



Training Patients in Europe for Effective Contribution to HTA

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- **Introduction: Maria Dutarte (EUPATI)**
- **Patient Involvement in HTA in Europe: Dr. Anke-Peggy Holtorf (HTAi)**
- **Patient Perspective: Lotte Klim (EUPATI Fellow)**
- **Audience Insights**
- **HTA4Patients: Maria Dutarte (EUPATI)**
- **ISPOR's Involvement: Clarissa Cooblall (ISPOR)**
- **Q&A/ Discussion**



PATIENT INVOLVEMENT IN HTA IN EUROPE

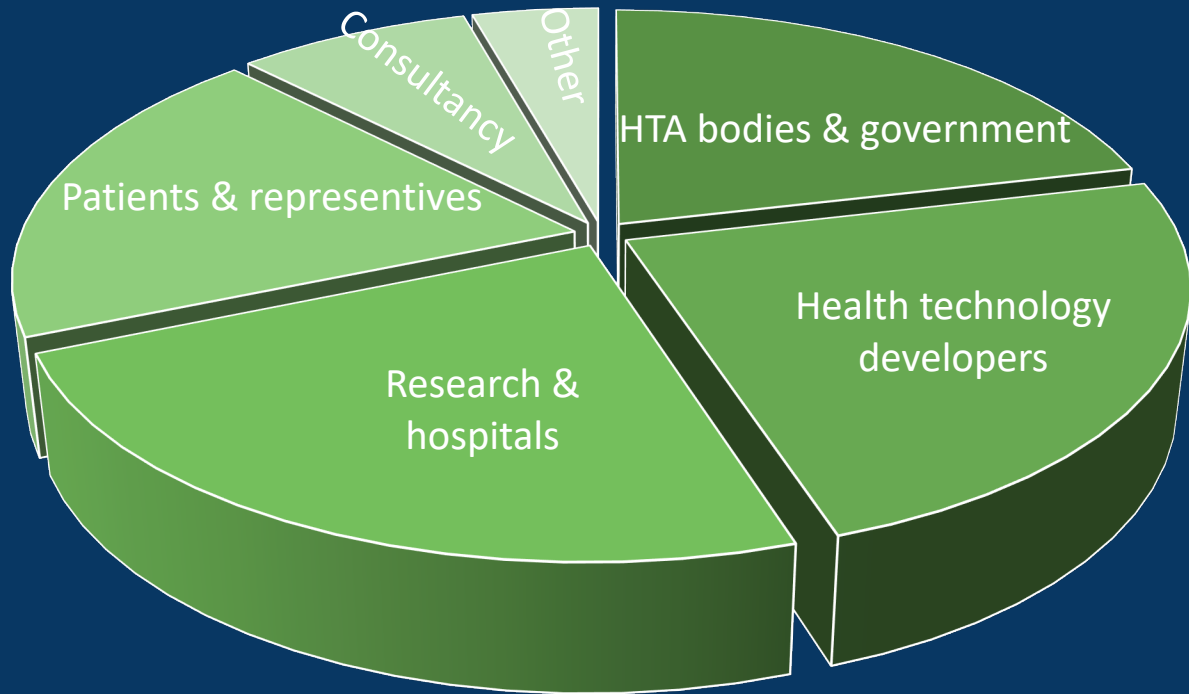


DR. ANKE-PEGGY HOLTORF, PATIENT AND CITIZEN INVOLVEMENT
IN HTA INTEREST GROUP (PCIG) AT www.HTAi.org

WWW.HEALTH-OS.COM / HEALTH OUTCOMES STRATEGIES GMBH (SWITZERLAND)

