HTAi HEALTH TECHNOLOGY ASSESSMENT iNTERNATIONAL

Patient involvement in HTA in Europe

Recommendations from a 360° perspective view of current patient involvement practices

A project of the HTAi Patient & Citizens Involvement in HTA Interest Group
https://htai.org/patient-and-citizen-involvement/

Authors:
Anke Peggy Holtorf: anke.holtorf@health-os.com
Neil Bertelsen: nb@neilbertelsen.com

Oct-2023

Foreword

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

Contents

Click on each of the sections to be taken directly to that part of the report

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 and 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)*

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ABOUT THIS RESEARCH

Objectives of the research

Purpose

- Generate clear and actionable recommendations on valued patient involvement processes used in HTA through a 360° analysis of current processes, experiences and perceptions

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

Outputs

- Published methods of current practices and experience of them for ongoing benchmarking
- General *360°* perceptions of patient involvement in HTA across Europe
- Analysis of evidence to identify valued practices to take forward and those to evolve
- A series of co-created recommendations for further evolution of the processes

Outcomes

A clear evidence-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

1. Scoping literature review
2. Interviews on process and experiences by participants: with HTA organisations, patient experts or patient organisations, industry representatives

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

1. Virtual roundtable to identify domains to include
2. Draft survey, pilot and validate, dissemination across Europe to all stakeholders

3 Co-create recommendations based on outputs of both tracks

Stepwise co-creation workshops with all stakeholder groups

PI = Patient Involvement

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CONTEXT

Patient Involvement in HTA - Foundations

CONTEXT HISTORY

1970s

USA Office of Technology Assessment (OTA) reported that decisions about the use of new health technologies should consider the impacts and implications for patients, patients' families, society, the healthcare system, legal and political systems and the economy[1] *(OTA was closed in 1995)*

1980s

Denmark and Sweden begin setting up organisations to undertake systematic assessments of all forms of health interventions to inform policy and practice and the modern use of Health Technology Assessment (HTA) was formed[2]

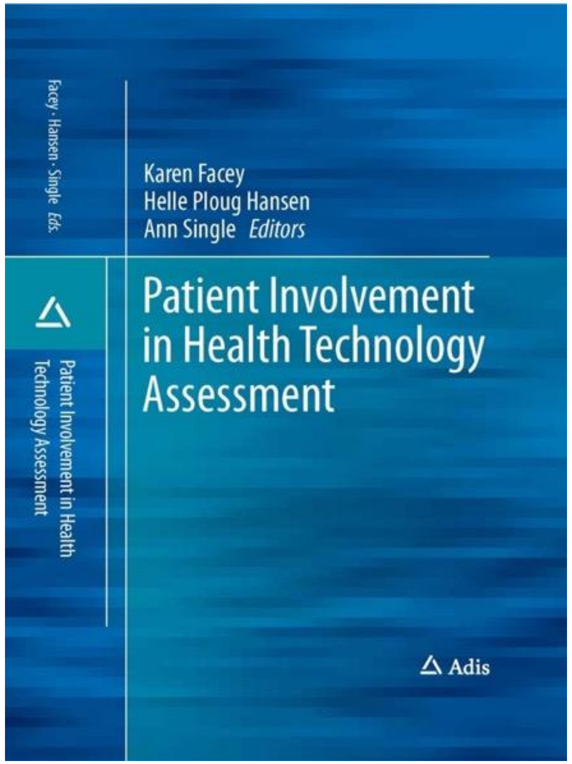
1990s-2000s

The Danish Centre for HTA published a detailed handbook that covered the detailed steps of planning and conducting an HTA. This included sections on primary research to understand different stakeholder perspectives, and considerations of patient aspects[3]

The 2000s also saw the rise of a more rapid form of HTA that was increasingly being used to inform reimbursement and coverage decisions of new health technologies[4]

2010s – 2020s

HTAi's Patient & Citizens Involvement in HTA group elaborated and published a range of papers, guides and templates to assist in patient involvement practices independent from jurisdictions. This included in 2014 conducting an international Delphi process to create consensus on the key values and standards that should underpin patient involvement in HTA.[5]



Patient Involvement in Health Technology Assessment: Springer
DOI 10.1007/978-981-10-4068-9

1: Patient Involvement in HTA book (K. Facey et al) pp4
2: Patient Involvement in HTA book (K. Facey et al) pp7
3: Patient Involvement in HTA book (K. Facey et al) pp9
4: Patient Involvement in HTA book (K. Facey et al) pp.10
5. HTAi Values and Quality Standards for Patient Involvement in HTA https://tinyurl.com/2p8zhvzb

HTAi Values and Standards

PURPOSE

The HTAi Patient & Citizens Group (PCIG) have previously developed a range of global values and quality standards to inform good practice in patient involvement in HTA.

For this research project, these values and standards were used to guide discussions during co-creation workshops on potential recommendations emerging from this research.

VALUES

RELEVANCE

Patients have knowledge, perspectives and experiences that are unique and contribute to essential evidence for HTA.

FAIRNESS

Patients have the same rights to contribute to the HTA process as other stakeholders and have access to processes that enable effective engagement.

EQUITY

Patient involvement in HTA contributes to equity by seeking to understand the diverse needs of patients with a particular health issue, balanced against the requirements of a health system that seeks to distribute resources fairly among all users.

LEGITIMACY

Patient involvement facilitates those affected by the HTA recommendations/decision to participate in the HTA; contributing to the transparency, accountability and credibility of the decision-making process.

CAPACITY BUILDING

Patient involvement processes address barriers to involving patients in HTA and build capacity for patients and HTA organizations to work together.

HTAi Values and Quality Standards for Patient Involvement in HTA
https://tinyurl.com/2p8zhvzb

HTAi Values and Standards

STANDARDS

GENERAL HTA PROCESS

HTA organizations have a strategy that outlines the processes and responsibilities for those working in HTA and serving on HTA committees to effectively involve patients.

HTA organizations designate appropriate resources to ensure and support effective patient involvement in HTA.

HTA participants (including researchers, staff, HTA reviewers and committee members) receive training about appropriate involvement of patients and consideration of patients' perspectives throughout the HTA process.

Patients and patient organizations are given the opportunity to participate in training to empower them so that they can best contribute to HTA.

Patient involvement processes in HTA are regularly reflected on and reviewed, taking account of the experiences of all those involved, with the intent to continuously improve them.

FOR INDIVIDUAL HTAs

Proactive communication strategies are used to effectively reach, inform and enable a wide range of patients to participate fully in each HTA.

Clear timelines are established for each HTA with advance notice of deadlines to ensure that appropriate input from a wide range of patients can be obtained.

For each HTA, HTA organizations identify a staff member whose role is to support patients to contribute effectively to HTA.

In each HTA, patients' perspectives and experiences are documented and the influence of patient contributions on conclusions and decisions is reported.

Feedback is given to patient organizations who have contributed to an HTA, to share what contributions were most helpful and provide suggestions to assist their future involvement.

Questions remain

OPEN QUESTIONS THIS RESEARCH WAS AIMED TO ANSWER

METHODS AND THEIR IMPLEMENTATION

Much has been written about the methods of HTA and the methods of patient involvement in HTA. Many HTA organisations now have official patient involvement processes that have become part of the overall process of assessing new and existing health technologies. No two HTA organisations have identical ways of doing this and there remains a lack of evidence on the comparative success of these methods in involving patients. Building from the work of the HTAi Values & Standards, this research aimed to answer the questions with a focus on countries in the region of Europe[1]:

- Which methods and forms of implementation are considered good practice by patients, the technology developer and the HTA body?
- Are there methods used that have not been documented within the HTA process, such as informal engagements, planning steps, adaptations to processes to address emerging stakeholder and HTA needs?

