Health Technology Assessment and Patient Involvement

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SPAGN Knowledge Spot
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Virtual

Source: HTA Consumer and Patient Glossary, Health Technology Assessment International. October 2009.

Become a part of the process. Learn more at www.meetforpatients.com

Health Technology Assessment (HTA)

- What is HTA?
  - Cost Effectiveness
- How can patients be involved in HTA?
  - Specific methods of participation

- New EU HTA Regulation

Regulatory process for medicine's marketing authorisation

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- Quality
- Safety
- Efficacy

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

MHRA
Regulating Medicines and Medical Devices

Difficult decisions

- Should our healthcare system invest in this new technology?
- If we invest in a new technology there is an “opportunity cost” - we must take investment away from somewhere else in the system
- How do we decide what the priorities are?
- How do we ensure equity of care in our healthcare system?

Health Technology Assessment (HTA)

HTA is a multidisciplinary process* that uses explicit methods to determine the **value** of using a *health technology* at different points in its lifecycle.

The purpose is to inform health policy and decision-making
(pricing and reimbursement/access)
to promote an equitable, efficient and high-quality health system.

*The process is formal, systematic, and transparent,
and uses state-of-the-art methods to consider the best available evidence.

O'Rourke B, Oortwijn W, Schuller T, the International Joint Task Group (2020). The new definition of health technology assessment: A milestone in international collaboration. International Journal of Technology Assessment in Health Care 1–4.

Value in HTA

The dimensions of value for a health technology may be assessed by examining the intended and unintended consequences of using a health technology compared to existing alternatives.

These dimensions often include

- clinical effectiveness and safety
- *costs and economic implications*
- *ethical, social, cultural and legal issues*
- *organisational and environmental aspects*
- *as well as wider implications for the patient, relatives caregivers & the population.*

The overall value may vary depending on the perspective taken, the stakeholders involved, and the decision context.

O'Rourke B, Oortwijn W, Schuller T, the International Joint Task Group (2020). The new definition of health technology assessment: A milestone in international collaboration. International Journal of Technology Assessment in Health Care 1–4.

Cost effectiveness
(Economic evaluation)

Economic evaluation is ‘ *the **comparative** analysis of alternative courses of action in terms of both their **costs** and **consequences***’
(Drummond & McGuire, 2001)

Modelling what happens to patients over their life time, measuring Quality Adjusted Life Years from generic PRO, to determine value for money

Issues in Economic Evaluation

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- Lots of uncertainties – particularly in rare, heterogeneous conditions
- Is there a COST/QALY threshold?

  (e.g. >£20,000 - £30,000/QALY; 3xGDP/capita; etc)

- Are there "modifiers" that would allow us to go above the threshold?
  - Severity of disease
  - Unmet need
  - End of life (survival estimated to be less than 2 or 3 years)
  - Orphan medicinal product

HTA Deliberation

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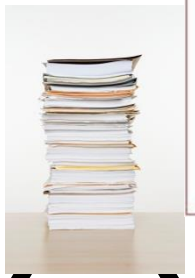
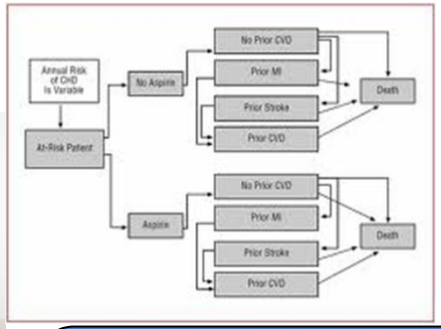
What is the benefit for patients? What outcomes are important?





What are the long-term effects of treatment?

What is the actual pathway of care? Are there variations in healthcare delivery?

Value Judgements about Uncertainties

Who will benefit most from this medicine?

DECISION PROVIDING ACCESS TO SPECIFIC PATIENT GROUPS
+ Pricing and Reimbursement
+ Additional evidence collection?

Value of patients' perspectives

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Living with an illness

- 'No one knows better what it is like to live with an illness day in, day out, than those who are doing this – patients, their family and those who care for them.'

