, research studies, etc.) is important; as are including groups that should have special considerations, including with disease sub-types and people with other disabilities and special needs.

**Patient evidence and the patient voice**

Patient evidence has been defined (Staniszewska and Werkö 2021) as coming from formal research methods, rather than social engagement.
Methods for gathering patient evidence include patient-relevant outcome measures (Haywood et al 2017, Ch9), Discrete Choice experiments (Tockhorn-Heidenreich et al 2017, Ch10), ethnographic fieldwork (Tjørnhøj-Thomsen and Ploug Hansen 2017, Ch12), and qualitative research (Booth 2017, Ch15). For example, a literature review can be followed by qualitative studies to inform quantitative patient preference research, where specific methods are very dependent on the diseases being investigated and patients’ awareness of the disease processes (Cook et al 2019). Patient evidence can be seen as important in HTA to inform needs and value for patients and their carers. Yet efforts are being made to not just seek input from patients into HTA processes, for example using templated questionnaires (HTAi PCIG resources), but to structure the patient voice so that it can be systematically incorporated as part of the evidence. Such structure is provided with patient preference studies and patient-reported outcome measures (Wale et al 2021).

**About HTA**

HTA is a field of scientific research used to inform policy and clinical decision-making on the introduction and use of health technologies. Health technologies include pharmaceuticals, devices, diagnostics, procedures and services, and other clinical, public health and organizational interventions including telehealth. HTA is often defined as a multidisciplinary field that addresses the clinical, economic, organizational, social, legal and ethical impacts of a health technology, considering its specific healthcare context as well as available alternatives. The scope and methods of HTA may be adapted to the needs of a particular health system, but HTA processes and methods should be transparent, systematic and rigorous (EUPATI).

HTA follows market authorisation and is often used to inform reimbursement and the choice of funding model for introduction of new technologies into clinical practice. Different HTA decisions can be made in different countries (Mamzer et al 2018; Nicod 2017). This is because HTA is context-dependent, not least of all in terms of budgetary impact and economic analyses (HTAsiaLink 2013). Service delivery opportunities need to be in place within a country, with appropriate staff, equipment, time and resources to provide the technology. The technology can then be accepted as part of health care. Countries may also seek clinical trials within their own countries, as for example occurs in Japan.
Social engagement in HTA

Countries such as England (NICE; Thomas et al 2017), Scotland (SMC), Germany (G-BA, Haefner and Danner 2017), Canada (CADTH, Weeks et al 2017) and Australia (PBAC, MSAC, Wortley and Wale 2017) have robust, clearly articulated patient and public participation strategies (Facey et al 2017). Support is often provided for social engagement through a public and patient involvement specialist team whose job it is to coordinate the engagement. These support teams can build in improvement mechanisms, for example by incorporating an advisory public, patient and carer group network (SMC, CADTH, EUnetHTA) and by offering feedback mechanisms, support and training. Meetings involving healthcare providers and patient organisation representatives together (as with PACE in Scotland, EUnetHTA) is an important development (Wale and Sullivan 2019).

The HTAi Patient and Citizen Involvement in HTA Interest Group (HTAi PCIG) used formal research methods with international participants to develop a generic template for obtaining relevant patient input on medicines to inform HTAs. Templates for medical devices and diagnostics were also developed (HTAi PCIG resources). The templates seek the unique knowledge and perspectives of patients most likely to inform HTAs and inform value assessments. The methodology involved a workshop using a nominal group technique and three stage Delphi process, with direction from an international steering committee. Guidelines were also developed for patient groups, adapted from a Canadian pan-Canadian Oncology Drug Review document (HTAi PCIG resources). The generic templates have been used by HTA agencies to inform their templates, for example EUnetHTA (EUnetHTA website), and have been translated into other languages (e.g., Italian, Lo Scalzo 2017).

For EUnetHTA, as with the Canadian Agency for Drugs and Technologies in Health (CADTH), patient input is received before developing the plan or protocol for the assessment. EUnetHTA planned to make the patient submissions it receives publicly available on a webpage at the timing of publication of the project plan. The Joint Assessment report will document how the information from patients was considered in developing the scope of the EUnetHTA Joint Assessment. This clarification of how the information is used is an important element of providing input (Wale and Sullivan 2019). The German G-BA (Haefner and Danner 2017, Ch25) stated that more and more, the acceptance of decisions will depend on the ability and commitment of the HTA body to describe how patient perspectives have been determined, involved and considered.” (p310)
Transparency is an important element as HTA decisions can have political implications and become a political construct (Haefner and Danner 2017; Lopes et al 2020).

PCIG Values for social engagement in HTAs
HTAi PCIG has developed values and standards for patient involvement in HTA (HTAi PCIG) to be applied by HTA bodies and national authorities, see Table 3.

Table 3. HTAi Patient and Citizen Involvement in HTA Interest Groups Values for HTA bodies working with patient groups

Why do we involve patients in HTA?

The values are: relevance (of patients’ experiential knowledge), fairness (that patients are involved), equity (in contributing to), legitimacy (of decision making), and capacity building (the need for). A specific skill set is required by staff of HTA bodies to enable social engagement, and the purpose of the social engagement needs to be clearly defined for those who are participating (HTAi PCIG resources).

Some key points in relation to social engagement
In a number of countries or regions within countries it is a legislative requirement that HTA is considered when introducing new medicines: Australia, Brazil (Lopes et al 2020), Canada, Colombia, Germany, Hungary, Kazakhstan (mandated), Poland, Portugal, Romania, UK; and that social participation is part of the HTA process (WHO 2015 survey).
Some countries stipulate one, or a few patient/consumer organization(s) to provide the voice of patients and the public in government policy. For example, in Germany the Federal Ministry of Health recognizes four relevant national umbrella patient organizations (Haefner and Danner 2017), the Australian Department of Health works predominantly with the Consumers Health Forum (Wortley and Wale 2017), in France Assos Santé is authorized to represent users with respect to public health authorities, information and public consultations on health-related issues in France (Mamzer et al 2018). For Brazil and its National Committee for Health Technology Incorporation (CONITEC website), participation of civil society in the HTA process is formalized through members of the National Health Council (CNS). All CONITEC recommendations are open to public consultation for 20 days (publicized through website content, email, and social media) – with summarized versions in simplified language. Suggestions (available on spreadsheet on website) are compiled and discussed as part of the final technical report, which appears on the CONITEC website.

Experience of developing HTA and patient input in Taiwan, China
Taiwan, China has shown that patient involvement can be delivered even when resources are significantly more limited than in many Western countries. It has found that patient engagement is important for understanding the needs of the target patient population, yet challenges remain in ensuring timely patient engagement and provision of relevant resources. So further efforts are needed to implement and improve the visibility of patient input into the HTA process.

