

Beyond guidelines – tools to support patient involvement in health technology assessment

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Key messages of this chapter

- Health technology assessment (HTA) considers patient involvement as both patient participation (such as committee membership and submissions) and research into patient aspects (such as patients' needs, preferences, expectations and experiences) using robust scientific methods. This is similar to patient involvement in clinical guideline development.
- As in clinical guideline development, patient involvement in HTA plays an essential role with patient input and research into patient aspects helping to identify what the traditional scientific evidence means for patient communities. It can also address gaps and uncertainties in that evidence.
- Choosing the approach to patient involvement and tools to use depends on the goal for involvement and context of implementation.

Top tips

- Start with a clear goal(s) agreed by guideline developers, staff within the organisation and patient groups, communities and key patients.
- In patient involvement, earlier is better, so begin by developing involvement processes with patient groups, communities and key patients.
- Manage expectations about what can and can't be achieved with patient involvement – explaining the purpose of the process and how decisions are made.
- Consider the ethical consequences – including harm and burden to patients and their representatives – and develop strategies with patient groups to manage them.
- Develop values and quality standards for patient involvement in guidelines internationally and encourage GIN members to adopt and enact them.

- Learn from the experiences of others and document and share your own experiences.

Aims of this chapter

This chapter gives an overview of tools to support patient involvement in health technology assessment (HTA). It begins by explaining the parallels and differences between HTA and clinical guideline development. It then discusses the barriers to patient involvement in HTA, outlines how patients participate in the HTA process, and how patient-based evidence is used. It presents tools developed to support patient involvement in HTA that may be adapted to suit the needs of clinical guideline development.

The HTA context

HTA can be defined as

‘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 deployed in more than 30 countries, using robust scientific evidence and deliberation to guide policy decisions about medicines, devices, interventions, procedures, and other health technologies. HTA often seeks to determine the value of a new health technology based on clinical and cost effectiveness evidence. However, when HTA was first described by the US Office of Technology Assessment in 1976 it was envisioned as a means of considering all the implications of introducing a new health technology. As such, wider societal aspects – including the impact for patients and their families, legal and ethical issues, and the environment – were considered essential to any assessment of the consequences of the way a health technology was or was not used. In some HTAs, this wider consideration of consequences continues to inform the determination of value and has important implications for the evidence considered and deliberative frameworks used.

One of the accompanying footnotes to O'Rourke et al.'s definition of HTA, Note 3, recognises that value has many dimensions and the overall value of a health technology 'may vary depending on the perspective taken, the stakeholders involved, and the decision context' (2020). As such, it appears to support a view that the evidence base for HTA is robust but not neutral. Determining value depends on your perspective, which shapes the questions you ask in an HTA, the evidence you consider, and how you interpret it. This understanding of value determination has implications for the goals of patient involvement and subsequent use of research into patient aspects (known as patient-based evidence) and approach to patient participation. For example, patient-based evidence and participation may be used in recognition that traditional scientific evidence (such as randomised controlled trials) may not capture the outcomes that are most important to patients. It needs to be interpreted in the light of patients' needs, preferences, expectations and experiences, especially for application in the local healthcare context. As such, it often provides an important opportunity for those with lived experience of a condition to challenge assumptions made about patients and to direct inquiry to more relevant issues. Thus, patient involvement in HTA can be viewed as a means to patient-centred healthcare policy, 'ensuring fair and transparent allocation of resources informed by the needs, preferences and experiences of patients' (Facey et al. 2018).

There are many parallels between clinical guideline development and HTA in terms of scientific rigour and fair processes to translate international evidence into improvements in healthcare at a national or regional level. However, there is a difference in the way the evidence flows into decision making. Although clinical guidelines inform improvement in the whole care pathway and are focused on informing clinicians of best practice (provider decision making), HTA focuses more on decisions about a specific item in the care pathway and may be linked directly to reimbursement (payer decision making). HTA is often described in 3 steps:

- **Assessment:** critical review of published or submitted evidence about the clinical effectiveness or cost effectiveness of a health intervention.
- **Appraisal:** wider consideration of the evidence in the local context with value judgements about value and appropriate use.

