Will patient input shape EU-level Health Technology Assessments? HTA representative perspectives on the EU HTA regulation

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Introduction

The EU HTA Regulation (EU HTAR) positions patient involvement as a core element of the Joint Clinical Assessment (JCA), including participation in scoping, reviews of draft assessments, and strategic feedback through the Stakeholder Network.[1,2] This aims to ensure Health Technology Assessment (HTA) reflects real-world patient experiences and preferences. However, it remains to be seen how effectively these provisions will be implemented.

Objective

This research explores HTA representatives' perceptions on the extent to which patient input will meaningfully shape JCAs under the EU HTAR.

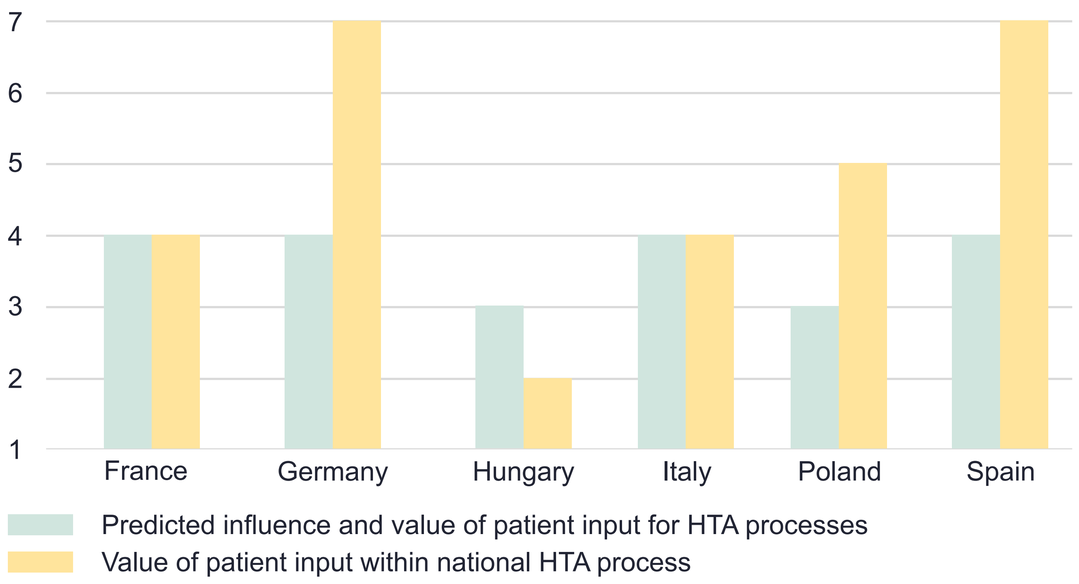
Method

Primary research was conducted using the Lightning Insights On-Demand Platform with a sample of six HTA representatives across France, Germany, Hungary, Italy, Poland, and Spain (n=1 per country). These countries were selected to capture differences in HTA systems and processes, enabling a comprehensive and holistic view on the EU HTA regulation. Insights were analysed to assess perceptions of the potential influence and value of patient input across different stages of the JCA process, identify key barriers and enablers affecting meaningful patient contribution, and develop recommendations to strengthen patient involvement across EU and national HTA systems.

Results

Likely influence of patient input on EU-level HTA

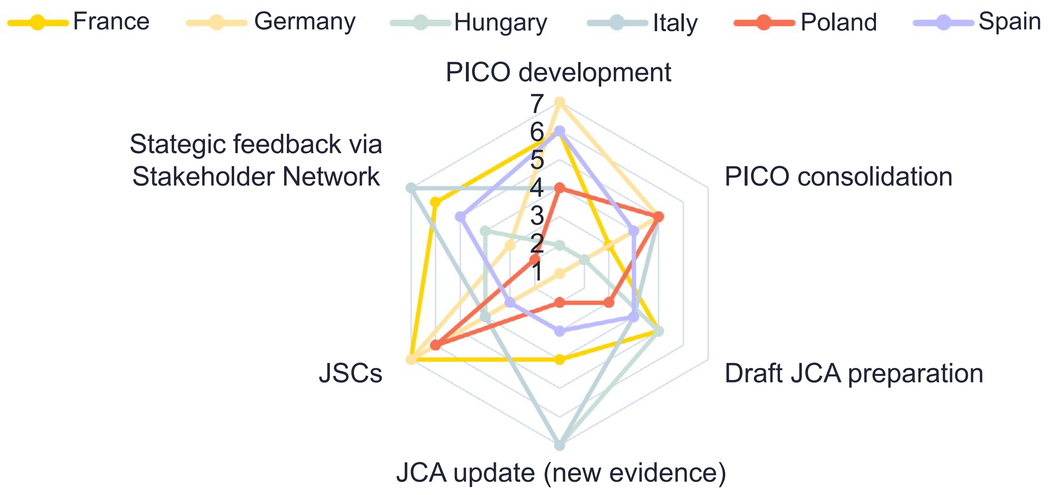
Figure 1: Predicted influence and value of patient input for HTA processes (1 = not at all likely; 7 = very likely); Value of patient input within national HTA process (1 = not valuable; 7 = highly valuable)