PCIG: Patient and Citizen Involvement in HTA Interest Group

More than 300 members spread across 43 countries

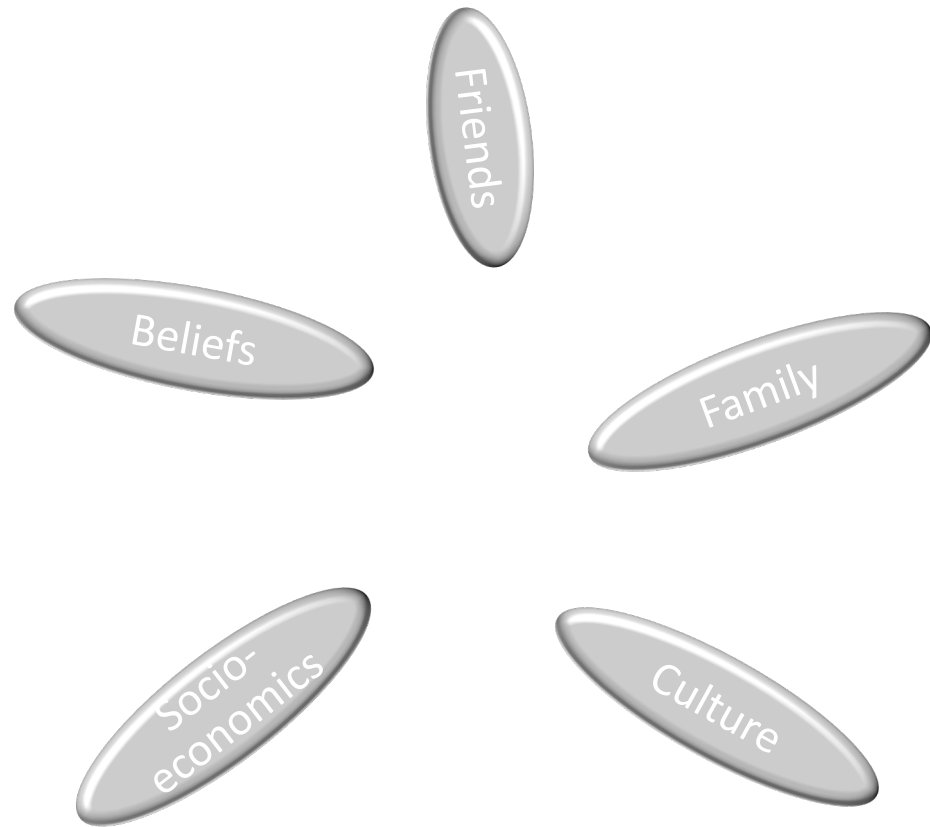


Established 2005

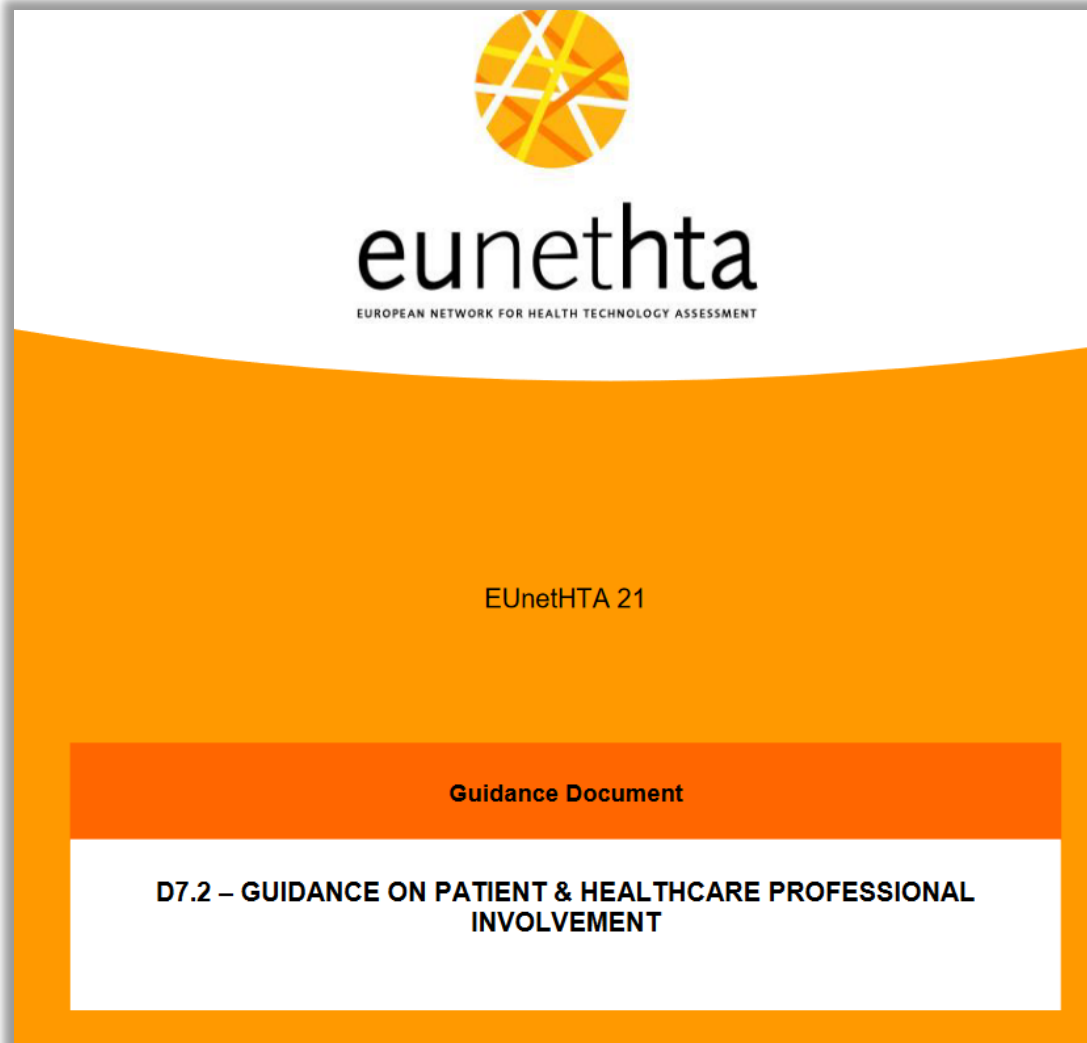
Patient and citizen perspectives improve HTA