- Decision making: decisions about whether health interventions are made available, and to whom, in a health system - access or reimbursement decisions. (Garrido et al. 2008.)

Bodies that undertake HTA vary widely and may be responsible for assessment, appraisal or both, but all seek to inform decision making in some way. Furthermore, many of the HTA bodies are part of larger organisations that undertake a range of evidence-based work in the health system and this often includes clinical guideline development. Hence sharing approaches, while recognising differences, seems appropriate.

As HTA has become increasingly associated with treatment reimbursement and access issues, it has become more contentious. In some countries this has resulted in strong patient advocacy challenges and political drives to involve patients in the processes. Some HTA bodies have responded by creating transparent processes for patient participation in the HTA process and developing approaches for obtaining patients' needs, preferences, expectations and experiences (Facey et al. 2010). However, this involvement is not widespread or consistent. Some HTA bodies are reticent about involving patients or including their perspectives, especially when a health technology's value is seen as scientifically determined and patient involvement considered a source of bias rather than evidence and perspective. If such concerns are not satisfactorily explored and resolved within an HTA body (and its stakeholders), patient involvement is unlikely, or may at best be tokenistic because of its perceived threat to the credibility and legitimacy of HTA rather than improving the robustness of HTA. Without careful consideration of an HTA body's beliefs and norms before committing to shared goals for patient involvement, HTA bodies risk setting up unrealistic expectations for patient communities. As a result, patient input and patient-based evidence will be perceived to have little or no consequence because of implicit or explicit barriers.

Hence guidance was needed to provide practical ways in which patients could contribute to HTA and decision making with credibility and legitimacy (Boivin et al. 2014). Clarification was also needed about the complementary but different roles for evidence generated from research into patients' needs, preferences and experiences using robust, scientific methodology and insights gained from patient participation in

HTA processes. Such participation includes patient input from written submissions and committee membership.

Barriers to patient involvement in the HTA process

Beyond organisational beliefs about HTA and patient involvement, a variety of barriers to involvement need consideration to operationalise it and avoid the features of tokenism, such as lack of transparency in decision making, lack of influence, and lack of inclusivity. In 2005, Hailey identified common themes that had been reported about consumer (patient and public) involvement in health research relevant to HTA. Facey updated this table in 2017 (Chapter 5). Table 1 presents a summarised version of Hailey and Facey's work. with some recent literature additions.

Table 1 Barriers to patient participation in HTA (adapted from Hailey [2005] and Facey [2017])

Challenge	Issues
Interaction of patients and researchers	<ul style="list-style-type: none"> • Time needed to develop a trusting, productive relationship
Resources	<ul style="list-style-type: none"> • Administrative, financial, staff support
Mechanisms of participation	<ul style="list-style-type: none"> • Lack of a comprehensive approach that sets the goals of participation for each stage of HTA (Gauvin et al. 2015) • Often chosen by the decision maker, who shapes it in a specific manner and so has control over the participation (Boivin et al. 2014)
Identifying a 'patient position'	<ul style="list-style-type: none"> • Recognising that there are differing values, expectations, environment, culture, genetics, and experience of the health system, and that it is not possible to canvass all
Nature and extent of patient representation	<ul style="list-style-type: none"> • Difficulty defining which patients should be involved • Questions about representativeness • Concerns about conflicts of interest and influence of health technology developers • Difficulty reaching marginalised populations
Technical demands	<ul style="list-style-type: none"> • Lack of knowledge, power, credentials or skills in scientific process and health care policy options
Training and education	<ul style="list-style-type: none"> • Lack of education and training developed specifically for consumers
Time demands and remuneration	<ul style="list-style-type: none"> • Time commitments, working to tight timetables, payments that should be made to patients
Balancing information from researchers, the literature, and patients	<ul style="list-style-type: none"> • Lack of concordance between issues that patients regard as important and those in which research has been conducted • Concern about methodology to balance qualitative and quantitative evidence and the role of costs, including questions about credibility of patient-based evidence • Devaluing patient-based evidence in evidence hierarchies (Gauvin et al. 2015)

