Patient Involvement in HTA

Can we learn from past experiences across Europe?

No two countries have the same process for determining if a medicine should be reimbursed and made available through public healthcare systems. Most use a form of Health Technology Assessment (HTA) [1] to assess the clinical and economic evidence, and many involve patient associations to provide additional insights which inform the decision [2]. However, how this patient involvement happens varies markedly from country to country [3].

To provide a more harmonised approach, the European Commission is establishing cross-national collaboration under the Regulation on HTA (HTAR) [4], with one important step in HTA conducted more consistently EU-wide. The Regulation comes into force in 2025.

Identifying good practices

The EFPIA Patient Think Tank views the introduction of the HTAR in 2025 as an opportunity to inform good practices for the future. EFPIA invited the Patient and Citizen Involvement in HTA Interest Group (PCIG) at HTAi to explore stakeholder experiences with patient involvement in HTA in collaboration with the European Patient Forum (EPF) and the European Patient Academy for Technological Innovation (EUPATI).

The aim was not only to understand how HTA agencies have involved patients in HTAs in European countries and to analyse the experiences of HTA researchers, patient stakeholders, and industry stakeholders, but also to extract those approaches that can be considered good practice.

The project was launched in 2022, with funding support from EFPIA and PhRMA.

What was done?

The research phase was structured along two tracks:

1) An in-depth interview-based exploration of processes and experiences related to patient involvement in HTA in selected European countries.
2) A Europe-wide survey with mostly multiple-choice questions related to the respondents' experiences of patient involvement in HTA.

From these two sources of information, suggestions for good practices were identified. Stakeholder workshops were conducted to refine the recommendations and build consensus, first separately by stakeholder type (HTA researchers, patient stakeholders, industry stakeholders) and finally, with all stakeholders together.

Patients can be involved at every step of HTA

The research found that patients can be involved in any step of the HTA across Europe (see Figure 1). Various methods are used when engaging patients, including written submissions, interviews, focus groups, deliberative multi-stakeholder discussions in appraisal committees, hearings, consultations, or by having a seat in an organisational committee.

However, no example was found where patients are consistently involved in all steps in one country, and there was low cross-agency consistency in how patients were involved or how their input was used in the HTA report, recommendations, or subsequent decisions. There were several examples in which previous guidance or tools from the PCIG was adopted or adapted for the use in a specific country.

