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Is it time we started asking manufacturers to be bolder when incorporating patient insights as part of HTA submissions?

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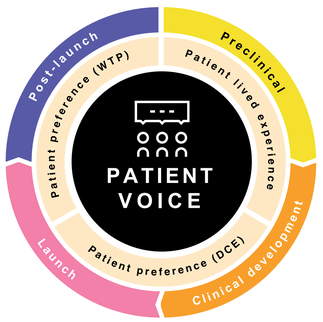
Introduction

'Patient centricity', 'patient engagement', and 'the patient voice' are terms that are used often and interchangeably and, as an industry, the value patients add throughout the product life cycle is not disputed. It is widely documented that understanding the patient experience when making healthcare assessments leads to more informed decision-making.[1] However, is it time to be more ambitious to ensure that the patient voice is truly central to any submission?

Figure 1: Patient voice: throughout the product life cycle



Methods

A targeted gray literature search explored how HTA organizations in England, France, and Germany incorporate the patient voice into their submissions. HTA websites were searched assessing their policies and processes for patient engagement as well as publications relating to general patient engagement and HTA. A selection of manufacturer submissions were explored to evaluate the types of evidence submitted.

Patient perspectives can be translated into patient evidence using various methodologies;[1] this evidence is then used as part of a HTA submission (for example: patient health-related quality-of-life [HRQoL], often through patient-reported outcome measures [PROMS], quantitative preference studies, and patient qualitative studies).

Results

All HTA organizations reviewed solicit input from patients; in the case of HAS (France), representatives sit on the committee and convey information collected via an online portal; for Germany, patients or their representatives contribute via the patient involvement office, which feeds into the IQWiG process; and at NICE (England), patient experts sit on the NICE committees. Some HTA submissions include patient preference studies, qualitative data from patients, and real-world evidence from patient registries; however, this is not mandatory. Guidance and tools are available on how to engage patients in the HTA process, but this is broad.

The NICE methods guide does not specifically cite patient preference studies but it does state that they may be deemed relevant and accepted alongside main evidence sources when the need is justified; it also invites written submissions from all patient and carer organizations involved in the evaluation to provide perspectives on experiences and preferences.

In 2020, NICE shared their opinion on the use of patient preference studies within HTA,[1] stating that these studies may provide valuable insights to a NICE committee into the preferences patients have for different treatment options, particularly if the study sample is representative of the wider population.[2]

From 2016-2018, NICE and Myeloma UK collaborated to further understand how organizations such as NICE can use insights from patients in their decision-making:[3]

1. They looked at current literature and research activities relating to patient preferences
2. A survey was completed by 97 myeloma patients and focus groups were held with patients and family members to understand more about what aspects of the patient experience should be considered by researchers and NICE
3. A workshop was held with researchers, healthcare professionals, policymakers, and other charities to discuss the findings.

The research suggested that discrete choice experiments could be extremely helpful to NICE in certain situations when several very different treatment options for the same condition exist (eg, a choice between taking a drug or a surgery, or between taking a pill daily versus receiving an injection monthly). This project suggested that there is clear scope for better use of quantitative patient preference studies. Table 1 provides a summary of patient involvement in HTA in England, France, and Germany.

Table 1: Summary of patient involvement in HTA by country*

Valuation of HRQoL (preferred source of valuation)	Public	Public	Patients
Qualitative surveys or impact statements			
Quantitative preference studies (DCE)			
Quantitative preference studies (WTP)			
Quantitative preference studies (Other)			
Patient participation in HTA decisions	Lay members	Patient advocates	

Recommended in formal guidance; No formal guidance but evidence in practice; Statement or evidence that is not activity considered; No identified evidence for or against activity; Some form of representation in formal guidance

*Adapted from: Kumar G, et al. https://www.ohe.org/publications/patient-voice-in-HTA; 2024 [accessed April 25, 2024]

HTA examples:

Somatrogon was recommended in 2023 – within its marketing authorization – as an option for treating growth disturbance caused by growth hormone deficiency in children and young people aged ≥3 years.[4] Somatrogon was previously recommended by NICE in the same indication, with the latest appraisal relating to a weekly injection rather than daily.

The manufacturer included data from a discrete choice experiment demonstrating that patients and caregivers prefer a less frequent injection regimen for treatment of growth hormone deficiency as part of the disease burden and unmet need section of the submission.

The study was not specifically discussed in the committee papers or final appraisals determination (FAD).

Upadacitinib is recommended as an option for treating active non-radiographic axial spondyloarthritis with objective signs of inflammation (shown by elevated C-reactive protein or MRI) that is not controlled well enough with non-steroidal anti-inflammatory drugs in adults.[5]

The EAG report that a survey was provided by the company which highlights the administration route is the third most important consideration (after symptom improvement and cost) when selecting treatment; it has been reported that 49.9% (198/397) of patients with axial spondyloarthritis prefer an oral treatment.

Eptinezumab is recommended as an option for preventing migraine in adults:[6]

- The manufacturer submitted a US study which used a discrete choice method to determine preference in onset of action and treating setting in advanced migraine prevention
- Patient preference towards a lower administration frequency was also highlighted by the manufacturer; the study used to gather these data was not specified
- The patient preference studies were not discussed in the FAD or by the EAG.

The treatments assessed by NICE were also assessed by the G-BA and HAS. The patient preference studies outlined by NICE were not detailed in the G-BA justification documents. Regarding current patient involvement, for most early drug assessments (according to the German AMNOG law), information on relevant outcomes and existence of patient subgroups is gained through a questionnaire that is completed by patient organizations. For non-drugs, patients are invited to IQWiG for a face-to-face discussion to identify important outcomes.[7-10]

For the somatrogon submission,[11] French and US patient preference studies were submitted by the manufacturer – HAS stated that the studies were not detailed, and not generalizable to the French population.

For both eptinezumab and upadacitinib,[12,13] patient preference studies were not mentioned.

Conclusions

HTA bodies and regulators do not specify detailed study design and evidence requirements for HTA submissions for patient involvement, but should more implicit guidance be provided by HTA bodies for technology manufacturers? To avoid patient studies being rejected due to lack of quality, generalizability, etc., should manufacturers be asked to provide more detailed evidence describing the patient voice in their HTA submissions (for example, including patient preference studies and clinical trial exit interviews to their evidence submissions to complement the patient representation at the HTA committee)?

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