Challenge	Issues
Use of patient input	<ul style="list-style-type: none"> • Unsure what to do with patients or how to involve them • Concern of tokenism • Impact on timelines • Poorly moderated discussions preventing patient contribution (Facey et al. 2010) • Researchers' or clinicians' concerns that scientific debate is softened by including patient perspectives • Possible distortion of funding decisions because of patients' biases • Selection bias – processes may be inaccessible to many patients and ignore, or aim to eliminate bias, rather than valuing the unique perspective of individual patient participants and developing more accessible and appealing processes (Vanstone et al. 2019) • Patient group concerns about how evidence from different sources is handled, weighed and valued, and that others have more influence • Power differences between patients and professionals (Boivin et al. 2014) – processes value clinical and economic evidence over lived experience and patient-based evidence (Vanstone et al. 2019)
Lack of awareness of HTA processes	<ul style="list-style-type: none"> • The implications of HTA processes for healthcare systems (including beyond yes or no funding decisions) are not understood • Patients do not know how HTA is used or how to participate
Few evaluations of patient input	<ul style="list-style-type: none"> • Absence of good quality research to show that patient involvement makes a difference • No demonstration that patient involvement improves quality of assessments
Burden, benefit or risk	<ul style="list-style-type: none"> • Poor consideration of the impact on patients or patient groups of involvement, including poor management of expectations • Benefit (for example, capacity building, learning, system change) should outweigh risk (for example, physical, emotional, spiritual, economic harm; Vanstone et al. 2019)

Although the research reflected in table 1 was initially done in the first decade of this millennium, many of these barriers still exist. They are probably applicable not only to HTA, but also to clinical guideline development.

Similarly, the variation in type and level of patient involvement in HTA identified by a European Patients' Forum (EPF) survey in 2011 remains a feature in this field. It

reflects the different rationale, motivation and approach applied in each country. The EPF confirmed that few HTA bodies and decision-making bodies involve and integrate patients' perspectives in their reports or conduct formal evaluation of the impact of patient involvement in HTA. Moreover, when there is some form of patient involvement this is often not done in a systematic, comprehensive and meaningful way. Apart from financial resource constraints, the main challenges were perceived to be the lack of capacity, time and good methodologies to involve patients. (EPF 2013.)

The first book in this field, *Patient involvement in health technology assessment* (Facey et al. 2017), sought to address the need for information about good methodologies and approaches to patient involvement in HTA. It drew on the expertise and experience of 80 authors from around the globe. In addition to providing case studies, the book aimed to be a cohesive guide to the field. It set out the rationale and detailed recognised approaches to participation and evaluation, and appropriate scientific methodologies for research into patients' needs preferences and experiences. The latter included the use of qualitative evidence synthesis, discrete choice experiments (DCEs), analytical hierarchy process (AHP), patient-reported and relevant outcome measures, ethnography field work, deliberative methods, and social media analysis.

Importantly, the book also sought to clarify issues that had arisen because of inconsistent terminology in the field and the resulting inappropriate use and treatment of patient involvement in HTA. Building on the work of the Health Technology Assessment international (HTAi) Patient and Citizen Involvement in HTA Interest Group (PCIG) in 2010, which described the 2 distinct but complementary approaches of patient involvement, that is, participation and robust evidence about patients' perspectives (Facey et al. 2010), the book expanded on the different roles and considerations for each. Participation was defined as a form of dialogue for shared learning and problem solving that can aid value judgements throughout the HTA process. Described as a mosaic of approaches selected according to the goal(s), participation is often sought to address gaps and uncertainty in the evidence and recognised for its role in interpreting evidence for real-world implementation. It commonly takes the form of patient input, such as written submissions and

committee membership, which is characterised by its source (patients and patient groups gathering and presenting information to aid decision making). Whereas evidence about patients' perspectives, known as patient-based evidence, is intended to provide evidence of patients' needs, preferences and experiences in a form that can be critically assessed, as are other forms of scientific evidence. Table 2, by Staniszewska and Werkö (2017), summarises key differences between the 2 approaches.