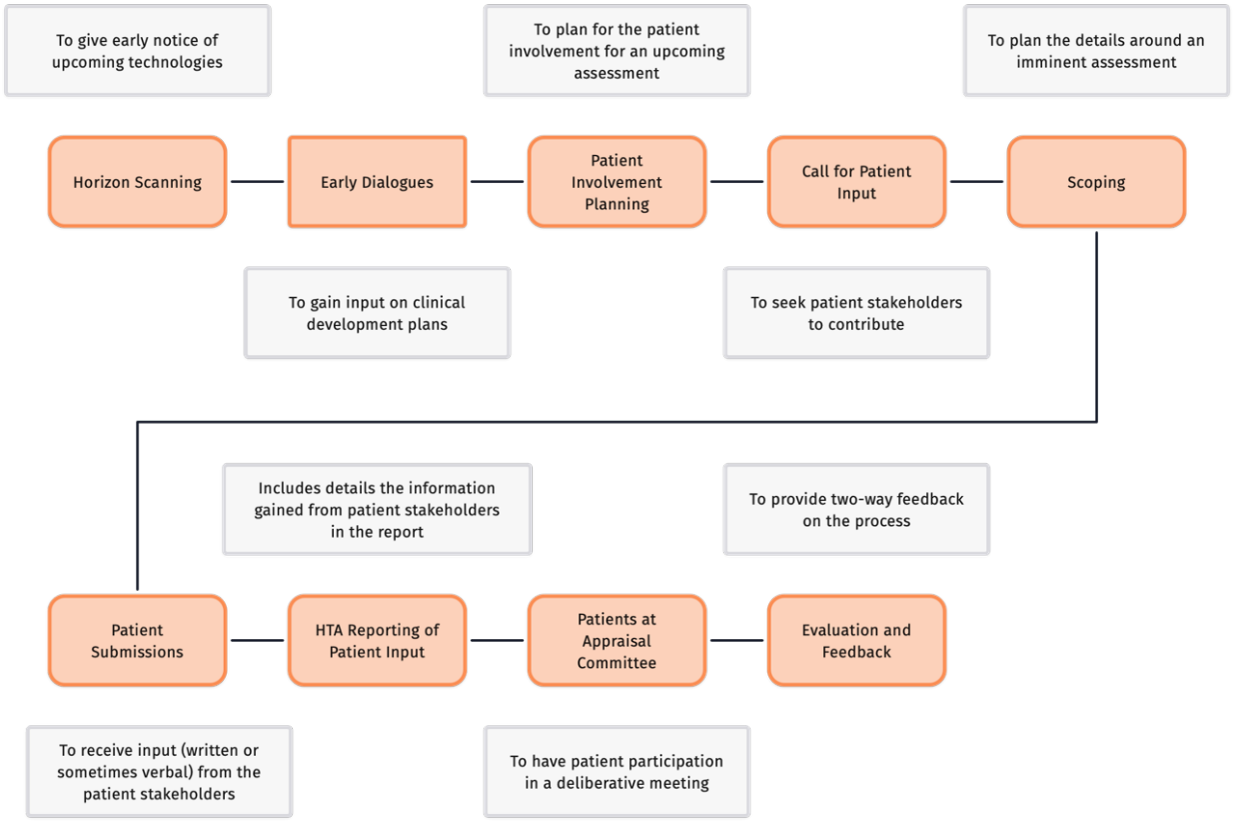

Figure 1: Elements of HTA that were mentioned during the interviews on experiences with patient involvement in HTA in Europe

Shortcomings were reported in several areas. These include:

- Reaching and motivating relevant patients
- Delivering fit-for-purpose training and information to enable effective involvement
- Guidance for reporting and evaluating patient involvement in HTA as well as giving feedback to those who contributed. This lack of feedback and reporting leads to resignation and very low motivation among patients to engage and be involved.

On the other hand, a growing trend was observed that patient stakeholders are involved at the organisational level. There are examples of standing patient committees (e.g. in Wales) or advisory boards that include patient stakeholders.

Finally, there is a lack of institutional memory, particularly among the patient community. In some organisations, patients who had been involved in HTA processes had moved on and there were no records or other ways to access the input given or any experience related to this process. Hence, it was much more difficult to get patient stakeholder input than originally expected. Due to the lack of such records, it can be assumed that the learning within the patient organisations on how to approach HTA may be limited.

Patients know little about HTA and need more information

The survey revealed important gaps that impede patient involvement in HTA. Among them was the fact that the relevant patients often are unaware of the opportunity to be involved or do not know how to become involved. In addition, they often do not have the capability or knowledge (lack of training or information) or the capacity (resources) to be involved within the tight timelines. The satisfaction of patients, who had been involved, with the different aspects of information they had received was relatively low (see Figure 2). While the information related to the technology to be assessed was perceived as somewhat satisfactory by 37% of the patient stakeholders, only a quarter of patients or less were satisfied with the information on what was asked of them. Even fewer were satisfied with the information on the HTA process, and on how their input had been used in the assessment or HTA recommendation.