Taiwan, China has a mandatory single-payer Health Insurance (NHI) program that covers more than 99% of Taiwan’s population. This program is known for its low premiums and co-payments. The Health Insurance Act, amended in 2013, protects patients’ rights and invites them to voice their opinions. Taiwan conducts health technology assessments (HTAs) to support the Health Insurance Administration’s (NHIA) reimbursement policies on new drugs. The HTAs consider the health and well-being of all citizens, medical ethics, and cost-effectiveness with regard to the financial framework of the NHI program. The HTA department operates under the supervision of the Center for Drug Evaluation (CDE). A short history of the HTA development process in Taiwan has been reported by Chiu et al (2015). Five social values must be considered for decision-making pertaining to the inclusion of new medicines within the domain of healthcare benefits. These are: the safety and efficacy of the drug; cost-effectiveness; the principle of equality and protection of the weaker sections of society; attention to the quality of life of patients; and the life-saving and urgency factor of the drug.
Patient groups are invited to participate in PBRS Committee meetings. For new drug applications, patient opinions are collected only if the product being discussed is related to treating the diseases included in the NHI’s major illnesses/injuries list. A summary of the submissions is developed by the CDE/HTA division and incorporated into the HTA report for consideration by the PBRS Joint Committee. The report is published before the PBRS Joint Committee meeting, allowing stakeholders to learn about patients' experiences.

The NHIA has a webpage that enables patients, caregivers, and patient groups to submit their opinions about new drugs and also medical devices (NHIA website). The online platform is supported by a patient involvement guideline to assist people making submissions in expressing their opinions. The initial guidelines were revised by the CDE/HTA team with more practical guidelines to help patients have their voices heard and so motivate patients and patient groups to contribute their views. The CDE/HTA also initiated a series of educational programs for patients, caregivers, volunteers in hospitals, and patient organisations. These training courses were held across Taiwan, China.

Patients can participate in PBRS Joint Committee meetings in two ways. First, two patient representatives attend the meetings. The CDE developed a project to assist patient representatives in understanding more about the HTA process, diseases, and patient voices. The CDE/HTA team also holds a pre-meeting for patient representatives, beneficiary representatives (consumers) and case-related patient organisations, who have provided input on the platform to discuss patients' perspectives before the PBRS Joint Committee meeting. In a resubmission case, the NHIA can invite two disease-specific patient representatives to voice their opinions during the meeting (NHIA website).

In some technology assessment projects, the CDE/HTA has conducted interviews or distributed questionnaires to patients regarding their experiences with a technology or medical procedure, such as robotic surgery, artificial electronic ears or transcatheter aortic valve replacement, chemotherapy, and radiation therapy. Patient organisations assisted the CDE/HTA in finding appropriate patients to ensure that the final report included the views of those who had experience of these procedures. Other projects related to the improvement of the patient involvement mechanism have also involved a high degree of cooperation with patient organizations – including interviews with patient organisations so that the patient involvement mechanism could be made more structured and adapted to local conditions.
In Taiwan, China, patients participating in HTA and the reimbursement decision-making process are supported by the NHIA. In this process, the CDE/HTA team plays a crucial role in supporting not only the NHIA, but also patients and patient organizations. However, some challenges remain. The impact of allowing patients to engage with the HTA and reimbursement process on decision making therefore remains unclear. Few patients have chosen to share their experiences, especially with medical devices, via the online platform. This is likely because many patient organizations are still not aware of the online platform. The HTA agency and patient organizations both lack human resources and windows of time are short for patients to contribute (Chen 2022).

**Why experiential knowledge is important**

Societal members can contribute with their unique experiential knowledge about a disease, its diagnosis, treatment, and impact on the health system. People with experience of a disease or health condition and its treatment can therefore provide valuable input into the assessment of the value of a new technology and on the clinical trials that support an HTA, as listed in Figure 1 for treatment interventions.

**Figure 1. Patient considerations when looking at a new technology and the clinical trial evidence that supports its use.**
Clinically held beliefs of what is important to patients may differ from the needs expressed by patients themselves (Gibofsky et al 2018; Morel and Cano 2017). Different populations may also show important differences in their needs and beliefs. As a result, knowledge of the benefits and side effects of technologies and of disease management on the outcomes most relevant to patients is important. If these are not addressed, the different perspectives can lead to uncertainty in an HTA appraisal (Menon et al 2015). Patients are also able to provide context to the use of health technologies, including unmet needs, disease and treatment burdens, and give personal accounts of quality of life to compare with clinical trial results (Wale and Sullivan 2019).

**Possible ways of engaging with society on HTAs**

Table 4 summarizes ways that social engagement can take place at different levels of participation. The following section details some of the concepts behind social engagement in health care; and strategies that can be utilised to capture societal viewpoints in HTAs (Scottish Health Council participation tools).

Table 4. Approaches to social engagement in HTAs at the different IAP2 levels of participation

<table>
<thead>
<tr>
<th>METHOD OF ENGAGEMENT</th>
<th>INFORM</th>
<th>CONSULT</th>
<th>PARTICIPATE</th>
<th>COLLABORATE</th>
<th>EMPOWER</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Websites and fact sheets, at conferences, media releases.</td>
<td>Surveys, focus groups, public meetings, hearings, on-line feedback and comments, online discussion.</td>
<td>Forums, including multi-stakeholder, in-depth discussion, committee involvement or membership.</td>
<td>Partnerships and joint projects.</td>
<td>Integration into governance.</td>
</tr>
</tbody>
</table>

**Social engagement concepts and strategies**

We recognize that increasing numbers of people are living with chronic illnesses (including mental illness and cancer survivorship). These people live with the demands of managing their health care 24 hours a day, seven days a week. More and more, doctors recognize that they have to involve patients and their families and carers in the continuity of care, and extend opportunities for shared decision-making and person-centred health care.

The internet has meant that the public has greater access to information on diseases and health conditions. People are able to recognise the different stages of disease – going from diagnosis, treatment, management of symptoms and any adverse effects of both the disease and its treatments; to prognosis and how health care is accessed.
People are able to investigate options for themselves (or their family members) and link up with other people living with a disease through the internet and social media. People can read scientific information on the internet and organise clinical data in open databases that they themselves manage, either by themselves, through advocates, or patient and consumer groups. They know what treatments are available and where they are being used or trialled; although their knowledge and understanding of how the treatments work and other relevant considerations may be limited.

**Community participation models**

Social movements are very effective in increasing social participation to include the community and patient voice in health care. Technology-based platforms are social networks formed by Facebook, Twitter, blogs and forums, patient association web-based platforms, patient online communities, and others (Ravoire et al 2017). These media have advantages in answering questions about health products, societal approaches, and the organization of care. Large numbers can quickly become involved and from varied sociodemographic groups, but with legal and methodological (such as verifying data and participants) constraints. Limitations include representativeness of participants and the influence of media reports. Potential biases include age, sociocultural and professional characteristics, living in urban or rural areas, and lifestyle. Patient opinion leaders also have potential influence, maybe in a particular condition, and they may be involved in patient-focused drug development. An example of a technology-based platform is the web-based platform PatientsLikeMe, which has developed structured methods of obtaining data from patients that is suitable for research and quality of care measures (Burstin et al 2017). Online surveys, discussion papers and questionnaires can be used by organizations including HTA bodies to target a broad population of people, if effectively disseminated.

Facilitated discussions with a knowledgeable person as facilitator can be an effective way of seeking people’s views and encouraging them to participate in discussions. These can be face-to-face, through telecommunications or online, including through online bulletin boards (Cook et al 2019). A proposal can be formed and developed by the group with clarification of opinions to come to a consensus view, if needed. Participants can be trained or coached in the processes before participating, and the approach can be multidisciplinary. Patient interactions on social media platforms are relatively quick to analyse to help identify what is important to patients, carers and families (Cook et al 2019).
The online discussions can provide insightful patient and disease relevant information (Street and Farrell 2017, Ch14). Such ‘listening’ to self-initiated online discussions does not impose a burden on patients.

