

Involvement of patients in HTA processes and an introduction to the HTx project

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Agenda



Mentimeter voting!

- Introduction
- What is health technology assessment (HTA)?

- Introduction to the HTx Project

- What is patient involvement in HTA and why is it important?
- What are the main barriers in CEE countries?
- Recommendations and takeaway messages

Introduction and acknowledgments

Ivett Jakab – former President of the European Patients' Forum Youth Group

Maria Dimitrova - associate professor at Faculty of pharmacy, Medical University of Sofia

+ partners at the **HTx Consortium** and external experts



How much do you know about HTA?



**Mentimeter
voting!**

<https://www.menti.com/>

Slide type: Multiple Choice

Question: How much do you know about health technology assessment (HTA)?

Options (4):

1. I have never heard of HTA
2. I have heard about it, but I have no experience
3. I have some experience with HTA processes
4. I participated in HTA processes or research about HTA

What is health technology assessment (HTA)?

Health technology:

- **any intervention** that may be used to **prevent, diagnose or treat diseases**
- Pharmaceuticals, devices, procedures, organizational systems used in health care and public health interventions

Health technology assessment:

“A multi-disciplinary field of analysis that examines the **medical, economic, social and ethical implications of the incremental value**, diffusion, and use of a health technology in health care.” – INAHTA

Why we use HTA?

- To make informed decisions
- Purchasing something is a decision
 - we assess the **value** ♥ against the **cost** 💰
- **Value** is a commonly used word, but it always has a very individual meaning - *the importance, worth, or usefulness of something*



Example of purchasing a car: 🚗 🚚 🚙 🚛

What are the most important attributes to consider when you purchase a car?

Maintenance cost, Price, Fuel Consumption, Engine Performance, Color, Style, Manufacturer, Carbon Footprint, Road Safety?

What are the most important attributes to consider when you buy a car? 🚗

Slide type: Ranking

Question: What are the most important attributes to consider when you buy a car?

Items:

1. Maintenance cost
2. Price
3. Fuel Consumption
4. Engine Performance
5. Color
6. Brand
7. Carbon Footprint
8. Road Safety



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Why we use HTA? (cont.)

- Same decision problem, but...
 - Complex technologies that directly influence people's health
 - More stakeholders from different perspectives on „what value is?“
 - Purchasing from public resources – greater impact of decisions
- All public health resources are limited in ALL countries
- **Evidence-based medicine:** Effective technologies should be selected in order to maximize health gain for patients
- **Health Technology Assessment:** Cost-effective technologies should be selected, because society cannot provide all effective health technologies to everyone from public resources

Components of health technology assessment

- clinical & economic burden
- unmet medical need
- public health priority
- clinical results (efficacy)
- health impact in real-world (effectiveness)
- cost-effectiveness
- budget impact
- equity aspects
- legal aspects
- social aspects
- infrastructural and organizational aspects

→ **health care priority?**

→ **health gain?**

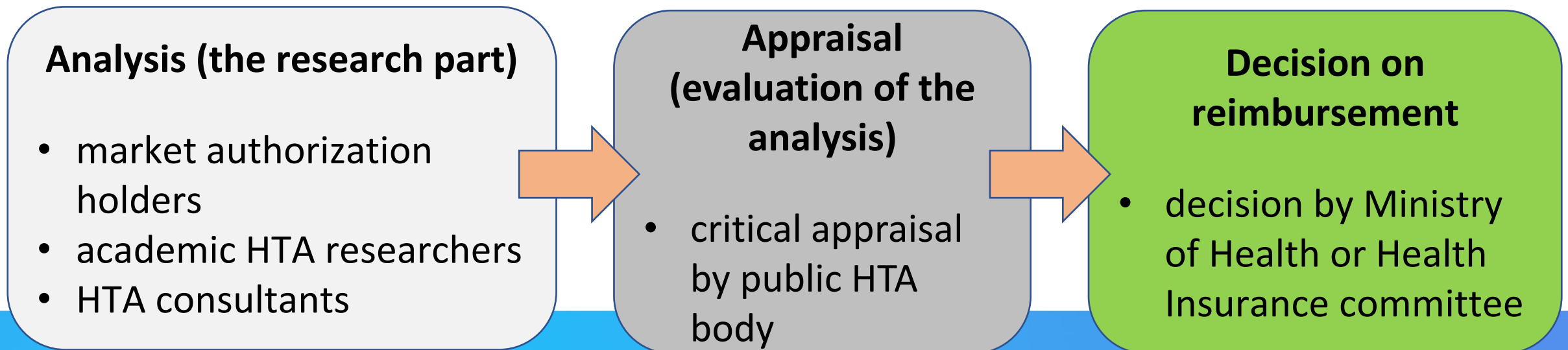
→ **value for money?**

→ **affordability?**

To help decision-makers answer these questions and make informed decisions

Most common HTA process

- HTA is heterogenous among EU Member States – all countries have individual HTA bodies
- EU Regulation on HTA entered into force in January 2022 and applies as of January 2025 (joint clinical assessment)





Next Generation Health Technology Assessment

How can Real World Evidence confirm/inform the added value of a health technology?

