



OHE WHITEPAPER

# Incorporating the Patient Voice in Health Technology Assessment



CONTRACT RESEARCH REPORT  
FEBRUARY 2024

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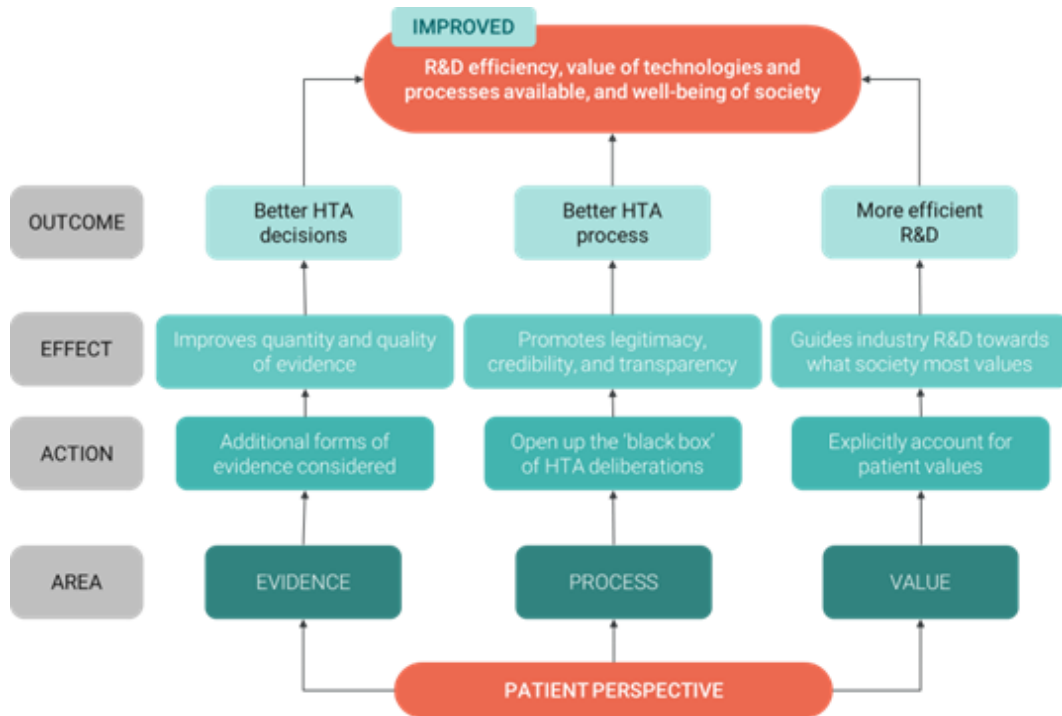
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# Executive Summary

The decision to adopt or not adopt a new healthcare technology, and the process by which this decision is made, affects patients, the health system, and wider society. Different interventions or technologies will have different impacts on a patient's outcomes, their experience during the treatment journey, and their financial situation. For health systems, adopting a new technology will impact the direct medical costs associated with treatment and will come with opportunity costs that affect other patients within the healthcare system. For wider society, a new intervention or technology may be associated with productivity gains, a reduction in welfare benefits or sick leave compensation, and a reduction in unpaid or informal care, freeing that money for other uses within the system.

In this Whitepaper, we suggest that the patient perspective is critical to making fully-informed decisions about the value of new technologies. However, this perspective has not always been given the same weight as clinical and economic aspects in health technology assessment (HTA). We argue that including the patient perspective in HTA has a number of potential benefits:

- First, incorporating perspectives and forms of evidence that may not have been considered within a narrower health system perspective can improve the quantity and quality of evidence, thereby improving HTA *decisions*.
- Second, opening the 'black box' of HTA deliberations to patients can reassure patients and the wider public that decisions are made on the basis that patients can trust, even if they may not agree with those decisions. This promotes the legitimacy, credibility, and transparency of decisions, improving the HTA *process*.
- Third, explicitly accounting for patient values in HTA decision-making can send a signal to innovators regarding what health systems value and are willing to pay for. This can guide industry research and development (R&D) towards the interventions, as well as the measurement of outcomes and experiences, that society and health systems value most highly. This improves the *efficiency* of research and development (R&D), the *value* of technologies and processes available to healthcare systems, and ultimately, the *well-being* of society.



The ‘patient voice’ refers to the opportunity for patients to participate – and especially to be *heard* – in HTA processes, and to offer the ‘patient perspective’ on aspects of need and value. This patient perspective refers to different experience-based perspectives on the burdens of a disease, the value of different treatments or interventions, and any unmet needs. Broadly speaking, we see three themes around which the patient perspective can bring valuable insights to the HTA decision problem: 1) measuring and contextualising clinical outcomes; 2) understanding the value of improvements in outcomes or processes; and 3) demonstrating novel and societal value elements.

We describe a number of qualitative and quantitative methods by which patient perspectives can be translated into ‘patient evidence’ that can be incorporated into HTA deliberations, but the most appropriate methods will depend on the nature of the decision problem. Ultimately, consideration of the patient perspective will depend more on the commitment of developers, regulators and assessors than the specific methods used to collect this information. As such, we make the following recommendations for ensuring that patients are ‘heard’ and that the patient perspective is appropriately incorporated into HTA:

1. **Developers, regulators and assessors** should engage with patients early and throughout the process of drug development and evidence generation, including when defining the key trial outcomes and measures. Patients and their advocates should be proactive in contributing to these processes, but this is not to suggest that they are responsible for ensuring that the patient voice is heard;
2. **Developers** should collect and present patient evidence that complements clinical and economic evidence;



3. **HTA bodies** should commit to considering patient evidence on an equal basis alongside clinical and economic evidence, including with explicit guidance and, potentially, by adapting their evidence paradigm to facilitate this consideration; and
4. **HTA bodies** should give patients a direct voice in HTA recommendations.