HTA representatives agree that patient input is overall somewhat likely to play a meaningful role in EU-level HTA. There is an observable link between the maturity of national level HTA patient involvement frameworks and stakeholder perceptions of the relative importance of patient input for the JCA. In Germany, Poland, and Spain, where patient involvement in HTA is formalised through mandated consultations, advisory body representation, and structured engagement channels, stakeholders view patient input as more important at the national HTA level than at the EU level. Conversely, in Hungary, where no formal patient engagement mechanisms exist, the EU framework is viewed as an opportunity to introduce clearer structures and guidance around patient involvement. This indicates that countries lacking well established national frameworks for patient involvement could potentially show a greater openness to the importance of patient involvement at the EU level.

Opportunities and challenges across stages of the EU HTA

Figure 2: Anticipated value of patient input at each stage related to the EU HTA process (1 = low value to decision making, 7 = high value to decision making)



JSCs and PICO development

Patient input is expected to be most valuable during the earlier stages of the EU HTA process, particularly during JSCs and PICO development in the JCA scoping phase. At these stages, patients can influence the parameters for clinically relevant value demonstration for a new therapy, including appropriate endpoint selection, relevant comparator(s), and the required duration of follow up. This is particularly important when selecting surrogate/intermediate outcomes to demonstrate patient-relevant value, and choosing QoL scales/PROs sensitive to functional changes within a specific indication.

The greatest value of patient involvement in the JSCs and PICO scoping is likely to apply for therapies for rare diseases without established treatment options (due to the potential lack of precedent regarding requirements for quantifying relevant clinical/humanistic value), and for the evaluation of treatments targeting indications where the primary symptoms are 'subjective' (e.g. pain and fatigue), to ensure the holistic impact of treatment is measured and interpreted appropriately.

> "As JSCs may happen before pivotal studies are designed, patient experts can influence endpoints (including PROs), acceptable comparators, feasibility, and burden, i.e., what evidence will exist when the JCA happens. I believe it's the single best point to ensure trials capture outcomes that matter to patients."
>
> **HTA representative, Poland**

However, the short statutory timeline of 87 days for PICO scoping could limit the depth of patient engagement and/or the time for the analysis and interpretation of patient representative input within this process. This highlights the importance of organisation structures to inform the timely identification and involvement of suitable patient advocates within this process, and ensuring sufficient time for the transparent incorporation of their feedback within the PICO scoping.

Furthermore, the impact of patient level input within the JSC and PICO scoping phases will likely be contingent on the 'technical literacy' of the patient advocates. For example, understanding the practical considerations for clinical study design and the ability to take a system-level view that balances individual preferences with broader population and healthcare system priorities, are key attributes that will support the value and impact of patient involvement within these processes.

It is also important to note that other evidence sources are expected to remain more influential than patient representative feedback in defining relevant PICOs. Across countries, country-specific guidelines and European and country level KOL feedback are consistently viewed as more influential than patient representative feedback for informing European level PICO requirements. In Hungary, France, and Italy, stakeholders also note that scepticism among HTA authorities regarding the potential subjectivity of patient input and the risk of bias associated with 'strong' opinions from a limited number of patient representatives reduces the value of this input compared to more 'robust' sources of data.

> "A fundamental prerequisite for meaningful input is that patient representatives possess a solid understanding of clinical study design, while also maintaining a broader, system-level perspective, balancing individual preferences with population and healthcare system priorities."
>
> **HTA representative, Germany**

PICO consolidation and draft JCA preparation

Later stages of the JCA process, such as PICO consolidation and the preparation of draft JCA reports, are seen as 'too late' and 'too technical' for significant patient representative influence. While patients could clarify aspects such as the relevance/impact of tolerability outcomes and alignment of comparators and outcomes with real-world practice, this is not viewed as transformative to the evaluation process.

