European
Patients' Forum

EU HTA SIMULATION WORKSHOP SUMMARY

EPF European Patients Forum

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Objective of the Workshop

Patient Organisations prepare for the EU-HTA and know the opportunities foreseen by the EU-HTA for involving patients. The long-term goal is that the value of patient involvement will have been demonstrated and patient involvement will be expanded and strengthened in the European HTA processes.

By simulating different aspects of Patient Involvement in Health Technology Assessment (HTA) after the introduction of the centralised European procedures for Joint Clinical Assessments (JCA) and Scientific Consultation (SC) in 2025, the participants should be sensitised to the upcoming challenges and be strategically prepared to overcome them.

Disease area	Country	Organisation	Level
	Germany, Greece	National Coalitions	National
Multiple Sclerosis, Cancer, hemophilia		European disease-specific platform	European
Rare diseases, Cancer, Multiple Sclerosis		National disease-specific organisations	National
Multiple Sclerosis		Local organisation	National
	Belgium, Greece	Individual patients	
		Facilitators	
	France	External expert	

Figure 1: Participants

Workshop set-up

The workshop was hosted by the European Patients' Forum (EPF) in Brussels on 11 December 2023. The invited participants reflected a mix of European patient organisations (European disease-specific platforms and national umbrella organisations) being active in the priority disease areas for the first wave of JCA under the HTA-Regulation (HTA-R).

In the first part of the workshop, introductory presentations were given on basic principles of HTA and patient involvement in HTA in Europe – especially under the new European HTA processes, the impact of involvement of patients in HTA and potential challenges and barriers for patient involvement in HTA. These were interspersed by two group work sessions related to values, barriers, and possible initiatives to overcome the barriers.

To follow up with the participants, a web-based meeting (90 minutes) was set up three months after the workshop (27 March 2024) to allow for an update on the newly published EU-HTA regulation's implementing act on JCA and to exchange recent experiences among the participants.

Introductory Sessions

HTA and the Role of Patients in HTA

Given complex choices regarding prioritisation of funding allocations to best meet the overall healthcare needs of the population, Health Technology Assessment (HTA) is increasingly used around the world. The purpose of HTA is to collect and assess all available evidence to inform decisions on access and reimbursement of health technologies in a country or region.

Terminology:

- A "**health technology**" is any intervention developed to prevent, diagnose or treat medical conditions, promote health, provide rehabilitation, or organise healthcare delivery (including medicines, devices, diagnostics, educational programs, or procedures).
- **HTA** is a multidisciplinary process that uses explicit methods to determine the value of health technology at different points in its lifecycle. The purpose is to inform decision-making in order to promote an equitable, efficient, and high-quality health system 1.

Value is determined through a variety of factors, whereby the essential ones are **differences in outcomes** (clinical effectiveness including safety aspects or quality of life) between the new technology and the current standard of care, and **differences in cost** (total cost of using the technology in the respective healthcare system). In addition, wider implications are considered such as the impact on environment or **care pathways**, **and organisational**, **cultural**, **ethical**, or **social** factors.

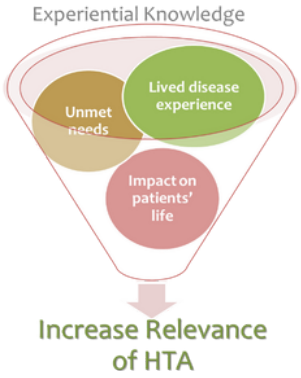
HTA is one step in the adoption pathway for new technologies, which follows the regulatory assessment and precedes the coverage decision.

HTA follows explicit and transparent processes following published national or regional guidelines. The steps in HTA processes generally start with **horizon scanning** and **topic selection**, followed by **scoping** of the HTA research based on the **PICO** (Patients, Intervention, Comparator, Outcomes) structure which defines the frame for **evidence collection**. After the **evidence analysis,** an **HTA report with preliminary recommendations** is drafted and published for **Professional and Public Consultation**; after **addressing the comments and revising the report** it is reviewed by an **appraisal or decision committee** and the **recommendations** are finalised into a **decision**. Usually, there is another legal provision for **appeals**.