Bertalan Németh
Syreon Research Institute

Data analysis in marketing - examples



- *“For example, let’s say you buy a blouse online. When you visit the [Burberry] store, an employee can see this purchase and recommend matching handbags or accessories. They can even suggest items other consumers have bought alongside the same blouse.”*
- *“McDonald’s [...] can see what foods and services customers order, how often or even whether they visit the drive-thru or go inside. In fact, Japanese customers using the company’s mobile app spend an average of 35 percent more because of spot-on recommendations just before they are ready to order food.”*
- *“Each week, Spotify offers every user a personalized playlist with music recommendations based on their listening and browsing history. It’s kind of like a curated mixtape from the platform, offering new tracks and artists, showing you new genres you might enjoy or even updating you on your favorite music.”*

Key question



If we can use modern methods
(artificial intelligence, big data,
machine learning, real-world data)
to **sell products**...

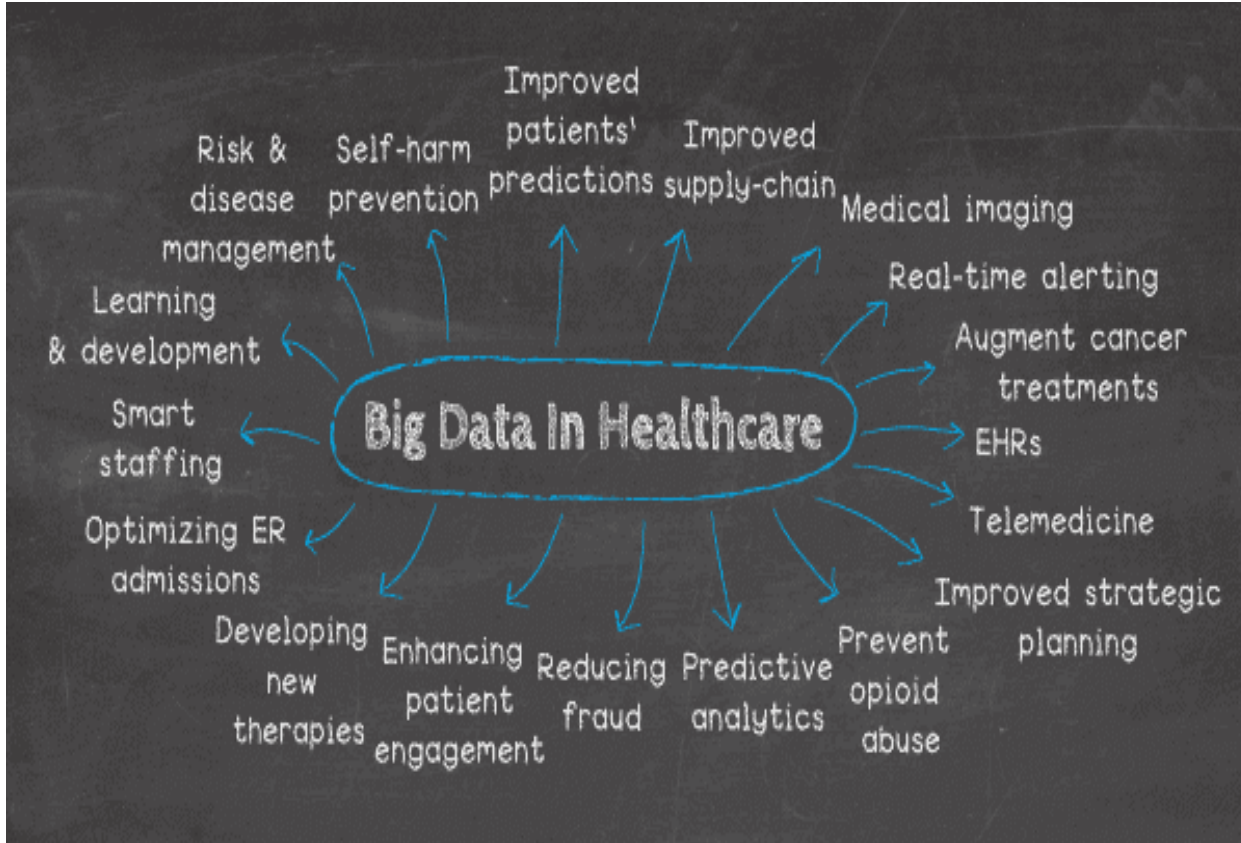
...can we also use them to **improve healthcare?**

Data analysis in healthcare - example



- *“We train a convolutional neural network (CNN) using a dataset of 129,450 clinical images consisting of 2,032 different diseases”*
- *“We test its performance against 21 board-certified dermatologists”*
- *“The CNN achieves performance on par with all tested experts across both tasks, demonstrating an artificial intelligence capable of classifying skin cancer with a level of competence comparable to dermatologists.”*

Data analysis in healthcare - example



Using Big Data to Prioritize Cancer Treatment During COVID-19

A new, web-based app draws on big data resources to create a personalized risk assessment for receiving cancer treatment during COVID-19.



Source: Getty Images

<https://www.datapine.com/blog/big-data-examples-in-healthcare/>

<https://healthitanalytics.com/news/using-big-data-to-prioritize-cancer-treatment-during-covid-19>

Key question #2



Can we use these methods to assess the **value** of health technologies?

What is value based price?