Table 2 Summary of the differences between patient-based evidence and input from patient participation in the HTA process (Staniszewska and Werkö 2017)

Patient-based evidence	Patient participation in the HTA process
Produced through research, generally published in peer-reviewed journals	Originates in perspectives of individuals, groups of patients or organisations
Draws on a range of methodologies	Does not necessarily use or need a specific methodology
Draws on robust scientific methods whose strengths and limitations are known, and provides a robust conclusion that can be clearly interpreted	The quality of the methods used to gather inputs may be unclear or not considered as important
Depends on appraisal of quality, including formal critical assessment and peer review	The concept of quality may depend on factors such as authenticity or diversity of perspectives
Research is based on research genres and specific research questions, and takes time to generate from either primary or secondary research	Patient participation can be used at any point in the HTA process, and may be in the form of a dialogue to enable immediate reaction to an emerging issue
May be more limited in accounting for context of the HTA, depending on whether studies have considered context	Can consider the context of the HTA question
Can be based on a synthesis of studies, which means a comprehensive, unbiased view of a patient issue can be summarised very effectively	Provides a selection of perspectives that may not be comprehensive but are informative
Research directly addresses questions of bias and balance, which provides some assurance of quality	Bias in relation to patient input is a complex concept that requires exploration in the future

In addition to clarifying these distinctions and describing appropriate methodologies and approaches, Facey et al. (2017) drew on the work of Abelson et al. (2016) and the OHTAC Public Engagement Subcommittee (2015) to suggest that patient involvement begins with defining the goals for involvement, which should then guide

decisions about approaches, methods and evaluation within the framework of the HTAi Values and Quality Standards for Patient Involvement in HTAi (see the [section on HTAi Values and Quality Standards for Patient Involvement in HTA](#) for more information). These goals may be instrumental, democratic, scientific or developmental (OHTAC Public Engagement Subcommittee 2015).

The editors and many of the authors of the book were active members of the HTAi PCIG. This interdisciplinary group, formed in 2005, promotes awareness of patient and citizen involvement, encourages methodological development, shares best practice, and supports jurisdictions seeking to introduce or develop involvement. PCIG has been active in developing tools for HTA bodies and patient groups to adapt for local involvement activities. Some of these tools may be suitable for adaptation for clinical guideline development. Key tools are described in the next section and further tools can be found on [PCIG's web pages](#).

HTAi Values and Quality Standards for Patient Involvement in HTA

Increased awareness of and interest in patient involvement in HTA has led to calls for guidance around 'best practice' from many stakeholder communities, including those comprising patients and families. In response, the PCIG produced HTAi Values and Quality Standards for Patient Involvement in HTA. These values and standards, shown in table 3, were developed through an international 3-round Delphi process. They can be applied or developed to suit the clinical guideline setting.

Table 3 HTAi Values and Quality Standards for Patient Involvement in HTA (2014)

