

From Participation to Partnership: Advancing Patient-Centric, Inclusive and Equitable Evidence Generation in HEOR

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Introduction

- Patients are increasingly interested in and calling for a more proactive role as partners with health technology developers. Understanding the burden and impact of disease as well as diagnosis and treatment pathways for patients is of paramount importance for developing health technologies.¹
- Engaging patients in market access and health economics and outcomes research (HEOR) activities can help to ensure that findings are both relevant to patients, and of utility to healthcare decision makers.²⁻⁴
- Health Technology Assessment (HTA) bodies such as the National Institute for Health and Care Excellence (NICE) and Canada's Drug Agency (CDA) weave patient and public engagement throughout their appraisal and assessment processes, reflecting a shift towards shared decision making.⁵⁻⁸
- Despite the recognised benefits, practical frameworks and solutions for meaningful patient and public engagement in HEOR remain underdeveloped and under recognised.⁹
- Patient-centric approaches and the patient voice can only be integrated in market access and HEOR if multi-stakeholder literacy and capacity is built.
- HTA bodies, payers, and researchers need to be aware of approaches and methodologies and be enabled to utilise them.
 - Furthermore, patients and members of the public need to feel empowered to be involved and trained to enable effective contribution.
- We reviewed existing frameworks advocating for the inclusion of patients in both research and planning, and precedent methodologies enabling patients to participate in HEOR based studies and aimed to generate an illustrative roadmap consolidating appropriate approaches to support advancement of knowledge of patient-centric, inclusive, and equitable evidence generation practices in market access and HEOR.

Methods

- A targeted evidence review was conducted to identify key published frameworks and established methodologies being used to capture the patient voice during evidence generation.
- Our review sought to capture frameworks and established methodologies that capture the patient voice either by informing research design or via research participation.
 - We identified evidence from the United Kingdom (UK), EU4 (France, Germany, Italy and Spain), Canada and the United States (US) published between January 2010 – October 2025.
- Following the evidence review, the frameworks and methodologies were collated into a roadmap highlighting the plausibility of patient-centric evidence generation throughout the product lifecycle.

Results

Our exploratory evidence review identified 18 key frameworks and 11 commonly used established qualitative methodologies that can generate patient-centric evidence in HEOR.

Published frameworks and guidance

- In initial searches, we identified broad frameworks that were not specific to types of evidence generation and decision-making activities but outlined principles for the incorporation of the public and patient voice in health research¹⁰
- However, we highlight a narrower range of 18 published frameworks and guidance that promote the incorporation of the public and patient voice within a range of activities most relevant to market access and HEOR. The commonalities between these frameworks illustrate key components that support public and patient engagement irrespective of the activity type.
- Future frameworks for evidence generation and decision-making activities would add to those identified if:
 - tailored to specific populations
 - they promote approaches for accessibility
 - they outline appropriate ethical standards
 - they promote consideration of equity and inclusivity of an approach.

Figure 1. Overview of frameworks identified enabling patient involvement in evidence generation to decision making activities

Categories of evidence generation	Priority setting	Patient-centred outcomes research	Clinical trials	Evidence syntheses	Health technology assessment
Examples	A. James Lind Alliance Priority Setting Partnership (PSP) Handbook ¹¹ B. Pratt, initial framework to construct power and dynamic balanced citizen engagement in priority-setting ¹² C. Pollock, New Model to Engage Patients and Clinicians in Setting Research Priorities ¹³	D. Consensus-based Standards for the Selection of health Measurement Instruments (COSMIN) ¹⁴ E. Camello Castillo, A framework for culturally sensitive approaches in patient centred outcome research ¹⁵ F. Kwon, How to translate common themes from community based participatory research into effective patient centred outcomes research ¹⁶ G. Wilson, A framework adapted from the Food and Drug Administration's roadmap for patient-focused clinical outcome assessment ¹⁷	H: Core Outcome Measures in Effectiveness Trials (COMET) Handbook ¹⁸ I: EUPATI: Guide for patient involvement in ethical trials of clinical trials ¹⁹ J: Patients active in research and dialogues for an improved generation of medicines (PARADIGM) ²⁰ K: Evans, A framework for involving service users in trials L: NIHR, Good practice guidelines on the recruitment and involvement of public members on trial and study steering committees ²¹	M: Johnson, Rapid Involvement of Patients and the Public in Evidence Synthesis (RIPPLES) ²² N: Pollock, ACTIVE ¹⁹ O: Abelson, A framework for action P: EUPATI Guidance for Patient Involvement in Medicines Research and Development ²³ Q: EUnetHTA D7.2 guidance ²⁴	
Overview of purpose:	A: Step by step guide to the process involved in a PSP with the aim of bringing patients, carers and clinicians together to identify uncertainties or unanswered questions for specific health issues B: Guidance on addressing ethical considerations and dimensions of power during priority-setting processes to accurately and equitably reflect patients' health needs C: To facilitate involvement through targeted engagement and assisted involvement to gather research priorities from people affected by stroke	H: Outlines how to develop core outcome sets and engage patients within this process I: Practical recommendations for ground rules and options for involving patients in the work of ethics committees and in the overall clinical trial process from concept development to trial result reporting in lay summaries J: A sustainable framework for meaningful, structured, and effective patient engagement (PE) across the entire medicines research and development (R&D) lifecycle – particularly where patient input can have the greatest impact K: Guidance to help researchers involve service users successfully in developing and conducting clinical trials and creating a culture of active involvement at all stages L: Guidance including definitions of public members roles in research oversight groups and good practice for recruitment and involvement of public members	M: A framework and toolkit to help researchers carrying out rapid evidence synthesis to embed patient and public involvement in their work N: A framework to enhance the relevance, quality, and applicability of systematic reviews by incorporating perspectives from various stakeholders, including patients, healthcare professionals, and the public	O: A comprehensive framework to involve patients and the public in the government's HTA process P: Guidance on patient involvement in industry-led medicines research and development covering the interaction between patients and the pharmaceutical industry within all functions throughout the medicines R&D lifecycle in relation to medicines for human use Q: A framework for involving patients and the European Union (EU)'s Joint Scientific Consultations (JSC) and Joint Clinical Assessments (JCA)	R: An overview of the values and standards for patient involvement in action at Canada's Drug Agency

