Critical appraisal of patient engagement frameworks in Health Economics and Outcomes Research (HEOR) and Health Technology Assessment (HTA)

Stone M [1], Bridge D [1], Heron L [1], Riley D [1], Pearson F [1]
[1]Adelphi Values PROVE, Bollington, Cheshire SK10 5JB, United Kingdom

ADELPHI VALUES | PROVE Expertise in Access and Value Evidence Outcomes

PCR157

Background and Objectives

- Understanding the burden and impact of disease as well as patient diagnosis and treatment pathways is fundamental to the development of health technologies which have meaningful impact for patients.[1]
- Patient and public involvement/engagement in health economics and outcomes research (HEOR) and Health Technology Assessment (HTA) has been shown to enhance the relevance and utility of evidence for both patients and decision-makers.[2-4]
- Major Health Technology Assessment (HTA) bodies such as the National Institute for Health and Care Excellence (NICE) and Canada's Drug Agency (CDA) have embedded patient and public involvement/engagement throughout their appraisal and assessment process, reflecting a shift towards shared decision making.[5-8]
- Despite these advances, practical frameworks and solutions enabling meaningful patient and public involvement/engagement in HEOR and HTA remain under-recognised and under used.[9] As a first step in combating this knowledge gap we previously undertook an evidence review of published approaches to patient involvement/engagement in HEOR and HTA to identify existing frameworks, guidance and commonly used methodologies producing an illustrative roadmap.[10]
- Pharmaceutical companies are under increasing pressure from regulators, HTA bodies, and patient advocacy groups to demonstrate early, meaningful, and sustained patient involvement/engagement throughout the product lifecycle. Beyond knowing what involvement/engagement approaches exist, they need to be able to differentiate between those which are rigorous, evidence based and applicable to HEOR or HTA-specific contexts avoiding those that are tokenistic, ad-hoc or simply perpetuate use of the status quo.
- Therefore, the objective of this work was to critically appraise the frameworks and guidance for patient involvement/engagement in HEOR and HTA that we had previously identified.

Methods

- A targeted evidence review identified key published frameworks being used to capture the patient voice during evidence generation, either via informing research design or via research participation.
- The review spanned publications from the United Kingdom (UK), EU4 (France, Germany, Italy and Spain), Canada and the United States (US) covering the period between January 2010 to October 2025.
- Ten of the frameworks or guidance for patient involvement/engagement identified across three areas of application (priority setting [n=3], evidence synthesis [n=2], and HTA [n=5]) were critically appraised systematically by two researchers using relevant domains of the Appraisal of Guidelines for REsearch & Evaluation (AGREE) II instrument. AGREE II is a validated and widely recognised tool for assessing the quality and rigour of guidance documents.
- Five of the six AGREE II domains were deemed relevant to this type of guidance and so questions from these applicable domains were used to assess: 1) scope and purpose, 2) stakeholder involvement in development, 3) rigor of development, 4) clarity of presentation and 5) applicability to the HEOR and HTA context. The questions from the domain on editorial independence were omitted.
- Individually assigned AGREEII Likert scale scores were assigned against each question and then agreed between two reviewers.
- The assessment of each framework or guidance document was then visualised using a heat map according to these scores, enabling a transparent comparison of quality and suitability for informing patient involvement/engagement in HEOR and HTA practice.

Key	
Shading	Reviewer assigned AGREEII rating
	6-7
	3-5
	1-2

Table 1. Visual overview of AGREE II appraisals conducted

			Domain	Scope and purpose		Stakeholder involvement		Rigour of development				Clarity of presentation		Applicability			
			Concept	The overall objective(s) of the guideline is (are) specifically described.	The population to whom the engagement guideline is meant to apply is specifically described.	The views and preferences of the target users and target population for engagement have been sought during development.	Intended users of the framework/ guidance are clearly defined.	The methods for formulating the framework are clearly described.	Systematic methods were used to search for evidence to inform development.	The criteria for selecting framework components are clearly described.	The guideline has been externally reviewed/ tested by experts.	The approaches are specific and unambiguous.	Different options for engagement are clearly presented.	The guideline provides advice and/or tools on how the methods can be put into practice.	The guideline describes facilitators and barriers to its application.	The potential resource implications of applying the recommendations have been considered.	The guideline presents monitoring and/ or auditing criteria for when used.
Activity	Priority Setting	Framework	JLA[11]														
			PEM[12]														
			PM[13]														
	Evidence Synthesis		Active[15]														
			RIPPLES[14]														
	HTA		Abelson[17]														
			EUPATI[16]														
			CDA[6]														
			EUnetHTA[6]														
			NICE[5]														