Values	<ul style="list-style-type: none"> • Relevance: Relevance refers to the fact that patients and families hold important knowledge and a unique perspective, which can only be obtained through 'lived' experiences with a particular disease or condition. Both are essential to the generation of HTA evidence that is comprehensive and captures the value of a technology to those directly affected by its use. • Fairness: Fairness relates to the need to create opportunities for patients to be engaged in the HTA process that are equivalent to those already available to other stakeholder communities, such as healthcare providers and industry. Therefore, patient involvement is
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	<p>viewed as a basic ‘right’ of patients and families affected by HTA-informed decisions.</p> <ul style="list-style-type: none"> • Equity: Equity is often defined as the absence of avoidable differences among groups within a population. Patient involvement in HTA helps to ensure that HTA evidence reflects an in-depth understanding of the diverse needs of various groups of patients. This information can reduce the risk of creating inequities in health status when healthcare systems are required to distribute health resources fairly among all users. • Legitimacy: Legitimacy refers to the acceptance of HTA-informed recommendations or decisions by affected individuals through appropriate patient involvement. Engagement of patients and families in HTA contributes to the transparency, accountability, and credibility of HTA-informed decision-making processes, which, in turn, enhances their legitimacy. • Capacity building: In general, adoption of formal mechanisms for involving patients in HTA not only addresses existing barriers to their engagement, but also provides an opportunity to build capacity for patients, families and HTA organisations to work together in a productive way.
<p>Quality Standards: General HTA process</p>	<ol style="list-style-type: none"> 1. HTA organisations have a strategy that outlines the processes and responsibilities for those working in HTA and serving on HTA committees to effectively involve patients. 2. HTA organisations designate appropriate resources to ensure and support effective patient involvement in HTA. 3. HTA participants (including researchers, staff, HTA reviewers and committee members) receive training about appropriate involvement of patients and consideration of patients’ perspectives through the HTA process. 4. Patients and patient organisations are given the opportunity to participate in training to empower them so that they can best contribute to HTA. 5. Patient involvement processes in HTA are regularly reflected on and reviewed, taking account of the experiences of all those involved, with the intent to continuously improve them.

Quality Standards: Individual HTAs	<p>The remaining 5 standards apply to specific steps followed during the assessment and formulation of a recommendation or decision about a particular health technology.</p> <p>6. Proactive communication strategies are used to effectively reach, inform, and enable a wide range of patients to participate fully in each HTA.</p> <p>7. Clear timelines are established for each HTA with advance notice of deadlines to ensure that appropriate input from a wide range of patients can be obtained.</p> <p>8. For each HTA, HTA organisations identify a staff member whose role is to support patients to contribute effectively to HTA.</p> <p>9. In each HTA, patients' perspectives and experiences are documented and the influence of patient contributions on conclusions and decisions are reported.</p> <p>10. Feedback is given to patient organisations who have contributed to an HTA, to share what contributions were most helpful and provide suggestions to assist their future involvement.</p>
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In developing these values and quality standards, the PCIG stressed that patient involvement should be seen as a journey. Every HTA body starts in a different place and the high requirements of the values and quality standards are intended to encourage them to take a step on the journey to involve patients in their processes. Those who already do, should evaluate what they do and make improvements. Since their publication in 2014, several HTA bodies have endorsed the Values and Quality Standards and used them to review their own processes. For example, CADTH has used it for their Framework for patient engagement in health technology assessment (2019).

Participation throughout the HTA process

Technology or topic selection

Most HTA bodies established to inform reimbursement or coverage recommendations review all new drugs and therefore do not need processes for identifying and selecting technologies for assessment. However, some jurisdictions require a sponsor to make a submission to trigger the assessment. Usually, the manufacturer is the sponsor. But when manufacturers do not submit a drug for assessment, other stakeholders, such as patient groups, may seek to make a submission so that patients can access the drug. Some HTA bodies, such as the Pharmaceutical Benefits Advisory Committee (PBAC) in Australia, accept

submissions from the public and patient groups. In practice this is rare because of the complexity of the technical process, but PBAC has supported and considered 1 submission from a patient group, Rare Cancers Australia.

However, HTA bodies with a mandate for assessing non-drug technologies do need processes for identifying and selecting technologies for assessment. For example, the Swedish Agency for HTA and Assessment of Social Services (SBU) and the Scottish Health Technologies Group (SHTG) accept topic proposals from patient groups and other stakeholders. Patient organisations have the right to request Germany's Joint Federal Committee (G-BA) for a decision on non-medicine technologies. This means that not only can they propose a topic, but a request must be discussed, and if it meets the conditions for an HTA, G-BA must conduct the HTA. Instead of using a form, a specialist team, available to patient organisations regardless of funding, skills or size, helps with drafting the request (Haefner and Danner 2017).