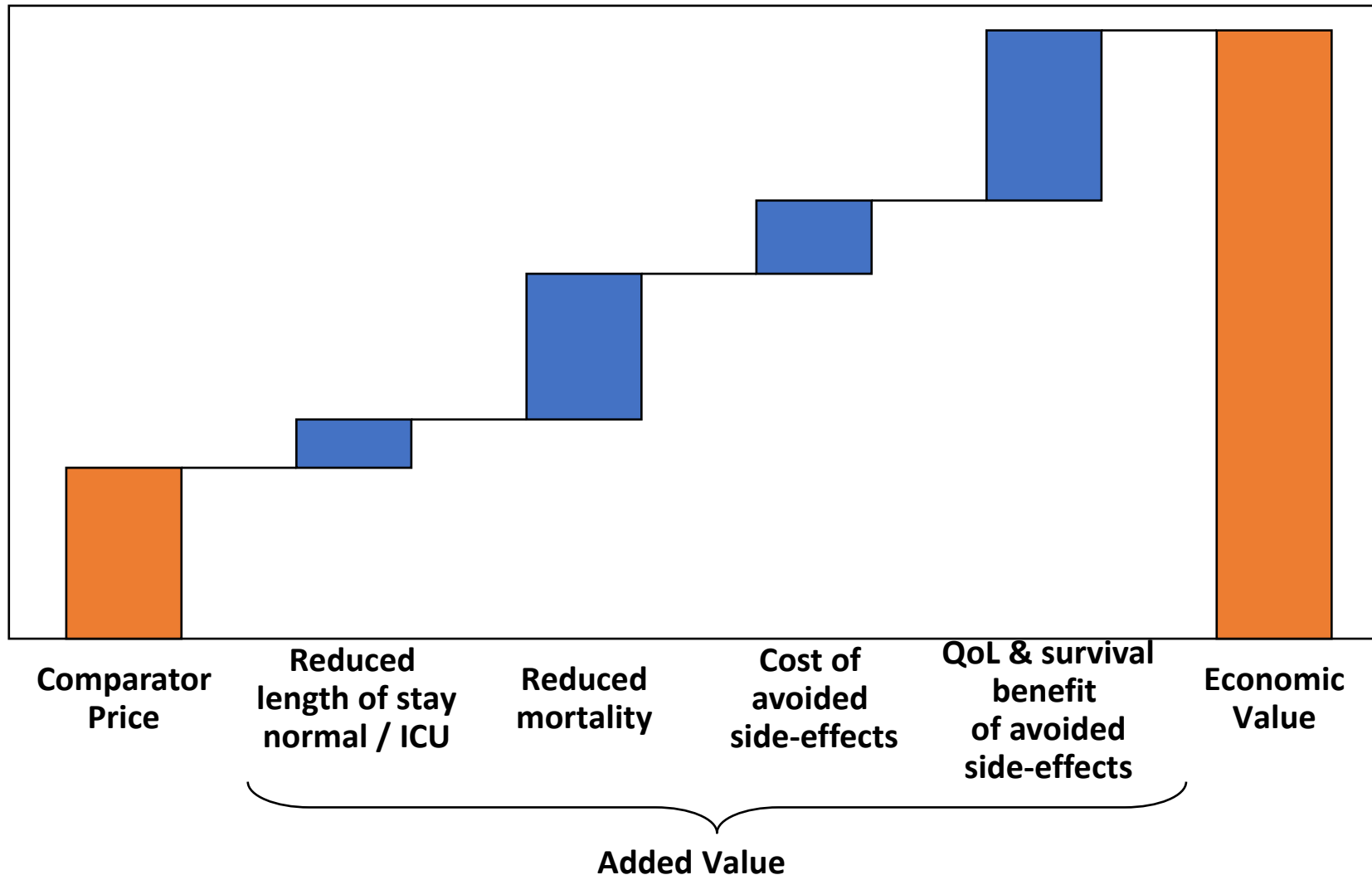


New technology is cost-effective at a given price

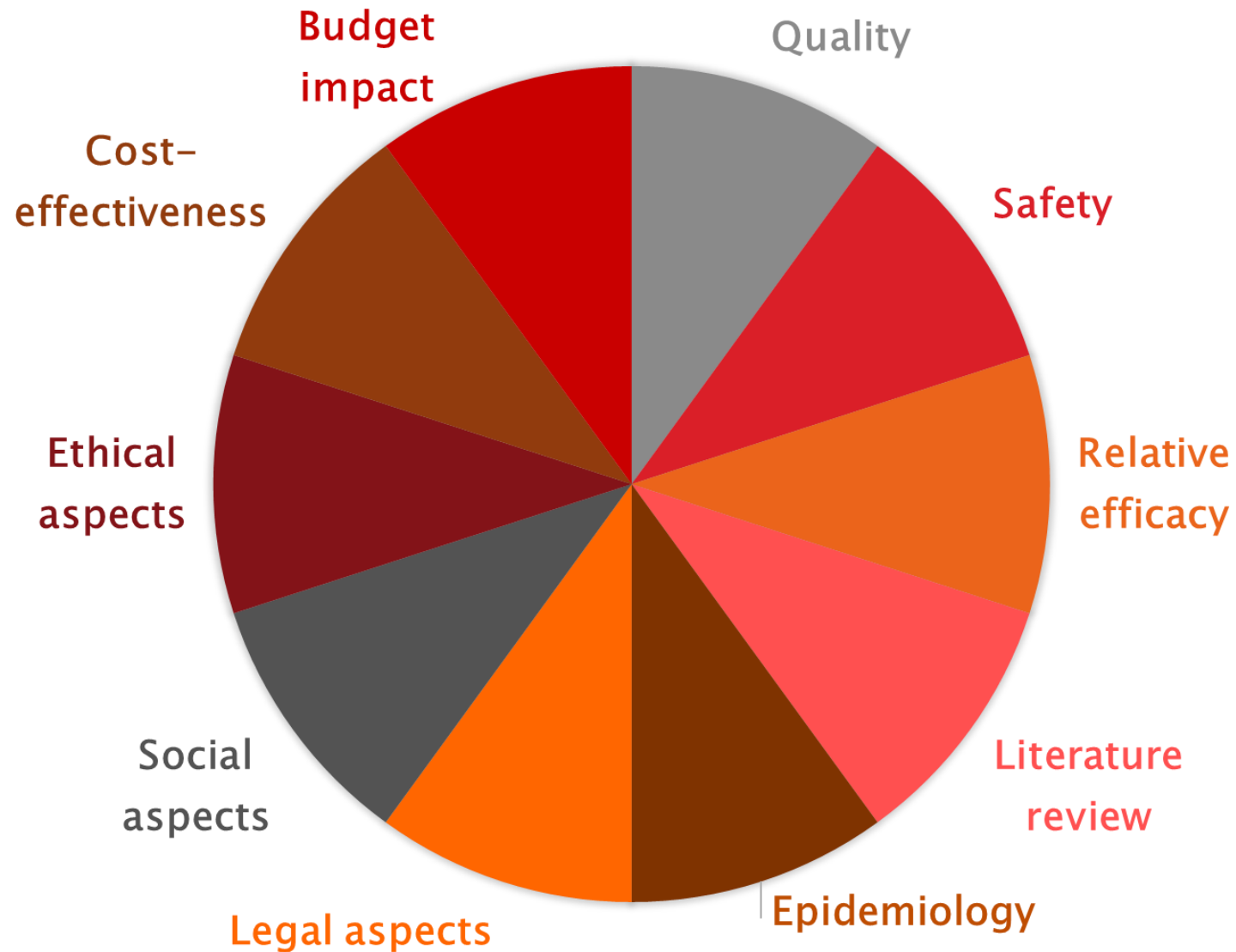
OR

The price premium of a new technology can be justified by additional health gain or savings in the health care budget

Elements of Added Value



What is Health Technology Assessment?

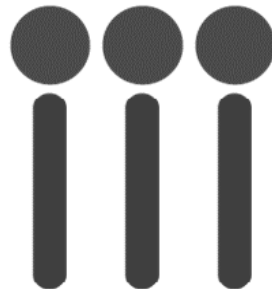


Patient input:
e.g. what are the
most important
outcomes?

Clinical trials versus real-world evidence



	Randomized Controlled trials	Real-World Evidence
Purpose	Efficacy	Effectiveness
Setting	Experimental setting	Real-world setting
Treatment	Fixed pattern	Variable pattern
Study group	Homogenous	Heterogeneous



Based on: Kim, H. S., Lee, S., & Kim, J. H. (2018). Real-world evidence versus randomized controlled trial: clinical research based on electronic medical records. *Journal of Korean medical science*, 33(34).

HTx: Vision for a new generation of HTA



- Imagine an individual patient who visits the doctor for a medical problem. The **doctor knows this patient's clinical history** (including her use of different health technologies, such as medical devices, e-health technologies and drugs), **her preferences and health outcomes**.



- Adequate clinical studies and real-world data analysis have resulted in a **real-time decision support system that the doctor and the patient can use to obtain person-centered information** (in a user-friendly format) about risks, benefits, outcomes and costs associated with a range of possible strategies to manage the patient's ailment.



- The **same information is made available to HTA agencies whose decisions are informed by means of this information**, analysed at the level of individuals and summarised at the subgroup and population level for the benefit of payers' decision-making. **This framework is what we envision as HTx.**