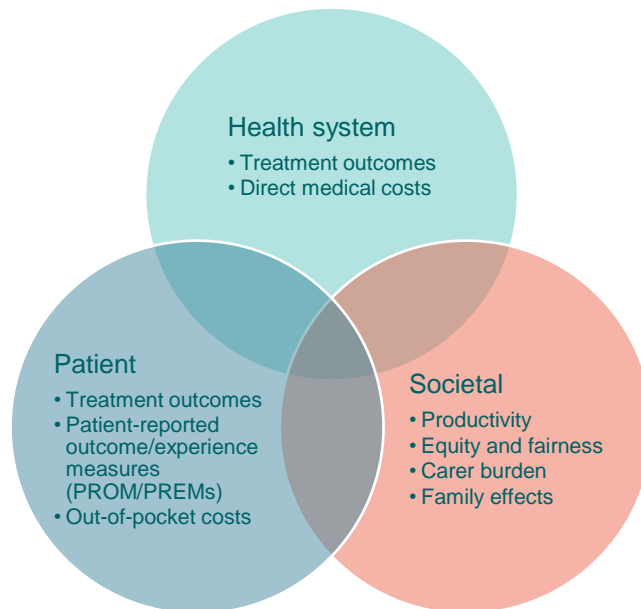
The patient voice matters in HTA decision-making because it ensures that healthcare assessments and decisions are aligned with patient-centred principles, reflect real-world experiences, and consider the values and preferences of the individuals who are most affected by those decisions. It ultimately leads to more informed, ethical, and patient-centric healthcare choices and should therefore play a pivotal role within HTA processes. We hope that this whitepaper and its recommendations can help make the ambition of greater patient-centricity in HTA a reality.

# 1 Why does the patient voice matter in HTA?

The decision to adopt a new technology has implications for patients, the health system, and wider society. Different interventions or technologies will have different impacts on a patient's outcomes, their experience during the treatment journey, and their financial situation. For health systems, adopting a new technology will impact the direct medical costs associated with treatment and will come with opportunity costs that affect other patients within the healthcare system. For wider society, a new intervention or technology may be associated with productivity gains, a reduction in welfare benefits or sick leave compensation, and a reduction in unpaid or informal care, freeing that money for other uses within the system.

Which of these considerations are included in a health technology assessment (HTA) depends on the 'analytical perspective' that is adopted. Figure 1 presents different perspectives on costs and outcomes that could be adopted as part of an HTA. Different HTA bodies may include a different mix of these perspectives but, in general, most take a health system perspective, focusing on direct costs and benefits to the health system. Consideration of the patient perspective is generally limited to descriptions of the health effects of the intervention. Even then, these health effects are often *valued* by the public rather than patients (Helgesson et al., 2020).

**FIGURE 1: DIFFERENT PERSPECTIVES ON COSTS AND OUTCOMES IN HEALTHCARE**



In this context, the term '*patient perspective*' refers to a range of perspectives, experiences and preferences of persons living with a disease and/or its treatment; paid or volunteer



carers supporting patients living with the disease; patient advocates (who may or may not have the disease themselves); and professional or volunteer patient organisations with a broader view of a disease and, sometimes, with experience around clinical, regulatory and HTA processes (Hunter et al., 2018). In some cases, the patient perspective may overlap with the societal perspective, for example when it comes to understanding how to prioritise different patients according to the rarity or severity of the disease or the socioeconomic characteristics of patients.

As we note, there is some overlap in the perspectives and priorities of patients, health systems, and society, but equally, different stakeholder groups will have concerns that are not recognised or prioritised by other groups. In particular, a health system perspective may miss elements that are important to patients, including the lived experience of a disease, the experience – as opposed to the *outcome* – of treatment, and aspects of treatment outcomes that may not be captured (or fully captured) in other perspectives. As a result, assessments of the value of different interventions based on a single perspective may be incomplete and overlook important elements of value.

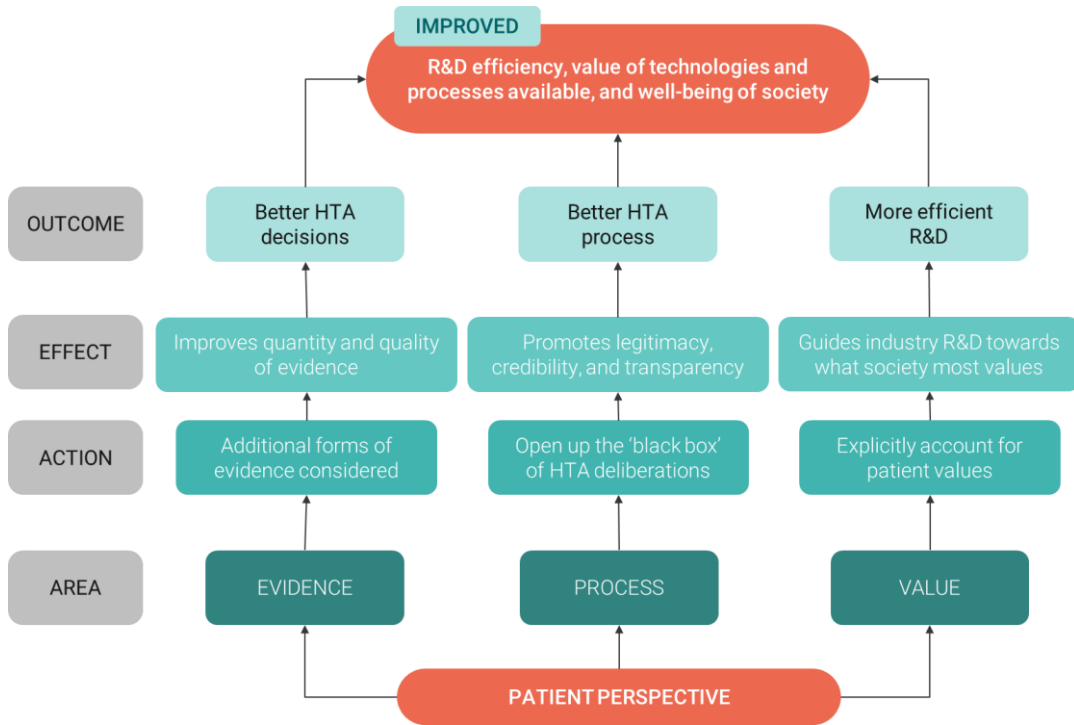
Recognising and accounting for these wider perspectives allows for a more comprehensive and meaningful definition of value. Specifically, inclusion of the patient perspective in HTA reflects principles of patient centricity that have become pillars of modern healthcare. A UK government consultation document articulated this trend towards patient centricity, stating that its aim to ensure “that all patients are fully involved in decisions about their own care and treatment so that the principle of shared decision-making - “no decision about me, without me”- becomes the norm across the NHS” (Department of Health, 2012). This principle of “no decision about me, without me” emphasises the role of the ‘patient voice’ in HTA: the opportunity for patients to participate and influence HTA decisions – that is, *to be heard*.