EXPERIENCE

For internal process improvement, some HTA organisations do ask for feedback from stakeholders involved in their processes. These do provide some insights into the processes that are valued by stakeholders and identify areas of improvement. However, there remains a lack of research that compares experience of multiple stakeholder groups across various HTA organisations. This research aimed to answer the questions:

- How do stakeholders experience the methods used to involve patient stakeholders[2] in an HTA?
- How could current methods and their implementation be improved to overcome any barriers identified by the stakeholder experiences?

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[1] Europe as defined by WHO

[2] For definitions of different types of patient stakeholders, see Hunter et al. 2018, Front. Med., Sec. Regulatory Science

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A changing landscape

EUROPEAN HTA REGULATION

AN OPPORTUNITY FOR REFLECTION

With the advent of the HTA Regulation in the European Union, bringing a new regional approach to some parts of the HTA process, there is an opportunity to reflect on the practices observed and experienced in national HTA processes. In particular, there are lessons to be learned from processes that are well regarded by those that take part in them, and to mitigate against processes that are less well experienced. This research concentrated on the European region to answer these questions:

- What can we learn from the experiences of those that have been involved in HTA processes?
- What steps, structures and resources can be applied to improve patient involvement in HTA?
- Are there methods that should be avoided or improved, as they are poorly experienced by those involved in HTA?
- Is reconsideration needed for the way that some standard involvement methods are implemented?
- How can the needs and expectations of stakeholders be better understood and aligned?

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METHODS

Methods – A two-track approach

1 Detailed process perceptions

To gain the deep insights needed to understand the step-wise patient involvement processes used across Europe

1. Literature review
2. Partner with 3-5 HTA organisations[1]
3. Identify up to three assessments for each body from 2019/2022 with PI[2] (formal or informal)
4. Detail the PI steps, timelines and outputs
5. Interview patient stakeholders involved
6. Interview industry representatives involved

2 Europe-wide perspectives

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

1. Virtual scoping workshop to identify domains to include
2. Draft survey, pilot and validate
3. Translation to 5 languages with post translation validation check
4. Promotion and recruitment of the survey across all relevant networks to stakeholders with experience in PI in HTA (Europe)

Extraction, analysis, and draft report

3 Co-create recommendations based on outputs of both tracks

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[1] The selection of interviewees was based on willingness to participate

[2] PI = Patient Involvement

Track 1: Interview structure

A discussion guide, built from the Values & Standards components and a Scoping Workshop was used to conduct the interviews. This was adapted slightly for each stakeholder group

1: Overview of patient involvement methods used / experienced in a particular case

2: Step-by-step experience of each stage of the patient involvement process

3: Reflections on the overall experience

4: Areas for improvement

5: Opinion on how the interviewee thought other stakeholders experienced this process

6: Identifying additional methods that may sometimes be used but were not part of this case

Track 1: Case studies used in the interviews with HTA organisations

EUnetHTA	PTJA17: Elivaldogene Autotemcel (Eli-cel) for Treatment of Cerebral Adrenoleukodystrophy (CALD) [2021] PTJA12: Glasdegib (in combination with low-dose cytarabine) [2020] PTJA06: Polatuzumab vedotin in combination with bendamustine and rituximab [2020]
SESCS, Spain	Neonatal screening for severe combined immunodeficiency [2019]
NICE, England	TA757, Cabotegravir with rilpivirine for treating HIV-1 [2021/22]
HAS, France	UPLIZNA / Inebilizuma [2022] REBLOZYL (luspatercept) [2021] RONAPREVE - Casirivimab/imdevimab [2021]
HTW, Wales	Strategies for Relatives (START) intervention to improve the mental health of carers of people with dementia [2021]

Track 2: Partner workshop on themes

WORKSHOP

A virtual scoping workshop using the GroupMap online collaboration tool was hosted by HTAi to gain multi-stakeholder initial insights from EUPATI, EPF, PhRMA and EFPIA on the themes identified in the literature search.

The outputs of this workshop were used to develop the outline for the online survey and to plan the discussion guide for the interviews.

KEY QUESTIONS EMERGING (SUMMARY)

REASONS / DRIVERS

- Is there alignment across stakeholders on the rationale for PI in HTA?
- Why do patient stakeholders want (or not want) to be involved in HTA processes?

PROCESS

- How is patient input seen from each stakeholder perspective?
- Which financial and human resources are allocated to patient involvement?

METHODS

- Are there knowledge and training gaps that need to be filled?
- Is there clear guidance on the appropriate methods to use?

IMPLEMENTATION

- Does the HTA body make it easy for patient stakeholders to understand their role?
- Is there a feedback/learning mechanism for continuous improvement of PI?

IMPACT

- How should we define and measure impact of patient involvement in HTA?
- What are the impact priorities for each stakeholder?

PI = Patient Involvement

Track 2: Survey development

SURVEY

An online survey was developed based on the themes identified in the scoping literature review and any additional themes emerging from the scoping workshop. This was available and promoted between 29th April 2022 and 7th September 2022.

The survey was provided in the following languages:

- English
- German
- French
- Italian
- Spanish
- Polish

STRUCTURE OF THE SURVEY

INTRODUCTION

- The rationale and purpose of the survey

OPINIONS

- Respondent's opinions on the rationale for patient involvement in HTA and opinions in general on the ideal process for patient involvement in HTA

EXPERIENCE OF PATIENT INVOLVEMENT IN AN HTA PROCESS

- **PRE-HTA:** Experience of the involvement process before the HTA was conducted
- **HTA:** Experience of the involvement in the actual assessment process
- **EVALUATION:** Experience of any feedback or evaluation processes after the HTA

Track 2: Survey workflow

- Scoping Literature review: Publications on processes of patient involvement in HTA
- Multi-stakeholder scoping workshop on survey content and design (domains, items)
- Translation (English, Italian, French, Spanish, Polish, German), programming
- Survey testing with patient representatives, revision
- Survey activation and roll-out (5 months collection phase)
- Evaluation, reporting

Section	No. of questions	Content	Target group
A: Introduction	9	Information on responder: country, responder type, with/without experience with Pi in HTA	All
B: Opinions	7	Opinion related to patient involvement in HTA	All
C: Pre-HTA	13	Experience with preparation of patients for participation in HTA	With experience of PI in HTA
D: HTA	17	Experiences / interactions during the HTA	With experience of PI in HTA
E: Evaluation	7	Experience related to evaluation; motivations, accelerators/inhibitors	With experience of PI in HTA; last question for all

PI = Patient Involvement

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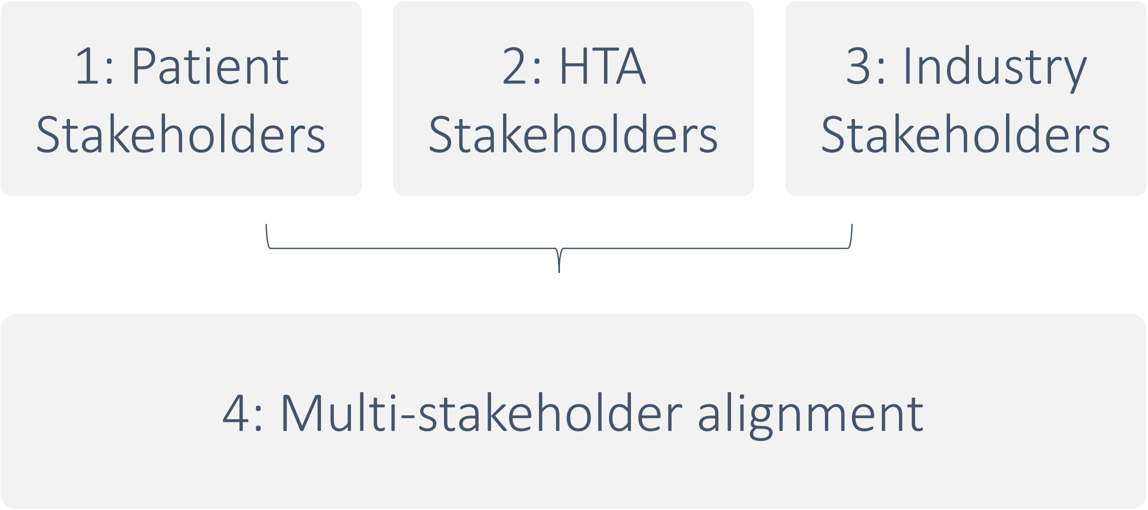
Track 3: Co-creation workshops