The definition of the **PICO** in the scoping phase is of essential importance and will impact how the assessment is conducted and may also impact the final recommendations and decisions.

Value of Being Involved

Patient Involvement in HTA encompasses both patient participation in terms of communication with or input to the HTA process and the use of Patient-Based Evidence.



The value of involving patients is to help ensure the relevance of the HTA research and report to the patient population and to bring clarity to areas of uncertainty due to insufficient or contradictory evidence.

In addition, patient involvement can help to understand the priorities of the patient population, and their real-life experiences and therapies, the consequences of therapy regimes on their life, and to understand what trade-offs patients would we willing to accept.

In terms of defining the **PICO**, patients can help to clarify what the experiences of the **patient** population in real life are, what variability there is between patients (subgroups, equity, etc), what **intervention** would mean to them or what their experiences with it are, what the real-life **comparator** is, the therapy-mix they use, and which **outcomes** are desired by or important to them. Herein, the patients' viewpoint may differ largely from those of other stakeholders such as clinicians, provider organisations, or researchers.

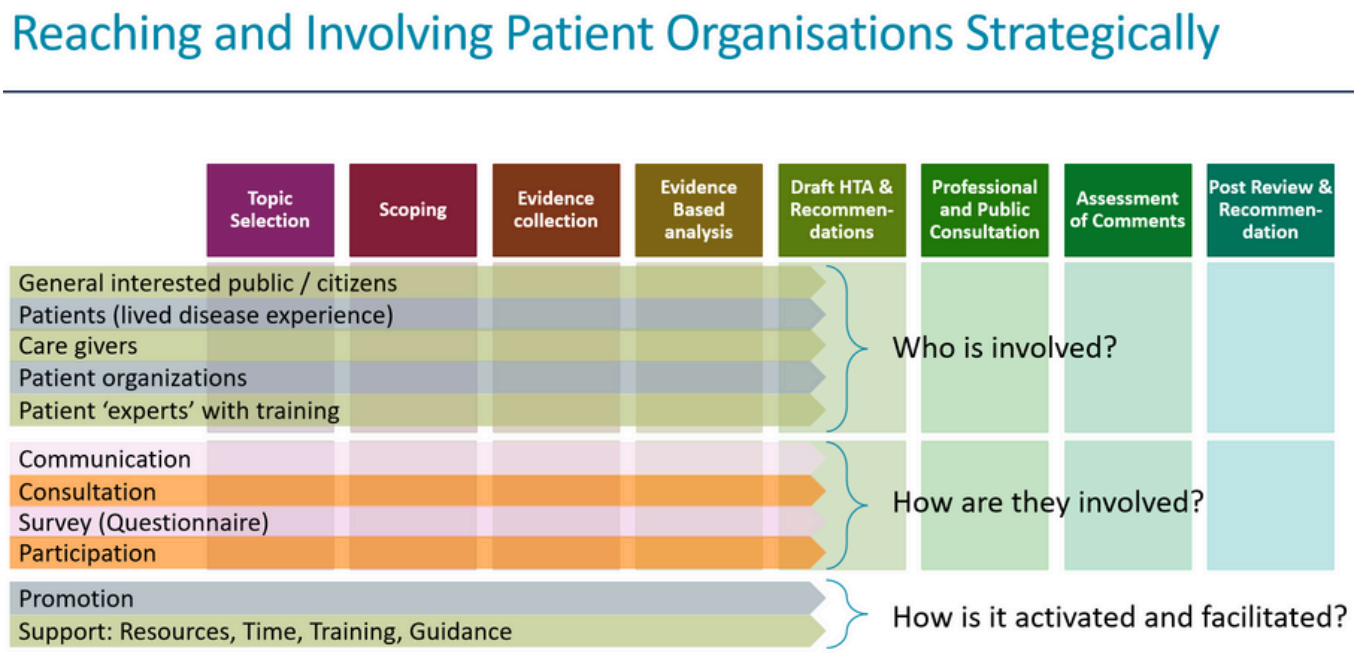
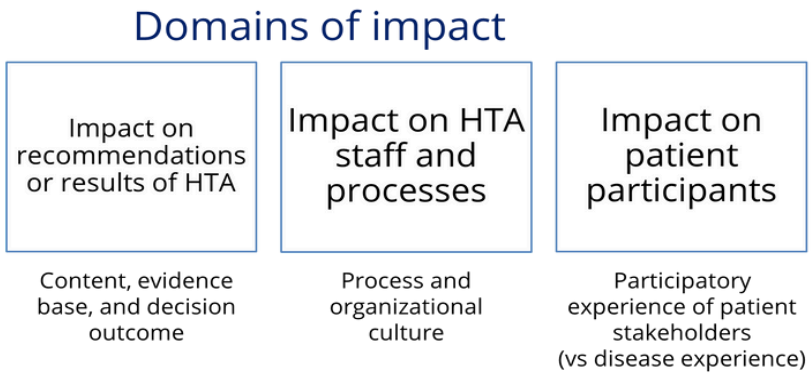