Additionally, [IQWiG \(Germany's Institute for Quality and Efficiency in Healthcare programme\) ThemenCheck Medizin \(TopicsCheck Medicine\)](#) allows anyone to propose a topic for an HTA. IQWiG uses a 2-stage selection process to determine up to 5 citizen proposed topics each year. Usually completing proposal forms can be challenging for any stakeholder even with support from the HTA body. However, IQWiG has developed a simple online process designed to enable people without medical or research knowledge submit a question.

In terms of prioritisation of work, SBU has involved patients and carers in prioritisation methods. Its process is based on work by the James Lind Alliance and is used to identify the 10 most important uncertainties for condition areas with many uncertainties (Werkö and Andersson 2017).

However, most HTA bodies need to develop opportunities for patient input into decisions around which technologies to assess. This may include the participation of patients and their families in the development of criteria used to select technologies for HTA.

Scoping

Patients and patient groups may be involved in scoping, which generally uses the PICO (population, intervention, comparator, outcome) framework. For example, the National Institute for Health and Care Excellence (NICE) elicits patient input by publishing its draft scoping documents for comment and then publishes all the comments and the new scope. An examination of the [NICE's public involvement webpage](#) reveals patient groups provide important input, particularly about patient subgroups, comparators and outcomes that matter.

Another example may be found in Australia's Medical Services Advisory Committee's (MSAC) PICO Advisory Sub-Committee. This group circulates the scope and a consultation survey to targeted patient groups.

EUnetHTA is a network of European agencies that collaborate in the joint production of HTA reports. The network uses the HTAi template (described in the [section on HTA tools for patient input](#)) to involve patients in [EUnetHTA's patient input in Relative Effectiveness Assessments](#) (reports that assess clinical effectiveness). The intention is to gain patient input to inform the development of the PICO table and provide the assessment team with insights into patient experiences.

Patient input at scoping has been shown to be valuable for highlighting outcomes that matter to patients and identifying appropriate comparators for HTA submissions.

HTA tools for patient input

With the adoption of more rapid HTAs, especially for drugs, many HTA bodies began accepting submissions in the form of a written template. Some, for example the Centre for Drug Evaluation (Taiwan), the National Committee for Health Technology Incorporation (CONITEC, Brazil), and PBAC and MSAC (Australia), accept submissions from any member of the public, including patient groups. Whereas others, such as NICE (England and Wales), the Scottish Medicines Consortium (SMC) and CADTH (Canada), apply criteria that usually limit submissions to patient groups and include a declaration of funding basis. Submissions contain insights from the lived experiences of patients and their families that may challenge assumptions or address gaps and uncertainties. Templates generally ask questions about the daily lives of patients and families affected by the particular condition or illness,

current treatment options and their impact, as well as the experiences of those who have had the technology being assessed.

The PCIG has worked with HTA bodies and patient organisations to review patient group submission forms. They have developed 3 standardised [HTAi patient group submission templates for medicines, non-medicines and diagnostics](#). These tools have been consulted upon internationally and translated from English into French, Spanish and Mandarin. The template for patient group submissions to the non-medicine's HTA could be adapted for clinical guidelines. This is accompanied by a cover note for HTA bodies, which stresses the need to adapt the template to suit their own circumstances, processes and the technology being assessed.

The HTAi templates highlight the information that is valued by decision makers but patients need to be well supported by training, guidance and feedback to realise their value. This is because patients may not know which areas of their lived experience knowledge are being assumed, misunderstood or missing in the evidence. Some HTA bodies have dedicated staff who provide support to patient organisations during the completion of submissions. SMC, for example, provides feedback on draft submissions and CADTH gives feedback in a letter after the assessment.