- Promoting & developing robust methodologies to incorporate patient/citizen perspectives, and sharing best practice in participation
- Strengthening HTA by systematic incorporation of patient/citizen perspectives
- Supporting countries with limited experience of HTA to elicit and incorporate patient/citizen perspectives

How can Patients help to ensure that HTA determines Value that is relevant?



Guidance by EUnetHTA



The value of patient and healthcare professional involvement in HTA work for Joint Scientific Consultations (JSCs) & Joint Clinical Assessments (JCAs);

How patient and healthcare professional (HCP) input can be gathered;

When and how patient and HCP contributions can be used;

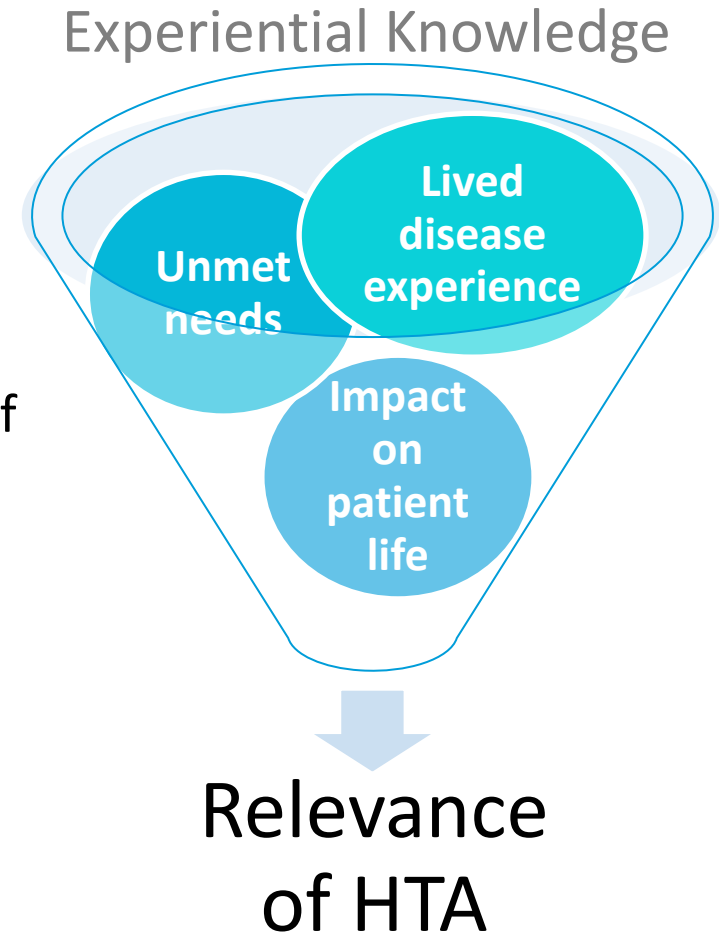
How patient and healthcare professional contributions should be reported.

CONTENT


• Version 1.0, 04. Apr 2023 <https://www.eunethta.eu/wp-content/uploads/2023/04/EUnetHTA-21-D7.2-Guidance-for-involvement-of-patient-and-clinical-expert-in-JSC-and-JCA-v1.0.pdf>

EUnetHTA21 D7.2 Recognizes the Value of Patient Involvement

- **Patients** provide important knowledge about a disease and insights into treatment pathways.
- ... important: are their values reflected (or not) by current treatments, e.g. in study designs or selection of relevant outcomes
- ... direct and indirect impact of the disease + characterisation of the appropriate patient population
- ... development of the PICO (Patient population, Intervention, Comparator(s) and Outcome(s)): Input from patients and HCPs ensures the relevance, legitimacy and transparency of assessments and recommendations.
- ... when taking part in HTA, patients and HCPs gain more understanding on how health technologies are assessed (transparency).



