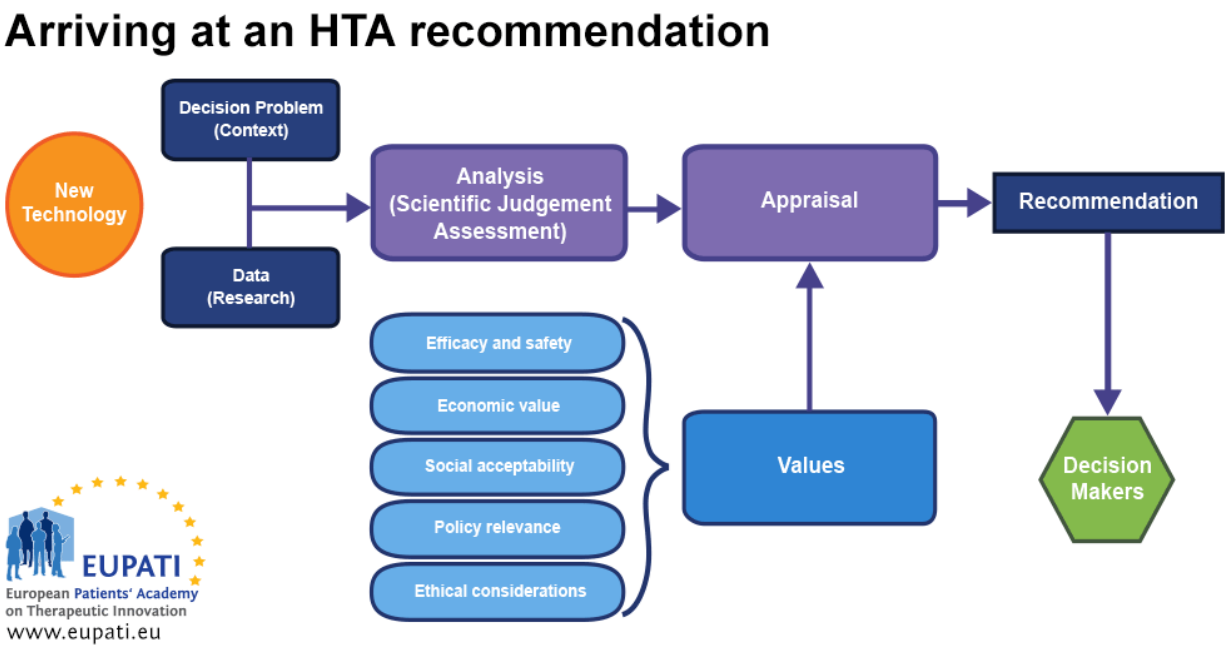
Patient involvement in the HTA decision-making process

Introduction

We can consider the entire HTA decision-making process as that shown in Figure 1. Patients can be involved in HTA decisions in many different ways.



The process and considerations of arriving at an HTA recommendation for decision makers.

Areas of patient involvement

Data (research)

HTA bodies need to make judgements about added value given the available information (data). Data in this context can range from clinical research to patient experiences. Assessments of

data will be in the form of either qualitative or quantitative research studies.

There are numerous ways in which patients can contribute 'data' to the HTA, for instance by:

- Developing or validating patient-reported outcome measures (PROMs), helping regulators, HTA bodies, and pharmaceutical companies understand what should be measured during clinical trials
- Participating in high-quality research
- Developing or completing surveys to:
    - Obtain information about the number of patients living with a condition
    - Document patients' experiences with a condition (for example, the number of patients with different forms of a condition)
    - Document patients' experiences with current treatments (for example, the number of patients experiencing certain adverse events and the proportion that rate these as extreme)
- Presenting patient experiences and stories to HTA bodies – HTA bodies use information from such presentations to examine the rest of the data. For example, if patients consistently report that having a disease is burdensome because of the need to take a wide range of medication, HTA bodies will look at all data to see if a new medicine, or a new way of delivering a medicine, will reduce this burden
- Providing patient submissions (for assessment) to HTA bodies, using a structured format that allows the HTA body to see impact across various decision criteria compared to current alternatives (such as equity, equality, legal, ethical, psycho-social). Submissions ideally seek to present information from a wide range of patients in a structured and unbiased way. They may include all of the above elements.