Abbreviations: CDA, Canada's drug agency; EUnetHTA, European network of HTA; EUPATI, European Patients' Academy on Therapeutic Innovation; HTA, Health Technology Assessment; JLA, James Lind Alliance; NICE, National Institute for Health and Care Excellence; PEM, Prat's Engagement Model; PM, Pollock's Model; RIPPLES, Rapid Involvement of Patients and the PubLic in Rapid Evidence Synthesis

Results

- Priority setting frameworks or guidance, such as the James Lind Alliance (JLA) Handbook, Pratt's engagement model, and Pollock's engagement model performed strongly on scope, purpose and stakeholder involvement.[11-13]
- All three frameworks clearly articulate their objectives and meaningfully embed patient and public involvement/engagement at their core. This is likely in part a reflection of their foundational design around inclusive, patient-centred processes.
- However, heterogeneity in clarity of presentation was evident. While the JLA Handbook offers structured, accessible guidance, Pratt's and Pollock's models are less extensively documented, which may limit their practical uptake by researchers and sponsors unfamiliar with involvement/engagement methodology.[11-13]
- Evidence synthesis frameworks, RIPPLES and ACTIVE, demonstrated strong performance across the majority of domains, scoring highly for scope and purpose, stakeholder involvement, rigour of development, and clarity of presentation.[14,15]
- However, both frameworks scored lower on applicability, suggesting that whilst methodologically robust, during translation into real-world HEOR and HTA practice there is likely a need for additional contextualisation and support for implementation across research settings and geographies.[14, 15]
- HTA frameworks from Abelson, EUPATI, EUnetHTA, CDA, and NICE provided the most comprehensive coverage of patient involvement/engagement within formal health technology assessment processes, with clear articulation of scope and purpose.[5-7, 16] Nevertheless, a key limitation across this group was inconsistent description and transparency around how the guidance itself was developed. This made it hard to understand what evidence underpins some of the recommendations the guidance makes.
- Across all ten frameworks, the quality of implementation guidance and accessibility of content for patients, researchers, and industry sponsors was highly variable. In addition, limited detail was given on resource implications of proposed approaches.
- Most of the frameworks are primarily oriented toward academic or policy audiences, with limited adaptation for patient or sponsor use.
- Critically, across all frameworks, resource implications of the proposed involvement/engagement approaches were inadequately addressed, with minimal detail provided on the time, cost, or infrastructure required to operationalise recommendations. This creates a significant knowledge gap for pharmaceutical companies and research teams seeking to adopt these approaches in practice.

Conclusions

- We have used a reproducible and methodologically robust approach to identify and appraise frameworks to guide future patient-centric evidence generation.
- If selected and applied appropriately all frameworks appraised do provide a defensible, systematic foundation from which to build a patient and public involvement/engagement strategy fit for purpose and aligned with HEOR and HTA expectations.
- While the frameworks appraised outlined valuable approaches to patient and public involvement/engagement, variation existed in their methodological rigor, accessibility and quality of allied implementation guidance. There is also a key difference between the applicability of the frameworks appraised, some are focussed on institutional policy and governance structures for involving patients and the public in health technology assessment processes, whereas some are methodological frameworks designed for involving stakeholders in specific project types or relevance to HEOR or HTA.
- This heterogeneity across assessed domains presents a practical challenge to companies seeking to embed patient-centric approaches within their HEOR and HTA practices. This work shows clearly to potential users where such variation exists and highlights the likely need for translation and implementation support when using methodological frameworks in different HEOR and HTA practice settings.[14, 15]
- Future frameworks and guidance should transparently report development processes and integrate approaches for the evaluation of their use and their impact on evidence generation for HEOR or HTA. This will enable continual evidence-based improvement in the frameworks and in patient-centric research practice.
- Using any of these frameworks during evidence generation would ensure that collection of patient-centric evidence is prospectively planned and methodologically grounded. Used in conjunction with one another, they can facilitate generated evidence packages to more holistically reflect the lived patient experience and voice.
- Patient and public involvement/engagement can strengthen value propositions to regulators, HTA bodies, and payers alike and demonstrate a genuine commitment to patient-centred medicine.
- Across our work, two important gaps have become apparent: a persistent knowledge gap regarding the practical application and comparative value of existing frameworks, and an implementation gap relating to their inconsistent and unsystematic use within HEOR and HTA research. Although multiple frameworks are available, there remains limited clarity on how they should be operationalised across different stages of evidence generation, appraisal, and stakeholder engagement. Addressing these will require more than increased awareness. A meaningful step change in current practice is likely to depend on the development of clear, practical guidance formally endorsed by HTA bodies, alongside stronger expectations for consistent implementation within HEOR and HTA processes.