Considering patient perspectives on the value of a new intervention or health technology can have benefits for HTA, the health system, and society, which are summarised in Figure 2. First, incorporating perspectives and forms of evidence that may not have been considered within a narrower health system perspective can improve the quantity and quality of evidence, thereby improving HTA *decisions*. Inclusion of patient evidence in HTA is likely to have the greatest potential impact when the clinical or economic evidence is finely balanced between acceptance and rejection. In these cases, the possibility that the incremental cost-effectiveness ratio and any additional payer criteria fails to tell the whole story can be the difference between recommending a health technology or not.

Second, opening up the 'black box' of HTA deliberations to patients can reassure patients and the wider public that decisions are made on bases that patients can trust, even if they may not agree with those decisions. This promotes the legitimacy, credibility and transparency of decisions, improving the HTA *process*.

Third, explicitly accounting for patient values in HTA decision-making can send a signal to innovators regarding what health systems value and are willing to pay for. This can guide industry research and development (R&D) towards the interventions, as well as the measurement of outcomes and experiences, that society and health systems value most highly. This improves the *efficiency* of research and development (R&D), the *value* of technologies and processes available to healthcare systems, and ultimately, the *well-being* of society.

**FIGURE 2: HOW CONSIDERATION OF PATIENT PERSPECTIVE BENEFITS HTA AND HEALTH CARE SYSTEMS**



In the first part of this report, we present a brief review of how different HTA bodies currently account for the patient perspective. Second, we describe how specific aspects of the HTA decision-problem could be informed by considering the patient perspective. Third, we describe research methods for generating rigorous evidence of patient value suitable for consideration during HTA alongside conventional clinical and economic data. Finally, we conclude by discussing how patients, health systems, and HTA bodies can work together to appropriately account for the patient perspective in valuing new health technologies.

## 2 How can the patient voice inform the HTA decision problem?

As noted above, the patient voice in HTA refers to the opportunity for patients (or others with direct experience of the disease, treatment, or caring) to be 'heard' in the HTA process and to contribute their perspective on value. Here, "patient perspective" refers to different experience-based perspectives on the burdens of a disease, the value of different treatments or interventions, and any unmet needs.

Broadly speaking, we see three key themes where the patient perspective can bring valuable insights to the HTA decision problem:

1. **Measuring and contextualising clinical outcomes**, including secondary endpoints and safety data, into day-to-day experiences, in terms of real-world consequences for the patient that may not have been previously considered from a clinical or HTA perspective. This can also include the identification of appropriate outcome measures, especially with respect to specific patient-reported outcome (PRO) or experience (PRE) instruments to ensure they fully capture the symptoms, functioning, and experiences that matter most to patients.
2. **Understanding the value of improvements** in the *process* of treatment and/or the *outcomes* of treatment beyond what may have been measured in clinical trials.
3. **Demonstrating novel and societal value elements**, such value of hope or real option value, as well as societal preferences over disease severity, rarity or unmet need.

We discuss each of these themes in more detail below.

### 1. Measuring and contextualising clinical outcomes

The first step in ensuring that patients can contribute to assessing the value of their treatments is to ensure that clinical trials and other evidence generation activities are measuring aspects that are relevant to patients. The importance of aligning the methodology of drug development and evidence generation with patient values and preferences is emphasised in the US Food and Drug Administration's (FDA) [Patient-Focused Drug Development \(PFDD\) Guidance Series](#) (Center for Drug Evaluation and Research, 2023). This guidance discusses recruiting patient collaborators, methods for identifying what is important to patients, developing 'fit-for-purpose' PROs, and incorporating these values and preferences into regulatory assessment.

Whilst clinical data provide valuable evidence around clinical efficacy and health outcomes, these may not provide a complete understanding of the distinctive benefits of different treatments to patients. Adverse effects and safety concerns that may seem minor from a clinical perspective might be critical to patients. Qualitative patient input, such as interviews or impact statements, can shed light on areas of treatment that impact patients'

lives but are not captured within clinical trials. For instance, minor adverse effects such as dizziness may not be seen as important from a clinical perspective but might prevent the patient from being able to drive to work or collect a child from school. From this perspective, the risk of dizziness might be a key factor in how a patient values a particular treatment. Utilising disease-specific PROs that include dimensions most relevant to patients represents another avenue through which the patient voice should be considered.

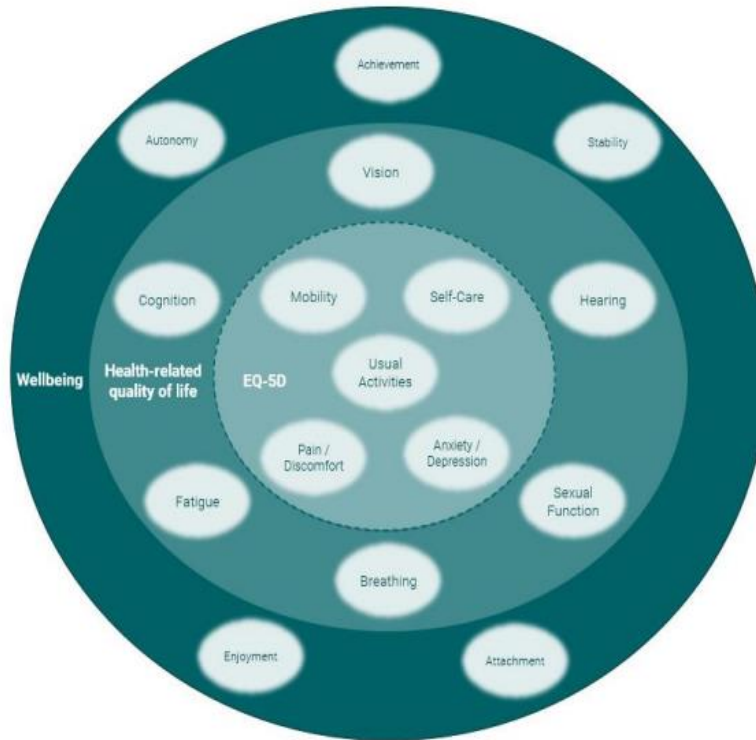
There is also increasing recognition of the importance of considering the impact of new medicines and technologies on the health and wellbeing of informal (unpaid) carers (Al-Janabi et al., 2016; Brouwer, 2019). As such, we would argue that carers may have as much of a role to play as patients in ensuring that all aspects of value are captured. This is particularly true in disease areas that impose a substantial carer burden such as congenital and neurodevelopmental disorders that occur in children, or neurological conditions such as Alzheimer’s disease that occur later in life. Indeed, we note that appropriate consideration of carer preferences is likely to be critical to making well-informed decisions around life-extending treatments for patients with high caring needs (Mott et al., 2023).