Four workshops held



1: Patient Stakeholders

2: HTA Stakeholders

3: Industry Stakeholders

4: Multi-stakeholder alignment

ACKNOWLEDGEMENTS: HTAi would like to thank all attendees of the interviews and workshops who all provided excellent insights and feedback

Contributors

HTA (10)

- EUnetHTA
- HAS, France
- SESCS, Spain
- HTW, Wales
- NICE, England
- KCE, BE (only in consensus workshops)

Industry (9)

- Novartis
- UCB
- Roche
- Bluebird Bio
- Takeda
- EFPIA

Patients, Public (12)

- Appraisal committee Lay Member of NICE, England
- Alzheimers' Soc, Wales, Wales
- AFM-Téléthon, FR
- Olijf, Patient organization for women with gynaecological cancer, NL
- EPF, BE
- EUPATI, DM, CZ, GR
- Myeloma Patients, EU

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Limitations and lessons

LIMITATIONS

Patient Stakeholders
In many cases, the patient stakeholder who had taken part on the HTA was no longer with the patient association, and there was a lack of retained knowledge in the association to share experiences. In these cases, patient stakeholders were found that had experience of HTA with the HTA body in question, but not from the particular case study under review

Industry Stakeholders
In some cases, the industry stakeholder who had taken part on the HTA was no longer with the company
In these cases, a stakeholders were found that had experience of HTA with the HTA body in question, but not from the particular case study under review

HTA Stakeholders
The plan was to include 5 HTA organisations. Not all HTA organisations approached were able to take part in the interviews, in which case an alternative HTA body was approached and enrolled into the research. Interviews were done with 1-2 HTA representatives that usually were the 'champions' of patient involvement in the respective agency

LESSONS

RETAINED KNOWLEDGE & EXPERIENCE
Turnover of patient participants or staff in the patient associations and industry is leading to a loss of retained knowledge and experience of patient involvement in HTA.

While industry often has systems to retain knowledge, and document experiences of individual HTA processes, patient associations often do not.

This is leading to a cycle of learning and experience building that is needed in patient associations that is a drain on their precious resources.

More needs to be done to support patient associations in documenting experiences and retaining knowledge of HTA processes.

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RESULTS OF TRACK 1 AND 2: GENERAL INSIGHTS

Track 1: Interviewee demographics

Interviews held with stakeholders who have experienced patient involvement in HTA in Europe

Practitioners from five HTA organisations:

- EUnetHTA
- Spain (SESCS)
- England (NICE)
- Wales (HTW)
- France (HAS)

Patients with experience of being involved with the following agencies:

- Patients involved with NICE (England)
- Patients involved with EUnetHTA
- Patients involved with ZIN (NL)
- Patients involved with HAS (FR)

Industry Stakeholders involved in a HTA process with patient involvement at:

- EUnetHTA
- HAS (FR)
- NICE (England)
- ZIN (NL)

LIMITATIONS: In contrast to the original plan, it was not possible to reach all of the different stakeholders for the HTA case that had been selected by the agencies. Some patients preferred to give only written input.

Track 2: Survey demographics

A total of 168 responses from European countries were received

HTA practitioners (n=33)

Patient Stakeholders (n=75)

Industry Stakeholders (n=42)

Providers (n=5)

Academics (n=7)

Others (n=5)

Of these, almost 57% had experienced an HTA with patient involvement (n=95)

The responses related to 32 European countries

The highest number of responses from:
United Kingdom (20), Spain (10), Luxembourg (10), Italy (10), France (8), the Netherlands (8), Germany (8) and Ireland (7)

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Track 1: SCOPING LITERATURE REVIEW

PURPOSE

To identify the key themes and topics to be included in both the interviews and the survey, a literature review was performed.

Published literature on patient involvement in HTA was searched to find reports of:

- What practices are used in patient involvement
- What challenges still exist
- Details of processes, methods and general implementation

KEY FINDINGS

Search in Pubmed / EMBASE revealed 90 potential references published between 2016 and 2021. The title and abstract screen of each reference was analysed.

58 papers were excluded (out of scope)
32 papers were included (in scope)

THEMES IDENTIFIED

- **Rationale/driver** of patient involvement in HTA
- The **process** of patient involvement in HTA
- Specific **methods** used during the involvement
- **Implementation** of patient involvement processes
- The **impact** of patient involvement

Track 1: SCOPING LITERATURE REVIEW

Culyer AJ. Use of evidence-informed deliberative processes – learning by doing:
Comment on “Use of evidence-informed deliberative processes by health technology assessment agencies around the globe.”
Int J Health Policy Manag. 2020; In Press. doi:10.15171/ ijhpm.2019.11

> *HTA will not need a fundamental change but rather a refocus on what matters to make fair and reasonable recommendations. In addition, a reasoned prioritization of interventions on which to make recommendations will be needed for HTA to contribute as much as possible to the creation of global value for patients and population served.*
>
> ***There is a need of ‘learning from doing what works best’ and that ‘In the absence of a theory of processes, we need to encourage imaginative innovation and much sharing of experience…. (from) which some general principles might eventually be inferred.’***

Overview of themes

Reasons/Drivers

- for Patient Involvement (Policy or 'HTA' perspective)
- for being involved (patient perspective)

Process

- Stages for PI in HTA
- 'Intensity' of inclusion
- Accountability
- Resources

Methods

- Type of Patients
- Role of Patient Representatives
- Reaching patients
- Involving patients
- Informing patients
- Evaluation of process
- Evaluation of impact

Implementation

- Accelerators
- Barriers
- Feasibility / Context / Evaluation / Sustainability

Impact

- Observations of Impact
- Reasons for Lack of Impact

PI = Patient Involvement

Reasons/Drivers

for Patient Involvement (Policy or 'HTA' perspective)

for being involved (patient perspective)

POLICY / HTA PERSPECTIVE

The public policy literature on legitimacy distinguishes between legitimacy related to:

- *INPUTS - democratic participation*
- *THROUGHPUTS - fair and transparent processes*
- *OUTPUTS - performance and effectiveness of policy outcomes*

Reasons/Drivers

for Patient Involvement (Policy or 'HTA' perspective)

for being involved (patient perspective)

PATIENT PERSPECTIVE

The patient focused literature outlines the following themes:

- *Frustration of patients (and industry) with purely 'technocratic' decision making.*
- *Current process was seen as too opaque, and the substantive outputs, which patient groups viewed as punitive and missing key patient-relevant information.*
- *Concern about choice of therapies and individual impacts that HTA processes are not necessarily designed to capture.*
- *Opportunity to provide HTA organisations with information on the experience of living with a condition or using existing treatments and the treatment under assessment.*
- *Provide information on the value and impact of the treatment from a patient perspective, to help agencies understand unmet needs, provide input more generally, or help to set the content of a health insurance package.*