• <https://www.eunetha.eu/wp-content/uploads/2023/04/EUnetHTA-21-D7.2-Guidance-for-involvement-of-patient-and-clinical-expert-in-JSC-and-JCA-v1.0.pdf>



GOOD PRACTICE RECOMMENDATIONS BASED ON 360° STAKEHOLDER EXPERIENCE WITH PATIENT INVOLVEMENT IN HTA IN EUROPE

360° Stakeholder Experience with PI in HTA in Europe



OVERVIEW

Health Technology Assessment (HTA) is a multidisciplinary process to assess health interventions such as medicines, diagnostics and procedures and to recommend their inclusion in publicly funded health systems.

It requires the input of many stakeholders, including patient stakeholders such as patient groups, patient experts, individual patients and care-givers.

Across Europe, countries have established different HTA approaches and processes. This has led to many varied methods for seeking the input of patient stakeholders.

The HTAi 360° Research Project aimed to identify good practices in patient involvement in HTA by seeking the perspectives, experiences and views of HTA organisations, patient stakeholders and the industry.



ACKNOWLEDGEMENTS

The global scientific society HTAi led this research, and in particular the Patient & Citizen Involvement in HTA interest group (PCIG) conducted the research with our partners



PARTNERS

This research would not have been possible without the partnership and support of the European Patients Forum and EUPATI



FUNDING

This research was funded by an unrestricted grant from EFPIA and PhRMA to HTAi



We thank our partners and funders for enabling this research

360° Stakeholder Experience with PI in HTA in Europe

Objectives of the research

Objectives

- Understand the current range of practices in use
- Build a picture of how these practices are experienced and perceived by stakeholders
- Develop concrete and actionable recommendations on valued processes

Outcomes

A experience-based set of recommendations, co-created by affected stakeholder communities to inform patient involvement in HTA practices for the future

Methods – A two-track approach

1 Detailed process perceptions

To gain the deep insights to understand the step-wise patient involvement processes and experiences across Europe from different perspectives

2 Europe-wide perspectives

Co-create a single survey instrument for all relevant stakeholders and capture experiences and perspectives on PI in HTA practices across Europe

3 Co-create recommendations based on outputs of both tracks

Stepwise co-creation workshops with all stakeholder groups

PI = Patient Involvement



OVERVIEW OF KEY RECOMMENDATIONS AT EACH STEP



Click on each of the process steps to be taken to the full recommendations for that step



HORIZON SCANNING

- *More collaboration between HTA organisations and umbrella Patient Organisations*
- *Efficient use of resources*



EARLY DIALOGUES

- *More guidance needed*
- *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 in purpose of patient involvement and useful information*



PATIENT SUBMISSIONS

- *Relevant templates built with patient input*
- *Options for support provided*
- *Multi-lingual 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 & support of 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 PI* and satisfaction with process*



INDUSTRY DOSSIERS

- *Report any patient engagement / involvement that occurred throughout R&D*

** Not all HTA organisations conduct all these steps. These are built from a diverse range of processes identified during the interviews and subsequent workshops

*PI = Patient Involvement



OVERARCHING PROCESSES THAT SUPPORT PATIENT INVOLVEMENT IN HTA



Click on each of the topics to be taken to the full recommendations for that topic

In addition to the discrete steps in the HTA process, the research identified broader processes and activities that are important to consider



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

Note communications recommendations are integrated into all recommendations and do not have a separate section

- *Consistent and two-way communication*
- *Timely communication*
- *Feedback opportunities (between patient stakeholders and HTA: receiving and giving feedback)*

HTA GOES EUROPE: HTAR & PATIENT INVOLVEMENT

HTAR: Involvement of Patient Organisations



- Selected POs as Members of the Stakeholder Network
- POs in capacity as SN members may join Coordination Group meetings as observers
- SN to be consulted on Annual Work Programme and Emerging Technologies, and whenever deemed relevant
- Active multipliers for HTAR - dissemination, awareness etc.