Figure 2: Different aspects of patient involvement along the process of HTA(4) *Adapted from J Abelson et al. IJTAHC, 2016, 1–9.*

Patients can be involved in any step of the HTA process and on the organisational, and strategic level. Depending on the objective of the involvement in the specific step, different types of patients may be best equipped for the involvement; sometimes – as depicted in *Figure 1* – it may be patients with the lived disease experience and other times, patients who have gained some experience with the healthcare system of the entire breadth of the patient population. Equally, different ways of gaining input may be chosen, which could involve direct communication (interviews, focus groups), written input (through a template questionnaire), through consultation, or through committee membership.

The processes and criteria applied for patient involvement in HTA should be published by the HTA organisation, and guidance or training should be available.

Impact of Patient Involvement in HTA

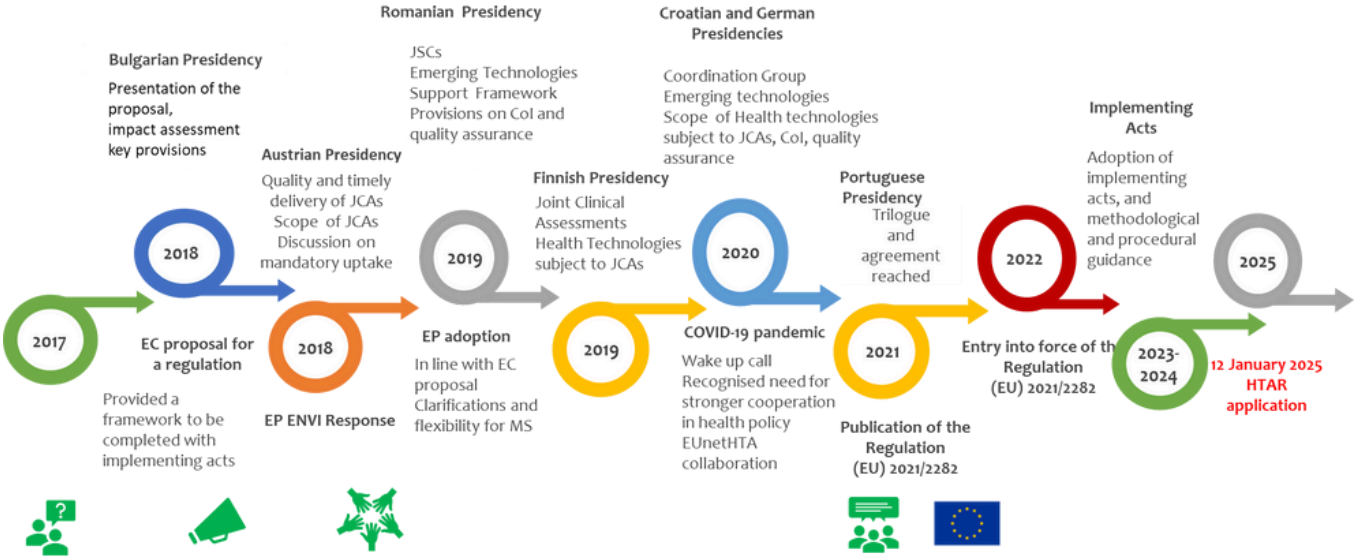


Although patient involvement is considered an essential component of HTA and several HTA agencies have set up guidelines and processes to support it, there is a scarcity of rigorous evaluations of patient involvement initiatives in HTA. If evaluation is done, the focus is mostly on process (how did it go?) rather than impact (what difference did it make?). Patient involvement can have an impact on both the processes and outcomes of HTA.

As there is a high diversity in the processes of how patients are involved in HTA, there is also a high diversity in the ways of evaluation and what is measured as impact or outcome. A **qualitative analysis by the patient and citizen involvement in the HTA interest group** (PCIG) at **HTAi.org** revealed a broad range of reported impacts across three different domains, the impact on the HTA results/recommendations, the impact on the HTA researchers and processes, and the impact on the participating patient stakeholders (*Figure 3*). Most frequently the impact was reported as "better understanding of patient experiences and needs and improved ability for data interpretation".

The EU HTA Regulation and Implementation