Community-based participatory research is where community members and researchers work together as equal partners – to increase skills, share relevant and effective ideas, and create connections and opportunities for learning and growth. Partners have diverse knowledge, skills, expertise and experiences to address complex problems. Benefits include increasing community trust in research; increasing likelihood that research results will lead to effective programs and products that communities want and can use; increasing validity of research results; creating connections between community organizations, healthcare organizations and researchers that support partnerships and share effective and relevant programs and products; increasing skills, connections and opportunities for growth for all partners (for example, SoLaHmo Partnership). Such participatory research is a type of “citizen science and/or citizen research” methodology.

The evolution of citizen science and/or citizen research
Citizen science and/or citizen research is an umbrella term that applies to a wide range of activities to engage society with science, acknowledging the need for scientists and members of society to work together to tackle complex challenges such as sustainable development. The term developed out of the work of UK-based Alan Irwin and US-based Rick Bonney in the mid-1990s (Riesch and Potter 2013). Within health care, patients and citizens establish social networks to share their experiences, symptoms and cures. They organize their own clinical trials (Wicks et al 2014) and join professional researchers for the advancement of large-scale research projects such as genomics, disease research, and microbiomes (for example, micro-organisms in the gut). Questions that professional researchers have been struggling with for years, or even decades, can be solved by people playing computer games (for example, designed to enable the public to help determine the folding structure of protein in Khatib et al 2011). The notion of ‘crowdsourcing’ has been applied by many authors to discuss novel forms of collective knowledge production and collective intelligence (Prainsack 2014; Woolley et al 2016; McGowan et al 2017). In this sense, the emergence of citizen science and/or citizen research has produced a paradigm shift on the pace and impact of collective knowledge production and innovation. This process is catalyzed by internet platforms (such as PatientsLikeMe, Citizen Science Alliance) and social media.
Citizen science and/or citizen research initiatives involve the participation of non-professional scientists at any and every stage of scientific knowledge production, from data collection or generation, data analysis and interpretation through to product application, dissemination, and evaluation. Differing visions for the role of scientific research in society call attention to the complex relationships between science, public goods, societal good, and public participation (Woolley et al 2016). Prainsack (2014) argues that “the increasing prominence of citizen science and/or citizen research challenges and reconfigures the ways in which knowledge production in the health domain takes place. At the same time, it raises ethical and regulatory questions pertaining, for example, to how contributions from citizens should be attributed in scientific publications; what research ethics procedures should apply to studies that are self-organized by patients; and how new modes of citizen participation in medical and bio-scientific knowledge production and decision-making can or should be integrated into existing institutional structures (e.g., into existing systems of healthcare delivery)”.

Across the spectrum, almost all citizen science and/or citizen research initiatives share three features that make its rhetoric particularly appealing to apply to large population-based biomedical research projects: (1) connections to the spreading popularity of personal information communication technologies (ICTs); (2) ‘crowdsourced’ problem-solving; and (3) the ‘grass-roots’ fundraising strategies that they facilitate. The recent explosion of citizen science and/or citizen research initiatives can largely be attributed to the integration of ICT into everyday life through computers, smartphones, the internet, and social media. As such, citizen science is touted as a way to tackle otherwise intractable, laborious, and potentially costly research problems. Being capable of coordinating the efforts of millions of lay people around the globe, it is said to allow researchers to think about data collection on a population-wide scale.

The core features of citizen science and/or citizen research, leveraging of widely distributed ICT, harnessing the ‘collective wisdom’ of the populace, and cultivating enthusiasm and support for science, make it very attractive to governments interested in propelling labour and data-intensive research in a cost efficient manner (Woolley et al 2016).
How is citizen science and/or citizen research relevant to HTA?
The key here is to consider the concept of technology. Researchers in the translational fields of political economy, science and technology studies and public health have outlined, in the 1980s that there are two types of technology: ‘hard’ or high-density technologies (that is, the conventional conceptualization of what technology means, such as diagnostic tests, vaccines, health programs etc.) and ‘soft’ or social technologies (that is processes, as a set of techniques, transformative methodologies, developed or applied in the interaction with and appropriated by the population, and which represent solutions for social inclusion and improvement of living conditions). Thus, outlining the relevance of constructing appropriate techniques also for the realm of human relations, so we can think of scientific ways to deal with social structures, human interaction processes, and motivational techniques. In this sense, social technologies can be used for the exercise of self-government and intercessor processes (Ferreira et al 2009; Campos 2011; Jin 2011; Dagnino 2014). If social participation models and methods are processes, that is ‘soft’ or social technologies, then patient and social engagement with both development and implementation of such technologies means that citizen science and/or citizen research is another type of technology within the scope of HTA. Citizen science and/or citizen research’s goal in HTA is to avoid tokenistic social participation approaches (that is, to involve society without effective participation) and to promote skills in members of society to contribute competently to the establishment of quality and effective deliberation processes. Furthermore, citizen science and/or citizen research approaches cover the need for early social engagement throughout the HTA processes, including at early stages (such as policy formulation and development) as well as at later stages (such as implementation and monitoring of (new) technologies in health systems).

In this sense, citizen science and/or citizen research initiatives should be both promoted and supported across the technology cycle (Shah et al 2009) by not only professionally-trained researchers but also HTA implementers, decision-makers and other social actors. Citizen scientists, better known as ‘patient research partners’, have been described in the Netherlands (Abma et al 2009) to meet HTA’s purposes in a more sustainable, responsible and equitable manner. As such, citizen science and/or citizen research comprises the ‘collaboration’ model referred to in Tables 8-2 and 8-4. Tools such as STARDIT that aim to standardise the way that information about such initiatives are reported, including information about which tasks were done by different people are key. Reports can be updated at all stages, from planning to evaluation, and can report impacts in many languages, using Wikidata.
STARDIT is free to use, and data can be submitted by anyone. Report authors can be verified to improve trust and transparency, and data checked for quality (Nunn et al 2021).

**Patient groups and participation in HTAs**
The traditional role of patient groups has been to provide support to patients, families and carers. In the late 1980s, the AIDS movement in the US led the way to patient involvement in changing health policy and access to new treatments, in a life and death situation. Breast cancer patients soon followed the lead. Patient groups have now moved beyond providing support in everyday care of patients to gaining experience and knowledge in scientific and clinical research to make demands for new treatments and early access to those treatments (Bedlington et al 2017). Patient advocates have been given ‘a seat at the table’ in research, health policy, service delivery and quality of healthcare committees, and HTA (Scott and Wale 2017). National, regional and international umbrella patient groups access greater expertise and provide stronger, more unified patient voices (Wagstaff 2018); and can more effectively apply citizen science and/or citizen research roles and provide value (Levitan et al 2018).

Social engagement in HTA decisions requires skills, resources and support, from both the HTA bodies and patient groups. For HTA bodies this work best when well resourced (Mamzer et al 2018). Addressing knowledge and skills requirements of patient groups to participate in HTAs and evidence-based healthcare decision-making and to manage patient expectations is another important element. Social participation opens the way for broader consideration of patient-relevant clinical trial outcomes and broader experience in and acceptance of qualitative and other forms of evidence. It is important that patients and the public can speak for themselves rather than through clinicians or researchers alone. Online resources and digital platforms are important enablers, in keeping with a person-centred approach to healthcare (Roy et al 2018). Attention must be given to how patient groups can interact effectively with HTA and policy processes.