One challenge is how to collect, analyse, and consider patient perspectives on individual attributes of a new technology. For example, the difference between an intravenous administration and a once a day tablet may be significant for the patient but not captured elsewhere in value assessment (for example, if assessing value using a quality-adjusted life year gained approach). Therefore, well-conducted preference studies – such as discrete choice experiments – may be a useful addition to the assessment of overall value. They can be anchored using data from the randomised controlled trials, and therefore provide appropriate scientific rigor to what otherwise may be seen as simple statements of preference ("I like this more than that") which carry little or no weight in well-conducted HTA.

Decision problem (context)

The questions to be addressed in research are often defined by clinicians. However, patients' perspectives should be considered at this stage, in order to:

- ensure that issues that are important to patients are considered in the way the research is framed and evidence is judged;
- assist with the scoping of the research questions and defining the decision problem for HTA bodies; **and**
- comment on draft recommendations issued for consultation to ensure that the recommendations are fair.

Scientific judgement and analysis

Scientific judgements are those methodical and systematic considerations on which the analysis of data is based. Poor or highly variable judgements can lead to poor or unpredictable findings and potentially bad decision-making. Patients and patient groups can:

- Verify that their local HTA body has an evaluation

manual or methods guidance (a manual of guidance on methods) and if not, recommend one. This could encourage consistency in scientific analysis and use of transparent and up-to-date comparable approaches.
- If a manual or methods guidance exists, check to see if methods of assessment of patient-reported outcome measures and other ways of incorporating the patient perspective also exist or ask for them to be included.
- Be involved with consultations or updates of manuals or methods guidance initiated by HTA bodies to ensure that processes for embedding patients' perspectives are explicitly stated.

Value

In this context, value means the priorities that individuals bring, on which the interpretation of evidence should be based. For example, how important is it to eliminate certain outcomes or diseases relative to others? Value may also include the relative importance of various factors for decision-making, such as the effect of a new technology on equity and equality, and its legal, ethical, and psycho-social implications.

Patients or patient groups can:

- Check that their local HTA body has a value or criteria-based framework for making decisions. Some HTA bodies apply consistent and transparent frameworks. In most cases, value frameworks do not exist or HTA bodies simply state that they consider clinical and cost-effectiveness. Since value is comparing costs to measures of benefit, a clinical/cost-effectiveness framework values clinical effects (health outcomes) only. Patients can promote a framework or, if one exists, advocate for inclusion.
- Check that their local HTA body has representatives who present submissions from patients and speak on behalf of

a wider range of citizens (by understanding common needs and patient-related information) and not a specific patient group.
- Use patient-group submissions to highlight the relative importance of the various decision criteria that should be considered (such as health outcomes, equity, equality, legal, ethical, psycho-social). This can either be an opinion or based on empirical research (such as surveys).

Recommendations (appraisals)

Recommendations should be consistent with data considered and the values incorporated. Patients or patient groups can:

- Check that their local HTA body has a mechanism to review and to give feedback on recommendations (or ask for one) to ensure recommendation procedures are accountable and fair
- If a review and feedback mechanism exists, review and provide feedback to recommendations to ensure patient evidence and information was considered and consistent with data and information on values provided
- Communicate summaries of recommendations that can be understood by patients.

Decision

Decisions should be consistent with recommendations or – if they are not – should provide some justification as to why there are differences. It is at this phase that patients can switch from their role of evidence and value-providers to their role of advocates.

Patients or patient groups can:

- Interact with local decision-makers before a recommendation regarding the relative importance of the

decision and ensure information is provided to HTA body.
- Monitor the time between when recommendations are made and when decisions are acted upon as a means of improving accountability in HTA processes.
- Engage in appropriate political processes (such as advocacy) after a decision is made or is unduly delayed.
- Advocate that any further research that is recommended is funded and participate in that research after a decision is made.

Governance

Outside the process for a single HTA, patients have important roles to play in the governance of an HTA body. For example, patients may contribute to reviews of the HTA process or indeed to help in the evaluation of patient involvement processes.