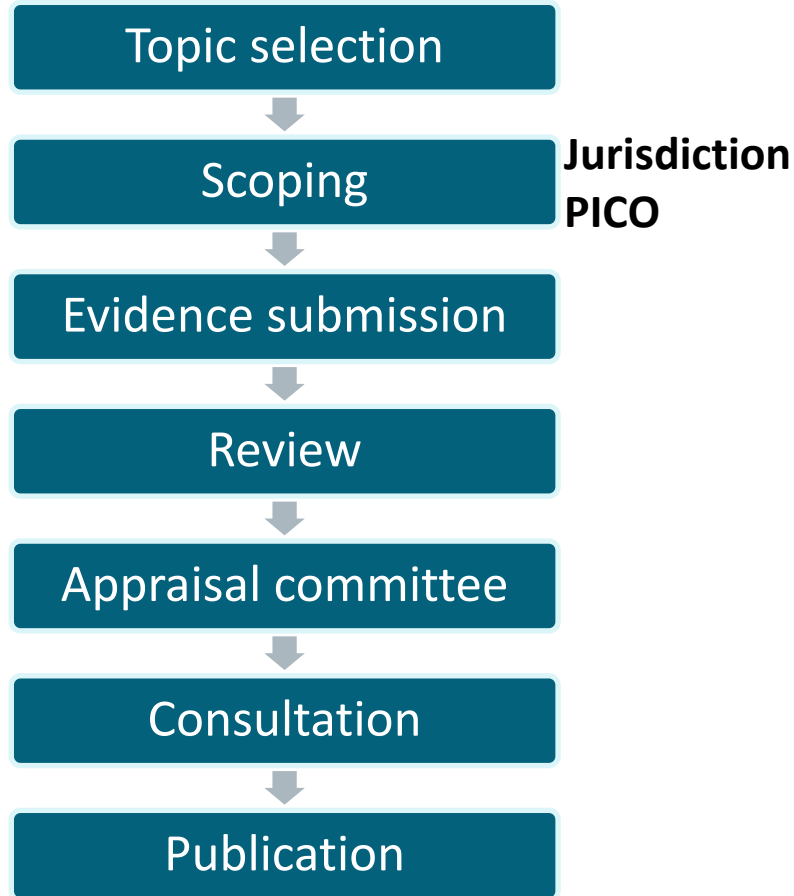
POs as 'Stakeholders'

Stakeholder Network consulted on Annual Work Programme

What will be different with HTAR?

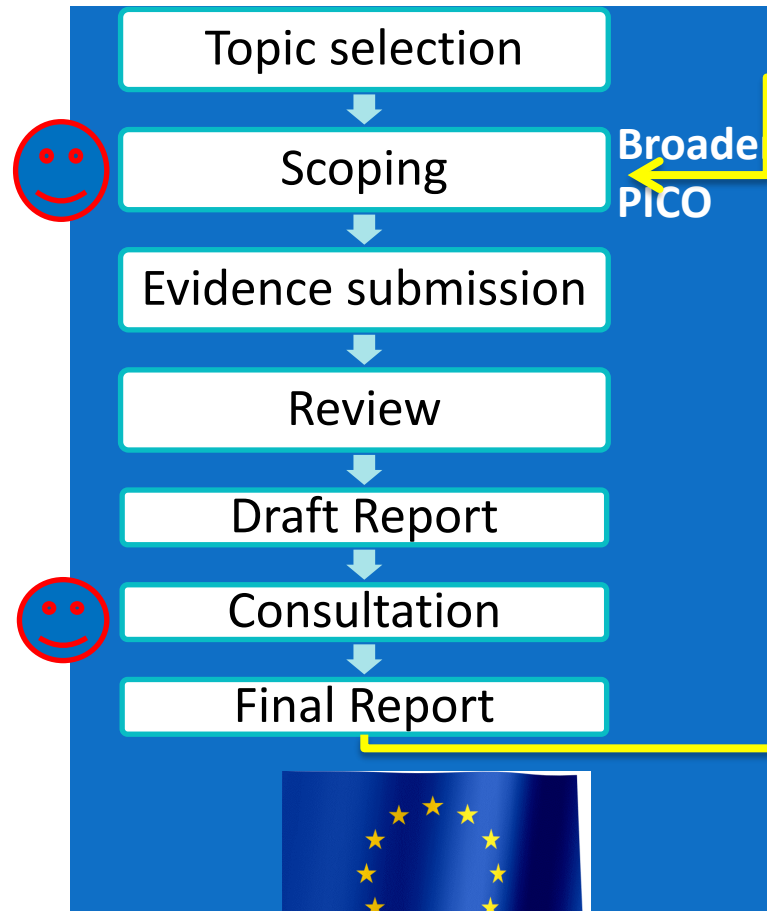
Until now

Q: Does the intervention offer useful, appropriate, and affordable benefits for patients ?