**Evaluation of social engagement**
Effective evaluation of social engagement in HTA and health policy is limited. Where it has been done, it is largely to improve processes for obtaining patient input (Weeks et al 2017). Any impact of patient input on individual HTAs is often opportunistic and cannot be systematically assessed as dependent on quality of patient input (Facey et al 2018). Indeed, patient involvement can act covertly, and rarely has a single impact.
This raises the question whether effective input is pragmatic, or if it can be driven by philosophical and cultural changes in processes and attitudes (Roy et al 2018; Wale and Sullivan 2019). Haefner and Danner (2017) identified that respectful, purposeful, inclusive, timely, transparent processes are important enablers for evaluation and impact.

Gagnon et al in their chapter on evaluation of patient involvement in HTA (Gagnon et al 2017) argued that rather than ‘impacts’ we would be better to talk about short and medium-term outcomes of patient involvement. Relevant dimensions (Esmail et al 2015) are the context of patient involvement, processes used, and impact. That is, the goals of patient involvement, roles played, expectations and process are important (Dipankui et al 2015). It is difficult to evaluate the input of patients and carers under these circumstances. A preferable way may be to accept that social engagement in HTA is important and to apply quality improvement methodologies to continuously improve how it is approached. This could be in the form of repeated plan-do-study-act (PDSA) cycles (Cleghorn and Headrick 1996). Each cycle begins with a plan and ends with an action. Including the right people on a process improvement team is critical to a successful improvement effort.

The impact of social engagement can ultimately be determined in terms of person-centred health care and partnerships in healthcare decision making, policy and practice (Table 5).

**Concluding Remarks**

To deliver affordable and equitable universal health coverage diverse HTAs are required, including for service delivery models, vaccines, drugs, and medical devices. This challenges a purely biomedical model to be broadly applied to HTA assessments. Social engagement has an important role in working together with researchers and clinicians to identify and prioritize technologies that best meet population needs and the needs of the health system.

The acceptance of decisions by HTA bodies has political aspects and depends on an HTA body’s ability and commitment to describing how patient and social perspectives have been determined and considered in decision making processes. The set goals of social engagement, roles played, expectations, and processes used are important and challenge evaluation. Respectful, purposeful, inclusive, timely, transparent processes are important enablers for social engagement to have an impact, and for it to be evaluated.
International HTA bodies and a supportive national policy context can have critical roles in driving patient involvement in HTA. Activities that address concerns about patient involvement among HTA professionals, for example any hidden interests (or industry connections) and challenging advocacy standpoints (lobbying positions), are important to address. Changing culture with respect to social engagement in HTA and introducing a person-centred input to policy making decisions in health care as well as in service delivery and quality of care are key.

Table 5. A conceptual model for goals of social engagement in research, clinical guidelines and HTA

<table>
<thead>
<tr>
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<tbody>
<tr>
<td>Culture of patient-centredness</td>
<td>Setting patient-centred scope</td>
<td>Clear processes and roles in HTA</td>
</tr>
<tr>
<td>Shaping discussions and how conducted</td>
<td>Describing personal impact of disease and its treatment options, comorbidities, unmet needs</td>
<td></td>
</tr>
<tr>
<td>Impacting how professional team members view patient involvement</td>
<td>With improved communication - providing upskilling, support</td>
<td></td>
</tr>
<tr>
<td>Meaningful and effective partnerships</td>
<td>Identifying themes, subpopulations, carer role that may be overlooked or given different priority by researchers and medical professionals</td>
<td></td>
</tr>
<tr>
<td>Relevance to patients/ stakeholders</td>
<td>Helping select patient-relevant topics and outcomes</td>
<td>Patient-important outcomes, key questions</td>
</tr>
<tr>
<td></td>
<td>Influencing guideline structure, development</td>
<td>Patient preferences provide context for cost effectiveness analysis and recommendations</td>
</tr>
<tr>
<td>Use of results in health decisions</td>
<td>Facilitating guideline dissemination, implementation</td>
<td>Noticeable effect on decisions - in appraisal committee recommendations, advice</td>
</tr>
<tr>
<td></td>
<td>Guidance for end-user uptake, actively disseminating to patient groups</td>
<td></td>
</tr>
</tbody>
</table>

Bibliographic sources and suggested readings


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HTA Publication, Dissemination and Implementation Support

Authors: Brendalynn Ens, Lisa Pyke, Barbara Greenwood-Dufour
**Publication**

Research results hold little chance of impact if they are not widely shared and communicated to those most likely to use them. Planning for dissemination is an early essential step towards realizing impact and practice change from your HTA report. It is important to remember that dissemination alone may not be enough to effect change in thinking, practice, or policy — rather than simply publishing your report, you may need to also engage in knowledge translation or mobilization activities — this will depend on the audience you are trying to reach, and the significance of your findings, and whether the findings have potential to change current health systems.

Having an article published in a peer-reviewed journal can be an effective way to disseminate research results. When selecting a journal to submit your research to, always consider the audience you are trying to reach and the types of journals that the audience will typically read. For example, front-line clinician leaders may never access journals geared to policy-makers. Choosing the appropriate journal to submit your article to is essential.

When selecting a journal for potential publication it is important to ensure the integrity of the journal. One should give careful consideration to ensure legitimate journal publications are deliberately selected; examples include open access journals such as those covered by Pub Med among others. Predatory journal publications are to be actively avoided as they solicit manuscripts and charge fees for publications, yet lack robust peer review processes, inhibit authorship rights, and potentially compromise the integrity of your published work.

Be sure that publication is right for your needs. HTA publication in reputable journals may not always be the best approach for disseminating HTA findings. While it can get your results disseminated to a wider audience, some drawbacks and challenges to publication processes include:

- Journal writing is an art and requires sufficient time and effort to frame the article correctly. For busy researchers, time may not be part of regular work hours.
- Journal publication takes an extensive amount of time and can be an arduous process; editorial steps may span many months before it is published, and sometimes this never occurs.
- If your article is published after lengthy editorial iterations, your information may be out of date by the time it becomes mainstream.
There needs to be a description of the ‘impact factor’ from your work. Many credible journal submission guidelines require you to include this up front.

Despite academic recognition of the publication, there are no guarantees of uptake and true utilization of HTA results from a publication – true outcomes remain unknown and there are few ways to determine the reach and value of your work to others who may have seen the publication.

With an estimated 1.8 million scientific articles (estimate) published each year, there is no guarantee that your article and results will be seen or read.

If you have decided to write for publication, carefully sequence your findings to clearly articulate the nature of your work and convince readers of its importance!

Some tips for doing so include the following:

- Remember that a publication is a passive form of knowledge dissemination (but may or may not lead to action on the part of those who read it).
- Have a colleague read your manuscript before submission. Disjointed and poorly written manuscript submissions may be declined by the journal's editors.
- Make sure your flow of ideas is sequential and that one paragraph leads logically to the next.
- Keep your writing style simple and understandable — avoid large complex words and scientific jargon.
- Generally speaking, manuscript guidelines require completion of several standard sections: introduction or background, methods, results, discussion, and conclusion.
- The discussion section is where you attach meaning to your findings and deliver the take-home message from the research. The sections that come before the discussion should be more factual in terms of what, why, how, and what you found.
- Although the abstract is the first part of your article after the title, you may want to write it last, as you may find it easier to use your article to inform the writing of it. Remember that the abstract is your opportunity to entice and influence readers to read your full paper.
- Look closely at the abstract before submission to ensure it appropriately reflects the key points and results within the main text of your work.
- Your conclusion should be short and precise, identifying just the key findings. Avoid sweeping generalizations and over-reaching conclusions not supported by your research.
- Run a spell check and grammar check before submitting your article — poor spelling and grammar will instantly reduce your chances of having your article accepted when the manuscript reviewers see your manuscript.
• After you have submitted your manuscript, expect reviews and edits from those at the journal who have been asked to review it. You may not always agree with their comments, but remember that they are considering the broader picture of the article’s relevance to the journal’s readership.