## 2. Understanding the value of improvements in process and outcomes

Clinical endpoints and generic measures of health-related quality of life (HRQoL) do not always fully capture the full range of a patient’s values or experiences. For example, the core dimensions of the Euroqol EQ-5D, the most common generic outcome measure in value assessment, include pain, mobility, ability to self-care, anxiety/depression, and ability to go about one’s usual activities. As illustrated in Figure 3 below, these ‘core’ dimensions can be supplemented by ‘bolt-ons’ that capture additional elements of HRQoL (Mott et al., 2021). Even with these bolt-ons, however, broader – and arguably fundamental – aspects of patient well-being, such as autonomy, social function, and life enjoyment are still neglected. This outer ring could also include elements of convenience related to the process of treatment, such as the frequency and (dis)comfort of a treatment (Higgins et al., 2014).

Alternative forms of patient evidence beyond generic measures of HRQoL can provide insights around these elements. This can include disease-specific PRO and PREs, that can provide insights around the outcomes and experiences of a specific disease and could supplement the ‘middle ring’ of Figure 3. Again, such instruments should be developed and validated in collaboration with patients. However, we suggest even PRO/PREs chosen in collaboration with patients may still neglect the *value* that patients assign to these outcomes. As such, we discuss alternative forms of evidence that can include direct measures of patient values and preferences in a later section.

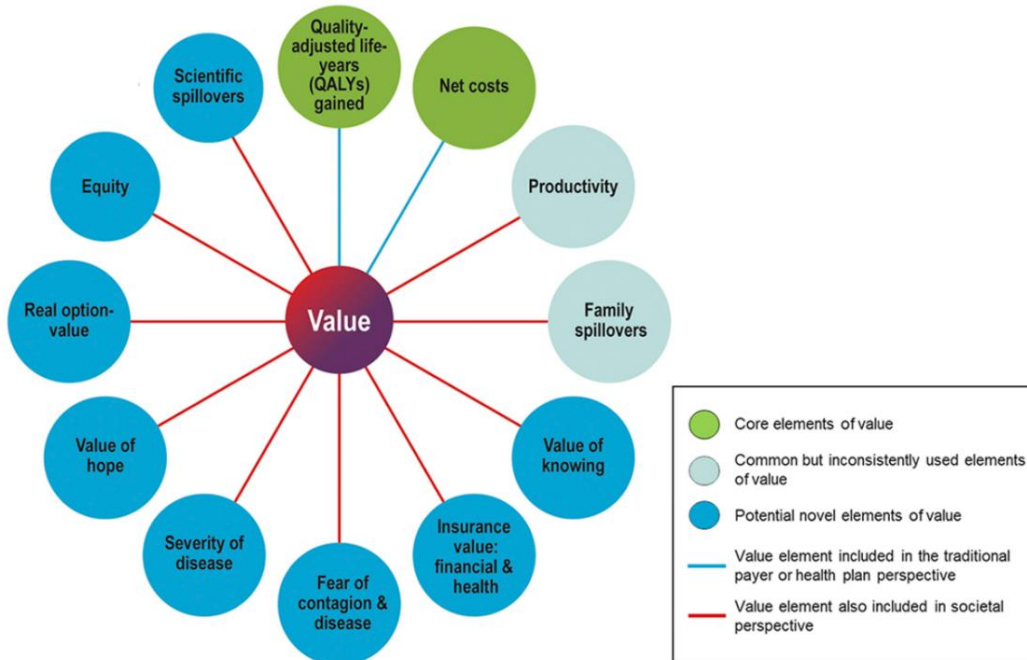
**FIGURE 3: EXPANDING PERSPECTIVES ON HEALTH AND WELLBEING (MOTT ET AL., 2021)**



### 3. Demonstrating novel value elements

Beyond the ‘conventional’ elements of clinical value discussed above, interventions may generate ‘novel’ elements of value that are meaningful to patients or society. The novel elements of value are depicted in a ‘value flower’ alongside more conventional (or ‘core’) elements of value in healthcare (Lakdawalla et al., 2018). This value flower is illustrated in Figure 4 below.

**FIGURE 4: THE ISPOR 'VALUE FLOWER' ILLUSTRATING ELEMENTS OF VALUE IN HEALTHCARE (LAKDAWALLA ET AL., 2018)**



These novel, and often less-tangible, elements of value can be as important, or even more important, to patients than conventional elements of value. Capturing the patient perspective on certain elements, such as real option value, severity of disease, value of hope, value of knowing and insurance value, could uncover additional value for a medicine that is not captured by looking at the direct health system perspective or societal perspective. For example, patients may assign *real option-value* to a sub-optimal treatment (e.g., a treatment that can extend life but at a poor HRQoL, or that slows disease progression without extension to life so that patients maintain their current functional status or quality-of-life for longer) if this treatment can bridge the gap between currently available options and some future innovation that could provide a ‘cure’ or more substantial benefit in terms of survival and quality-of-life. In this context, an ‘interim’ treatment may be valued disproportionately to its direct health effects.

Patients may also prefer treatments with a positively skewed outcome distribution (*value of hope*). For example, a patient who is closer to the end of their life may value a treatment with a high variability in outcomes, with the hope of experiencing a substantially better, albeit less probable, outcome, over a treatment with a similar expected outcome and less variability (Neumann, Garrison and Willke, 2022; Garrison, Kamal-Bahl and Towse, 2017). Empirical studies in oncology suggest a divergence between patients and clinicians on the value of hope, with clinicians expressing a preference for certainty over durability of outcomes (Shafrin et al., 2017; Hauber et al., 2020), therefore it is important to consider the patient as a separate stakeholder perspective from healthcare professionals.