References
1. Fredriksson M, Sampaio F, Moberg L. The impact of patient and public involvement in healthcare services: A conceptual review spanning social sciences and health sciences. SSM - Qualitative Research in Health. 2025;7:100517.
2. Duffy J, Cairns AE, Richards-Doran D, van 't Hooft J, Gale C, Brown M, et al. A core outcome set for pre-eclampsia research: an international consensus development study. BJOG. 2020;127(12):1516-26.
3. Thomson KH, O'Connor N, Dangova KT, Gill S, Jackson S, Bliss DZ, et al. Rapid priority setting exercise on faecal incontinence for Cochrane Incontinence. BMJ Open Gastroenterol. 2022;9(1).
4. ISPOR. Patient Engagement in HEOR 2024 [Available from: https://www.ispor.org/strategic-initiatives/patient-initiatives.]
5. NICE. Patient and public involvement policy 2013 [Available from: https://a.storyblok.com/f/243782/x/c7f3c64edd/patient-and-public-involvement-policy-november-2013.pdf.]
6. CDA-AMC. Framework for Patient Engagement in Health Technology Assessment 2019 [Available from: https://www.cda-amc.ca/framework-patient-engagement-health-technology-assessment.]
7. EUnetHTA. 21. D7.2 – Guidance on patient and healthcare professional involvement. 2023.
8. Johnson EE, Lally J, Farnworth A, Pearson F. Involving people with a lived experience when developing a proposal for Health Technology Assessment research of nonsurgical treatments for pelvic organ prolapse: Process and reflections. Health Expect. 2023;26(3):1127-36.
9. Johnson EE, Gill S, Still M, Trenchard D, Smith D, Harmston R, et al. Understanding the Scope, Intent and Extent of Published Conceptual Frameworks of Frameworks for Patient and Public Involvement in Health and Social Care Research: A Rapid Scoping Review. Health Expect. 2025;28(5):e70425.
10. Pearson, F; Bridge, D; Stone, M; Heron, L; Riley, D. From Participation to Partnership: Advancing Patient-Centric Inclusive and Equitable Evidence Generation in HEOR. Value in Health. 2025; 28(12): S653
11. The James Lind Alliance Guidebook. 2021.
12. Pratt B. Constructing citizen engagement in health research priority-setting to attend to dynamics of power and difference. Dev World Bioeth. 2019;19(1):45-60.
13. Pollock A, St George B, Fenton M, Crowe S, Firkins L. Development of a new model to engage patients and clinicians in setting research priorities. J Health Serv Res Policy. 2014;19(1):12-8.
14. Johnson, E.E., Gill, S., Still, M. et al. Development of the RIPPLES framework for patient and public involvement in rapid evidence syntheses. Res Involv Engagem (2026). https://doi.org/10.1186/s40900-026-00878-5
15. Pollock A, Campbell P, Struthers C, Synnot A, Nunn J, Hill S, et al. Development of the ACTIVE framework to describe stakeholder involvement in systematic reviews. J Health Serv Res Policy. 2019;24(4):245-55.
16. Warner K, See W, Haerry D, Klingmann I, Hunter A, May M. EUPATI Guidance for Patient Involvement in Medicines Research and Development (R&D); Guidance for Pharmaceutical Industry-Led Medicines R&D. Front Med (Lausanne). 2018;5:270.
17. Abelson J, Tripp L, MacNeil M, et al. Development of the Engage with Impact Toolkit: a comprehensive resource to support the evaluation of patient, family and caregiver engagement in health systems. Health Expect. 2023;26:1255-1265. doi:10.1111/hex.13742