About the HTx project



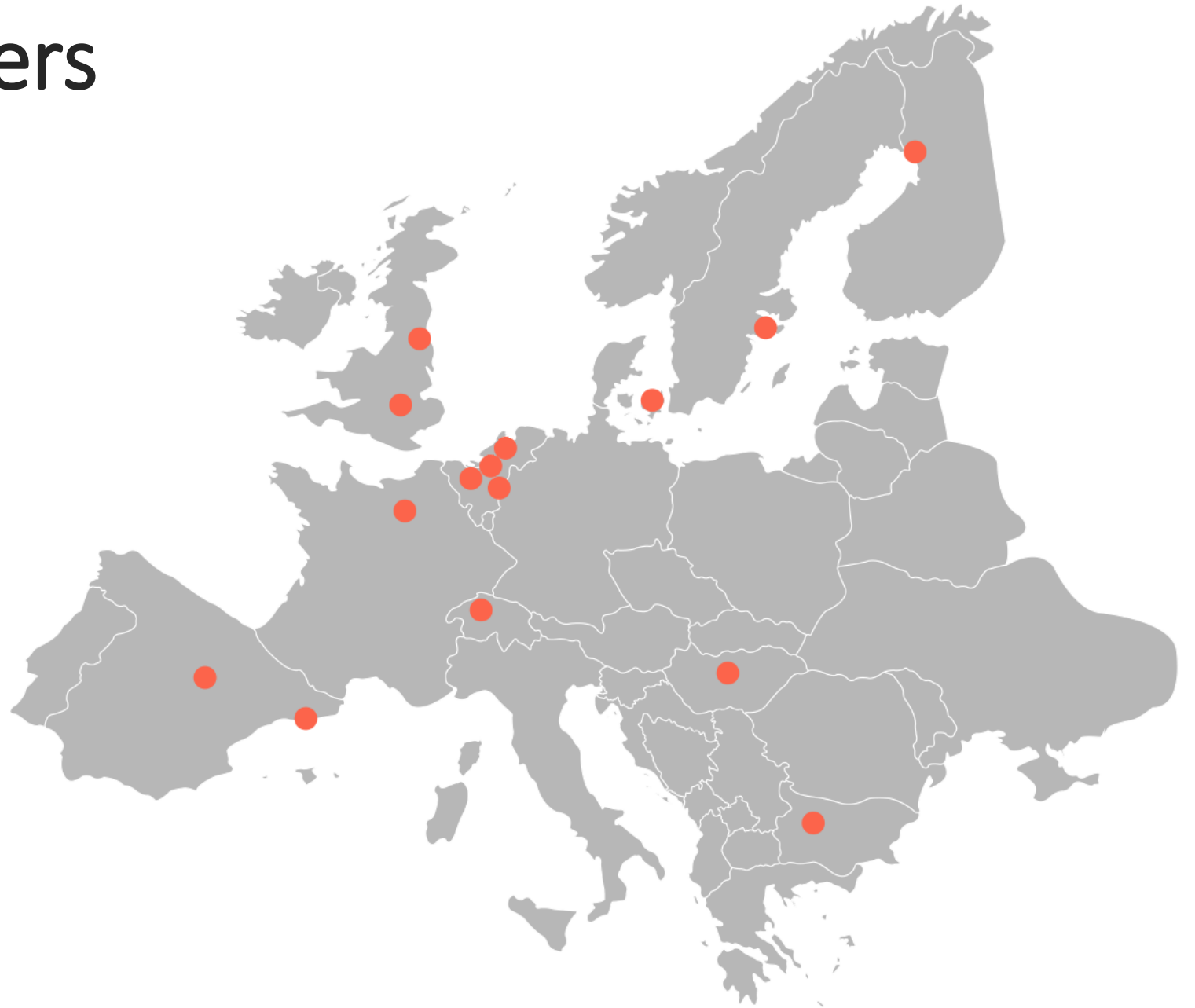
- HTx is a **Horizon 2020** project supported by the **European Union**, kicking-off in **January 2019** and lasting for **5 (and a half) years**.
- The main aim of HTx is to create a framework for the Next Generation Health Technology Assessment (HTA) to support **patient-centered, societally oriented, real-time decision-making on access to and reimbursement** for health technologies throughout Europe



Consortium partners



University of Utrecht
Project Coordinator



Advisory boards – HTx Forum

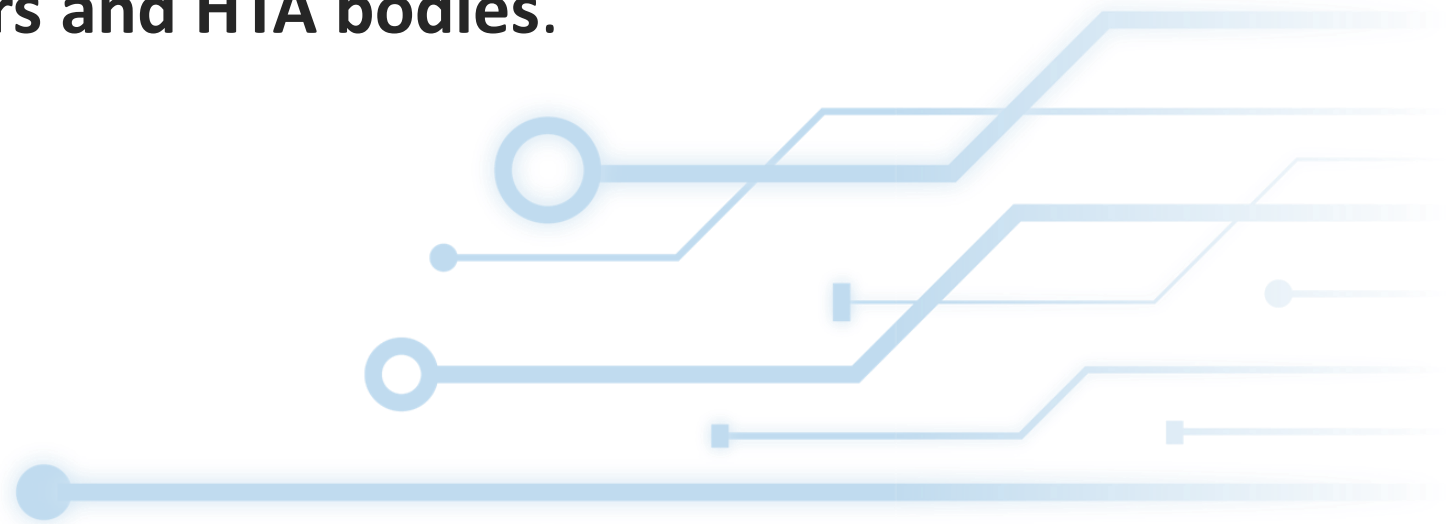


Role:

- to discuss the **broader implications of methods and tools** developed in project for society and healthcare systems.

Participants:

- senior representatives of the most important stakeholder groups, which are **patients and consumers, payers, healthcare providers, technology producers** and also **regulators and HTA bodies**.



