HTA203

Insights on Progress Toward Patient Centred HTA Through Evaluation of PRO Integration, Value Disconnects, and Policy Change in the EU4 + UK

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1 INTRODUCTION

Patient-reported outcomes (PROs) are increasingly recognised in European health technology assessments (HTAs) as key evidence of patient experience, yet research has shown that their influence on reimbursement and value decisions remains limited and inconsistent. This study explores how emerging policy initiatives, particularly those aimed at harmonising processes at the EU level, may help strengthen the role and methodological acceptance of PROs, supporting more patient-centred and aligned value evaluations across Member States.

2 OBJECTIVES

This research aims to:

1. Evaluate the integration and influence of PROs within EU4 + UK HTA frameworks and compare patient-prioritised outcomes with HTA drivers of value across select therapeutic areas (TAs).
2. Examine recent policy developments that may enhance patient-aligned evidence generation and value communication in future assessments.

3 METHODS

- Reviewed HTA guidelines, methods papers, and assessment reports from HAS, IQWiG/G-BA, NICE, AIFA, and AEMPS to characterise PRO inclusion, evidentiary standards, and influence on value conclusions.
- Identified patient-prioritised outcomes via targeted literature review of burden of disease and advocacy surveys.
- Analysed recent EU and national policy documents (2024–2025), including the EU HTA Regulation, EHDS, NICE 2025 Modular Update, and EMA/EUPATI initiatives, to assess implications for patient-centred evidence and PRO integration.

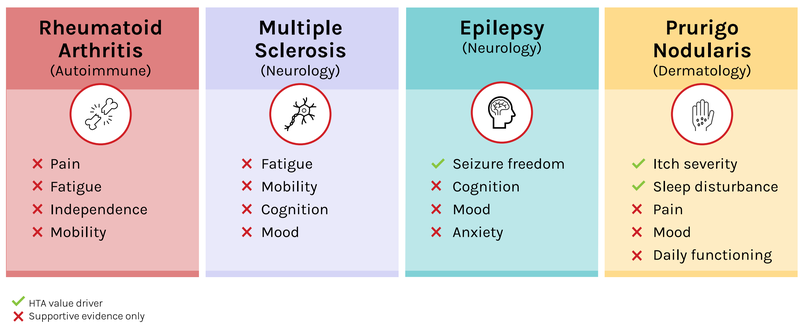
4 RESULTS

- Across the EU4 + UK, PROs are routinely collected and submitted in reimbursement dossiers, yet they rarely influence final value determinations **(Table 1)**.
- An examination of patient-prioritised outcomes (all PRO domains) versus HTA-valued endpoints highlights persistent misalignment between what patients consider most meaningful and what drives HTA conclusions **(Figure 1)**.

Table 1. Integration and Influence of PROs Across EU4 + UK HTA Frameworks

COUNTRY	INTEGRATION OF PROS	PRACTICAL IMPACT
(HAS)	• PROs routinely included in 2023–2025 HTA reports, with strongest emphasis on HRQoL • HRQoL data may support higher ASMR if robust and clinically relevant • Methodological guidance in development to improve PRO integration and interpretation	• Symptom-based PROs can be decisive in symptom-led diseases (e.g., pruritus in prurigo nodularis) • In most areas, PROs remain supportive rather than value-driving • 2023 analysis: >75% of 2021–2022 submissions excluded HRQoL data for methodological reasons
(G-BA/IQWiG)	• Recognised as patient-relevant dimensions of benefit for morbidity and HRQoL • Validated instruments and pre-specified analyses required • Evidence must show ≥15% response and <30% missing PRO data	• 2024 oncology dossiers review: PROs included in ~95% of dossiers, but only ~40% contributed to added-benefit ratings • Robust, validated PROs can influence outcomes (e.g., SGRQ in dupilumab COPD supported a minor added benefit)
(NICE)	• Manual recognises PROs for domains such as HRQoL, symptom burden, and health-related behaviours • EQ-5D-3L preferred for utilities, though may under-detect fatigue, cognition, and social participation changes	• HRQoL utilities dominate cost-effectiveness modelling • Symptom/function PROs typically supportive, not decisive • Recent appraisals note clinical relevance but limited standalone value except for EQ-5D
(AIFA)	• No formal PRO guidance • PROs on HRQoL, symptoms, or function may feature in evidence but are not standalone criteria • Can support "innovativeness" if robust	• 2017–2021 dossiers review: ~49% of dossiers included PROs; only ~20% of "innovative" reports cited them explicitly • Recent AIFA reports summarise PROs as supportive evidence
(AEMPS)	• No formal PRO guidance. • PROs routinely appear in Therapeutic Positioning Reports (IPTs) but seldom value-determining	• Transparency improving, but IPTs still provide limited detail on how PRO evidence informs conclusions

Figure 1. Alignment Between Patient-Prioritised Outcomes and HTA Value Drivers Across Four Diseases



- A review of recent EU and national policy initiatives reveals ongoing efforts to strengthen the methodological acceptance and decision impact of PROs across HTA frameworks (**Table 2**).

Table 2. Emerging EU and National Policy Initiatives Supporting PRO Integration in HTA

POLICY/INITIATIVE	OBJECTIVE	POTENTIAL IMPACT ON PRO INTEGRATION
EU HTA Regulation (2025)	Establishes Joint Clinical Assessments (JCAs) and Joint Scientific Consultations (JSCs) for early dialogue between manufacturers, regulators, HTA bodies, and patients/clinicians	Embeds patient and clinician perspectives early, providing a more consistent space for PROs in core evidence packages and less national variance
EUPATI HTA4Patients (2024-2025)	EU-funded initiative training patient representatives to contribute effectively to national and EU HTA processes, including JCAs and early advice	Strengthens patient advocacy capacity so patient input highlights the most relevant outcomes and supports clearer valuation of PRO findings in HTAs
EHDS Regulation (2025)	Creates a harmonised framework for cross-border reuse of health data, including clinical registries and electronic records, under strict governance	Expands access to real-world PRO and patient-relevant data to complement trial evidence, enabling more patient-centred assessments
NICE Modular Update (2025)	Introduces adoption of the new UK-specific EQ-5D-5L value set in assessments	Produces utilities more sensitive to moderate symptom or function gains, reducing "ceiling effects" and better reflecting gains seen in chronic conditions
EMA Patient Experience Data (PED) Reflection Paper (2025)	Clarifies EMA expectations for integrating PED (evidence generated by patients without input or interpretation by a clinician) across drug development and marketing authorisation	Strengthens the evidence pipeline from regulatory to HTA, ensuring trials collect PROs aligned with patient value

5 CONCLUSION

Across EU4 + UK HTA frameworks, PROs are now routinely generated and reported but their influence on HTA decisions remains limited, constrained by methodological scrutiny, comparator evidence, and limited translation into QALY utilities. While Germany has established the most structured approach, other countries require further methodological guidance. Although primary endpoint selection is largely shaped at the EMA level, HTA bodies retain control over how PRO evidence is interpreted and weighted, often privileging disease progression measures or clinical response outcomes. Emerging policies create tangible avenues to enhance the visibility and credibility of PROs in future assessments.

For manufacturers, this shift underscores the need to align trial design and evidence strategies early, ensuring PROs reflect regulatory, HTA, and patient expectations. Proactive alignment on validated instruments, responder definitions, and transparent analytic methods will be critical to demonstrate and effectively communicate the value of PROs in an increasingly patient-centred HTA environment.

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