Patients may also value the reduction in uncertainty, or additional information provided by an intervention (*value of knowing*), even if there is no immediate health impact (e.g., a



diagnostic test). Even if a treatment is not currently available, patients may value information that allows them to plan their remaining life-years (Neumann et al., 2012).

Finally, the patient perspective can extend to identifying unmet medical needs around diseases with no appropriate treatments, or with treatment that fail to address key aspect of patient concern or value, such as an unacceptable risk-benefit trade-offs or intolerable adverse effects (Zhang, Kumar and Skedgel, 2021). The availability of a treatment is only one aspect of unmet need, and the identification of other aspects of unmet medical need often relies on patient evidence to highlight sub-optimal aspects of an existing treatment.

## 3 What methods can be used to develop ‘patient evidence’?

There are many possible methods available for translating the patient perspective into ‘patient evidence’ that can be incorporated into HTA decision making. Which method is most appropriate will ultimately depend upon the research question(s) most relevant to the therapeutic area and the acceptability of the methods by HTA agencies.

Broadly, one can identify three forms of patient evidence:

1. Valuation of patient health-related quality-of-life (HRQOL), often through patient-reported outcome measures (PROMs)
2. Quantitative preference studies
3. Qualitative studies

Each form of evidence has specific strengths or advantages in demonstrating particular aspects of patient value.

- Generic measures of HRQoL and other PROMs tend to focus on relatively narrow aspects of the patient experience, particularly in terms of clinical outcomes, with little or no consideration of broader value elements or the process by which those outcomes are reached. The narrow scope of generic PROMs means they may not be sensitive enough to capture important changes in the patient’s condition.
- Condition-specific PROMs or HRQoL measures, such as the EORTC QLQ C30, may be more sensitive to aspects of a particular disease than generic measures such as EQ-5D, but most HTA bodies prefer generic measures to facilitate comparisons across different diseases on the same scale and tend to give less weight to disease-specific measures.
- Quantitative preference studies are effective at understanding the trade-offs or relative value of different aspects of treatment but may be less useful for understanding the holistic ‘experience’ of a disease.
- Finally, qualitative studies are particularly useful for understanding the holistic patient perspective but are less useful for quantifying the relative value of different aspects of treatment and outcomes.

Some examples of different methodological approaches for each form of evidence are shown below. This list is not exhaustive but intended to illustrate how different types of patient evidence are better at demonstrating different aspects of value. The relevant aspects of value and the best approach(es) for capturing this value should be considered as early as possible in any clinical trial or other evidence generation programme. We highlight which methods are most suited, or most commonly used, for demonstrating different aspects of value.



**TABLE 1: EXAMPLES OF STUDIES SUPPORTING KEY THEMES OF PATIENT EVIDENCE**

	Demonstrating value of clinical outcomes	Demonstrating value of alternative treatment processes	Assessing novel value elements
<b>Valuation of HRQoL</b>			
Direct time trade-off	✓		
Indirect EQ-5D	✓		
<b>Quantitative preferences studies</b>			
Discrete Choice Experiment	✓	✓	
Contingent valuation (willingness-to-pay)	✓	✓	✓
Threshold techniques	✓	✓	✓
Best-worst scaling	✓	✓	
<b>Qualitative studies</b>			
Qualitative Impact Statements	✓	✓	✓
Patient experience questionnaires		✓	✓
Interviews and focus groups	✓	✓	✓

## 1. Valuation of HRQoL

HRQoL methods such as direct time trade-off (TTO) valuation or, more commonly, indirect valuation via EQ-5D, are typical in most clinical trials. These HRQoL measures describe the ‘quality’ of a particular health state and are the foundation of the quality adjusted life-year (QALY).

In general, direct TTO elicitation, where respondents are directly asked how much survival they would be willing to trade-off for improved quality of life, are able to capture broader elements of value than indirect EQ-5D. Indirect EQ-5D asks patients to describe their health state in terms of different levels on the EQ-5D dimensions, and this ‘description’ is valued by a representative sample of the general population. It is an ‘indirect’ evaluation in the sense that the state is valued by someone other than the person in that state. Direct elicitation can be useful in demonstrating the value of outcomes that may be neglected by EQ-5D, or not well-understood by members of the public with no direct knowledge of the condition. However, the process of eliciting TTO values in specific indications is substantially more demanding than indirect valuations, and critically, direct patient elicitation are typically not accepted by HTA bodies such as NICE on the grounds that decisions over the allocation of public healthcare resources should be informed by (theoretically disinterested) public, not (potentially self-interested) patient values and preferences.

## 2. Quantitative preference studies

Whereas HRQoL methods seek to estimate a single value or utility for a specific health state, quantitative preference methods can explore the relative value of different aspects of treatment, or the acceptable trade-offs between positive and negative aspects of treatment. Such quantitative preference methods include discrete choice experiments (DCE), contingent valuation (CV) or willingness-to-pay (WTP) studies, threshold techniques, and best-worst scaling.

These methods typically present one or more hypothetical scenarios to participants and asks them to choose between them based on their preferences.

- DCEs ask respondents to choose between alternative scenarios defined on the basis of different levels of key treatment 'attributes'.
- CV/WTP studies present similar tasks, typically in a more descriptive form, and ask respondents to express how much they would be willing to pay to move from one scenario to the other. However, participants may not be familiar with the idea of paying directly for healthcare, either because they are in a publicly funded system or have health insurance that obscures the full costs. Therefore, participants may struggle to provide meaningful WTP estimates in the decision contexts likely to be considered as part of HTA deliberations. Such studies are typically more useful in contexts where patients may be used to paying for treatment in the form of co-payments or over-the-counter medicines, or where WTP is expressed in terms of an increase in taxes or insurance premiums.
- Threshold techniques are similar to DCEs but ask respondents to identify the critical value of some key treatment characteristic, such as risk of adverse effects, that would cause them to switch from one treatment to another. Such techniques are often used in understanding acceptable risk-benefit trade-offs.
- Best-worst scaling (BWS) methods are less common but are most useful when seeking to understand the relative importance of different aspects of treatment. In the most typical version of BWS, respondents are shown a hypothetical treatment scenario, similar to a DCE scenario, and asked to indicate which aspect of treatment is 'best' (e.g., a high probability of treatment success) and which is 'worst' (e.g. a high risk of adverse effects, or a long stay in hospital). The objective is to place all aspects of treatment on a common scale of "relative importance" so that they can be compared directly.