• Provide courteous response to reviewers. They have been carefully selected to review and provide a wider perspective. If you disagree with a reviewer’s commentary, be clear and professional in your response while providing supporting information to illustrate your perspective.

**Dissemination**

HTA evidence can help inform decisions regarding access to and the appropriateness, affordability, and availability of health technologies. Whether it actually will or not depends on whether the results are clear, actionable, and relate to an issue that is a priority to decision-makers. Research has confirmed uncertainty as to which specific dissemination strategies (including publications) work best.

Dissemination begins with re-engaging with the original requestors of the HTA work. As applicable, key decision makers and HTA requestors may seek research consultation to verify results, confirm conclusions or request a high-level synopsis. Evidence has the best chance at having an impact through dissemination if it’s adequately communicated in a way that has relevance to recipients.

Unexpected HTA conclusions may catch original requestors off guard if they have not been apprised early of the results. If conclusions are surprising, further consultation and conversation with interested parties aids smooth dissemination once the final report is delivered.

Formatting of HTA reports should consider ease of navigation to key sections or sub-sections of the report and be respectful of limited (paper) printing policies. An 800 page HTA report in paper copy is a daunting item for even the most seasoned decision maker!

An accompanying 1-3 page plain-language summary is an excellent way to engage health technology decision-makers at multiple levels to improve dissemination of findings and to enhance likelihood of utilization of results. It is one of a number of knowledge mobilization tools featured in section C to efficiently provide stakeholders and decision makers with research, promoting meaningful discussion and ultimately supporting utilization of your work.
What is a plain-language summary?
A plain-language summary is typically one or two pages long and written using plain and everyday language — that is, with a choice of wording, structure, and layout that is appropriate for a non-research audience. Plain-language summaries are intended to be a quick and easy read, quite in contrast to the HTA report.

For decision makers, a plain-language summary may be necessary to capture their attention and make them aware of the research findings fast. Decision makers often deal with many issues simultaneously and therefore may not have time to devote to a lengthy, technical HTA document. However, they likely will have time to read a concise plain-language summary. For decision-makers who have several HTA reports on their desk and are deciding which of them to actually read, providing them a summary of your HTA will allow them to quickly assess whether your HTA is of interest to them. Then they might decide to read your report before or instead of others.

Secondary stakeholders — such as clinicians, health service administrators/managers, patients, and the general public — may not have experience with research science and HTA methods backgrounds necessary to make sense of the language and structure of research reports. Providing a summary in plain, straightforward, and jargon-free language can make research findings accessible to these audiences and ensure accurate interpretation of the conclusions. Even many primary stakeholders may not have appropriate backgrounds; so, in fact, plain language can benefit all stakeholder audiences.

A final advantage of plain-language summaries is they allow you to address and tailor the specific decision-making needs of your audience. Whereas the structure and language requirements of a formal HTA report are geared to the needs of other researchers or HTA producers, the format of a plain-language summary allows you to contextualize your key messages and the present the information in a way that resonates with a specific audience. It also allows you to focus on only those findings that are of relevance to the audience and to frame that information in a way that illustrates how it could address their particular decision-making needs.

Here’s a quick guide to planning a plain-language summary. Plan to include:
- What the HTA tells us
- The current problem that the HTA addresses
- How this information revealed by the HTA is new or different from what we knew before
- Why the information is important
How the reader will benefit from it; use action words to convey this.
What action could be taken as a result.

Here are some common headings to include in your plain-language summary:

- Key Messages: These are the key findings that would be relevant to your audience. Keep in mind that these messages might not include all the conclusions of the HTA. You should include only those findings that would be considered as factors in the readers’ decision making.
- Context: Include some brief background information as context. This should include a description of the condition the technology is intended to do and could also include the prevalence of the condition and what is currently the standard practice. This information can usually be found in the Introduction section of the HTA report.
- Technology: Briefly describe the technology, how it is thought to work, and why it may be a preferable alternative to current practice.
- Issue: Include a statement explaining the reason the HTA was performed. This statement is usually based on the research question.
- Methods: Include a couple of sentences describing the methodology used for finding and selecting the sources of information as well as the appraisal approaches used to analyze the information.
- Results: State how many sources of information were identified and how many met the criteria for inclusion.

Keep the summary to one or two pages, with each section containing only one or two short paragraphs and most sentences fairly short. Cover each major idea in its own paragraph and make sure each idea flows logically from one paragraph to the next.

Additional Options for Knowledge Dissemination Tools
Like plain-language summaries, knowledge mobilization (KM) tools or support documents can help overcome barriers to evidence adoption. Often however, it won’t be useful to develop a tool at all, so be clear on intended purpose for additional KM tool development.

KM tools most often are prepared to strategically support the implementation of practice, behavior, or policy change. Through such tools you can further connect stakeholders with research information in a way that they understand, is relevant to them, and is actionable by them. Examples include a half-page or full page guide for changing prescribing behavior, disinvesting in a drug no longer reimbursed or to help change treatment choices.
They must therefore be planned with the intended impact in mind. View them as persuasive tools and ensure they accurately reflect HTA research results. A targeted KM tool allows you to present information to a specific stakeholder group, with consideration of the group’s unique information needs and perspective. Whereas a plain-language summary is meant for the broader non-scientific audience, in a targeted KM tool you can use more tailored plain language. Tools can be prepared in many different forms:

- A policy brief or briefing note
- A basic handout or brochure
- A 1-page newsletter
- An infographic or shared decision-making illustration
- A slide set or presentation
- A video or blog-spot,
- An internet-based interactive learning event, or
- A social media campaign engaging Facebook™, Twitter™, or other platforms.

Not all HTA research requires the planning for additional dissemination and knowledge mobilization tools. Understanding your original requestor needs for information and further KM work is a good starting point as the results may be sensitive or not warrant further communication strategies. Furthermore, KM tools:

- Can be time-consuming to plan and prepare
- Require extensive consultation and multiple iterations and editing before they are complete
- Are highly focused on strategic needs of single groups or decision-makers so transferability may require further consultations and changes
- May need specialized graphic support
- Need to be available to accompany the release of the larger HTA report for optimal value and understanding
- Require additional copyright approvals for use of pictures, graphics and designs
- Require updating when new research is available.

Depending on the nature of your HTA work, tools can be very helpful ways to assist in broad dissemination and sharing of research results. In some instances, several KM tools — or a KM toolkit — may be required to give your research the best chance at being put into policy and/or practice at multiple levels in health systems. In all cases, the style of the tool must “fit” the intended audience. A tool should be created only when there is a sense that they could help result in impact — that is, that the end user of the tool will be able to use the information provided in them to inform their decision-making.
Some tips for getting started in KM tool preparation include:

- Carefully tailor tools to each specific audience and with an understanding of:
  - their point of view
  - their current concerns and interests
  - what they already know (or think they know) and what they don’t know
  - how receptive they will be to the new information and the barriers to accepting that information.