HTx Case Studies

- **CASE 1** on the use of proton-therapy in patients with **in head and neck cancer**. In collaboration with EORTC, the study is delivering an evaluation of an expensive non-divisible medical device delivered intervention for which individualisation of treatment is crucial to maximise its effectiveness and cost-effectiveness.

HTx Case Studies

- **CASE 2** in which we address combinations of health technologies **diabetes type 1 (T1DM) and type 2 (T2DM)**. In this case study, we use the Maastricht Study (NL), a telemonitoring study from the University of Pécs Medical School (HU), data from the National Health Insurance Fund of Hungary(33), and data from the Pediatric Diabetes Clinic, Oulu University Hospital (FI) about patients with T1DM

Based on such a linkage we should be able to provide more accurate estimations of the differential health impact of the technologies in specific subgroups of patients and predict which treatments combinations are most beneficial and cost-effective.

HTx Case Studies



- In **CASE 3** we focus on optimal treatment for relapsing-remitting **Multiple Sclerosis (MS)**: For four of the included drugs the manufacturing company (Biogen) provides individual patient data, which together with the Sylvia Lawry Centre for MS Research creates a unique opportunity to combine randomised and real-world data on a large set of treatment options and treatment sequences with the ability to estimate treatment effects within patient's subgroups to present the basis for an individualised treatment decision-making.

HTx Case Studies



- For **CASE 4** we concentrate on **myelodysplastic syndrome (MDS)**; **together with** the H2020 MDS-RIGHT study we focus on evaluating the effectiveness and cost-effectiveness of treatment sequences, combination of therapies, the development of prediction models for individualised treatment decisions and the analysis of relevant patient reported outcomes (PROMS).



Next Generation Health Technology Assessment

Website: www.htx-h2020.eu

E-mail: info@htx-h2020.eu

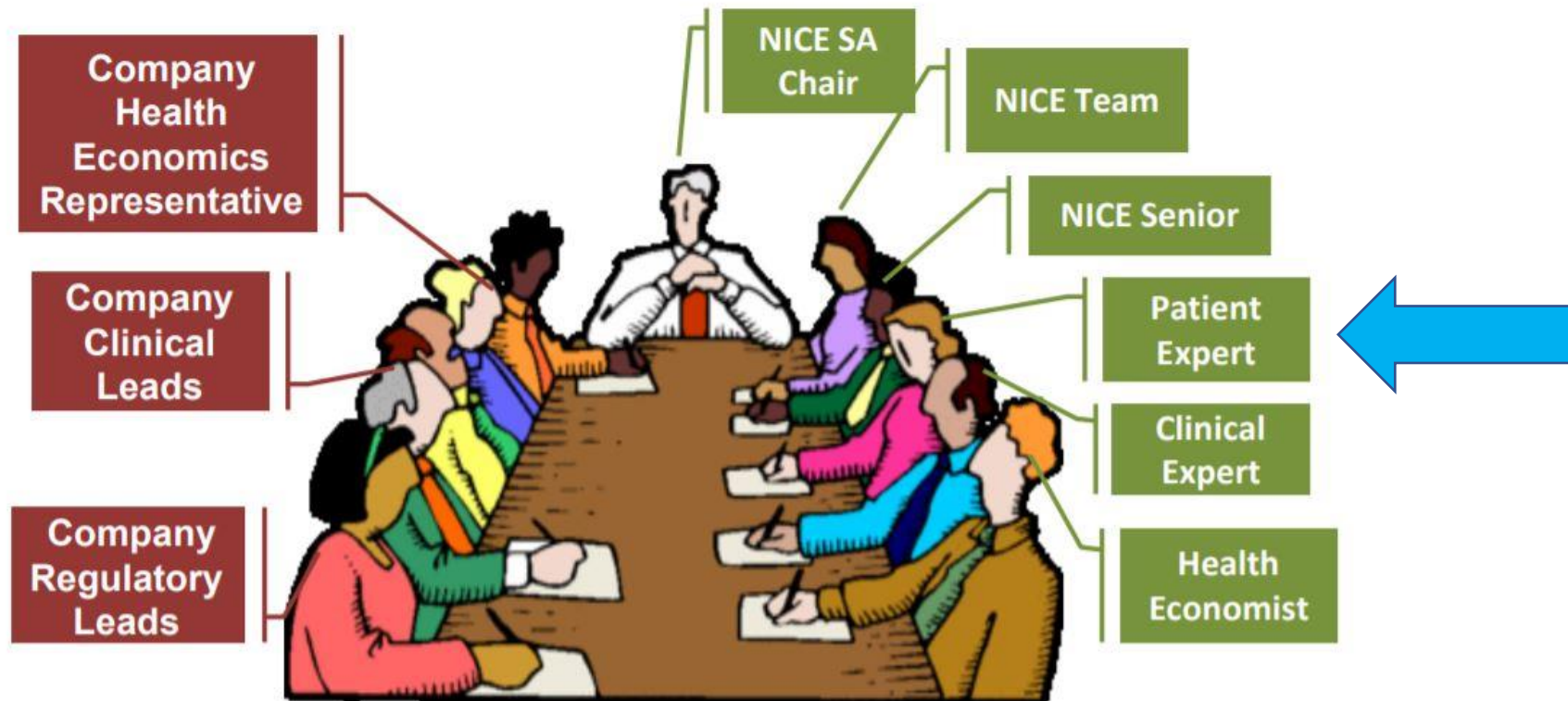


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But...where is the patient in this ?