Examining patients' experiences in the HTA process

In some HTA processes, patients are asked to describe what it is like to live with a disease and what it might mean to have a new treatment or medicine. These patient experiences relay several important pieces of information to the HTA body:

- They are considered a real world 'case'. However, individual cases are not particularly strong forms of evidence. HTA bodies are interested in the experience of entire populations, which is better captured by qualitative research of groups of patients with the disease in question. This is an area that can be supported by patient organisations who can draw on the breadth of their membership's collective experiences to supplement any testimony by an individual patient.
- They give a preliminary indication of which outcomes are important. This can give an idea of how the clinical

data will be used and what outcomes should be examined. It can also create a ‘value’ perspective – pointing to which outcomes are most important and what an acceptable ‘meaningful’ difference is for patients. Again, HTA bodies are more interested in entire populations and population-based research will be more informative than an individual ‘case’. However, in the absence of research, cases can serve as a useful starting point, and can provide perspectives not found elsewhere.

Examining input – ‘advocacy’ versus ‘evidence’

HTA processes are intended to be helpful for decision-making – they should use appropriate data and analysis coupled with a fair and accountable process to produce recommendations on which authorities can base their decisions (such as making a new health technology available to the patients who need it and how). In this process, patient inputs are crucial to make sure that the value of the new technology for their life is taken into consideration. Patients may consider it extremely important for their life to have that technology available. In this case, the decision that comes at the end of the HTA process can play a major role in their health. To ensure that their priorities are taken into account, patients should provide information to decision makers to better explain **how** important a decision is and **why** it is so important. This is what decision-makers need to know to justify their decisions based on available information using a defensible process.

The role of patients should be to make sure that an appropriate HTA process is put in place. This includes:

- ensuring that explicit and transparent processes are used for analysis and recommendation;
- facilitating the involvement of patients in defining the analysis;

- participating in committees making HTA recommendations;
- providing feedback during recommendations and analysis; **and**
- contributing with their perspectives consistent with the principles already discussed (including what it is like living with the condition in question, what the limitations of currently available treatments are, and – in some cases where the information is likely to be helpful in assessing overall value – preferences for specific product attributes).

Many HTA processes fall short of these important practices, considered 'key' principles. For example, many HTA committees lack patient representation – someone who understands the process and can speak on behalf of patients during the recommendation phase.

[glossary_exclude]Values and quality standards for patient involvement in HTA

In 2014, Health Technology Assessment International (HTAi) worked with a wide range of stakeholders internationally to develop values and quality standards for patient involvement in HTA. These values are the underpinning principles that indicate why it is important to involve patients in HTA. The quality standards are practical steps that HTA bodies can take to ensure effective involvement of patients in an individual HTA and when shaping the general process for an HTA. (For full text, see HTAi 'Values and Quality Standards for Patient Involvement in HTA')[/glossary_exclude]

The implementation of these values and quality standards is at an early stage, but patient groups have an important role to play in promoting them with HTA bodies and engaging in HTAi activities to encourage their use.

Conclusions

There are many different aspects of HTA where patients can contribute. The patient role begins before a new medicine is developed and can continue within HTA and after HTA recommendations are made. A starting point for any patient or patient group is to examine the inputs to their local HTA process, and assess whether it is fair and accountable.

Ultimately, HTA bodies need to understand how all patients with a condition will be affected by a new technology. Research that involves a large representative sample of local patients and using good research approaches will be most useful for HTA bodies. Beyond relaying personal accounts, patient groups may also consider how they can best develop or provide population-based research to these HTA processes.

[glossary_exclude]Further Resources

- Health Technology Assessment International (2014). 'Values and Quality Standards for Patient Involvement in HTA'. Retrieved 11 July, 2021, from https://htai.org/interest-groups/pcig/values-and-standards/
- Health Technology Assessment International (2015). 'FOR PATIENT GROUPS AND INDIVIDUAL PATIENTS'. Retrieved 11 July, 2021, from https://htai.org/interest-groups/pcig/resources/for-patients-and-patient-groups/
- Health Technology Assessment International (2014). *Completing a patient group submission template: Guidance for patient organisations.* Retrieved 11 July, 2021, from https://past.htai.org/wp-content/uploads/2018/02/PCISG-Resource-GuidanceandChecklist-Dec14.pdf[/glossary_exclude]

References

1. Eddy, D.M. (1990) ‘Clinical decision making: from theory to practice. Anatomy of a decision.’ *Journal of the American Medical Association,* 263(2), 441-3.

[glossary_exclude]Attachments

- Presentation: Patient Involvement in the HTA Decision Making Process

  Size: 486,275 bytes, Format: .pptx

  A presentation describing the patient involvement in the HTA decision making process, which can be adapted for own use.

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