JCA update (new evidence)

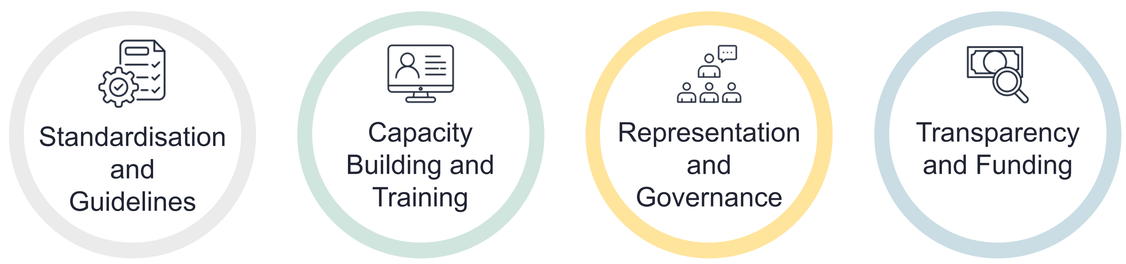
If updates are made to published JCAs, the significance of patient representative input depends on the type of new evidence provided. Patient impact will likely be greater in cases where new evidence incorporates new patient reported and/or surrogate outcomes (where there will be high interest from assessors in understanding the validity of the outcome and practical implications for how the patient feels/functions), and where the new data changes the scope or conclusions of the original JCA.

Strategic feedback via the Stakeholder Network

The Stakeholder Network is viewed as an opportunity to establish a formal, continuous channel of engagement between patient representatives, the HTA Secretariat, and JCA subgroups, helping patient representatives build credibility and expertise over time, likely increasing their influence on the JCA process (particularly concerning interpretation of the relevance/validity of clinical/QoL outcomes, interpretation of RWE, and discussing wider issues concerning equitable access). Nevertheless, without structural solutions, such as addressing language barriers and the absence of any formal obligation to consult patients, there is a risk that this channel remains largely symbolic.

Proposed solutions for meaningful patient involvement

Figure 3: Proposed solutions at the EU-level to ensure more meaningful patient involvement in EU-level HTA



HTA representatives identify several EU-level solutions to enhance the value and impact of patient involvement within EU-level HTA, which can be grouped into the following categories:

- **Standardisation and guidelines:** Establish clear EU-wide standards and good practice frameworks, supported by case studies to clarify patient representative roles across assessment stages
- **Capacity building and training:** Develop virtual training modules and EU-funded initiatives to strengthen technical understanding and evidence-based contributions from patient representatives
- **Representation and governance:** Ensure representativeness of the populations concerned and perspectives across Member States, leveraging the Stakeholder Network and the EMA for recruitment. Formalise regular patient involvement across multiple JCA phases given that under the EU HTAR patients are mandated to contribute only during PICO consolidation and revision of the final JCA draft (while at formative stages such as PICO scoping they 'may' be consulted at the assessor's discretion)
- **Transparency and funding:** Introduce transparent compensation mechanisms, early notification of EMA submissions to allow preparation, plain-language summaries of JCA reports, and tracking indicators (e.g., number and diversity of patients engaged) to measure progress over time

Supporting national-level actions could further help improve the effectiveness of patient involvement. National agencies could appoint Patient Involvement Liaison Officers to track EU 'calls for experts', guide domestic patient organisations in applying, and maintain calendars of upcoming JCAs and JSCs. Member States could also support the creation of pools of patient experts that can be engaged by the HTA Secretariat and JCA subgroups. Finally, the importance of building national capacity to capture and use patient-relevant data is highlighted.

Conclusion

Patient input is viewed as somewhat likely to meaningfully influence EU-level HTA, particularly at earlier stages such as JSCs and PICO development, when the evidence framework shaping the scope and criteria of the EU JCAs is still being established and remains open to refinement. However, the extent of that influence will depend on overcoming several barriers: strict timelines that may limit meaningful patient involvement, the need for adequate technical knowledge among patient representatives, and prevailing scepticism among HTA stakeholders that patient input may not be objective/representative and should be deprioritised. Across all countries, HTA representatives emphasise the need for clear standards and guidelines for patient involvement, systematic training, adequate representation through formalised involvement, and sustainable funding to ensure patient voices are consistently and credibly integrated.

Abbreviations: EMA: European Medicines Agency; EU: European Union; EU HTA: European Union Health Technology Assessment; EU HTAR: European Union Health Technology Assessment Regulation; HTA: Health Technology Assessment; JCA: Joint Clinical Assessment; JSC: Joint Scientific Consultation; PICO: Population, Intervention, Comparator, Outcome; QoL: Quality of Life; PRO: Patient-Reported Outcome; RWE: Real-World Evidence; KOL: Key Opinion Leader.

References: 1. Regulation (EU) 2021/2282, European Union. 15 December 2021. Available from: https://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:32021R2282&from=EN. 2. Implementing Regulation (EU) 2024/1381. European Commission, 23 May 2024. Available from: https://eur-lex.europa.eu/eli/reg_impl/2024/1381/oj/eng.