METHODS OUTLINED IN THE LITERATURE

Methods

Type of Patients

Role of Patient Representatives

Reaching patients

Involving patients

Informing patients

Evaluation of process

Evaluation of impact

- *Type of Patients*
- *Role of Patient Representatives*
- *Reaching patients*
- *Involving patients*
- *Informing Patient participants (on the process, technology, context)*
- *Materials provided*
- *Training*
- *Evaluation*
- *Process*
- *Impact*

MOST IMPORTANT BARRIERS TO OVERCOME WHEN INVOLVING PATIENT STAKEHOLDERS IN HTA

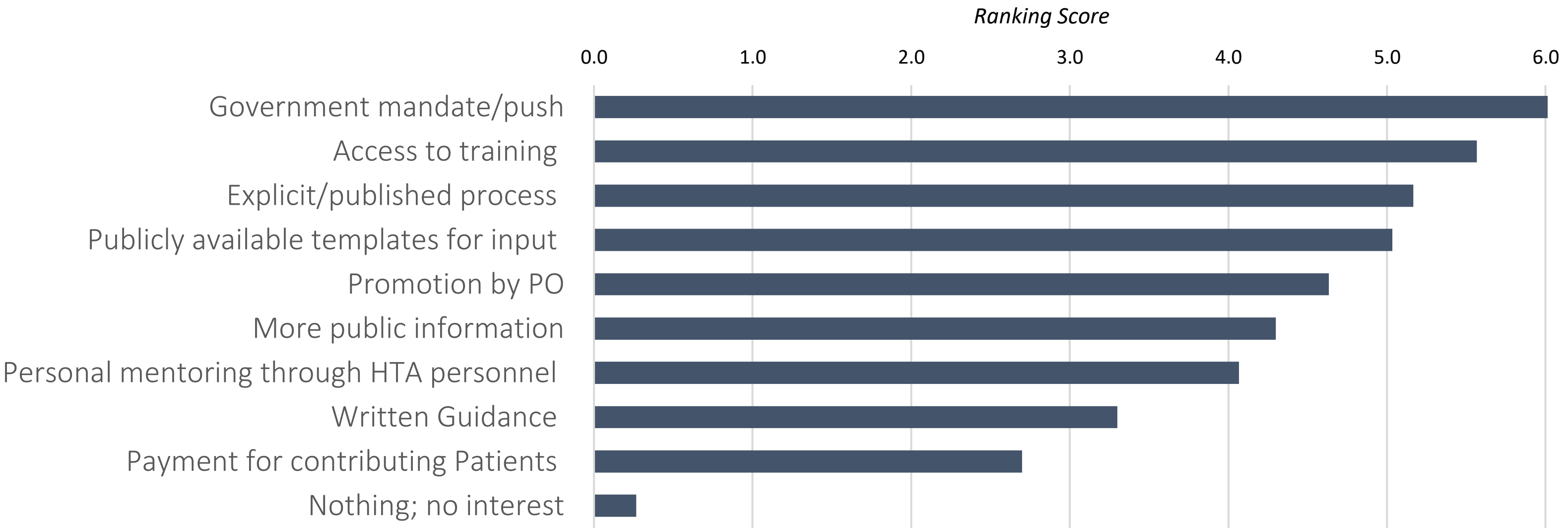
Relevant patients **do not know about the opportunity**

Patient stakeholders **do not know how to get involved**

Patient stakeholders do not have sufficient knowledge (**capability**) as well as sufficient resources (**capacity**) to be able to provide input

Some patient organisations may also not consider this activity to be a **priority**

All patients who had not participated in an HTA were asked, what would motivate them to do so in future (right graphic)



PATIENT STAKEHOLDER SATISFACTION WITH THE INVOLVEMENT ★☆☆

Satisfaction with…

The technology-related information

☺ 37% ☹ 47%

The information on what was asked of them in their involvement (briefings)

☺ 26% ☹ 42%

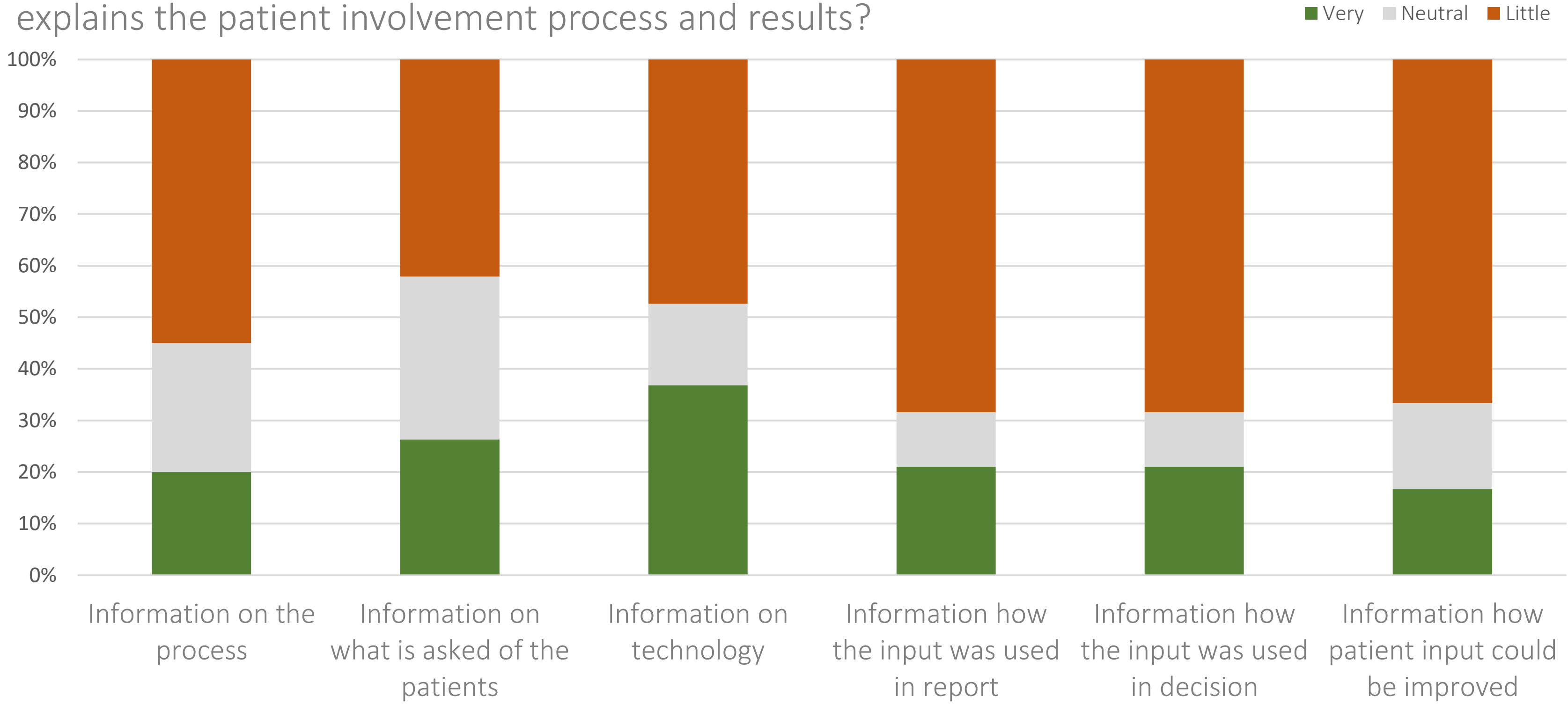
Information relating to the HTA process

☺ 20% ☹ 55%

Information explaining how their input was used in the HTA report, and on how their input was used in the decision

☺ 21% ☹ 68%

How satisfied were you with the different types of information provided that explains the patient involvement process and results?



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RESULTS OF TRACK 3: OVERALL FINDINGS & RECOMMENDATIONS

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KEY STEPS TO CONSIDER IN PATIENT INVOLVEMENT PROCESSES[1]

Interviewees identified the detailed process steps that need to be considered when conducting patient involvement in HTA. These were validated and built upon in the stakeholder workshops. The steps identified form the structure of this report.