The PCIG has adapted a guide developed by CADTH's pan-Canadian Oncology Drug Review that helps patients complete a patient group input submission template. The [HTAi's guidance for providing patient input using the templates](#) helps patient groups understand what kind of information will have most impact and gives guidance on how to undertake surveys and conduct interviews with patients and report findings. Recognising that patient groups usually conduct this work without an ethics committee review, the PCIG also developed an [HTAi short guide and a long guide on ethical issues for patient groups to consider when collecting and reporting information for HTA submissions](#).

Additionally, in 2020 the PCIG released a [Summary of Information for Patients](#) template and guidance based on a research project and SMC's experience in providing information to patient groups from the sponsors about the medicine being assessed. The rationale for the template is that people, especially those in smaller or less HTA-experienced patient groups, require information about the product being

assessed to target their input. The PCIG intends that HTA bodies will adapt the template.

Patient input in submissions has helped HTA bodies understand:

- the trade-offs patients might make
- the consequences of variations in service provision
- the potential real-world value of small clinical benefits to patients
- the consequences of treatment pathways
- how a treatment is administered for patient subgroups not identified in clinical trials.

Berglas et al. (2016) studied how patient input was integrated in 30 assessments by CADTH's Common Drug Review. They found that CADTH reviewers used patient insights about health status achieved, progress of recovery, and longer-term consequences of illness and treatment to frame the assessment. The CADTH Canadian Drug Expert Committee uses these insights to aid the interpretation of evidence. They also found patient input identified outcomes that are important to patients, but which may not be measured in clinical trials.

Committee membership

Patients and their representatives may be included on advisory and appraisal committees. To overcome ongoing confusion about the roles of patient and public members, Street et al. (2020) found identifying members by the interest and values they are tasked to represent provided clarity. They defined a patient member as someone 'who has been selected to support the inclusion of the interests of patients in HTA processes on a committee', whereas a public member 'supports the inclusion of the interests of the society at large'. Patient members may be nominated for a committee to give specific expertise based on their lived experience. As committee members, they can also present the wider experiences and perspectives of their patient communities on a particular condition or issue. Furthermore, patient and public members play an important role in ensuring that patient involvement processes are appropriately enacted, can reflect on improvements, and provide training to those providing patient input. G-BA appraisal committees are an example

of HTA bodies using this approach. They include patient representatives throughout the appraisal process and in all sessions of the committee.

Hearings

Australia's PBAC conducts consumer hearings when there is greater uncertainty in interpreting benefit and harm evidence, such as some medicines for rare diseases. In Brazil, public hearings are legally provided for, but have not yet occurred. The intention is that public hearings be held before the final decision is taken for cases in which the secretary of the Ministry of Health's Secretariat of Science, Technology and Strategic Inputs determines that the relevance of the matter justifies a hearing. The hearings are envisaged as a face-to-face consultative mechanism open to anyone and participants will have the opportunity to speak. (Silva et al. 2019.)

Consultation

Several HTA bodies, including the Italian National Agency for Regional Healthcare Services (AGENAS), CONITEC (Brazil) and NICE (England and Wales), publish consultation reports to seek feedback from a wider range of stakeholders, including patients and patient groups. Because these reports can be quite technical, patient involvement is better supported if patient-friendly versions are prepared and workshops or meetings are held to discuss the issues with relevant patient communities.

Dissemination

HTA bodies use patient-friendly versions of HTA reports and recommendations to communicate how recommendations were formed and what this means for patients. For example, working with Health Improvement Scotland's public partners and drawing on the guidance from the [DECIDE project](#), the SHTG produced a patient guide to its HTA on wound dressings.

Beyond individual HTAs

The use of patient participation beyond individual HTAs is less described in the literature. Examples include the formation of advisory groups at SMC, NICE and Health Technology Wales who involve patients in developing and reviewing patient involvement processes. Another example is CADTH's involvement of patients in

shaping and contributing to the agenda of its key capacity building activity, that is, its annual symposium. PCIG is currently undertaking a study to describe patient participation at the organisational level and may develop tools to support this area if a need is identified.

