

Background and objectives

HTA plays an important role in healthcare decision-making through assessment of clinical effectiveness and cost-effectiveness of new health technologies (1). Patient engagement in the HTA process can bring additional value to decision-making (2). Building in this perspective upholds patients' rights and responsibilities for contribution, ensures decisions align with patient values, and leverages insights into living with a health condition and unmet need with respect to condition-related services and treatments. Patient engagement also enriches HTA knowledge and advances its methodologies (2).

We evaluated patient engagement within the context of the evolving collaborative European HTA landscape. The objectives were to explore the current level of patient engagement and its impact, to identify and assess the approaches taken by EUnetHTA to promote patient engagement, and to recognise opportunities and challenges for engagement initiatives.

Methods

A targeted review of patient engagement in the EU HTA process was carried out via analysis of EUnetHTA 21 deliverables, guidance documents, methodologies, and updates from the websites of EUnetHTA (EUnetHTA.eu) and European Commission (commission.europa.eu).

Results

Patient engagement in JA3: Building a comprehensive framework

- As shown in Figure 1, JA3 aimed to create a comprehensive framework to engage patients, their representatives, and their organisations in JSCs and JCAs/CAs (3)
- Patient engagement to support JSCs and associated meetings was initiated via direct contact with patient associations, offering valuable insights in interviews about their diseases and treatments (3), with their input summarised in final recommendations (4). EUnetHTA reported that patient input in early dialogues had valuable impact on JCA recommendations (4)
- In JCAs/CAs, the preferred approach was to gather patient input early in the scoping phase, using a patient submission template and open calls for input, tailored to the topic, to inform PICO development (3)

EUnetHTA 21: Nurturing patient engagement methods and guidance

- Frameworks developed as part of JA3 facilitated engagement approaches for the EUnetHTA 21 initiative (Figure 1)
- Table 1 illustrates patient engagement at both the European and national levels in the 7 JSCs conducted during EUnetHTA 21 (5)

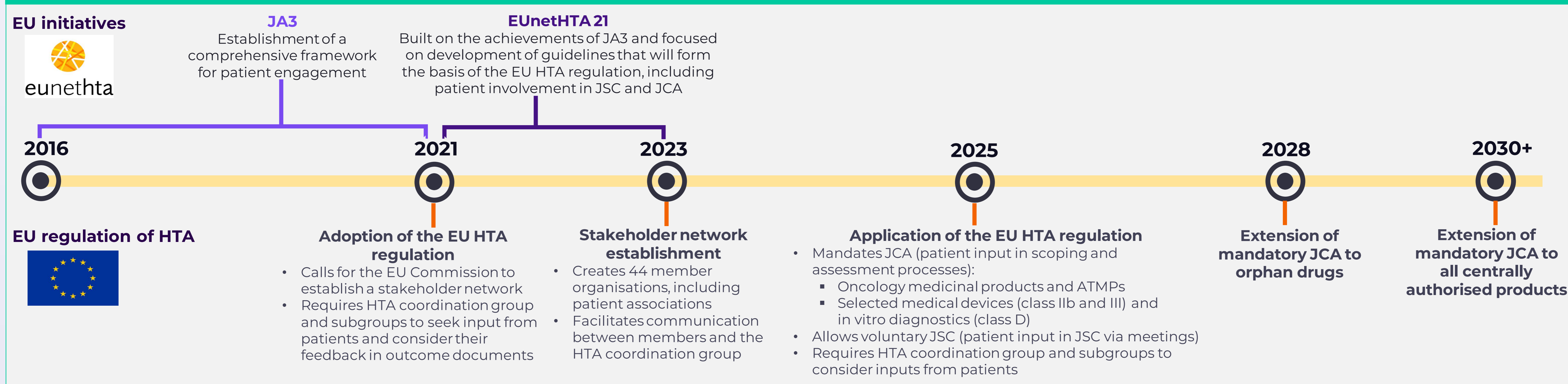
Table 1. Patient engagement levels in JSCs at EU and national levels

EUnetHTA 21 JSC	Patient engagement	
	European level	National level
JSC 001	+	+
JSC 002	+	+
JSC 003	-	+
JSC 004	+	-
JSC 005	+	+
JSC 006	+	+
JSC 008	+	-

Note: Based on the service contract, EUnetHTA 21 was required to conduct 6 to 8 JSCs. JSC 007 was withdrawn by the applicant.