Understanding HTA. Health Equality Europe. 2008

(Available in several languages). http://www.htai.org/index.php?id=744

Unique knowledge

- Experiences (good and bad) of the health care system in terms of diagnosis, disease management and treatment
- Preferences, needs

How can patients get involved in HTA?

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- Many HTA bodies believe that patients' views are subjective:
  - Are they representative?
  - Are patient groups biased by industry?

- How can patients' and care-givers' (carers') perspectives be combined with evidence from controlled clinical trials or complicated economic models of cost and benefit?

Patients' perspectives in HTA: a route to robust evidence and fair deliberation

Int. J. Tech Assess Health Care, 2010, 334-340

37 chapters:
I: Conceptualization
II: Methods
III: Country experiences

Patient involvement in HTA:

- Patient-based evidence: robust qualitative and quantitative research about patients' perspectives, experiences & preferences
- **Patient and patient group participation in the HTA process**

“Patient” participation in HTA

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At every stage:

- Study design to produce evidence (individual patient)
- HTA topic selection (“public/citizen/lay” representative)
- Scoping meeting or interview (patient group)
- Submission of information
- Presentation of patient experience to expert committee
- Development of a joint statement with clinicians
- Sitting on an HTA decision-making committee
- Development of a plan for additional data collection
- Consultation on recommendations
- Patient friendly summaries
- Dissemination/communication
- Approval of Managed Entry Agreements/collection of PROs
- Designing and reviewing patient involvement processes
- Using HTA to inform charity investments
- Contributing to governmental policies relating to HTA

Ways patients/patient groups can participate in HTA

- Online suggestions – topics, process
- Participation in a multi-stakeholder meeting
- Structured submission of information
- Public consultation response – open or structured

- HTA researchers interview individual patients
- Workshops (ala Health Technology Wales/FDA)
- Co-production of new processes

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Patient Group Submissions in

https://htai.org/interest-groups/pcig/resources/for-patients-and-patient-groups/

- What it is like to live with the illness
- Ex
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COMPLETING A PATIENT GROUP SUBMISSION TEMPLATE: GUIDANCE FOR PATIENT ORGANISATIONS

for Health Technology Assessment and Appraisal of Medicines

Prepared by HTAi Patient and Citizen Involvement in HTA Interest Group
Version 2-2015

Note:
We would be grateful to know if and how you have used this document, and how we can make it better. All comments and document will be reviewed in February 2017.
Comments to: Ann Single, email: singlehaworth@gmail.com

Health Technology Assessment international

Key ethical considerations for patient groups collecting and reporting information for HTA submissions

Short guide

Purpose

To complete submissions for health technology assessments (HTAs), patient groups may gather information about patients' and caregivers' experiences of living with a condition, preferences and unmet needs for treatment. This may involve (but is not limited to) conducting interviews, focus groups and surveys and collecting input using social media. As a result, patient groups need to think about the ethical and legal issues involved when engaging with people and using their personal information. This document aims to help your patient group identify and respond to those issues. It is not mandatory guidance and can be adapted to meet your needs.

Issue	Consider
1. Need for activity	○ Do you already have information that can answer the HTA submission questions? ○ Have you found a gap in the available information? Does this gap mean you need to collect new information? ○ Have you planned and tested the way you will collect the information to make sure it meets your needs?
2. Inclusivity	Have you taken steps to reach out to as broad a population (including vulnerable groups) as feasible?
3. Informed consent	Is each person who is asked to take part competent to consent? If yes, have they been told: ○ how the information being collected will be used and shared? ○ who is collecting the information? ○ that they can refuse to take part, stop taking part at any time, or choose not to answer all the questions without this being held against them? ○ any perceived or potential conflicts of interest of the person(s) or group collecting the information? ○ what is involved in taking part (how much time, what will be discussed, possible use of their actual words or stories in the submission)? ○ the realistic potential benefits? ○ the risks or potential harm of taking part (such as distressing thoughts, sense of stigma)? ○ That they will not be able to be identified from the submission?