HORIZON SCANNING	*Identifying upcoming technologies likely to undergo a HTA and of alerting stakeholders to an upcoming HTA in order to allow stakeholders to prepare*
EARLY DIALOGUES	*Also known as 'scientific advice'. Engagement between a HTA body and a technology developer to discuss evidence generation plans*
PATIENT INVOLVEMENT PLANNING	*In preparation for an upcoming HTA, the HTA body will plan how it will reach out to and promote the opportunity of involvement*
CALL FOR PATIENT INPUT	*A call for patient participation is published at the start of a specific assessment and outreach to relevant patient stakeholders occur*
SCOPING	*At initiation of each HTA, the scope (and PICO*) of the research for an upcoming HTA is defined*
PATIENT SUBMISSIONS	*Patient stakeholder written submission detailing the patient perspective and evidence relating to an assessment/appraisal*
INTERVIEWS / FOCUS GROUPS	*When HTA organisations conduct interviews or focus groups with patient stakeholders instead of (or as well as) a written submission process*
REPORTING OF THE PATIENT INPUTS	*How the patient input is detailed in the report alongside any consideration of this input in the evidence assessment or any deliberations*
APPRAISAL	*A deliberative meeting in which the HTA body, the manufacturer and expert stakeholders including patient stakeholders attend*
EVALUATION AND FEEDBACK	*When the impact of patient input on the HTA is evaluated and communicated to those who were involved and published*

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

[2] PICO – Population, Intervention, Comparator, Outcomes are defined to frame the research question of each HTA

OVERARCHING PROCESSES THAT SUPPORT PATIENT INVOLVEMENT IN HTA

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	*How patient stakeholders input into the HTA body organisation and general processes, outside of a specific assessment. This includes, for example, standing committees, boards and advisory groups that input into a HTA body's process, methods and general approach*
GUIDANCE AND TRAINING	*The general guides and educational resources and events provided by a HTA body to patient stakeholders to explain the process, their role in the process, and to provide tips and advice on presenting their evidence during the process*
COMMUNICATION	*The process, types and quality of communication to patient stakeholders at all points through the the assessment process*

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PATIENT INVOLVEMENT AT THE ORGANISATIONAL LEVEL

DESCRIPTION

Patient stakeholder standing committees, boards and advisory groups that input into a HTA body's process, methods and general approach

STEPS*

- Establish a patient involvement advisory board or standing group
- Open call for members via advertised positions
- Are independent of the HTA body and see all planned HTAs
- Regular meetings (e.g. quarterly)
- Clear focus to advise of patient involvement issues
    - E.g. Relevance of a technology to patients
    - E.g. Types of interaction needed
- Some members part of appraisal panel, other members part of operational group

WHAT WORKS WELL

- ● A patient involvement advisory board helps to assure quality, consistency, and effectiveness of Patient Involvement
- ● Independence of advisors ensures that processes, activities and decisions are meaningful to patients and the public
- ● Board members bring a range of different perspectives into the HTA body and advise on how to motivate patient organisations to be involved
- ● Members are well networked to the patient and patient advocate community and so can advise and assist with recruitment to a HTA

WHAT WORKS LESS WELL

- Nothing identified from the interviews

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

○ *HTA* ○ *Industry* ○ *Patients/Citizens*

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PATIENT INVOLVEMENT AT THE ORGANISATIONAL LEVEL

1. HTAs 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 HC system
    a. A process exists to gain regular feedback from the board, to advise on organisational processes as well as specific advice for upcoming HTAs that further strengthens patient involvement practices
    b. The terms of reference of such a board is published and accessible
    c. The expected composition of the 'patient involvement board /committee' including the expected competencies and qualifications are defined and published
2. For these boards, an open call is used to advertise for positions to ensure a diversity of applicants
3. The members of such a committee / board are trained and prepared appropriately for their task
4. All processes for patient involvement are transparent and each type of involvement is defined along the HTA process. The board may advise on when & how to involve patients in each specific upcoming HTA (example checklist Wales)
5. Overall quality assurance for PPI is one of the responsibilities of the board

PPI = Patient and Public Involvement

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GUIDANCE AND TRAINING PROVIDED

DESCRIPTION

Published guidance and training materials and courses provided by the HTA body or third parties that explains the process, the patient stakeholder role and the evidence to bring into HTA

STEPS*

- Regular training seminars provided by the HTA body
- Specific guidance on collecting the evidence from the patient community for submissions
- Individual support for a particular HTA offered through preparatory calls and guidance calls
- Published guidance and slide-sets on HTA body websites explaining process and roles
- Use of patient language summaries (Summary Information for Patients) to ensure contributors have the relevant information
- Templates (e.g. submission questionnaires) with guidance on how to answer each question

WHAT WORKS WELL

🔵 Training and guidance that ensures participating patient organisations know what is expected

🔵 Targeting promotion of training to patient organisations in disease areas with upcoming HTAs

🔵 Invitation to input into a HTA includes links to guidance and training so patient organisations can go straight to it

🟢 Industry and third-party training on the HTA process and how patient input is important to HTA

🟡 Preparatory support by the patient organisation or umbrella organisation for those attending HTA meetings

WHAT WORKS LESS WELL

🟡 People need a lot of support to be well prepared for giving input (especially if they have limited time in appraisal committee meetings) – the support provided is not always enough

🟡 Often, the details of the HTA are not known to patients

🟢 General awareness of patient organisations about patient stakeholder role in HTA is too low

🔵 Guidance for assessor on how to conduct and use patient stakeholder involvement is too general and leads to high variability

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

🔵 *HTA* 🟢 *Industry* 🟡 *Patients/Citizens*

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GUIDANCE AND TRAINING PROVIDED

GUIDANCE FOR PATIENT STAKEHOLDERS:

1. Education and information is easily accessible and explains, in a timely and fit-for-purpose manner, the principles of HTA, the process, the purpose and intended use of patient input, and how to give relevant input. This includes the differentiation between HTA and regulatory process.
2. Training and information is available in relevant local & plain language versions
3. Guidance on collecting evidence from patient communities is available for patient organisations (best practices, privacy and data management, burden of disease data)
4. Training or information given to the patient community that explains the opportunities and value of patient involvement and puts into context the multi stakeholder roles in the process to mitigate against any potential expectation that the final recommendation is follows th wish of the patient stakeholder contributors
5. Regular trainings are accessible, particularly for patient groups for upcoming HTAs (well in advance of the call to submit)
6. Where possible and appropriate, training includes case examples and mock HTAs for practice
7. A dedicated contact person at HTA organisation or umbrella patient organisation helps with advice or support during preparation
8. Guidance is provided on receiving and using Summary Information for Patients (SIP) to participating stakeholders in a specific HTA where available

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GUIDANCE AND TRAINING PROVIDED

GUIDANCE FOR HTA RESEARCHERS:

9. Explicit internal HTA guidance to all relevant staff on how patient involvement within an assessment shall be conducted and used in the assessment
10. Reporting of patient input: There is guidance for HTA researchers on how methods and results of patient input shall be reported. The guidance includes best practice examples

CONFLICT OF INTEREST AND DATA USE:

11. Definition and public communication of a conflict-of-interest policy for all involved stakeholders. Specifically, guidance for patients who have been engaged by clinical study sponsors during technology development.
12. Guidance on implication of GDPR* for collecting, analysing, using, and communicating patient information

* *GDPR = General Data Protection Regulation, Regulation (EU) 2016/679 of the European Parliament and of the Council of 27 April 2016*