- Two JCAs were conducted for medical devices and followed EUnetHTA-recommended processes for external expert and stakeholder involvement (5). Both JCA reports were recently published; patients were consulted early in the scoping process and participated through open calls and online submissions (6,7)
- When establishing their 2021-2023 joint work plan, EUnetHTA 21 and the EMA prioritised patient engagement in HTA (8)
- EUnetHTA acknowledges the importance of patient insights and has developed a guidance for engaging patients, their caregivers, and representatives (9). The deliverable, titled *Guidance for the interaction with patient representative, healthcare professional and other experts*, presents a structured process for selecting, recruiting, and evaluating patient and clinical experts, as well as patient and healthcare professional organisations in JSCs and JCAs (3). This resource will benefit HTA organisations at the national level (3)

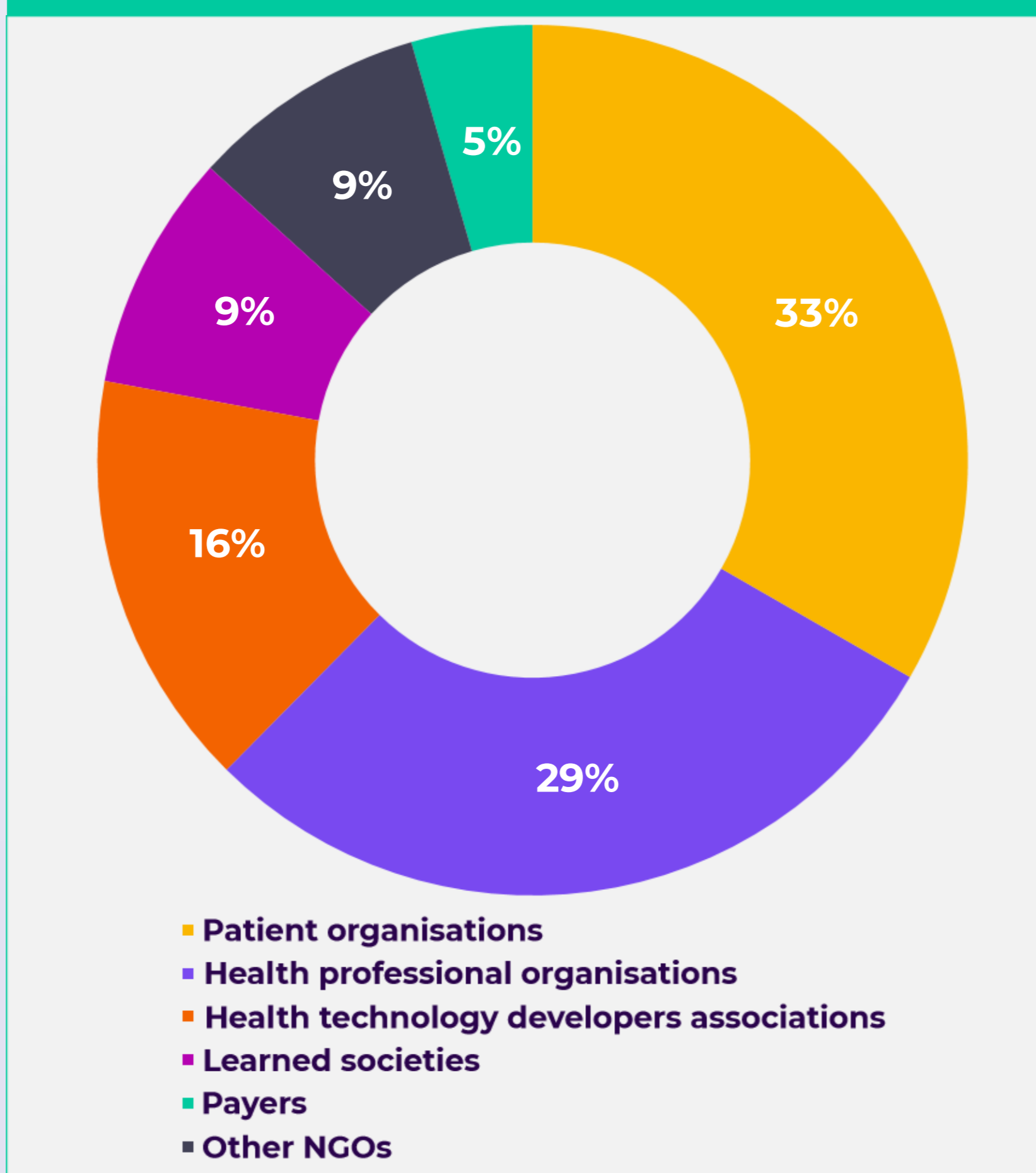
Figure 1. Overview of patient engagement within EUnetHTA initiatives and forthcoming EU HTA regulation