PCIG's resources to involve patient groups and individual patients in HTA, include the [HTAi Online Resource Directory](#). The directory aims to make it easier to locate useful resources shared by HTA bodies, not-for-profit organisations and other relevant organisations.

Use of patient-based evidence

HTA bodies that perform their own literature reviews, such as SBU, DEFACTUM (part of Corporate Quality in Central Denmark Region), AGENAS, CADTH, RedETS (the Spanish Network of Health Technology Assessment Agencies and Benefits of the National Health System) and SHTG, may undertake specific literature searches to determine patient issues. They use iterative processes to identify issues of importance to patients, and then search for literature (often qualitative research studies) that describes patients' perspectives and experiences about those issues. Such studies provide evidence of how people, including patients, carers and family members, perceive and experience a condition and its treatment. They are in a form that can be critically reviewed and is explicit about the strengths, limitations and bias of its methods. Systematic processes, such as qualitative evidence synthesis, can be used to critically appraise such qualitative research and synthesise it using methodologies from social and humanistic research (Swedish Council on HTA 2013). If evidence is lacking, primary research can be commissioned and reported as part of the HTA (Danish National Board of Health 2007).

EUnetHTA's HTA Core Model Online (2017) includes a patient and social aspects domain that focuses on patients' and their significant others' considerations, worries and experiences before, during and after the implementation of the technology. The [EUnetHTA HTA Core Model Handbook](#) provides guidance on conducting research into patients' perspectives that could be used for a variety of needs. In such processes, patient groups or patient experts can also:

- provide helpful input to the protocol that defines the research questions

- identify outcomes that matter most to patients
- provide important consultation comments on the draft guideline and recommendations.

In recognition of the increasing use of rapid assessments, Health Improvement Scotland has produced 3 resources, which they are trialling. See the [HTAi website for the guide to conducting rapid qualitative evidence synthesis for HTA, the methodology and the coding template](#).

In addition to qualitative research, patient preference methodologies, such as DCEs and AHPs, may provide a useful additional source of evidence to inform HTA recommendations. Some HTA bodies are exploring these methodologies. Patient preference research may be especially useful when a technology is being compared with a standard treatment that has different features, such as mode, ease of administration, side effects, and the risk of serious side effects (Bouvy et al. 2020). However, further research is needed to ascertain its optimal use in HTA and the health technology development lifecycle (Danner and Gerber-Grote 2017). Limitations associated with stated preference methods, such as participant innumeracy, hypothetical bias, variation among subgroups, and inert or flexible preferences need to be considered. A PCIG project subcommittee is investigating these issues.

IQWiG conducted 2 preference elicitation studies – one using DCE and one using AHP - in which patients valued the importance of treatment outcomes in different indications. It found the studies had potential to generate weights or prioritise outcome-specific HTA results. The AHP study demonstrated that patients valued different outcomes to clinicians and the DCE study in lung cancer identified important alternative endpoints (Egbrink and IJzerman 2014).

Impact

Inconsistent terminology, limited goal descriptions, and poor documentation of patient involvement's use and influence have made it challenging to evaluate their impact. Despite this, the need to evaluate the impact of patient involvement is increasingly recognised, especially to improve practice. Evaluation has been used to determine if and how patient insights were integrated into assessment reports, and if

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the presence of written patient statements are associated with positive reimbursement decisions. It has also been used to assess the impact of written statements (Mason et al. 2020). The number of sources of evidence and variables in an HTA make such evaluations problematic. An alternative approach to understanding the impact of patient involvement in HTA is case studies in the form of stories (for example, as described by Single et al. 2019). The PCIG is developing this work using [a template to collect further case studies](#) or stakeholders' experiences of patient involvement in HTA. Such information could provide reflections on the perceived impact of patient involvement from the perspective of anyone involved in an HTA, including patients and people working in HTA or industry.

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