NICE

Source: https://www.ispor.org/docs/default-source/presentations/ispor-2018-implementation-of-patient-preferences-in-hta_261118.pdf?sfvrsn=d6f60999_0

What is patient involvement in HTA?

WHO?

A “patient” involved in HTA refers to anyone...

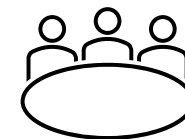
- **who can communicate the lived experience of a health condition, healthcare technology, or healthcare system**
- E.g., Patients, Patient experts, Patient representative, Patient advocate, Patient advisor

What is patient involvement in HTA? (cont.)



Considering patient-centric value elements in value assessment

Considering transparent and verifiable patient experience (PEX)-related criteria in the value judgment of marketed health technologies



Involving patients in HTA processes

The timely involvement of patient representatives in healthcare research, product development and HTA processes.

Value pillars to be considered in HTA

Patient-centric value elements

- Patient and caregiver reported outcomes
- Household's financial burden
- Adherence
- Caregiver burden

Traditional value elements

- Efficacy
- Health-related quality of life
- Direct healthcare costs

Societal value elements

- Improved equity
- Increased productivity

Ranking of patient-centric value elements (Voting)

1. Ability to reach important **personal milestones**
2. **Patient's financial burden**
3. **Value of hope/balance** or timing of risks and benefits
4. **Uncertainty about long-term** benefits and safety of the treatment
5. **Patient empowerment** through therapeutic advancement and technology
6. **Caregiver/family's financial burden**
7. Patient experience related to **treatment regimen**
8. Therapeutic **options**
9. **Caregiver/family's quality of life**

Name	Description
Ability to reach important personal milestones	Patients' ability to achieve major life goals related to education, work, family affairs (e.g., pregnancy), and social integration
Patient's financial burden	The financial impact of the disease and treatment on the patient including drug cost, medical costs of treatment and disease management (e.g., hospitalization), and nonmedical costs (e.g., transportation costs, absence from work) borne by the patients
Value of hope/balance or timing of risks and benefits	Potential of a treatment to provide the chance of "cure" at a great risk
Uncertainty about long-term benefits and safety of the treatment	Uncertainty about the long-term benefit and safety
Patient empowerment through therapeutic advancement and technology	Therapeutic advancement or improved technology (e.g., digital health tools) combined with the treatment to empower patients in self-management and to allow their involvement as equal partners through their patient journey (e.g., through shared decision making)
Caregiver/family's financial burden	The financial impact of the disease and treatment on the caregiver(s) (e.g., travel costs, absence from work)
Patient experience related to treatment regimen	The patient's experience related to the management of the condition with available therapies (e.g., infusion-related reactions, site of injection reaction, pain, frequency of taking the treatment, location, and time needed for administration) and the potential of the new treatment to influence it
Therapeutic options	The treatment provides the first/only option for the patient and/or extends current or future treatment options, for example, by promoting the possibility of individualized therapy
Caregiver/family's quality of life	The caregiver's general perception of how the patient's disease and treatment affect their own physical, physiological and social aspects of everyday life (e.g. anxiety, social isolation, exhaustion, health consequences)

Ranking of patient-centric value elements

Slide type: Multiple choice

Question: What are the 3 most important patient-centric value elements from the list from your perspective?

Options (9):

1. Ability to reach important personal milestones
2. Patient's financial burden
3. Value of hope (balance or timing of risks and benefits)
4. Uncertainty about long-term benefits and safety of the treatment
5. Patient empowerment through therapeutic advancement and technology
6. Caregiver/family's financial burden
7. Patient experience related to treatment regimen
8. Therapeutic options
9. Caregiver/family's quality of life



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2 Case Studies

Neuromyelitis optica spectrum disorder (NMOSD)

- 8 patients ranked the value elements by anonymous voting (Mentimeter)
- Their 3 most important were:
 1. Uncertainty about long-term benefits and safety of the treatment
 2. Patient experience related to treatment regimen
 3. Patient's financial burden

Spinal muscular atrophy (SMA type II-III)

- 9 patients ranked the value elements by coin allocation
- Their 3 most important were:
 1. Ability to reach personal milestones
 2. Patient's financial burden
 3. Value of Hope/Balance timing of risks and benefits

Summary or exercise

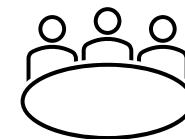
- **Traditional value elements are essential** (efficacy, safety, HRQoL, costs)
- **But not enough** to capture the total patient experience spectrum
- Identifying the most important value elements is a **complex task, with high cognitive burden**
- Preferences for value elements are **likely to be disease specific**
 - Nature and progression of disease
 - Time since receiving diagnosis
 - Age of affected patients
 - Available treatments at the time of ranking

What is patient involvement in HTA? (cont.)



Considering patient-centric value elements in value assessment

Considering transparent and verifiable patient experience (PEX)-related criteria in the value judgment of marketed health technologies



Involving patients in HTA processes

The timely involvement of patients and patient representatives in healthcare research, product development and HTA processes.

What is patient involvement in HTA? (cont.)

WHAT?

Several frameworks exist

- Participating in consultations (patient present at meetings)
- Shaping conclusions and recommendations in final documents that inform decision-making (reviewing documents)
- Producing/reviewing plain language summaries of assessments that will be shared with the public
- Being involved in designing and reviewing HTA processes/methods

Why is patient involvement in HTA processes important?