Established methods commonly used to capture patient insights

- Following our evidence review, commonly used methods to capture the patient voice within evidence generation in HEOR and considerations for their use have been highlighted in Table 1.
- Our review highlighted a range of methodologies including:
 - Qualitative research methods such as interviews and focus groups.²⁵
 - Mixed methods research including triangulation of findings.²⁶
 - Preference and valuation-based elicitation techniques, such as Discrete Choice Experiments (DCE), best - worst scaling, patient preference studies, or swing interviews) and utility elicitation via the use of Time Trade-Off analyses, Standard Gamble, or Visual Analogue Scales.²⁷⁻²⁹
 - Consensus based approaches, such as traditional Delphi panels, modified Delphi panels and nominal group techniques and Structured Expert Elicitation.^{30,31}
- Across a range of methodologies, the patient voice was often depicted through the participation of a range of stakeholder types. These included:
 - Caregivers, such as family members, partners, or friends who provide day-to-day care and support to the patient, particularly in paediatric, geriatric, or cognitively impaired populations.^{32,33,34}
 - Patient advocates and advocacy organizations, such as representatives from or leaders of patient support groups, charities, or advocacy groups.^{35,36}
 - Legal guardians, often used on behalf of patients who cannot consent for themselves (e.g., minors, incapacitated adults).^{37,38}
 - Alied health professions or healthcare professionals with close patient relationships, such as nurses, social workers, physiotherapists, or occupational therapists.³⁹
- Across all methodologies and research including the above range of stakeholder types, the importance of transparent communication, respect for participant well-being, support for accessibility and autonomy, and sensitivity to the emotional and cognitive demands of participation were highlighted as key considerations to ensure ethical patient engagement from which meaningful outcomes can be derived.

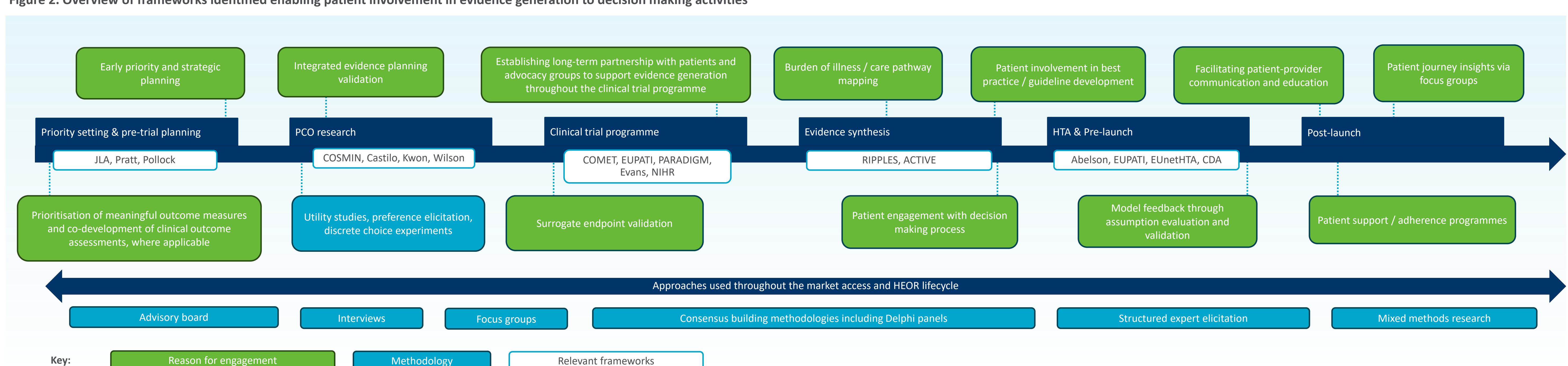
Table 1. Approaches for capturing the patient voice in HEOR with key considerations