- Develop tools that are:
  - clear, concise, and brief — instead of giving the audience every detail of what the HTA uncovered, present them with only the information that they need
  - geared specifically for the audience they are intended to reach
  - include only the findings and messages that are necessary for the specific decision-making needs of that audience.

- Because there isn’t one format that would be suitable for all audiences, you will likely need to have tools available in a variety of different versions and formats to appeal to different audiences.

- To improve the chances of impact, ask a sample of your end users for input to inform the tool development, and ask them to review the final product.

- Produce a tool only if there’s an audience for it — that is, end users who can use the tools (and the information provided by them) to inform their decision-making.

One final note: An HTA program or research unit should have authorization and clearance to create knowledge mobilization tools before undertaking some of the examples discussed above. Preferences of HTA requestors or HTA funders need to be respected when the nature of HTA work may be confidential, sensitive or require time to consider potentially controversial perspectives. In these instances, wider tool development and dissemination beyond the original requestor is not suitable.

**Basics of Implementation Support**

As directed by HTA requestors or funders, there may be times when further assistance to specific groups is warranted to understand research findings, consult on dissemination tool development to assist practice change, or to nudge grassroots efforts along to align with evidence results. These more extensive outreach efforts are often viewed as “implementation support” and they represent a collection of carefully planned, coordinated efforts grounded in scientific implementation research.
The realistic uptake of HTA information can be impeded by various barriers and challenges, a common one being a lack of alignment of the priorities and principles of the various stakeholders. Stakeholders will often have differing opinions on whether a given health technology is appropriate, affordable, and offers good value for money, and who (if anyone) should have access to it.

Implementation support strategies attempt to ensure that HTA findings have the best chance of getting to those who can use it in time to inform their decisions and in a way that supports identification of challenges/gaps where additional research might help. Implementation support practitioners often prepare a “plan” or strategy to guide their efforts. A framework or plan for implementation approaches is recommended to guide actions.

It is important to note that such additional implementation support strategies are not an essential component of all HTA products. High impact / significant change HTA research may benefit the most from this added service provision. Further, an HTA program or research unit should have authorization and clearance to explore more complex implementation strategies before undertaking such work.

Benefits of implementation support include:
- Building awareness with a wide range of groups (clinical and decision levels) to ensure clear messaging and understanding of research findings
- Identifying practical and unintended challenges or barriers facing those trying to implement
- Facilitating dialogue across contradictory groups to arrive at common ground
- Direct linkages to be able to track and report impact from HTA research
- Identifying need for reassessment of technologies, need for real-world evidence consideration, or disinvestment.

Drawbacks and challenges to implementation support should be noted as:
- Implementation support requires trust to be established with decision-makers and researchers and clinicians, exceptional communication and facilitation skills. These are developed only over time and relationship development with decision makers and stakeholders.
- Requires advanced skills including abilities to understand and convey research findings efficiently, while gauging research knowledge of audiences.
- In some situations, partnerships and coalitions must be formed to support collaborative activities for practice change.
• Audience needs and priorities need to be assessed quickly and plans adjusted to meet multi-stakeholder concerns. Listening effectively to issues and challenges is critical for success.
• Implementation support requires extensive resources, dedicated staff, users understanding of the role(s), and dedicated funding to ensure success.
**Bibliographic sources and suggested readings**


International Journal of Technology Assessment in Health Care (Web Page); https://www.cambridge.org/core/journals/international-journal-of-technology-assessment-in-health-care/information/instructions-contributors#


Health Professionals’ Involvement in HTA

Author: Iñaki Gutierrez Ibarluzea
The participation of health professionals and citizens in the assessment of health technologies facilitates the evaluation process and contributes to improving the quality of evaluation reports and the acceptance and implementation of their conclusions.

Participation is relevant in all phases of the process: guidance on the development of the protocol, help in the critical appraisal of the clinical evidence and interpretation of the clinical relevance of the results.

There have been several efforts to ensure the involvement of patients in HTA (see specific chapter in this Handbook) and even the society HTAi holds a patients and citizens interest group since the very beginning (https://htai.org/interest-groups/pcig/). Notwithstanding, although clinicians or health professionals' involvement in HTA processes is seen as crucial, critical or at least, relevant, no such groups are established, in which discussions around their roles, processes in which they should participate and how and defined functions.

**What do we mean by health professional?**
A health professional or healthcare professional is a provider of health care that embraces the overall management of a patient, a group of patients or a group of citizens and in concrete, activities such as prevention, diagnosis, treatment, follow up, promotion and advice based on certified training and personal expertise and experience. The field includes those who work as a nurse, physician (such as primary or community care physician), internist, radiologists, psychiatrist, radiologist, surgeon etc.), physician assistant, registered dietitian, veterinarian, veterinary technician, optometrist, pharmacist, pharmacy technician, medical assistant, physical therapist, occupational therapist, dentist, midwife, psychologist, or who perform services in allied health professions. Experts in public health and community health are also considered as health professionals.

**Why health professionals are so important in HTA processes?**
As part of the group of stakeholders that should be considered when starting a HTA report or exercise, health professionals are not more important than others in HTA processes, however their participation should not be circumvented or replaced in certain phases. In fact, health professionals maintain health in humans through the application of the principles and procedures of evidence-based medicine (EBM) and caring. In accordance to HTA definition (O'Rourke et al, 2020), HTA uses the principles and procedures of EBM, in order to help decision-makers reaching to the best decision possible in the context of application.
Furthermore, health professionals give advice on or apply preventive and curative measures and promote health with the ultimate goal of meeting the health needs and expectations of individuals and/or populations, and as such, improving individuals and population’s health outcomes.

**Types of health professionals and their role**

It has been stated that health professionals belong to a wide range of specialties and as such their role could differ along the HTA process. In fact, there is a need to differentiate between those that are going to contribute in the scoping process and those that are advising on certain parts of the methodological roadmap of building up a HTA report. In accordance with the EUnetHTA Handbook of Building Capacities in HTA, 2008, not all the HTA agencies have all the capacities installed to complete a HTA report as required, there are certain skills that are fundamental in the HTA process, meanwhile there are others that are commonly hired or requested when needed, such as biostatistics, bioethics, legal or social skills or more recently environmental advice on the impact of technologies. Same can apply to biomedical engineers, experts in regulatory aspects or social or mass media experts. These experts are fundamental in many occasions in contributing to the development of HTA exercise. The inclusion of professionals that bring expertise in certain areas of the process and especially in methods and analysis is more common in small HTA teams or units, meanwhile big organizations of more than 30-40 professionals, they have all those capacities among their workforce. Although some of those could be considered as health professionals, from now on we will refer as such to those that contribute to the scoping process and along the elaboration of the HTA report and its discussion, due to their expertise in the specific questions that are related to the characteristics of the patients, the pathology or condition, the care pathway and possible alternatives and the outcomes of interest to be measured in accordance to their expertise.

Scoping is the exercise by which HTA doers define the research questions within the HTA process. This stage should address five major components: a) target population, b) technology or intervention, c) comparator/s and d) outcomes of interest, and e) perspective (Tanvejsilp and Ngorsuraches, 2014).