HTA is historically performed for a specific country, region, or institution. Therefore, a multitude of HTA organisations have been working in parallel in Europe with a lot of overlap in their work and a high variability in the results and conclusions. After much discussion and debate on how these processes could be performed more efficiently and also more harmonised across the European Union, the EU Commission released a regulation (HTA-R) in 2021 that mandated the introduction of a European collaboration on the clinical assessment part of the HTA starting in 2025.



Starting in January 2025, all clinical domains of the HTA for technologies targeting cancer and rare disease areas will be assessed in a so-called Joint Clinical Assessment (JCA). In addition, there will be the opportunity for early interaction with the EU-HTA community during the clinical development of new technologies, the Joint Scientific Consultation (JSC).

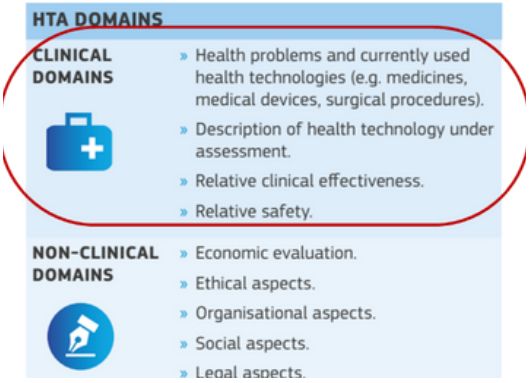


Figure 3: Three domains of impact of patient involvement in HTA (Lopez Gousset 2024)

The exact processes and details on how this is going to happen are currently elaborated by the Member State Coordination Group for HTA and will be published successively throughout 2024. Preliminary processes have been elaborated as deliverables of a previous EU project called EUnetHTA21, that can serve as an indicator for how future processes may look.

In terms of patient involvement, the Coordination Group has already published a few standards in a fact sheet, which include the establishment of an HTA Stakeholder Network that will act as a standing committee, that advises the Coordination Group, the HTA secretary, and the HT assessors in relation to stakeholder (including patients) viewpoints and expectations related to the EU-HTA. It is also clearly stipulated by the regulation, that patients – together with other stakeholders – need to be involved. However, what type of involvement, who and how is not yet described in sufficient detail beyond that any patient stakeholder participating in the EU-HTA needs to have a European (multi-national) perspective. In addition, there will be clear regulations for how to identify and protect against Conflict of Interest (CoI) among the stakeholders.