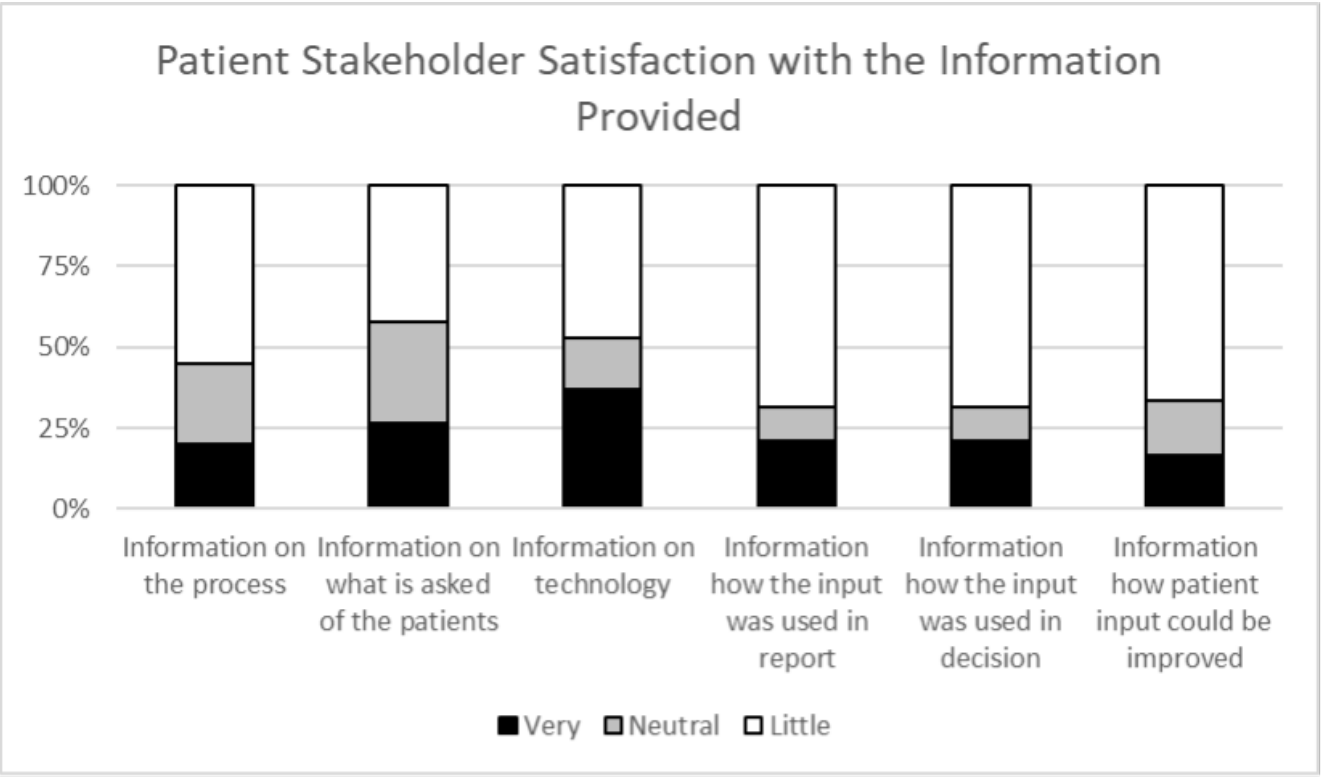

Figure 2: Level of satisfaction of patient stakeholders with the information received during the process of their involvement in HTA

HTA & patient involvement: Good practice recommendations

If involving patients, do it well (and make it a motivating experience for them)

The consensus process resulted in good practice recommendations (see Table 1). As an overarching principle, an advisory board or standing committee to advise on patient and public involvement activities should be considered. Such **organisational level involvement** could help to meet demands for quality assurance, purposefulness, transparency, and consistency of patient involvement. At the same time, it could be a good channel for improving awareness and opening doors for more engagement in HTA among the patient community. **Guidance and training** should be fit-for-purpose, available to patients and researchers, transparent and easily accessible. **Communication** should be timely and consistent in a two-way manner and should include feedback opportunities.

Among the **process-level recommendations** related to specific aspects of health technology assessment, a few recurring themes were observed, including **low barrier information and training possibilities** for each aspect of involvement; **early outreach and active communication** in addition to the standard publication of calls on agency websites; **collaboration and communication** between stakeholder groups to improve the effectiveness and experience of the involvement; and the importance of **reporting, evaluating, and giving feedback**.

In addition, it is recommended that technical information (dossier) should be complemented by a **lay language summary of the key items of relevance to patients**[5].

When establishing processes for involving patients in HTA, it is advisable to use these recommendations as a compass. Involving patients is a learning process for patient stakeholders, HTA organisations and researchers. The aim should be to make this experience fruitful and productive, and to ensure all stakeholders feel that their time and resources are well spent.

ORGANISATIONAL LEVEL INVOLVEMENT ▪ HTA organisations should consider an advisory board or standing committee to advise on patient and public involvement activities. Such a board may also be an umbrella board for multiple platforms in the healthcare system (e.g. regulatory, HTA, MoH) GUIDANCE AND TRAINING ▪ Fit-for-purpose training and guidance (for each type of input) for patients and researchers ▪ Transparency, accessibility ▪ Collaboration on training and materials COMMUNICATION ▪ Consistent and two-way communication ▪ Timely communication ▪ Feedback opportunities (between patient stakeholders and HTA: receiving and giving feedback)	HORIZON SCANNING	• More collaboration between HTA organisations and umbrella Patient Organisations • Efficient use of resources
	EARLY DIALOGUES	• Specific guidance needed (why/what/how) • Recruitment and involvement practices follow those for assessments
	PATIENT INVOLVEMENT PLANNING	• Early alert systems to increase preparation time • Transparency and explicit criteria
	CALL FOR PATIENT INPUT	• Active outreach • Collaboration to maximise outreach • Motivational language
	SCOPING	• Clarity on purpose of patient involvement and provision of useful information
	PATIENT SUBMISSIONS	• Relevant templates built with patient input • Options for support provided • Multilingual to ensure all can take part
	INTERVIEWS / FOCUS GROUPS	• Discussion guide relevance/suitability • Support options (e.g. buddying)
	REPORTING OF THE PATIENT INPUTS	• Reporting of patient input (standards, quality) • Guidance for researchers on use and reporting of input
	APPRAISAL	• Guidance and support for committee members • Importance of leadership – dedicated space for patient expertise
	EVALUATION AND FEEDBACK	• Tracking, evaluation, and communication on use of input • Evaluation of impact of patient involvement and satisfaction with process
	INDUSTRY DOSSIERS	• Report any patient engagement / involvement that occurred throughout R&D including participation and dialogue as well as research / data

Table 1: Good practice recommendations resulting from the 360° Stakeholder experiences research and subsequent stakeholder consensus process. Overarching themes are described in the left column, items relating to specific steps or themes, are listed in the right column.

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Since its foundation in 2005, the PCIG has acted as a platform to discuss, exchange, and improve methods for patient and citizen involvement in HTA, to develop tools that support HTA agencies and patients in this endeavour, and to foster knowledge about good practices in this field.

EFPIA approached the PCIG to plan and lead a European project on stakeholder experiences with patient involvement in HTA in collaboration with the European Patient Forum (EPF) and the European Patient Academy for Technological Innovation (EUPATI). The aim was not only to understand how HTA agencies have involved patients in HTAs in different European countries and how the experiences of HTA researchers, patient stakeholders, and industry stakeholders had been, but also to extract those approaches that can be considered good practice.

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