HTA doers are not specialists in all the fields of healthcare and apart from reading the literature and the evidence, it is crucial to have the perspective of those that are linked to the patients, their characteristics and the way systems could manage or are managing them, and which are the desired outcomes to be changed from the different perspectives that can be adopted.
The identification of the values and the diversity of approaches to determining the value and the possible inconsistencies requires a dialogue among the different stakeholders including the HTA team. The role of the HTA team is identifying the divergences and discuss them with the stakeholders, among those, health professionals, in order to resolve the controversies, or at least to characterize the reasons or the argues behind those differences.

Approaching the health professionals
The HTA Team should establish a clear strategy to approach stakeholders and specifically health professionals. This strategy should include: a) what HTA is; b) what HTA is for; c) which the methods that are used are; d) how the project will be structure and e) their role in the HTA analysis. In this sense, it is important clarifying which the role of them could be.

In certain occasions, the HTA teams include health professionals at the end of the process as peer-reviewers or discussants of the results and conclusions of the analysis. This should not be considered a good practice, but it is sometimes needed when fast approaches to evidence gathering and analysis is needed due to constraint timeframe to provide evidence to decision-makers. Thus, the HTA teams should plan ahead which the necessities of stakeholders’ engagement are, when they are going to occur and how conflicts will be resolved in order to deliver the final product/report on time.

Figure 1. Validate-HTA concept of looking for values in the determination of value (used with permission from VALIDATE-HTA consortium)
Which health professionals should be included
There is an eternal debate on who should be included and why. Notwithstanding, this applies to any stakeholders' group. In this regard, the level of representation and the characteristics of the health professional is key. There is no way to ascertain that the health professionals that we have chosen are the right ones, same applies to patients and patients' representatives. However, there are some tips that could be useful when choosing the adequate people. First of all, which the objectives that we are prosecuting are. Among those we can mention at least three: a) obtaining health professionals views and their values; b) ascertaining that this group of stakeholders understand what HTA work means and which the strengths and limitations of a HTA exercise are (“proselytism”); c) improving the acceptance of HTA reports and their conclusions and recommendations by groups of stakeholders, in this case, health professionals. There are other objectives that can also be covered with this exercise. It is worth mentioning, a) accuracy in the way the reports are written; b) accountability to other health professionals and; c) reliability by health professionals and thus, improvement in the subsequent processes of dissemination and diffusion of the final HTA reports and their conclusions/recommendations.

The dilemma here is who we should choose. There is a trend to identify key opinion leaders (KOLs) in the area or the theme we are working on. By choosing KOLs, we are ensuring that when finishing the report and disseminating the conclusions, it is more likely that the conclusions and recommendations are followed and accepted by a wide range of professionals. It occurs similarly when clinical practice guidelines are elaborated and finally published (Borbas et al, 2000). If those are supported, endorsed or accepted by KOLs, the impact of their recommendations and their use in real practice is expected to be higher. Nevertheless, the selection of KOLs could pose another issue, that is the management of time and agendas and their capacity to contribute to the discussions needed with the process of elaborating the HTA report. Therefore, it is worth considering including “second-line” health professionals when building the HTA project team. Furthermore, a strategy that can also be applicable or useful in this case would be to be in touch with local/national health professionals' societies and ask them for representatives of the society in the area or theme we are starting. It is also worth considering including health professionals we have already worked with, and the work they have developed is adequate. In this sense, there are agencies that have networks or lists of professionals “trustful professionals” they are involving when elaborating the reports.
The professionals finally involved need to be conscious that the process is demanding, and the timeframe is sometimes tight. So, we need to be very careful when detailing the tasks and phases, deliverables and milestones that meeting deadlines is part of a well-developed HTA project.

A clear strategy and project plan on health professionals’ involvement including the phases in which their participation is required and their role in each of the phases could help reducing the pressure on professionals and managing their expectancies. Sometimes it helps also having their acceptance or renounce to participate in the project. For that, a detailed Gantt chart, in which milestones, meetings and deliverables are detailed could be useful.

If finally, KOLs are not considered for HTA project team, we can always engage them at the end of the process, that is peer-review phase. This is also a way to confront the recommendations, inform around the report and improve the level of acceptance of other professionals by convincing them that the course of action has been sound, and the final conclusions are accurate. The final endorsement of KOLs and scientific societies also can be sought for when publishing the final report, within a so-called, public open consultation.

**When health professionals should/could be involved**

The HTA project, as it has been pointed out in other chapters of this monography, encompasses an iterative process that consist of different phases such as: a) identification; b) filtration; c) prioritization; d) scoping; e) analysis; f) findings discussion; g) conclusions and/or recommendations’ elaboration; h) peer-reviewing; i) public consultation; j) dissemination and; k) impact analysis. See Figure 2.

In previous paragraphs, we have mentioned when the role of professionals could be key, but it has not been revealed, in detail, in which phases of the HTA process, health professionals’ involvement makes sense, or it is needed/required.
Figure 2. Phases of the HTA process and health professionals’ involvement

- Identification: *Health professionals **
- Filtration: *Health professionals *
- Prioritization: *Health professionals **
- Scoping: *Health professionals ***
- Analysis: *Health professionals *
- Findings’ discussion: *Health professionals ***
- Conclusions and/or recommendations’ elaboration: *Health professionals ***
- Peer-reviewing: *Health professionals **
- Public consultation: *Health professionals **
- Dissemination: *Health professionals *
- Impact analysis: *Health professionals *

* health professionals’ involvement low
** health professionals’ involvement medium
*** health professionals’ involvement high.
First things first. Managing the conflict of interests
Prior to start any process and when we are considering the involvement of any stakeholders, but specifically health professionals, there is a need to record their possible conflict of interests. Why in this case, it is even more crucial than in any other cases. Health professionals are those that are using, prescribing, diagnosing and managing patients by means of health technologies. On many occasions, they have relation with technology producers such as: grants to attend meetings, funds for research projects or funds for publications, involvement in commercial trials and or companies funded registries among others. This does not discard them from becoming part of the team at the very beginning, but the declared conflicts and their influence on the assessment should be considered by the HTA team and when it is anticipated that a conflict could arise, the health professional involved should not participate in the discussion or should be discarded from the project team. Sometimes, it is difficult to discard certain professionals from processes of analysis of certain topics, because they are the main experts. In those occasions the best way to manage it, it is to transparently declare the conflicts and make them publicly aware. The minimization of any conflicts and their consequences should be attentively considered by the HTA team at any time. Although each organization could have a conflict of interests disclosure form, there are standard ones that could be used such as that of the International Committee of Medical Journal Editors (ICMJE, 2021: https://www.icmje.org/disclosure-of-interest/) or a specific one from EUnetHTA and how to manage declarations of interests (https://www.eunethta.eu/wp-content/uploads/2019/11/EUnetHTA-Procedure-Guidelines-DOI.pdf).