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HORIZON SCANNING*

1. Use existing or develop horizon scanning process and inform patient organisations early about the upcoming possibility to become involved
2. Link and promote existing horizon scanning services (European & National) to umbrella organizations so that they can alert members early to contribute where they have knowledge about upcoming technologies

Organisational and general considerations

It was noted that all stakeholders may have horizon scanning information to contribute and that a joint approach with excellent communications will be needed…

ORGANISATIONAL LEVEL INVOLVEMENT

3. *Explore partnership opportunities with patient stakeholders*
4. *Liaise with Patient Organisations to maximise effectiveness of communication of scanning outputs*

COMMUNICATION

5. *Strengthen communications between umbrella patient organizations and HTA organisations so that horizon scanning insights are shared between them in a timely manner*

*Horizon scanning emerged as a step to consider during the multi-stakeholder workshops; it was not part of the interview findings

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EARLY DIALOGUES / SCIENTIFIC ADVICE

DESCRIPTION

The engagement between a HTA body and a medicine developer to discuss evidence generation plans for a medicine in development

STEPS*

- Medicine developer requests early dialogue
- HTA body identifies potential patient contributors
- Briefing book developed by medicine developer
- Lay summary (Summary Information for Patients) shared with patient contributor(s)
- Briefing of patients by HTA body in preparation of the early dialogue
- HTA body hosts meeting with patient(s) included
- Patient(s) invited to respond to all the main questions posed

WHAT WORKS WELL

- 🟢 Overall process to plan and prepare the early dialogue collaboratively
- 🟢 Patient expert was well briefed and prepared by the HTA body
- 🟢 2-hour prep meeting ensured patient expert knew what to expect
- 🟢 During the meeting there was time made for the patient input
- 🟢 Patient input was valued as equal to other stakeholder input

WHAT WORKS LESS WELL

- 🟢 Only one patient expert invited – would have been better to have more
- 🟢 Insights from patients involved in early dialogues could inform later discussion on the PICO at the assessment phase but firewall between early dialogues and assessment phase prevents the sharing of learnings and insights
- 🟢 Difficult to motivate patients to take part (low priority for them)

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

🔵 *HTA* 🟢 *Industry* 🟡 *Patients/Citizens*

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EARLY DIALOGUES / SCIENTIFIC ADVICE

1. Patient stakeholders are part of early dialogues as they have valuable contributions to the discussions on PICO and the feasibility of proposed clinical studies
2. Preparatory briefing with patient contributors are a part of this process
3. Time is ring-fenced on each key topic at the meeting to gain patient input
4. Ideally, more than one patient contributor is involved
5. More communication, education developed and promoted on the Early Dialogue process to encourage more patient stakeholders to take part

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PATIENT INVOLVEMENT PLANNING

DESCRIPTION

In preparation for an upcoming HTA, the HTA body will plan how it will reach out to and promote the opportunity of involvement

STEPS*

- Pre-HTA engagement by industry to confirm timelines and likely submission dates
- HTA body defines the involvement need (decision to involve patient stakeholders, which kinds of stakeholders to involve, and at what process steps)
- A checklist and the advice from a standing committee to advise on the need to involve and advise on the stakeholder types to involve
- Identifying relevant patient stakeholders and patient organisations
- Alerting the patient stakeholders to the timeline

WHAT WORKS WELL

- 🟢 Increased patient engagement throughout the development of new medicines
- 🔵 Checklists for patient involvement decisions
- 🔵 Repository and databases of patient organisations where existing
- 🔵 Disease specific stakeholder lists
- 🔵 Complementing existing lists/databases with external search
- 🔵 Roles/responsibilities matrix so stakeholders understand their role

WHAT WORKS LESS WELL

- 🔵 Time constraint; Restriction on when agency can reach out to POs
- 🔵 Confidentiality concerns of industry can hinder outreach
- 🟢 Low continuity of involvement along the assessment phases
- 🟢 Low consistency of processes across countries
- 🔵 Challenging in some disease areas to identify stakeholders
- 🟢 Variability across HTAs of who is involved (e.g. Patient organisations versus individual expert patients)

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

🔵 *HTA* 🟢 *Industry* 🟡 *Patients/Citizens*

PATIENT INVOLVEMENT PLANNING

1. At the EU-level, strengthen co-ordination with other health system players (e.g. regulatory) to plan involvement
2. Overcome any concerns over earlier outreach (level of information sharing, confidentiality solutions)
3. When relevant patient stakeholders cannot be found, enable the use of other stakeholders' networks (e.g., industry, regulatory) to augment the search

Organisational and general considerations

ORGANISATIONAL LEVEL INVOLVEMENT	4. *Strategic planning of patient involvement (e.g. checklist / pathways for planning when and how patient stakeholders should be involved) to minimize burden on patients and to ensure most appropriate method is used and the burden on patient stakeholders does not outweigh the possible impact of patient input*
COMMUNICATION	5. *Stakeholders contacted earlier than the usual scoping timepoint so that they can prepare. For HTA organisations with yearly planning cycles, communicate plans to umbrella patient organisations once finalised* 6. *Early alert systems with public and direct alerts of upcoming opportunities to patient organisations to increase awareness*

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CALL FOR PATIENT INPUT

DESCRIPTION

A call for patient participation is published at the start of a specific assessment and outreach to relevant patient stakeholders occur

STEPS*

- Open call published on the HTA body website to officially announce the opportunity to input
- Individual outreach to Patient Organisations known to the HTA body (in databases or lists maintained by the HTA body)
- Reminders and telephone outreach if no response
- Leveraging existing relationships with patient groups and umbrella organisations to further promote the opportunity
- Social media and muti-channel outreach in some cases

WHAT WORKS WELL

- 🔵 Early publication of the call improves the chances of gaining patient stakeholder input as does flexibility in the timelines
- 🔵 Working with the patient stakeholders and/or a standing committee to determine the appropriate form of input needed
- 🔵 Multi-channel outreach to spread the call further
- 🔵 Previous relationships smooth the process
- 🟡 Patient Organisations can select most appropriate patient expert
- 🟡 Umbrella patient organisations do their own horizon scanning or are in contact with the agency's horizon scanning unit and so can alert patient organisations early

WHAT WORKS LESS WELL

- 🔵 If no direct outreach, patient organisations need to constantly monitor HTA website – may miss a lot of eligible organisations
- 🟡 Hard to gain support of patient organisations and/or patient experts when no support is offered for the work and prep
- 🔵🟢 Pan-EU: Language barriers restrict who can take part
- 🔵 Without dedicated patient involvement personnel at the HTA body, this can be an 'additional task' on top of the day job of the HTA researcher
- 🔵 Process depends on the individual HTA personnel and is done with high variability

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

🔵 *HTA* 🟢 *Industry* 🟡 *Patients/Citizens*

CALL FOR PATIENT INPUT

1. Timelines allow for both the recruitment time and meaningful participation
2. Key success factors:
    a. Strong relationships with umbrella groups assist with recruitment of patient organisations and expert patients
    b. If the call describes how the involvement can benefit the patient community, patients may realise the value of their involvement.
3. Clear inclusion criteria for patient stakeholders are defined and published
4. When HTA organisations consider voices other than patients (e.g. parents, care persons), it is described, how they are selected and involved

Organisational and general considerations

ORGANISATIONAL LEVEL INVOLVEMENT	*5. Patient stakeholders advise on most effective interactions and pathways to promote the opportunity and improve the experience of giving input*
GUIDANCE AND TRAINING	*6. A dedicated contact is assigned at the HTA body to liaise with patient stakeholders and has the time reserved to administer the call and answer questions / provide guidance*
COMMUNICATION	*7. Direct outreach to patient organisations by HTA body (umbrella and disease-specific) happens alongside the publication of the call on the website (plus multi-channel).*

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SCOPING

DESCRIPTION

At initiation of each HTA, the scope (and PICO) of the research for an upcoming HTA is defined.