EUPATI

HTA) on
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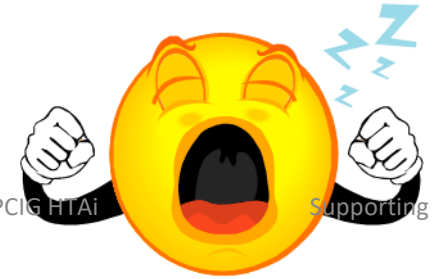
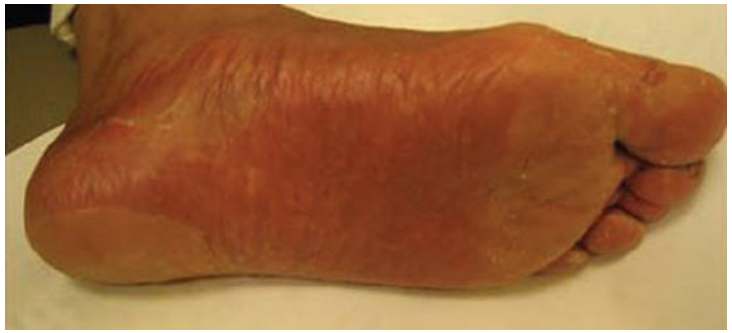
Burden of disease

Current treatments



Outcomes that matter

Impact of outcomes



Public influence on health service planning
(Boivin 2014)

- Credibility – ability to contribute knowledge that is considered valid and relevant will result in mutual learning and generation of new solutions
- Legitimacy – to speak on behalf of others
- Power – ability to influence

Patient Involvement in HTA

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Patient-Centred HTA: Putting a Human Face on the Evidence

(Facey, Bedlington, Berglas, Bertelsen, Single, Thomas, *The Patient*, 2018)

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Patient involvement in NICE and CADTH (Canada) has:

- Clarified outcomes that matter to patients
- Addressed gaps in clinical evidence
- Identified how clinical trials differ from experience in the local healthcare setting
- Questioned assumptions in modelling
- Contributed to deliberative discussions about trade-offs

EU HTA cooperation

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ECHTA
2002-2005

eunethta
EUROPEAN NETWORK FOR HEALTH TECHNOLOGY ASSESSMENT

Project (2006-2009)
JA1 (2010 – 2012)
JA2 (2012 – 2015)
JA3 (2016 – 2021)


Ref. Ares(2016)6599082 - 24/11/2016

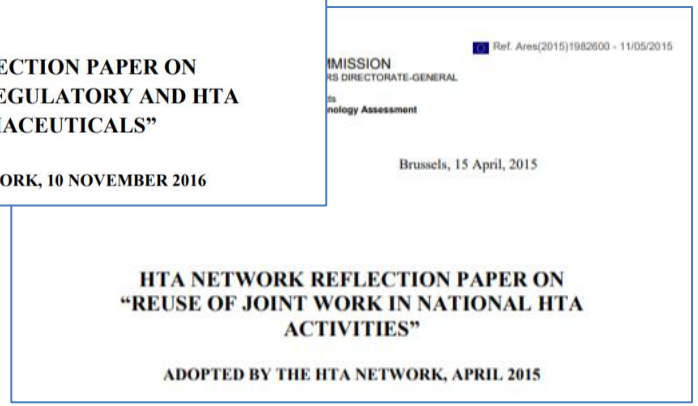
EUROPEAN COMMISSION
DIRECTORATE-GENERAL FOR HEALTH AND FOOD SAFETY

Health systems, medical products and innovation
Medical products: quality, safety, innovation

Brussels, 10 November 2016

HTA NETWORK REFLECTION PAPER ON "SYNERGIES BETWEEN REGULATORY AND HTA ISSUES ON PHARMACEUTICALS"

ADOPTED BY THE HTA NETWORK, 10 NOVEMBER 2016


Ref. Ares(2015)1982600 - 11/05/2015

Brussels, 15 April, 2015

HTA NETWORK REFLECTION PAPER ON "REUSE OF JOINT WORK IN NATIONAL HTA ACTIVITIES"

ADOPTED BY THE HTA NETWORK, APRIL 2015

HTA Regulation

HTA Regulation (HTAR)