Identification
This phase describes the step by which topics that are potentially suitable for evaluation by the HTA process are identified. Typically, these could be topics that are publicly financed, covered by private insurances or covered by compulsory health insurance or included in publicly or privately funded health benefits' packages. Each healthcare system could have different needs due to their characteristics or the epidemiological paradigm the country or the region may have. The identification phase could differ from country to country and from system to system. Thus, some healthcare systems use horizon scanning that include health professionals among those that identify the items to be evaluated (EuroScan toolkit, 2014. Health professionals are a good source of identification because they know their needs, they are connected with advancements in their field of knowledge, and they can contrast those advancement with their practice and the current value of their practice.
Filtration
Once topics are identified not all of them are suitable for evaluation or they could be covered by the HTA team. Filtration means discard, there are different reasons by which technologies or topics are discarded, among those it can be mentioned that: a) they are not covered by the healthcare benefit; b) they are already being evaluated; c) potentially they do not respond to the needs of the systems; d) the level and quality of the information is not sufficient for the assessment; e) the technology is at an early stage of development and it is unlikely that it will impact the system in a short term. Filtration with explicit criteria is normally done by the HTA team. Notwithstanding, health professionals can potentially provide arguments or information to aid in this process.

Prioritization
Prioritization relates to the process by which the healthcare systems and HTA teams decide on what is going to be assessed first or even, what is not going to be evaluated due to the limited capacity of a HTA team to evaluate all the things that have been identified and filtrated. In big agencies or systems, prioritization defines a process of establishing the importance of a topic in accordance with the characteristics of the healthcare and the patients/citizens’ needs. In big agencies with huge capacities none of the topics are discarded and prioritization just defines the order of assessment, which topic will be first, second and so on. Health professionals could play a double role in prioritization, first defining the criterion or set of criteria that will be used to judge and prioritize the technology (Varela-Lema et al, 2014) and then to prioritize as part of stakeholders’ groups the topics that have been identified and filtrated.

Scoping
Once the topics to be evaluated have been selected and the HTA team starts to define the HTA project, the first exercise to be done is scoping. Within the scoping process, the questions to be addressed (normally PICO system is used) and the divergences among stakeholders should be identified and if possible, resolved. The involvement of health professionals at this stage is essential. They could even constitute part of the evaluation team as researchers within the project or just limit their role to give opinion at the first stage and then participate in the ulterior discussion. As it has been mentioned before, health professionals know better than any other stakeholders how the technology could be used in real world and all those aspects related to patients’ management, specifically organizational aspects at the micro level. This could also aid helping when building up the economic models and the micro-costing analysis (health professionals involved, time devoted, phases, etc.).
The Validate-HTA consortium (Validate-HTA Handbook, 2022) considers this phase critical as it could bring out the intended and unintended consequences of using or discarding the health technology/ies that are going to be evaluated.

**Analysis or assessment**
The analysis or assessment includes information gathering, critical appraisal of the information/evidence and summary of the final evidence that will be included in the report and it is normally conducted by the HTA team. On some occasions, the process of discarding pieces of evidence could be discussed with stakeholders, principally patients and health professionals, but it is more a mechanistic, transparent and well-defined process that has its own rules (mainly good practices in systematic reviews are followed). The process finish with presenting the evidence gathered in the form of evidence tables and when GRADE methodology is used (https://www.gradeworkinggroup.org/), the HTA team fills the GRADE forms for the posterior discussion of the findings with the stakeholders.

Findings’ discussion and conclusions/recommendations elaboration
Once the evidence has been found, analyzed and summarized, the HTA team proceeds to discuss the findings with the stakeholders. This is a process in which health professionals and patients’ involvement is essential. It is obviously needed that the stakeholders understand which their role in this process is and which will be the final output and to whom the conclusions/recommendations are directed. When GRADE methodology is used to reach to conclusions and recommendations, this is also a well-defined, standard and structured process.

**Peer-review**
HTA reports being scientific documents follow a process of peer-review before publication. This is one of the last chances to have specialized feedback from the stakeholders. Most HTA bodies have formal processes for peer-reviewing, and they adhere to publications best-practices. It has been pointed out that perhaps this is a good chance to involve KOLs as they will open the window for disseminating the results if they agree with the method and its conclusions. Health professionals are normally involved in this phase and depending on the topic different professionals are included. The HTA Teams have the right to include or not the comments provided by the peer-reviewers but they need to at least rebate or give argues on the changes that have been proposed.

**Public Open consultation**
Some HTA bodies provide the chance to comment or give feed-back at different stages of the HTA projects.
Normally that is accessible before development, during development or after publication. The most common one is just before or when the publication is to be published. The open-consultation process is another chance for interaction with stakeholders including health professionals (https://www.nice.org.uk/get-involved/consultations). The difference with involving directly the stakeholders is that the process is not structured, the interaction and roles are not well-defined and that the feedback though valuable could be diverse depending on the topic and the sole interest of the stakeholders. Open consultations are more adequate for further involvement of patients and citizens than health professionals. In the case of the health professionals interaction with scientific societies and professionals colleges seems to be more efficient.

**Dissemination and diffusion**
As part of the HTA projects, there is a phase of diffusing or disseminating the results and recommendations. One of the target audiences is health professionals and as such their role is passive, although in some occasions HTA bodies contact with scientific societies to help them distributing the results or including the conclusions among the newsletters, mass media, social media or journals. It is also common that some reports are adapted for publication in scientific journals and dedicated specialized journals are chosen to increase the impact of the results and the diffusion of the recommendations/conclusions.

**Impact analysis of the HTA work**
This phase is also a duty that is played mainly by the HTA team. Once again, the role of health professionals is minimum, and they are mainly the actors that are selected to analyze if the conclusions/recommendations have been adopted when managing the patients or groups of patients. Variability in practice and its translation on health results could be also analyzed. However, most of the HTA organizations analyze the adoption, inclusion, exclusion or modification of use of the assessed technology without considering the value that those actions could have in the final patient or group of patients. Nevertheless, HTA practices are changing and the analysis of Real World Data and the monitoring of technologies after their introduction in practice is becoming more and more demanded (Serrano et al, 2021). In this case, the role of health professionals is becoming more important as they are the actors that use the technology and participate in the elaboration of the protocol for data gathering and collect the data that is used for ulterior analysis. Health Professionals’ role in other processes such as reassessment and disinvestment, considering the life cycle of health technologies needs to be also carefully analyzed, as such, it could be even more crucial than in assessment activities, because they are already using the technology/ies and now the value they are obtaining by means of them.
Concluding Remarks
Health professionals are part of the stakeholders that play an essential role in HTA processes. Their involvement is key in certain phases but especially in the case of topic identification, scoping, prioritization, findings discussion, recommendations elaboration and peer-reviewing, although their role in other phases could be important and as such it is worth mentioning their role in defining the value of the technology once it has been implemented in the system. Main aspects to be considered when selecting health professionals is what we are going to demand from them and which their role and rights will be. In this regard, the planning of the HTA project is critical and the communication to those involved important in conducting a smooth and successful project.

The management of conflict of interests in the different phases of the HTA project and the inclusion, exclusion or consideration of the involvement of health professionals in accordance with what they have declared in the disclosure of interests need to be carefully discussed by the HTA team. The use of KOLs in the different phases needs also to be counterbalanced.
**Bibliographic sources and suggested readings**


EUnetHTA JA2 Declaration of Interest and Confidentiality Understating-FINAL -STATEMENT ON TERMS OF PARTICIPATION AND EXTERNAL ACTIVITIES OF EXPERTS (potential conflict of interest and confidentiality undertaking), 20140502 (as endorsed by Plenary Assembly, Madrid, 2014.04.10).

EUnetHTA Standard Operating Procedures (SOP) Manual, EUnetHTA Joint Action2; 2012-15; Developed by the EUnetHTA Secretariat July 2013; Amended and approved April 2014.