After the clinical assessment in the EU-HTA, the member states will receive the report and build on it for the second part of the assessment, which will be performed nationally or regionally as previously. This includes consideration of the national context and the economic components of the assessment. Patients could also participate in the national assessment; in this case, it will be national or regional patient stakeholders who will be involved.

In addition, at the very beginning of the EU-HTA, the member states will be able to define the key PICO (Patient, Intervention, Comparator, Outcomes) parameters, they would like to see covered in the scoping of the HTA. In this phase, patient stakeholders should also be able to voice their perspectives.

Group-Work

The following sections summarise the results from sequential sessions of work performed by 3 work groups with a focus on JCA, JSC, or national HTA respectively. This started by brainstorming what ‘good’ could look like in the future HTA scenario.

A Good Scenario for Patient Involvement under the HTA-R in the EU

Ideally, experienced patient stakeholders should participate in each EU-HTA who are well informed on the full range of needs and experiences of the specific patient groups across Europe, or who have access to such information through patient experience data. There should be sufficient access to appropriate training and information for patients so that they know how to give input in an effective and efficient manner.

Umbrella organisations should actively screen the upcoming opportunities for patient involvement in HTA and alert the patient organisations concerned. In addition, they should be able to draw on a pool of patient representatives, who can fulfil these tasks and are qualified for participation (little risk of conflict of interest, trained, experienced with the role as patient representative in their disease area, etc.).

The ‘ideal scenario’ is equally true for involvement at the EU level or national level.

Challenges

The typical challenges for patient involvement in HTA from the patient perspective are that often patient organisations don’t know about the opportunity to be involved, they don’t know how to respond to the call, don’t have the resources to give the appropriate feedback within the requested time, or that they don’t see this activity as an important priority. In addition, HTA dossiers are often highly technical and difficult to understand and there is little support given by the HTA agencies to facilitate patient participation.

It is anticipated that there will only be a limited number of patient stakeholders who will qualify for participation in an EU-HTA, due to the requirements defined in the process or to language or other barriers such as narrow timelines, resource or mobility limitations, as well as the turnover in the patient organisations resulting in the lack of organisational or individual memory (e.g. for repeated involvement).

Despite high expectations towards the willingness of patient stakeholders to participate in the EU-HTA, it seems currently unlikely that there will be any mechanisms to reimburse the participating patients for their expenses and time.

Addressing the Challenges

Each group selected one challenge to 'design' an initiative for improving or overcoming the challenge.

JCA Group

Barrier	Work Group
Difficulty in building a sufficiently big pool of experts	Jana Hlavacova, Vassillis Katatzias, Natasha Muench, Mitchell Silva, Juan Jose Ventura

Description of the Barrier

There is a lack of individual experts who can provide good quality input on short notice. This is due to a lack of knowledge about who belongs to the right target group and who has sufficient understanding of the HTA requirements to provide inputs.

Root causes of the barrier	Idea pool
• Limited number of patients in some of the primary target diseases • Limitations through bad health status and prognosis • Lack of expertise • Lack of time • Limited to no capacity from the smaller patient organisations to build and maintain the expertise	• Build a database storing the available expertise (at the national level) • Set a standard, fit-for-purpose training format • E-learning, simple material • Explaining the importance of Pi in HTA to improve motivation • Show the effect (measure, report) • Build momentum through a community approach

Plan for Action	Key Stakeholders
• Build and offer access to a simple e-learning platform (low threshold) • With fit-for-purpose training • Need to be engaging • Successful participants can register to be in the patient expert pool **Milestones and important intermediate steps** • Patients involved in procedures, planning • Creation of a database (infrastructure) • Training materials **Resources required** • Knowledge • Hosting and maintenance • Expertise, experts • Funding **Measures for monitoring success** • Number of graduates Number of registrants in database	**Actors:** • Umbrella patient organisations (EPF, EURORDIS) • EUPATI, EUCAPA **Allies and supporters:** • Research, scientific associations • HTA-bodies • Industry • Insurance funds • IT companies **Oppositions:** • HTA Bodies (they have their database which is not open to the public) **Key success factors:** • Infrastructure • Sustainability of expert pool • Updating **Enablers and accelerators:** • Funding • Cross-country collaboration • Sharing, transferability • Language, translation **Risks:** • Poor engagement or retention • Training not fit-for-purpose

The JCA Group aimed to address the lack of individual experts who can provide good quality input on short notice.