- Decisions are made that have significant impacts on patients' lives
- Involving patients...
 - ensures that decision-makers have sufficient patient-level input
 - increases the acceptance of HTA recommendations and reimbursement decisions
 - ensures that HTA outputs are relevant to patients and society in real life

But what is the problem then ?

Previous research conducted within HTx

Scoping literature review

Basis of the list of barriers

Webinar with CEE stakeholders

Feedback on barrier list, concept presented

Survey for CEE stakeholders

Top barriers identified

Workshop with CEE stakeholders (Pula, 2022)

Top barriers with potential solutions

Potential Barriers of Patient Involvement in Health Technology Assessment in Central and Eastern European Countries

Maria Dimitrova^{1}, Ivett Jakab^{2†}, Zornitsa Mitkova¹, Maria Kamusheva¹, Konstantin Tachkov¹, Bertalan Nemeth², Antal Zemplyeni^{2,3}, Dalia Dawoud^{4,5}, Diana M. J. Delnoij^{6,7}, François Houyez⁸ and Zoltan Kalo^{2,9}*

1. Patient Perspective
2. Payer/HTA Perspective

Why focus on CEE countries?

- The approach to patient involvement **differs greatly** by country and regions
- CEE countries generally have a **worse health status** and **less public resources for healthcare** → evidence-based policy decisions are needed
- Surveys by EPF and Health Technology Assessment International's Patient and Citizen Involvement Group (HTAi PCIG) reported that many Western European HTA bodies involve patients in their processes
- There was **only one** HTA organization from the CEE region reporting on patient involvement activities—**Poland**

What are the main barriers? - Patients

PATIENT PERSPECTIVE	CATEGORIES	PRIORITISED BARRIERS
	Lack of understanding the decision context	<ul style="list-style-type: none"> • Patient representatives' lack of basic knowledge in HTA • Patient representatives' lack of knowledge of the local regulatory processes including how they can get involved
	Lack of knowledge and guidance of evidence-based advocacy	<ul style="list-style-type: none"> • No methodological guidance to support the activities of patient organisations in collecting data (e.g., survey) valuable for HTA • Patients' lack of experience in searching and/or interpreting information from independent resources (i.e., scientific articles)
	Lack of resources to be spent on meaningful patient representation	General lack of capacities of patient organizations due to financial constrains
	Difficulties to finding the 'right' patient	Lack of support and supporting tools (e.g., registries or networks) to help disease-specific patient recruitment

What are the main barriers? – Payers/HTA

	CATEGORIES	PRIORITISED BARRIERS
PAYER / HTA PERSPECTIVE	Limited willingness to involve patients	<ul style="list-style-type: none"> • Limited consideration of societal factors on pricing and reimbursement decisions • Lack of understanding of the added value of involving patients in the HTA process • Patient involvement in HTA is not mandatory / is not mentioned in the local HTA guideline
	Lack of human resources at relevant public institutes	Payer or HTA organisations do not have enough human resources/time to involve patients (even though they would intend to)
	Not knowing how to involve patients	<ul style="list-style-type: none"> • Lack of experience / training / skills in HTA and payer organisations of knowing how and when to incorporate patient perspectives • Lack of local (regional or country-specific) guidelines on best practices of patient involvement in HTA

Recommendations for involving patients into HTA in CEE countries

1. Educate HTA/payer organizations on the value of patient involvement
2. Acknowledge patients as experts of their condition similar to health care professionals
3. Dedicate a person/team to be responsible for patient involvement activities at HTA bodies
4. Set a certain percentage of the HTA annual budget to be spent on patient involvement



Recommendations for involving patients into HTA in CEE countries (cont.)

5. Compensate patient experts (involved in the HTA process) for time and transportation fairly
6. Launch EU-funded calls for the implementation of patient-centric value assessment of health technologies (especially in countries with limited experience in patient involvement)
7. Set up a training center to provide tailored trainings for patients on local HTA, health policy decision-making procedures and the generation of patient-centric scientific evidence



Ranking of recommendations



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Slide type: Ranking

Question: Please rank the presented recommendations by relevance from your perspective!

Items (7):

1. Educate HTA/payer organizations on the value of patient involvement
2. Acknowledge patients as experts of their condition similar to health care professionals
3. Dedicate a person/team to be responsible for patient involvement activities at HTA bodies
4. Set a certain percentage of the HTA annual budget to be spent on patient involvement
5. Compensate patient experts (involved in the HTA process) for time and transportation fairly
6. Launch EU-funded calls for the implementation of patient-centric value assessment of health technologies (especially in countries with limited experience in patient involvement)
7. Set up a training center to provide tailored training for patients on local HTA, health policy decision-making procedures and the generation of patient-centric scientific evidence

Available Resources

- **EURORDIS on new HTA legislation**
<https://www.eurordis.org/demystifying-hta/>
- **INAHTA Position Statement: Patient Involvement**
<https://www.inahta.org/position-statements/>
- **EUnetHTA on patient involvement in relative effectiveness assessments**
https://www.eunetha.eu/wp-content/uploads/2019/06/Final_290519_Patient-Input-in-REAs.pdf?x69613
- **The European Patients Academy on Therapeutic Innovation (EUPATI) guideline on patient involvement in HTA**
<https://www.frontiersin.org/articles/10.3389/fmed.2018.00231/full>
- **HTAi PCIG**
<https://htai.org/patient-and-citizen-involvement/>