REGULATION (EU) 2021/2282 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 15 December 2021
on health technology assessment and amending Directive 2011/24/EU

- **Establishing a support framework and procedures for cooperation of Member States on health technologies at EU level, with a mechanism for submission of evidence and commons rules and methodologies for joint assessments**
- To ensure **high quality, timeliness and transparency**
- Ensure **use of joint work in national HTA processes**
- **Member States** remain responsible for:
  - Drawing **conclusions on added value** for their health system
  - Taking **decisions on pricing & reimbursement**

HTAR - joint work

Joint clinical assessments (JCAs) (medicines, medical devices) published within 40 days of Marketing Authorisation

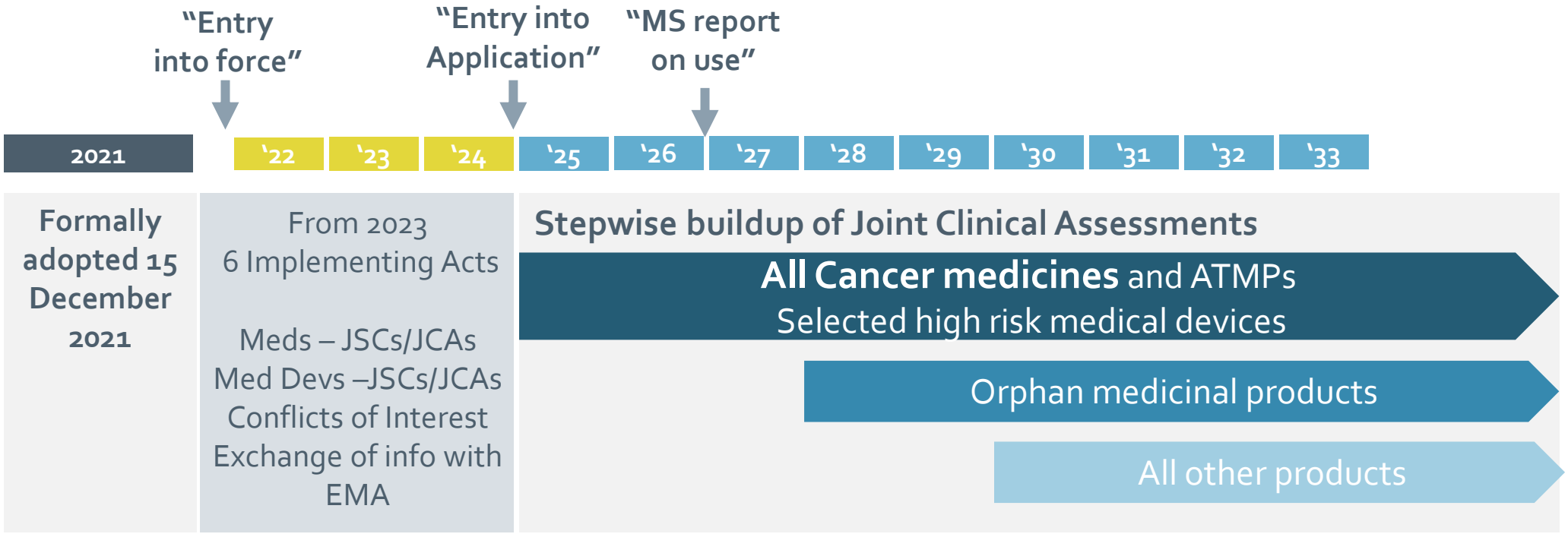
Joint scientific consultations
(scientific advice/Early Dialogues to health technology developers on clinical study design; parallel HTA-EMA advice for medicines possible)

Identification of emerging health technologies
(horizon scanning in collaboration with EMA to determine workplan)

Voluntary cooperation in other areas
(e.g. on other health technologies or non-clinical HTA aspects)

FIPRA

HTAR progressive implementation



Coordination Group

All MS

- Horizon scanning (for workplan)
- Joint Scientific Consultations
- JCAs
- Methods