Method type	Key considerations during planning and implementation
Advisory Boards	It is important to ensure diversity within the group, provide clear roles for each member, support ongoing engagement, and avoid surface level involvement by empowering patients to contribute meaningfully.
Focus Groups	Focus groups should be inclusive and foster open discussion. Consider group dynamics and make arrangements for accessibility so that all participants can share their views comfortably.
Interviews	Interviews allow for in-depth insights and should accommodate privacy and confidentiality. The approach should be tailored to the specific needs of each participant.
Delphi Panels	Delphi panels benefit from iterative rounds of input, clear materials, and opportunities for participants to provide feedback throughout the consensus-building process.
Nominal group technique	When involving patients in nominal group techniques, it is critical to prioritise process that promote equality across all participants, and to foster a supportive environment to obtain meaningful, clear, concise and representative input.
Structured Expert Elicitation	When involving patients as experts, it is essential to define the criteria for "expertise," provide sufficient background information, and take steps to minimize bias in responses.
Patient Preference Studies	These studies should use accessible formats and strive for representativeness. For example, selecting appropriate elicitation methods (such as discrete choice experiments, best-worst scaling, or interviews) is imperative for these types of activities to ensure the approach matches the cognitive, linguistic, and cultural attributes of the patients.
Co-design Workshops	Co-design workshops work best when collaboration is fostered, participants are equipped with relevant background knowledge, and group size is managed to enable effective engagement.
Discrete Choice Experiments	Choices should be simplified and pre-tested for understanding, with particular attention to health literacy to ensure meaningful participation.
Utility studies	Health state vignettes or descriptions must be understandable, comprehensive, and relevant to patients' experiences. Involving patients or caregivers in the development or validation of these descriptions to ensure content validity.
Mixed Methods Research	Mixed methods approaches should integrate both qualitative and quantitative input, and the methodology should be clearly explained to all participants.

Roadmap of approaches

- Combining insights from both the frameworks and methodologies identified, we developed an illustrative roadmap that can be used to identify ways and means to capture the patient voice, including early and sustained patient engagement across the product lifecycle (Figure 2).
- The appropriate approach to capturing the patient voice and involving patients as active participants in HEOR depends upon the objective and if patient perspectives are to inform study design, endpoint selection, model assumptions, or evidence interpretation.

Figure 2. Overview of frameworks identified enabling patient involvement in evidence generation to decision making activities



Stages and approaches can be interchangeable but have been overlaid for illustrative purposes

Conclusions

- Insights from our review reiterated that capturing the patient voice and involving patients as active participants in HEOR is crucial for generating evidence that truly reflects real-world experiences and outcomes that matter most to those affected.
- Published frameworks were identified that promote the incorporation of the public and patient voice in HEOR. However, frameworks were not specific to types of evidence generation and decision-making activities. Future frameworks should provide clear, practical guidance on how to involve patients in specific HEOR activities, including how to recruit and engage different groups and consider equity and inclusivity.
- In addition to frameworks that enhance patient involvement in research planning and design, our review identified a range of methodologies that can be utilised for evidence generation in HEOR to enhance the patient voice in the evidence base for novel therapies.
 - Patient and public involvement in research planning and design helps to ensure that research questions reflect real patients' concerns and addresses matters of importance to them.
 - Involvement in research planning also supports patient recruitment to and engagement with HEOR, which further enriches evidence generation, leading to more impactful and actionable insights.
- Patient and public involvement can highlight outcomes that truly affect individuals moving beyond clinical markers of effect and ensuring incorporation of outcomes, preferences, and priorities that ensure research is meaningful and relevant to patients. It can also ensure findings are interpreted in an appropriate language and within a meaningful context for non-clinical audiences. It can also improve language of materials making them accessible to wider communities.
- Our illustrative roadmap provides an overview of use cases whereby patient involvement could be considered across the product lifecycle, be that as participants or research planning and design partners. This roadmap aims to provide drug developers with an outline on where and how to incorporate the patient voice in HEOR and market access, including frameworks to use at each stage.
- By leveraging existing frameworks and established methodologies, we can move toward a more inclusive and credible paradigm ensuring that HEOR evidence in decision-making reflects the needs and values of all stakeholders.

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