The JSC Group

The JSC Group aimed to improve the agility to respond to calls for patient involvement by establishing a consensus on a process and responsibilities among European umbrella patient groups and other patient organisations.

Barrier	Work Group
Identifying patient experts	Konstantina Boumaki, Martinus Desmet, Cees Smith, Lene Kaa Meier

Essence of barrier

When the call for patient inputs comes, there is a very limited time to identify and prepare the patient expert(s)

Root causes of the barrier	Idea pool
• Insufficient communication of call • Lack of education of the potential target patients • No platform for retrieving those patients • Lack of organisation • Unclear information on the processes and information	• Form a ''EU-HTA-R Group • Develop a consensus on clear communication pathways: EPF → National umbrella organisations → Identification of disease-specific patient organisations and patient experts • Pre-emptive actions (good preparation enabled through early alerts for upcoming JSC's)

Plan for Action	Key Stakeholders

• Establish a communication process through EPF
  ◦ Horizon scanning, collaboration and communication with the EU-HTA coordination group
  ◦ Network and consensus-building with European-level organisations and national patient organisations
• Develop an agenda and processes
• Implement
Resources required
• Staff
Funding
	Actors:
	• EU-HTA Coordination Group & EPF
	Allies and supporters:
	• European and/or National Umbrella Patient Organisation
	Oppositions:
	• Other umbrella organisations that want to lead
	• EU-HTA organisations if they don't cooperate
	Key success factors:
	• Awareness created in 2024
	• Education and preparation of patient communities
	Enablers and accelerators:
	• EU support
	• Country support
	Risks:
	• Bureaucracy
	• Administrative barriers
	• Lack of consensus between different stakeholders

The National HTA Group

Barrier	Work Group
Low capacity for HTA in patient organisations (POs)	Martin Danner, Julie Spony, Nikolaos Dedes, Christiane Tihon-De Cokele

Description of the barrier

- There's no way to find out who could be interested in HTA and knows how to participate in it
- There is no coordination unit in the organisation
- It's difficult to find representative patient perspectives, few people engaged in this
- Lack of early alerts

Root causes of the barrier	Idea pool
• Lack of understanding of the role of NGOs and patient organisations • No strategy for PI in HTA and lack of institutional funding in the sector, no public funding for infrastructure and core business of POs • Lack of trust into the professionalism of POs • Lack of participation culture (except guidelines) • Not enough knowledge of HTA • POs prioritise supporting individuals	• Culture change • Evidence of the importance of participation • Umbrella organisation • Build patient organisations, where gaps exist • Recognition by national government

Plan for Action	Key Stakeholders
• EPF acting like a guardian (chiedi Vale) • EUCAPA and HTA4Patients can contribute in upskilling • Participation in implementation plan at EU-HTS] • Early political action: statements to proactively use opportunities for consultation and clarify the perspective of POs. It's necessary to act at all levels: EU and Member State • Develop a political strategy and action process	**Actors:** • Governments • Community of patient organisaitons **Allies and supporters:** • Scientific / research community **Oppositions:** • Industry **Key success factors:** • / **Enablers and accelerators:** • / **Risks:** • The role of the industry

The Group considering the National HTA perspective aimed to address the low capacity for HTA in the Patient Organisations and the limited ability and capacity for doing so.

Follow-up Webinar (27 March 2024)

Eight of the workshop participants joined the follow-up webinar, which aimed to summarise the workshop report, to give an update on further developments related to the EU-HTA and to foster the exchange between participants.