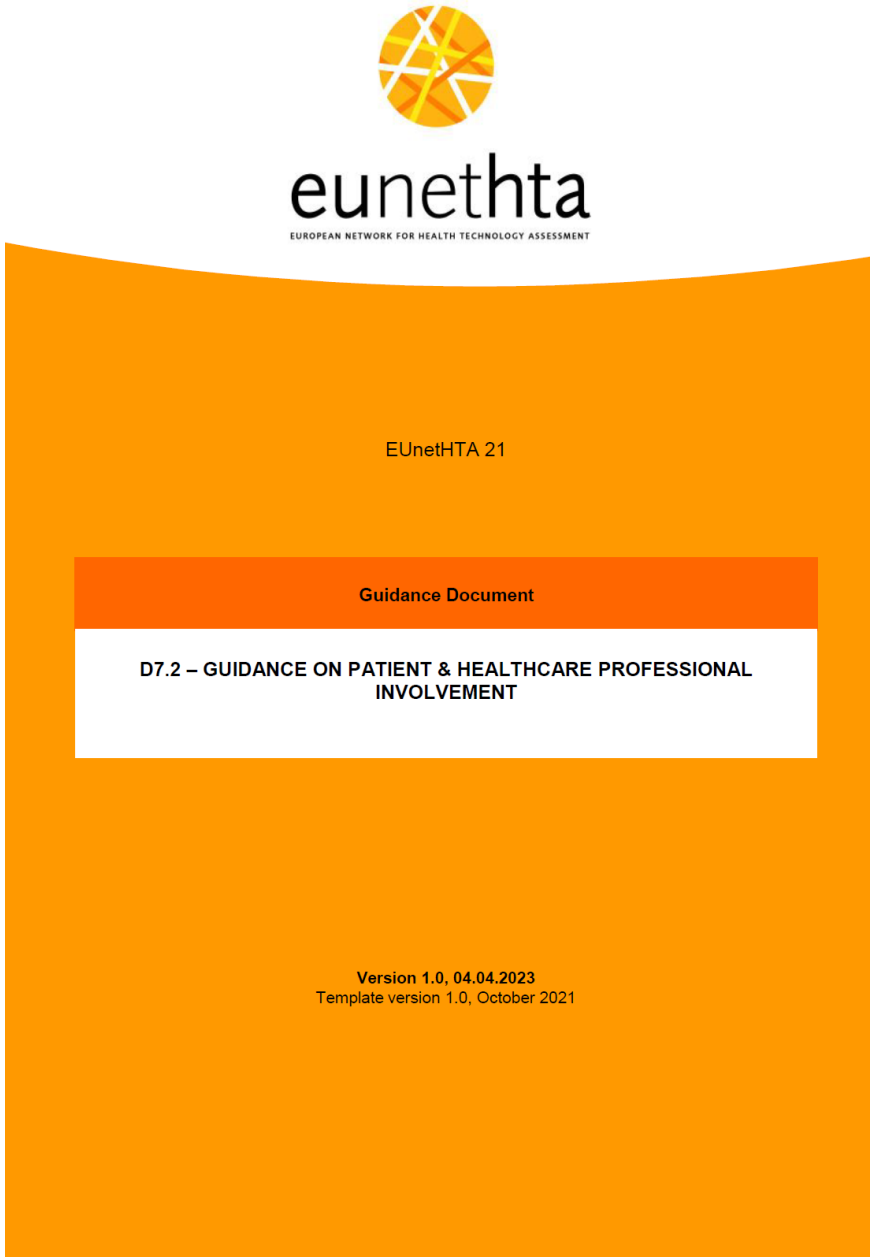
Stakeholder Network

EU stakeholder groups/socs

Support Actions

EU4Health Stakeholder Training - EUPATI and EPF

FIPRA



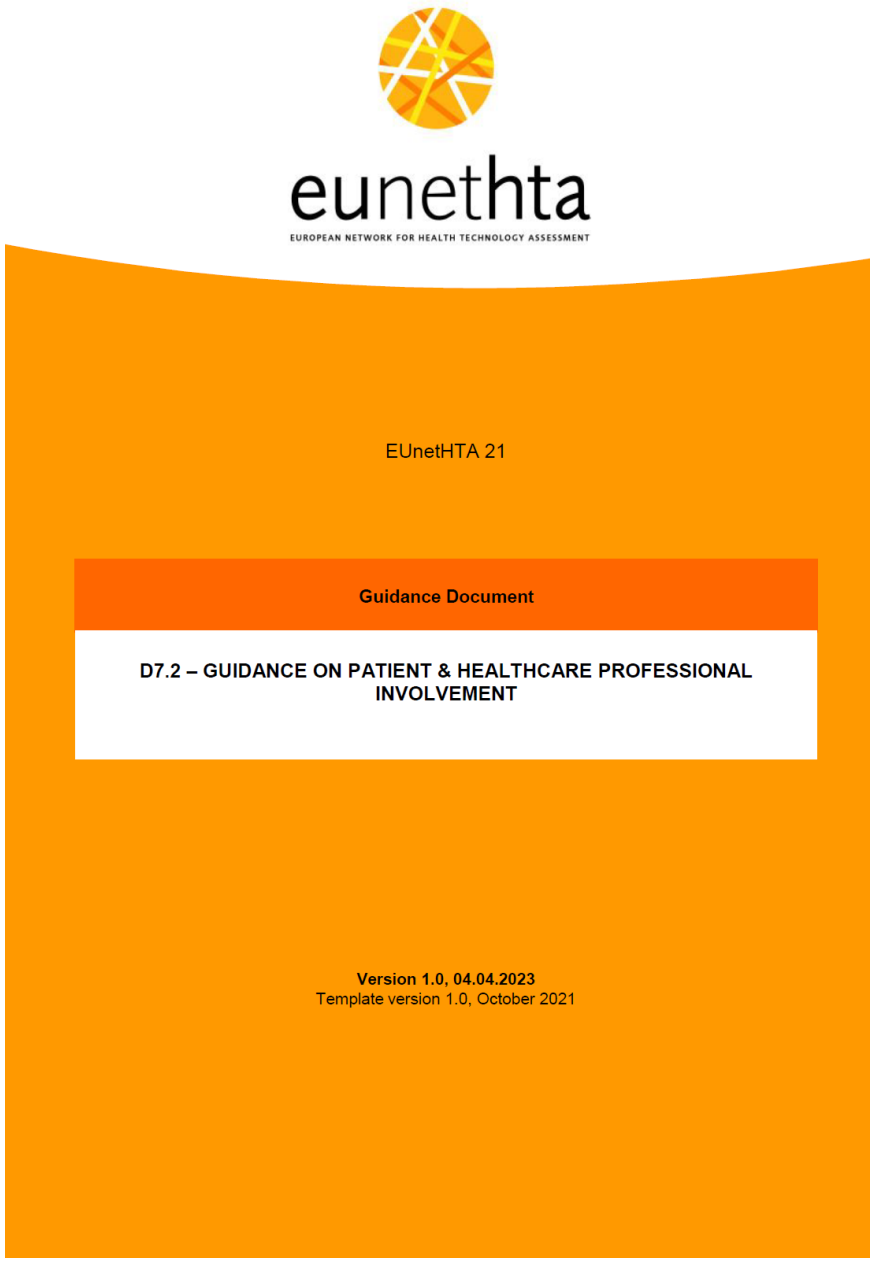
Views from Valentina Strammiello EPF on Final Guidance Submitted to EC

European Access Academy, 21 April 2023

Individual patient experts

- Involved in JSCs and JCAs
- Patient experts with experiential knowledge (individual with collective perspective)
- More flexible about link to patient organisation and need for different types of experts at different points
- Not necessarily trained

- Conflicts of Interest (CoI) = Link to patient organisaitons with >40% industry funding



Patient organisations

- Addition after consultation to return in some way to EUnetHTA JA3 process (going beyond regulation)
- Submission template for patient groups to inform scoping in JCAs (PICOs coming from MS)
- CoI rules unclear *(and unfair given effort needed)*

Thank you!

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