STEPS*

- Assessment if stakeholder involvement is needed (e.g. new disease area that needs input from stakeholders)
- HTA body creates draft remit, draft scope and stakeholder list
- Stakeholders invited to a scoping process or draft protocol is shared for feedback
- Confidentiality forms may need to be signed before details of the draft protocol (remit, scope) are shared
- If a meeting, HTA body hosts the scoping meeting

WHAT WORKS WELL

🔵 Scoping can start early, before the official dossier submission, which also alerts patient organisations that a HTA is upcoming

🟢 Multi-stakeholder interactions so that patient stakeholders also hear the views of HCP and other stakeholders

🔵 An interactive discussion on the scope can lead to more nuanced research questions and outcomes can be weighted for stakeholder importance

WHAT WORKS LESS WELL

🔵 Cannot alert patient stakeholders earlier due to confidentiality needs of technology developer

🟢 No industry representation in scoping meetings which can mean that specific knowledge is missing from the discussion

🟢 Not a systematic process in all cases – meaning that patient input is often missing from the PICO discussions

🔵 A challenge getting some patient organisations to be interested in the process and take part (capacity and knowledge gaps)

*PICO – Population, Intervention, Comparator, Outcomes are defined to frame the research question of each HTA

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

🔵 *HTA* 🟢 *Industry* 🟡 *Patients/Citizens*

SCOPING

1. Promotion of involvement in scoping activities takes place in a similar way, using similar methods as recruiting for the actual HTA (see recommendations for 'Call for Patient Input')

Organisational and general considerations

ORGANISATIONAL LEVEL INVOLVEMENT	2. *Transparent published criteria for when stakeholder involvement in scoping happens and when it does not*
GUIDANCE AND TRAINING	3. *A definition of scoping, the process and its objectives laid out in plain language*
	4. *Guidance is provided to patient organisations on the kinds of insights useful to a scoping process and their role in it, with case studies and examples to illustrate the value of patient input into scoping*
	5. *Explicit guidance is given on the options, requirements, and quality expectations for submission of patient-based evidence*

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PATIENT SUBMISSIONS

DESCRIPTION

Patient organisations (and sometimes patient experts) are invited to submit a written document detailing the patient perspective and evidence relating to an assessment/appraisal

STEPS*

- Planning and timeline based on when manufacturer indicates they are likely to be ready to submit a dossier
- Once start date is known stakeholders, including patient stakeholders are invited to submit
- Often a 4-8-week window to submit
- Variety of processes, online questionnaire, standard submission templates, request for data (e.g. survey)
- Confidentiality forms need to be signed in many processes

WHAT WORKS WELL

- 🔵 Having a dedicated support person to manage process (invites, reminders, conflict of interest forms, legal forms, etc.)
- 🔵 Flexibility on timelines and reminders maximise the likelihood of gaining submissions
- 🔵 Good communication between HTA body and manufacturer helps to better manage timelines (e.g. early alert for o opportunity to submit, clarification of confidentiality issues)
- 🔵 Patient organisations with HTA experience give more focused input or know what is needed
- 🟡 Larger patient organisations often have dedicated personnel to input
- 🔵 HTAi questionnaire used as the basis for many processes, gaining some consistency and confidence in the approach

WHAT WORKS LESS WELL

- 🔵 If can only input through online form, may restrict accessibility (technical or capability restrictions)
- 🔵 Language limitations exist for EU-wide processes
- 🔵🟡🟢 Costs significant time and resources to the patient organisation. Some smaller organisations do not have the capacity
- 🔵🟡 Time for input can be too short
- 🔵🟡 Formal forms and legal documents deter some organisations
- 🔵 Standard questions not always relevant to population or disease

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

🔵 *HTA* 🟢 *Industry* 🟡 *Patients/Citizens*

PATIENT SUBMISSIONS

1. Online questionnaires are user friendly and allow flexibility including the ability to save progress (with barrier free off-line alternatives).
2. All question are relevant to the disease area and population and can be answered in the time allowed for submission (e.g. disease specific examples)
3. If possible, patient organisation input and review for question / template development to sense-check they are clear, relevant, and understandable to the target audience
4. Create / communicate options for practical support for the patients submitting information (e.g., buddying, peer support, umbrella org)
5. Investigate financial support to help cover the resources needed to provide submissions
6. EU-level: Responses can be submitted in all member state languages. Country-level: submission possible in all relevant national languages

Organisational and general considerations

GUIDANCE AND TRAINING

7. *For patient submissions, guidance is provided in relation to each specific question on different types of information that can be submitted and what types are useful to the HTA process, with examples*
8. *The guidance describes how the input will be used*
9. *Guidance on collecting evidence from patient communities is available for patient organisations (best practices, privacy and data management, burden of disease data)*

COMMUNICATION

10. *Good and frequent communication exists between the patient involvement managers and those receiving the input (HTA researchers / appraisal committees) to effective input and use of information*

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INTERVIEWS & FOCUS GROUPS

DESCRIPTION

Some HTA organisations conduct interviews or focus groups with patient stakeholders instead of (or as well as) a written submission process

STEPS*

- Assessment team identifies the need for interviews / focus groups
- Interviewees are identified and recruited
- Pre-interview discussion to let patient stakeholders know what to expect (often based on the same questions as the template submission)
- Patient organisation can propose changes to the interview questions and structure
- In some cases, a focus group is held, hosted and managed by a patient organisation

WHAT WORKS WELL

- Interviews can help to confirm other collected information or to bring additional context to the evidence provided by others
- Focus groups provide an opportunity to discuss the issues in depth and gain a variety of inputs
- Focus groups led by a patient organisation may lead to more involvement and more comfortable experience for participants
- Involving a patient organisation in the structure of the interview or focus group can can improve comprehension by target group and improve participation

WHAT WORKS LESS WELL

- HTA practitioners are not prepared for dealing with the emotional aspects of the direct reports by care-givers and patients
- Interviewing one patient is not sufficient - there is a need to develop methods and processes that allow for wider input

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

● HTA ● Industry ● Patients/Citizens

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INTERVIEWS & FOCUS GROUPS

1. A quality check of the discussion guide with a PO as well as other stakeholders (e.g. an experienced researcher) can improve quality and relevance of interviews, especially for topics or wordings that are likely to be sensitive
2. For focus groups, having a PO host the meeting can create a more comfortable environment where participants feel freer to voice sensitive subjects.
3. The work invested by POs to host and organise a focus group should be paid for
4. Smaller size of focus groups ensure that it is not too daunting for participants to share their experiences
5. Provide a clear list of who will be in the room/call, their role/expertise, and the purpose of them being there
6. Explain, why questions are asked
7. Due to the small number of individual views that are considered, findings from interviews and focus groups should be validated against the other patient-based evidence that was submitted, such as patient group submissions or patient preference studies.