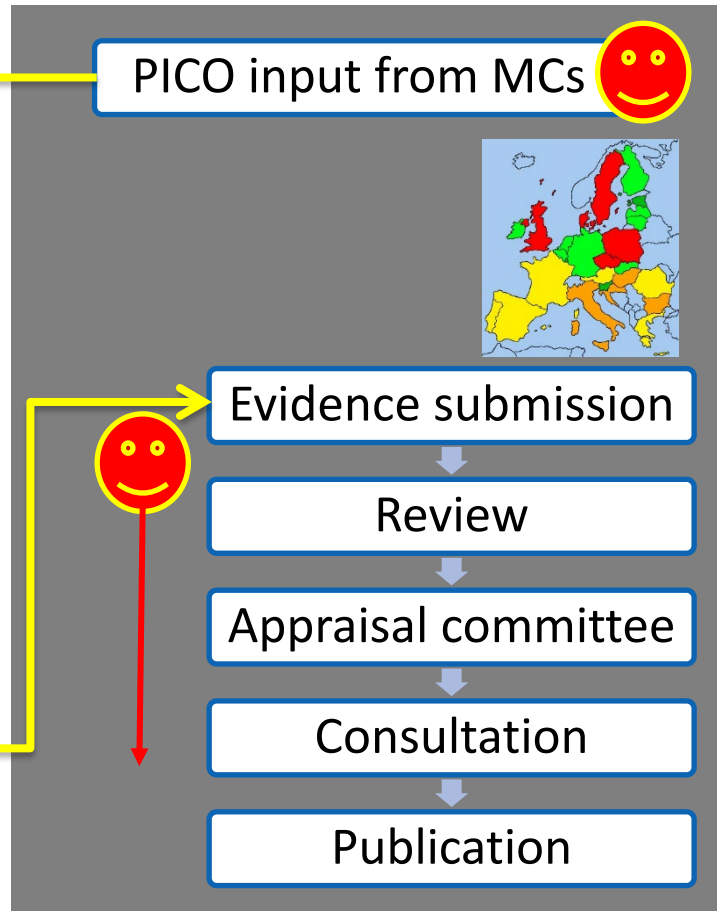


Starting 2025 with phase 1 of HTAR

Q: Does the intervention offer useful and appropriate benefits for patients ?



Q: Does the intervention offer affordable / feasible benefits for patients in this country?



Contact



HEALTH OUTCOMES STRATEGIES

strategies for value in healthcare



www.health-os.com

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The University of Utah
Pharmacotherapy Outcomes
Research Center

Visit our website www.health-os.com and have a look at our Edu-Clips on

- What is Value?
- How is Health-Economics used in Healthcare?
- Generics & Bioequivalence
- Patients in Research and Development
- What is MCDA
- And others in future



Patient Perspective: Lotte Klim (EUPATI Fellow)



Patient Perspectives

Opportunities & Challenges

Lotte Klim
EUPATI Fellow

Patient Involvement in EU HTA



Opportunities

- Stakeholder Network
- Horizon Scanning
- JSC – Joint Scientific Consultation
- JCA – Joint Clinical Assessment

Ethical obligation

Democratic process

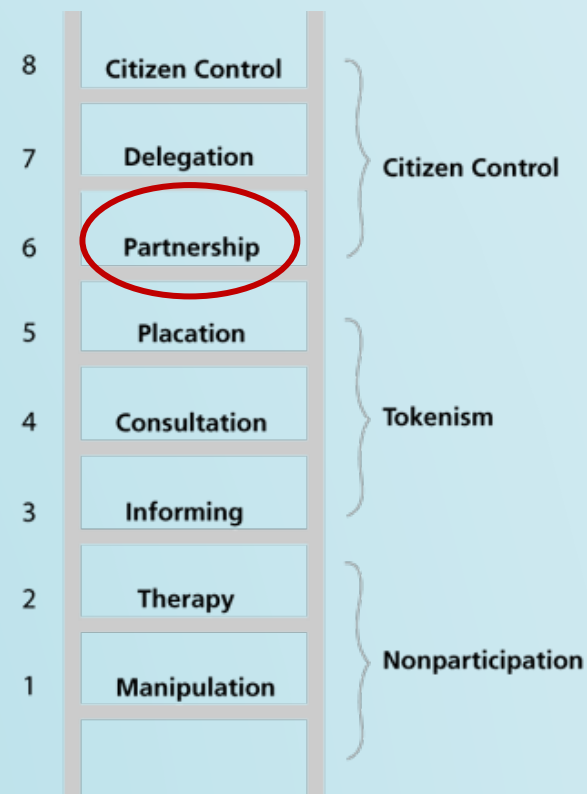
Scientific concepts

Patient impact in EU HTA



Opportunities

- Stakeholder Network
- Horizon Scanning
- JSC – Joint Scientific Consultation
- JCA – Joint Clinical Assessment