Compliance and collaboration: Patient engagement under the EU HTA regulation

- Following EUnetHTA 21 initiatives, the HTA regulation emphasised the importance of patient engagement in the JCA scoping process. The HTA coordination group and its subgroups are legally obligated to consider inputs from patients, clinical experts, and others in the JCA scoping process. As shown in Figure 1, this ensures their input in draft JCA and JSC outcome documents and promotes information exchange during JSCs (10)
- The EU HTA regulation (Article 29) establishes a stakeholder network with 44 member organisations, including patient associations and 2 observers (11). Their collaboration could enhance the patient engagement (12,13). Notably, patient associations are the most prevalent type of organisation in the stakeholder network, as depicted in Figure 2 (14)

Figure 2. Different types of organisations within the EU HTA stakeholder network (14)



Beyond regulation: Additional activities promoting patient engagement

The rolling plan for 2023-2024 implementation of the HTA regulation also identifies other activities that will support EU HTA (12). Among these activities, the EUPATI EU4Health Work Programme 2022 supports patient engagement through the EU4Patients project that focuses on training patients and enhancing knowledge of the joint health technology activities (14,15). This 3-year project, which began in March 2023, updates patient training content, designs an e-learning course, develops interactive training sessions for JCA and JSC, and implements sustainability measures to ensure alignment with the HTA regulation and continuity of the project outputs into the future (16).

Considerations for patient engagement going forward

- Challenges remain from both the patients' perspective and the pharmaceutical industry's perspective (17,18)
 - A survey with 54 responses, including 9 from patients, reported low self-ratings for engagement in the EU HTA process (17)
 - Challenges from the patients' perspective include lack of capacity and resources, expertise and training, alignment of organisations, conflict management, and shared valuation of patient input (17)
 - European pharmaceutical industries have previously expressed their concerns regarding the implementation of the HTA regulation and stated that the comments from various key stakeholders—including patient organisations—on development documents of EUnetHTA appear not to have been taken into consideration (18)
- The JCA with EUnetHTA and HTA regulation only covers the relative efficacy assessment, and the recommendations made are not binding. Value assessments for decision-making continue to be conducted at the national level (10)
- In fact, a study conducted in Central and Eastern Europe showed that patient engagement at the national level is limited. Thus, there is a need for EU countries to adapt their frameworks to include patient input to align with the joint HTA process (19)

Conclusions

From the patients' perspective, the patient engagement initiatives of EUnetHTA remain limited despite its best efforts. HTA regulation offers a unique opportunity to enhance this engagement. Furthermore, the full impact of current initiatives is a work in progress, with recent efforts like EUPATI still evolving. To strengthen the framework and ensure efficient HTA, patient engagement should be increased at national levels, and a clear guidance for alignment between joint and national HTA processes should be provided. The EU countries should recognise the importance of patient insights for efficiency. Moreover, additional avenues for patient input on engagement frameworks should be established.

Abbreviations: ATMP, advanced therapy medicinal product; CA, clinical assessment; EMA, European Medicines Agency; EU, European Union; EUnetHTA, European Network for Health Technology Assessment; EUPATI, European Patients Academy on Therapeutic Innovation; HTA, health technology assessment; JA, joint action; JCA, joint clinical assessment; JSC, joint scientific consultation; NGO, non-governmental organisation; PICO, population, intervention, comparison, and outcome

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