Several participants reported that they have discussed the workshop with their colleagues in their respective patient organisations. Generally, the awareness of the new European-level HTA and the patient involvement in the processes is still very low among patient organisations. Hence, more will have to be done to increase the awareness and readiness to contribute once the guidance documents are published. It was emphasised that one local patient organisation cannot change much and that therefore, collaboration across organisations is essential – mediated by EPF or other umbrella organisations.

The HTA agencies on the national level – as far as existing - are not yet impacted by the change of processes and continue their work like previously. However, the subject is discussed and recognized in policy meetings and patients and/or patient organisations are invited to present their perspectives. This is an opportunity to share slides and coordinate messages. Otherwise, there is a risk of fragmentation by the various inputs.

Some organisations have started to address the issue of capacity building and training. Two training initiatives are available at the European level (funded by European Commission grants under the EU4Health Programme):

1. **EUCAPA**: offers training with different intensity levels including various formats. Registration is open through the website.
   a. A 2-hour online introduction to the European HTA Regulation and Patient Participation Opportunities
   b. An 8-hour online training program in Health Technology Assessment (HTA) for patients and patient representatives (fast track)
   c. A 3-day in-person training program in Health Technology Assessment (HTA) for patients and patient representatives at UMIT TIROL, in Austria (extended training)
2. **HTA4Patients**: HTA4Patients will enhance the education, training and information that EUPATI already provides on HTA via its **Open Classroom** and **Toolbox** empowering patients and patient organisations to play a vital role in the implementation of the new framework.

In early March, the Coordination Group for the EU-HTA published the draft JCA implementing act for consultation (consultation closed on 3 April) and both EPF and PCIG as well as some other patient organisations have submitted their comments. IQVIA has published **a perspective** on it.

Cees Smit reported that he and Annemarie van Eekelen recently published **a book** on cell and gene therapy, in Dutch. The book may also be published in an English version in the future (2025 or later), and will then include a chapter on the European HTA – this is an opportunity for increasing awareness among the community.

Feedback from Participants on the Workshop Experience

What worked well	To be improved
• The workshop led to a better understanding of the issues around PI in HTA, what is currently happening, what can be expected in terms of PI in HTA in EU-HTA • The format is a good opportunity to meet EPF and understand how EPF can support the member organisations	• There was a lot of new content and intensive work for one day. • Conducting this workshop as a 2 x half a day would be better.

Additional considerations

- It would be good for future workshops to bring together POs for the same disease areas on the European level (umbrella) and national level to help them build consensus on how to collaborate better for giving input into the EU-HTA
- Once the processes are more defined, it will be important to understand what exactly happens once individual patient experts are selected from the database. How are they contacted, prepared, briefed, informed, and interacted with? How can POs support them best?
- It was proposed for EPF to produce a 2-page factsheet on PI in HTA, which can be distributed to patient organisations in the member states.
  - What is HTA-R and EU-HTA?
  - What does it mean for patient organisations and patient participation?
- Would it be possible to involve patients / patient organisations in the upcoming mock PICO exercises for the member states?
  - National POs should be informed to contact their national HTA bodies and demand to be involved.

Conclusion

An interactive workshop format was used to simulate – with patient representatives from the primary target disease areas for the European Joint Clinical Assessment – the opportunities and challenges on central and national levels with patient involvement in the upcoming EU HTA systems. Although it was an intensive day of work for the participants, the workshop was considered by the participants informative and an important contribution to activate and align European patient organisations for the new processes. An important role was seen for the European Patients' Forum to engage more patient organisations in such workshops and to sensitize them for the need to prepare for being involved in future HTA processes across Europe. Preparation for patient organisations will include participation in preparatory training formats such as those offered through EUCAPA or HTA4Patients and strengthening of the cross-organisational and trans-European networks for efficient identification of qualified patients who can give input to the upcoming HTAs.

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