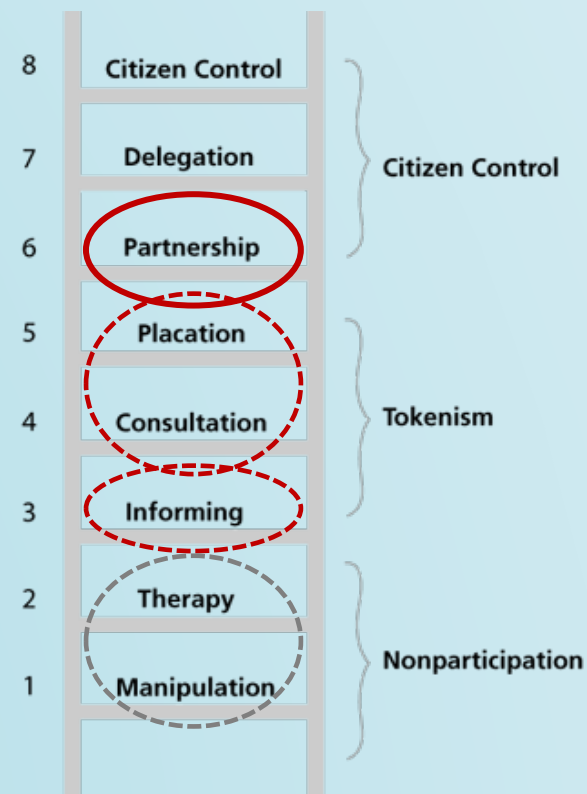
Arnstein's Ladder (1969)
Degrees of Citizen Participation

Patient impact in EU HTA



Opportunities

- Stakeholder Network
- Horizon Scanning
- JSC – Joint Scientific Consultation
- JCA – Joint Clinical Assessment



Arnstein's Ladder (1969)
Degrees of Citizen Participation

Patient perspectives on EU HTA



Opportunities

- **Stakeholder Network**
- **Horizon Scanning**
- **JSC – Joint Scientific Consultation**
- **JCA – Joint Clinical Assessment**



Challenges

- **Risk of climbing down the ladder**
 - Limited influence on EU HTA procedure
 - Input to the PICO survey is a black box
- **‘Irrelevant’ HTA causes delay in access to new technologies**
 - Comparators and outcomes varies

A seat at the table is not enough

Equal partnership requires training

Processes

Conflict of interest

EU HTAR

Member state HTA

ethical - data

organizational
economic
societal

Medicine vs. medical dev.

Definitions & methods

Patient-relevant

Outcomes

Endpoints

Evidence – what and how

Methodologies

Instruments e.g. PROMs

Statistics

Scoping & evidence

Population

Intervention / technology

Comparator

Outcomes

Patient Experiences

Patient Preferences

Key messages on effective patient involvement

- Patients need training at EU and at member state level
- Clear pathway for patients who are not represented in the Stakeholder Network
- Types of input accepted
 - What
 - How
 - When
- Patient organizations should collaborate strategically at EU level



HTA4Patients: Maria Dutarte (EUPATI)