## 3. Qualitative surveys and impact statements

The methods above often rely on some form of qualitative research to understand the patient's holistic perspective on treatment. Structured or unstructured qualitative studies can identify the aspects of treatment that should be included in a quantitative study. Qualitative studies can also stand on their own, illustrating the patient's holistic "lived experience" in a way that may not be captured in more quantitative approaches. It is not always necessary to be able to quantify some aspect of a disease or its treatment to be able to understand that it is important to patients.

Structured, semi-structured or unstructured patient interviews can reveal unexpected values over different aspects of a condition or treatment but are time and resource intensive. Such approaches are typically intended to allow for the 'patient voice' to be heard, but because of their resource intensity, are not typically 'representative' of a particular population in the same way that more scalable quantitative approaches can be. Focus groups of 6-8 people may be seen as a less resource-intensive version of patient interviews. Moreover, one patient's comment may trigger reflection in other participants, producing information that may not have otherwise been considered by an individual patient. The reverse, however, may also be true, where participants converge on a "consensus" position that may not accurately reflect the individual experiences within the group. This is known as "consensus bias". (The Health Foundation, 2013)

Patient surveys exist somewhere in-between patient interviews and quantitative preference studies. They can be standardised enough to allow for large-scale sampling but, under some circumstances, can be flexible enough to reveal unexpected perspectives on the patient experience. Owing to their impersonal nature, though, most surveys are more useful for aggregating a wide range of patient experiences into summary statistics or figures. These can include open-ended questions with free text responses or closed-ended with multiple choice responses or Likert scales. Patient experience questionnaires can allow for standardisation of the questions, process, and method of analysis, and can be implemented relatively quickly and inexpensively with a larger sample of patients. However, the questionnaire format may mean that it is difficult to probe into responses and handle sensitive topics (The Health Foundation, 2013).

## 4 How do HTA bodies currently incorporate patient evidence and the patient voice?















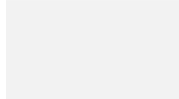
In general, we see what might be termed ‘more established’ HTA systems, such as the UK’s the National Institute for Health and Care Excellence (NICE), Germany’s Institute for Quality and Efficiency in Health Care (Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen, IQWiG), France’s Haute Autorité de santé (HAS), the Netherlands’ Zorginstituut (ZIN), Canada’s Agency for Drugs and Technology in Health (CADTH), and Sweden’s Dental and Pharmaceutical Benefits Agency (TLV), as moving towards more ‘patient-centric’ approaches. This includes broadening the forms of evidence they will consider as part of their assessments, formalising or even expanding the role of patients in the assessment process, and, in some cases, even including patients in making the final recommendation. Sweden’s TLV is notable amongst this group in adopting a flexible and pragmatic approach in its willingness to consider different forms of evidence, especially with respect to patient value (TLV, 2021). They recognise that in certain circumstances, a conventional QALY-based approach may not demonstrate the full value of a treatment and consequently, a flexible approach can lead to better HTA decisions and better value for patients and society.

What might be termed ‘less well-established’ systems, such as those in Italy’s Agenzia Italiana del Farmaco (AIFA) and Spain’s Agencia Española de Medicamentos y Productos Sanitarios (AEMPS), specify few, if any, opportunities for patients to participate in their guidance documents. Their methodological guidance generally has little to say about the role of patients in their assessment processes. Arguably, this reflects an emphasis on affordability and budget impact over ‘patient value’ in these systems.

The Institute of Clinical and Economic Review (ICER) in US differs from the bodies above in that it is a non-profit organisation funded by non-governmental actors and has no formal role in formulary decision-making. However, ICER does seek to inform value-based decision-making around health technologies, and there is evidence that their assessments are increasingly considered by payers making coverage or formulary decisions (Faraci et al., 2022). As such, we have included ICER alongside the more formal HTA bodies discussed above in our review of current HTA approaches to incorporating the patient voice.

Figure 5 summarises a brief review of different HTA bodies in terms of their acceptance of different forms of patient evidence and the participation of patient evidence in their decision-making. We highlight the source of HRQoL valuation used in conventional health economic evaluation, and the role of patient impact statements and quantitative preference studies, and patient participation in recommendations.

**FIGURE 5: SUMMARY OF PATIENT INVOLVEMENT IN HTA BY COUNTRY**

										
	France	Germany	Italy	Netherlands	Spain	Sweden	UK (England)	UK (Scotland)	Canada	USA <sup>1</sup>
Valuation of HRQoL <sup>2</sup> (preferred source of valuation)	Public	Patients	Preferred source not specified	Public	Preferred source not specified	Patients or public	Public	Public	Public	Public
Qualitative surveys or impact statements										
Quantitative preference studies (DCE)										
Quantitative preference studies (WTP)										
Quantitative preferences studies (Others)										
Patient participation in HTA decisions <sup>3</sup>	Patient advocates						Lay members	Public partner		
	Recommended in formal guidance		Some form of representation in formal guidance		No formal guidance but evidence in practice		Statement or evidence that activity is not considered		No identified evidence for or against activity	

<sup>1</sup> USA does not have a formal, national HTA body. We have included ICER guidelines to represent US practice.

<sup>2</sup> We distinguish between countries that recommend public valuation of health states and those that accept patient valuations.

<sup>3</sup> Cross-hatched cells indicate the involvement of public members or patient 'representatives' or 'advocates' but not necessarily patients themselves.

## Source of HRQoL valuations

Most of the HTA bodies we examined use preferences from the general public to value HRQoL, although Germany and Sweden are distinct in accepting patient valuations. Likewise, Germany and Sweden, as well as France, do not rely (or do not rely primarily) on 'cost per QALY' measures of value. In the Netherlands, patients are often consulted at the start of the evidence generation process to aid in the selection of patient-relevant outcomes such as quality of life measures, either individually or through patient organizations, (IQWiG, 2011; ZIN, 2023).