Organisational and general considerations

GUIDANCE AND TRAINING

8. *'Buddying' or support through a (umbrella) patient organisation may help the interviewees to prepare and respond effectively.*

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HTA REPORTING OF THE PATIENT INPUT

DESCRIPTION

How the patient input is detailed in the report alongside any consideration of this input in the evidence assessment or any deliberations

STEPS*

- Assessment team, HTA researchers or lay members of a HTA process provide a summary of the patient input received
- In some cases, this summary is developed by the participating patient organisations as part of the submission template
- Reports may have a dedicated chapter on the patient input received, others may include this within the report alongside other evidence
- Not all HTA organisations review, comment or make considerations of the patient input, but those that do often include consideration of the patient input in the conclusion

WHAT WORKS WELL

- 🔵 Checking the patient input summary with patient contributors helps to ensure it is an accurate reflection
- 🔵 Secretariat offers to check the submissions for grammar and spelling when patient groups submit in English but this is not their first language
- 🔵 When patients' direct input at hearings is transcribed into the report, this is fully transparent

WHAT WORKS LESS WELL

- 🔵 Not all HTA reports detail the patient input
- 🟢 There is a lack of feedback in some cases to the patient stakeholders to let them know what has been included and why
- 🟢 Many reports do not detail how the patient input was considered
- 🔵 Clearer guidance is needed for assessors and report writers to ensure a consistent way of detailing the patient input (and the way it was considered)

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

🔵 *HTA* 🟢 *Industry* 🟡 *Patients/Citizens*

HTA REPORTING OF THE PATIENT INPUT

1. Processes close the circle by feeding back to the patient stakeholders how their contribution has been reported and how it was considered
2. Patient organisations are consulted for their agreement on how their input is reported
3. Methods and results of patient input are reported as a standard component in the main report. Reporting includes (1) disease& therapy level learnings and (2) the use of the input in the appraisal, the recommendation, and the decision
4. Where no patient input was received, it is described how it was sought and/or why none was received
5. An annex provides the full patient submissions (where approval is needed from the patient organisation, this is sought as part of the submission process)
6. When patient input is summarised, this is shared with the relevant patient stakeholders for their agreement before publication

Organisational and general considerations

GUIDANCE AND TRAINING	7. *There is guidance for HTA researchers on how methods and results of patient input shall be reported. The guidance includes best practice examples*
COMMUNICATION	8. *Upcoming consultations are announced timely to make the patient organizations aware of the opportunity and process (purpose, process and timing of consultation)*

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APPRAISAL COMMITTEES

DESCRIPTION

A deliberative meeting in which the HTA body, the manufacturer and expert stakeholders including patient stakeholders attend

STEPS*

- Invitees selected and invites sent to patient stakeholders
- Preparatory materials sent to attendees
- Sometimes, Patient Stakeholders or Lay members have a seat(s) in the appraisal committee
- Often a liaison is assigned to walk the patient stakeholders through the process (sometimes supported by lay member)
- Often a briefing with all external experts about one month before the meeting
- On the day, a named person looks after patient stakeholders (HTA or Patient Organisation)

WHAT WORKS WELL

- ● If well prepared, the patient input at the committee meeting or through consultation can make an important difference
- ● A welcoming atmosphere (leadership)and interviews by an informed, supportive committee member helps with relevant patient input
- ● A patient expert support is present at the time of the committee meeting to facilitate the participation
- ● Stakeholders can better respond (more relevance) to questions if the rational for the question is explained
- ● If well prepared and getting sufficient ‘space’ in the meeting, the patient representation in the committee is very impactful and important (e.g. for QoL / PRO consideration)

WHAT WORKS LESS WELL

- ● If too many people are there for the hearings, the patients may be overwhelmed, and the strict format may be intimidating
- ● The materials/technical documents are not easy to understand for patients
- ● A lack of ‘role specification’ means that everybody interprets their role differently

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

● HTA ● Industry ● Patients/Citizens

APPRAISAL COMMITTEES

1. The patient stakeholder roles are clearly defined so that all members are aware of them as integral to the committee and/or the process.
2. Patient stakeholders are appropriately informed / prepared (e.g. according to PFMD criteria) and supported to ensure that their input is relevant to the discussions
3. The agenda includes a specific agenda item on 'patient perspectives' to ensure a dedicated 'space' for the patient input
4. Considering patient ability and limitations (e.g. employment, accessibility) will maximize likelihood for participation
5. Meetings with patients who are not committee members are ideally held in smaller groups to reduce the perceived hierarchical barriers

Organisational and general considerations

GUIDANCE AND TRAINING	6. *The chair is trained and supported to ensure that an appropriate welcoming environment exists in the meeting* 7. *Participating patient stakeholders receive guidance on what they can expect and what is expected from them (potentially using a checklist to ensure all guidance is given and understood).*
COMMUNICATION	8. *Summaries of all key (relevant) materials are provided in plain language versions or summaries*

EVALUATION AND FEEDBACK

DESCRIPTION

The impact of patient input on the HTA (e.g., scoping, quality, assessment, appraisal) is evaluated and communicated to those who were involved and published

STEPS*

- Patient involvement is evaluated after completion of each HTA or regularly
- Feedback is shared with those who contributed
- Feedback from HTA to patients can include information on how their input was used or how it could be improved

WHAT WORKS WELL

- 🔵 Evaluation can be collected through open questions from research team (What impact had the patient involvement, did it provide new evidence or corroborate other evidence?)
- 🔵🟡 Feedback to patient organisations can help to improve future involvement of the same organisation and of others (if it was published)
- 🔵 Feedback to HTA agency can help to improve the patient involvement process and training
- 🟡 The more interactive the involvement, the easier is the feedback

WHAT WORKS LESS WELL

- 🔵 Feedback is not given consistently
- 🔵 (Patient) Stakeholders become frustrated if they don't see what difference their input makes
- 🔵 Collecting feedback always is much work, therefore it's only collected occasionally
- 🟢 It would be good to know what happens next (after decision)

*Steps differ across HTA systems - this lists the steps the interviewees outlined in their specific cases

🔵 *HTA* 🟢 *Industry* 🟡 *Patients/Citizens*

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EVALUATION AND FEEDBACK

1. The patient involvement is evaluated regularly. HTA organisations monitor and track how the submitted information from patient stakeholders was used in the HTA and give feedback to the contributing patient stakeholders
2. Evaluation may cover the aspects of process satisfaction (by patients and HTA) and impact (Patients and HTA).
3. The results of the evaluation are published so that they are accessible and can support preparation for future processes and collective learning
4. There is transparency of the processes for evaluation and feedback
5. The outputs of the tracking of patient stakeholder inputs and use is collected to form an evolving practice guide for both HTA and patient stakeholder learning

Organisational and general considerations

GUIDANCE AND TRAINING	*6. There is guidance for HTA researchers on how to provide constructive feedback to stakeholders. The guidance includes best practice examples*
COMMUNICATION	*7. Feedback is shared with those who contributed and potentially advice how future input could be improved to be more relevant*

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INDUSTRY DOSSIERS*

1. As standard, company dossiers should report any patient engagement / involvement that occurred throughout R&D
2. A lay language summary from the medicine developer submitted as a standard part of the dossier following guidance to industry on how to structure and produce balanced lay language summaries (e.g., Summary Information for Patients (SIP)

* These recommendations emerged from discussions in the multistakeholder alignment workshops

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CONCLUSION

Overall Conclusions

A detailed 360° review of stakeholder experiences with involvement processes can help to identify best practices

Patient involvement on the organizational level can help to create and apply processes in a way that they are feasible and relevant

Good preparation, early alerts, cross-stakeholder collaboration can help to maximise the level of involvement

Evaluation and feedback mechanisms can help to improve quality, consistency, and effectiveness of patient involvement

SUGGESTED FURTHER RESEARCH AND ACTIVITIES

EXTENDED SCOPE OF 360° RESEARCH

This research shows the value of conducting multi-stakeholder experiences and perception research to identify good and emerging practices in patient involvement in HTA. Only a limited numbers of countries could be involved in this piece of research and it would be advantageous to conduct this type of research with many more HTA organisations, patient stakeholders and industry stakeholders.

INSTITUTIONAL MEMORY OF STAKEHOLDER ORGANISATIONS

In conducting this research, it was found that many organisations lose key members of staff that were instrumental in the HTA process, and with these losses, key experiences and knowledge is lost from the organisations. This was particularly seen with Patient Organisation stakeholders. More research needs to be conducted to quantify the scale of this loss and to develop practical ways of retaining institutional memory of HTA processes within organisations.