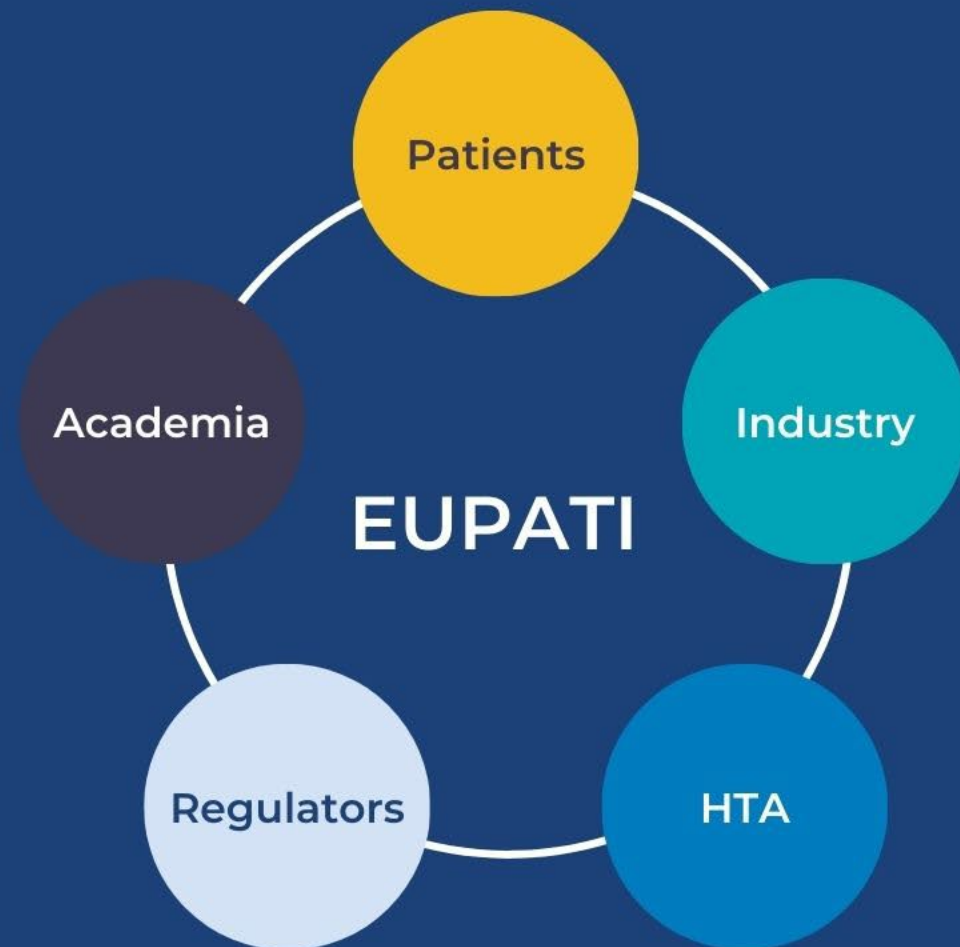





EUPATI

Enhancing patient engagement through education

Education that empowers
patients to engage and
partner with all stakeholders
in the medicines R&D process





Advanced EUPATI Training for Increased Patient Involvement in HTA: HTA4Patients



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Co-funded by
the European Union

Project Outcomes for Patients

- A new online training course on the EU HTAR offered in 6 EU languages by August 2025
- 12 online training sessions for different patient communities from September 2024 onwards
- Identify and train key 'HTA Ambassadors' within the patient community



Co-funded by
the European Union

EUPATI Open Classroom



Introduction to Health Technology Assessment (HTA)

After completing this course, you will be able to:

- Unders...

HTA Bodies and Principles

After completing this course, you will be able to:

- Describe the fundamentals of ...

HTA and Evaluation Methods: Quantitative

After completing the course, you will be able to:

- Understand the differences between HTA

HTA and Evaluation Methods: Qualitative

After completing the course, you will be able to:

- Understand the difference between quantitative and...

Patient Involvement in HTA

After completing the course, you will be able to:

- Understand how patients can contribute ...

EU HTAR

New course
to be added





Sister Project



<https://www.eucapa.eu>





Find Out More



European Patients' Academy on Therapeutic Innovation
EUPATI
www.eupati.eu





ISPOR Patient Initiatives

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14 November 2023

Patients in the Fabric of ISPOR

| Advocacy | Dialogue | Education | Publications | Research | Collaboration | Conference |
|---|--|---|---|--|---|--|
| <ul style="list-style-type: none"> • Responses to call for comments on relevant guidance documents | <ul style="list-style-type: none"> • Regional Roundtables <ul style="list-style-type: none"> • North America • Europe • Latin America • Asia Pacific | <ul style="list-style-type: none"> • ISPOR Short Course on “Patient Engagement in Medical-Product Research and Development” • Educational Webinars <ul style="list-style-type: none"> • 3-part webinar series on recent FDA PFDD Guidance Documents | <ul style="list-style-type: none"> • <i>Themed Issues</i> <ul style="list-style-type: none"> • <i>Value in Health</i> – The Patient Journey • <i>Value and Outcomes Spotlight 2023</i> – Patient Centricity in HEOR | <ul style="list-style-type: none"> • Patient Centered Special Interest Group • Clinical Outcome Assessment Special Interest Group • Rare Disease Special Interest Group • Health Preference Research Special Interest Group • Task Forces | <ul style="list-style-type: none"> • EUPATI • EURORDIS • National Health Council | <ul style="list-style-type: none"> • Programming: <ul style="list-style-type: none"> • Patient experts included in program committee and on session panels • Patient Travel Grants |

ISPOR Resources

- **ISPOR Partnership with EUPATI since 2013**
 - ISPOR developed first HTA module for training program
 - Sustaining Partners Assembly
 - Contributing to content development for the HTA4Patients Training
- **ISPOR Activities and Training**
 - ISPOR Patient Representatives Roundtable – Europe focusing on JCA: Will it improve the patient experience of involvement in HTA?
 - Plenary 3: The Calm Before the Storm? Delivering the New Reality for EU HTA
 - ISPOR Short Course “Unveiling the EU-HTA Regulation: A Deep Dive Into the Joint Clinical Assessment (JCA)”
 - Upcoming event: Real World Evidence (RWE) in JCA
- **ISPOR Special Interest Groups**
 - Patient-Centered
 - Rare Disease
- For more information on ISPOR Patient Initiatives, visit <https://www.ispor.org/strategic-initiatives/patient-initiatives>