## Qualitative patient evidence

Roughly half of the HTA bodies we reviewed formally recognise a role for qualitative patient evidence in their assessments and there is evidence that the remaining bodies consider such evidence in practice. For example, HAS (France) has identified patient engagement as a priority in its 2019-2024 strategic plan (Haute Autorite de Sante, 2020). There is little evidence, though, to suggest that patient impact statements or other forms of qualitative evidence currently receive much weight in decision-making. A 2020 Canadian study found that among patient groups that had contributed to a CADTH HTA process, most were uncertain whether their submissions had any impact on the outcome of the process (Mercer et al., 2020). Perhaps consistent with this sense of uncertainty, and the limited capacity of patient organizations to develop qualitative submissions also identified in the Canadian study, a 2020 French study found that patient association input was included in only 20% of value dossiers submitted to HAS over the period 2017-2018 (Brogan et al., 2020). This may indicate a need for HTA bodies to provide greater support to patient associations if they genuinely value patient engagement.

The Scottish Medicines Consortium's (SMC) Patient and Clinician Engagement (PACE) process utilises patient evidence in situations where end-of-life or orphan medicines have received a negative preliminary decision. The PACE process allows the committee to consider (typically but not exclusively qualitative) evidence from patient groups and clinicians on the added benefit of the treatment to patients and their families that may not have been captured in the prior conventional assessment. A recent assessment of the PACE process showed that one-third of submissions that initially received a negative recommendation received a full or restricted approval on the strength of patient and clinician input with no price discounts or other patient access schemes (Barham, 2019). This finding suggests that patient input can have a meaningful marginal impact on HTA decisions.

CADTH (Canada) aims to include patients from the outset of the HTA process, consulting with patients regarding the specification of the decision problem to ensure the most meaningful outcomes are identified. In particular, CADTH's pan-Canadian Oncology Drug Review (pCODR) process, patient groups are invited to make submissions at the start of the review and feedback on initial drug recommendations, with 97% of pCODR reviews including patient input as of June 2019. However, as noted earlier, patients have suggested there are substantial resource challenges associated with submissions, and they are unclear about impact of these submissions on the final decision (Mercer et al., 2020).

## Quantitative patient evidence

Guidelines around consideration of quantitative patient evidence is even more mixed than qualitative evidence. In this context, we are referring to quantitative techniques such as DCE or WTP that allow for the statistical estimation of patient preferences and trade-offs, distinct from the descriptive statistics that might be generated from an opinion survey. A 2020 review of the use of patient preference data in European HTA found that whilst Sweden's TLV is open to considering willingness-to-pay (WTP) evidence, France's HAS formally rejects a role for such evidence in HTA (Marsh et al., 2020; HAS, 2020). Likewise, whilst Germany's IQWiG, along with Sweden's TLV, acknowledge a role of discrete choice experiments (DCE) in their guidelines, there is little evidence of IQWiG considering DCEs in practice (Marsh et al., 2020). NICE's (England) position is similarly ambiguous. NICE acknowledges a role for better use of quantitative patient preference, and suggests that such studies might offer valuable insights as part of the HTA process, but, at present, concludes that it "do[es] not see a role for the direct integration of patient preference data into economic models..." (Bouvy et al. (2020).) This ambiguity is echoed in research conducted with HTA representatives from Germany, Belgium and Canada. Participants were "interested" in using quantitative preference data, but they found it difficult to determine how such evidence should be considered alongside conventional clinical and economic evidence, or how much weight it should be given (van Overbeeke et al., 2019)

Quantitative patient evidence is more likely to be considered under specific circumstances. In Germany, for example, patient evidence can be critical when there are concerns around the safety or tolerability of a medicine or its comparator. Likewise, the Food and Drug Administration (FDA) in the US encourages both qualitative and quantitative patient evidence in judging the acceptability of risk-benefit trade-offs (Flythe and West, 2021; Health, 2020). While the FDA is not an HTA body, its judgements around the acceptability of risk-benefit trade-offs have implications for value assessment of health technologies around the world. ICER's Value Framework considers the "potential other benefits or disadvantages" of a new intervention to patients, caregivers and the public, which can be informed by quantitative and qualitative evidence (Institute for Clinical and Economic Review, 2020).

As noted, TLV (Sweden) explicitly encourages the use of different forms of quantitative patient preference information, including willingness-to-pay, ranking, rating, matching, and DCE, "to estimate the value of impacts on treatments more accurately than would be possible with the QALY, such as when valuing short-term acute pain." (Evidera, 2019; Marsh et al., 2020). In Italy, there is greater importance placed on patient information/preferences in rare or less common diseases by AIFA due to the relative lack of information about such diseases (Brogan et al., 2020). Likewise, ICER (US), as part of its Patient Engagement Program, states that it conducts surveys to elicit patient preferences for treatment and to inform model inputs when data is not adequately captured in the literature (ICER, 2020). The first manifestation of such a survey was in sickle cell disease, with the results informing health utilities and inputs related to the societal perspective model analysis. However, this approach has not been consistently implemented in other disease areas, even where patient input could have addressed gaps in available data (Xcenda, 2020).

## Patients as decision-makers

Beyond contributing data or preferences to the HTA process, patients have little direct role in most HTA decision-making. ZIN, in the Netherlands, gives patients a role in decisions, as does Sweden's TLV, but none of the other bodies give patients a formal vote in final decisions. NICE, in England and Wales, includes members of the public in their deliberations, but these are not specifically patient representatives. HAS, in France, gives patients voting rights at particular stages of appraisals but these votes are primarily consultative and do not play a role in final coverage decisions (Brogan et al., 2020; Sowell et al., 2018). Although Spain has undergone changes in its HTA processes, the role of patient organisations and scientific societies in HTA deliberations is limited to consultation only on the therapeutic position reports and not in the development of the reports (Pinilla-Dominguez and Pinilla-Dominguez, 2023).

The latest EU HTA regulations have emphasized the importance of including patient experts throughout the process, proposing that patients be consulted to define the parameters for assessment, comment on draft reports of clinical assessment, and provide input into scientific consultation outcome documents (Regulation (EU) 2021/2282 of the European Parliament and of the Council of 15 December 2021 on health technology assessment and amending Directive 2011/24/EU (Text with EEA relevance)). An earlier EUnetHTA guidance document on patient input in rapid evidence assessments highlights the specific areas for gathering evidence on the patient experience, including their disease, unmet needs, currently available treatments, new treatment expectations, quality of life issues and other outcomes that are relevant to the patient (EUnetHTA, 2019). In addition to informing the selection of outcomes for the rapid evidence assessment, the guidance highlights the benefits of improving the "relevance, legitimacy and transparency" of the assessment through collecting direct patient input. Like the national HTA bodies, though, EUnetHTA does not specify a decision-making role for patients.



## 5 How can the patient voice be incorporated in HTA?

To ensure that patient values are appropriately considered in HTA, we recommend the following:

1. **Developers, regulators and assessors**, in collaboration with researchers and scientists, should engage with patients early and throughout the process of technology development and evidence generation, including around defining the key trial outcomes and measures. Patients and their advocates should be proactive in contributing to these processes, but this is not to suggest that they are *responsible* for ensuring that the patient voice is heard.
2. **Developers** should collect and present patient evidence that complements clinical and economic evidence.
3. **HTA bodies** should commit to considering patient evidence on an equal basis alongside clinical and economic evidence, including with explicit guidance and, potentially, by adapting their evidence paradigm to facilitate this consideration.
4. **HTA bodies** should give patients a direct voice in HTA recommendations.

### 1. Engage with patients early and throughout the process of drug development and evidence generation

Including the patient voice in the development and assessment of new medicines and health technologies requires that patients (or their advocates) *engage* and *are engaged* as early as possible in the development and evidence generation processes. Processes should explicitly incorporate the patient voice, but equally, patients and their advocates should be proactive in contributing to these processes. This is not to say, however, that patients and their advocates are *responsible* for ensuring their voice is heard.

These contributions include patient input into *which* products are developed, not just how these products are valued. At a minimum, developers should embed patient evidence into the design of their evidence generation programmes from the start, alongside clinical and economic evidence. Engagement with patients throughout development and HTA processes should be done in a structured and consistent way.

Evidence should demonstrate how a new treatment addresses unmet needs identified by patients. In France, HAS recommends that patient evidence should provide information about *the impact of the drug*. HAS is less interested in evidence of the burden of a disease without a clear demonstration of how this burden would be reduced with a particular treatment (HAS, 2020). This demonstrates the importance of engaging with patients early, so they can assist in identifying the most relevant and impactful outcomes and measures.

## 2. Collect and present patient evidence that complements clinical and economic evidence

The most appropriate source of patient evidence will depend on the particular characteristics and context of the decision-problem. Patient evidence should be used to *complement* aspects of value that may not be (well-)addressed or demonstrated by conventional clinical and economic evidence. As noted earlier, this can include evidence that demonstrates the impact of outcomes that might otherwise be seen, from a clinical perspective, as less relevant to a patient's day-to-day experience with a disease or treatment.

Complementary patient evidence allows HTA bodies to understand the holistic value of a new medicine or technology. This can contextualise clinical and economic evidence and, in some cases, may help to resolve decision uncertainty. This will present a challenge, though, to evidence generators as well as assessors. By its nature, patient evidence, especially around patient values, illustrates trade-offs and relative priorities that are context-dependent and more heterogeneous than most clinical or economic outcomes. Researchers must continue to refine their methods to provide accurate and reliable information, whilst HTA bodies must be willing to incorporate evidence that does not align with their typical expectations for clinical and economic evidence. Both aspects could be improved by collaborative discussions between patient, developer, researcher, and HTA stakeholders.

We encourage the opportunity for patients to make direct submissions to the HTA process, although the presentation of patient evidence may often be in the form of a patient value dossier prepared and submitted by the manufacturer. In either case, patient evidence should be presented in a succinct manner that complements and contextualises the clinical and economic evidence.

## 3. HTA bodies should commit to routine consideration of patient evidence alongside clinical and economic evidence

We argue that accounting for the patient voice, and consideration of patient evidence, can improve the quantity and quality of evidence, improving HTA *decisions*, and accounting for patient values in HTA decision-making can signal to innovators regarding what patients and health systems value, improving the *efficiency* of research and development (R&D), the *value* of technologies available to healthcare systems, and ultimately, the *well-being* of society. Furthermore, committing to routine consideration of patient evidence as part of HTA deliberations will encourage developers to improve the scope and quality of their patient evidence. This can include going beyond conventional measures of HRQoL and accepting PRO/PREs that better capture outcomes and experiences of value to patients.

By failing to fully account for patient values and preferences, HTA bodies risk misallocating resources away from interventions that are of the highest value. The impact of omitting – and then reconsidering – the patient voice was clearly demonstrated in Sweden, where increased consideration of the patient voice led TLV to consider additional endpoints in economic models and subsequently change their HTA decision in five oncology cases between 2018 to 2020 alone, including acute lymphocytic leukaemia, breast cancer, non-small cell lung cancer, lymphoma and CAR-T (Brogan et al., 2020). The substantial impact

of involving the patient perspective within one disease area suggests routine recognition will identify further treatments for which decisions can be better informed and subsequently improved.

#### 4. Patients should be given a direct role in HTA decisions

Finally, HTA bodies should give persons with direct experience of the relevant condition a more direct voice to in decision-making. This means going beyond a purely consultative roles with little direct influence. Allowing patients to contribute evidence but excluding them from decision-making can lead to resentment and mistrust of the decision-making process. Giving patients token responsibilities is little better, as they will likely come to perceive their role as 'window dressing', again damaging trust and legitimacy. On the other hand, genuine patient involvement in the HTA process can also open the 'black box' and improve the transparency, credibility, and ultimately, the legitimacy of HTA.

## 6 Conclusion

We have explained how the patient voice can ensure that HTA *decisions* are based on the fullest and most relevant understanding of value, as well as ensuring the legitimacy and ongoing public support for the HTA *process*. However, whilst many HTA body guidelines acknowledge a role for patient evidence and patient involvement in decision-making, the practical impact of such aspirations remains limited. At the very least, the *perception* is that patient evidence plays a limited role in HTA decision making, and patient involvement on committees and advisory boards is often largely symbolic.

The patient voice matters in HTA decision-making because it ensures that healthcare assessments and decisions are aligned with patient-centred principles, reflect real-world experiences, and consider the values and preferences of the individuals who are most affected by those decisions. It ultimately leads to more informed, ethical, and patient-centric healthcare choices and should therefore play a pivotal role within HTA processes.

We have described different methods for collecting evidence around patient values and preferences, and provided recommendations for how this evidence can be more routinely considered as part of HTA decision-making, including by giving patients a role in making decisions about their treatments. We hope that these recommendations can help make the ambition of greater patient-centricity in HTA a reality.

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