Volunteer engagement across the medicine's lifecycle and healthcare

(Part 2)

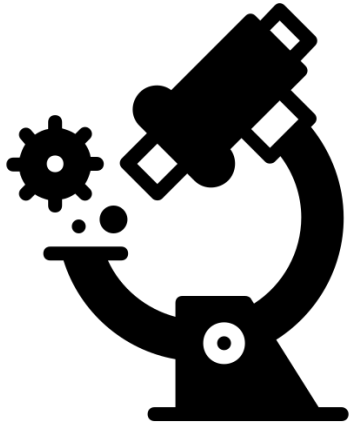
Julien Delaye, Patient Engagement Manager, EURORDIS

Aims of this session

- Follows Volunteer engagement across the medicine lifecycle and healthcare session
- Explore key aspects of HTA and the HTAR
- Get familiar with how patients are engaged in EU HTA
- Get informed about EURORDIS' HTA and DITA task-forces
- Delve into concrete example through questionnaires presented by volunteer
- Learn about Community Advisory Boards (CABs)

Where are we in the product lifecylce?

Research and discovery → Clinical trials → Regulatory → HTA → National use



Decision-making in healthcare

- In health, decision-makers (Ministry of health, health insurance funds, hospital management boards, etc.) cannot purchase, use and reimburse all new technologies for prevention, diagnosis, treatment, and rehabilitation of disease
- They have to make a choice and decide whether a new health technology brings **added value** to standard of care/current treatment
- **Investment and disinvestment decisions should be well informed and evidence-based**

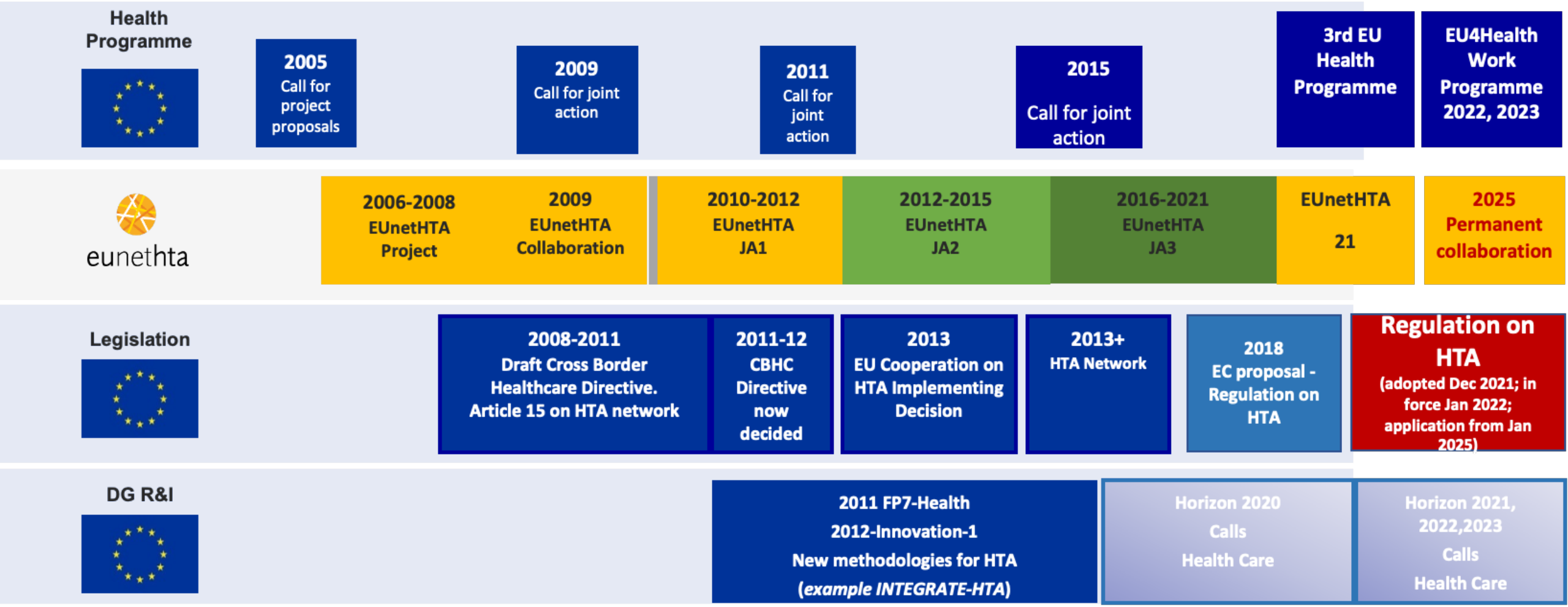
HTA 1.01 – A bridge between research evidence and health policies

- Policymakers need a tool that provides them with the **best available evidence to inform decision-making** and **develop guidance on the reimbursement** and administration of new health technologies
- They need **Health Technology Assessments (HTAs)**
  - A multidisciplinary approach that **compares new technologies with an already existing one** (or the standard of care) to assess whether it is more effective, equally effective, or less effective
    - **Dimensions of value:** clinical effectiveness, costs and economic implications, ethical, social, cultural and legal issues, organisational and environmental aspects, as well as wider implications for the patient, relatives, caregivers, and the population

HTA in Europe and the HTAR

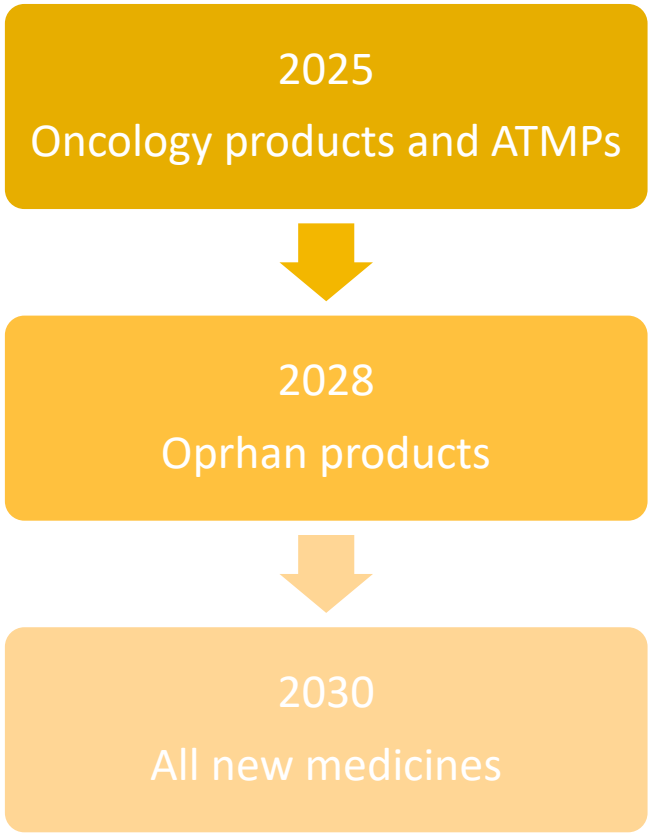
- HTA processes in the EU are fragmented. **Each Member State conducts its own evaluations**, leading to duplicated efforts, inconsistent outcomes, and delays in patient access to innovative therapies
- The introduction the new HTA Regulation has changed/will change this dynamic, streamlining the HTA process across the European Union, although some competences (such as the decision on whether or not to reimburse a new technology) will remain a national prerogative

A long European history to get there



Key aspects of the HTAR

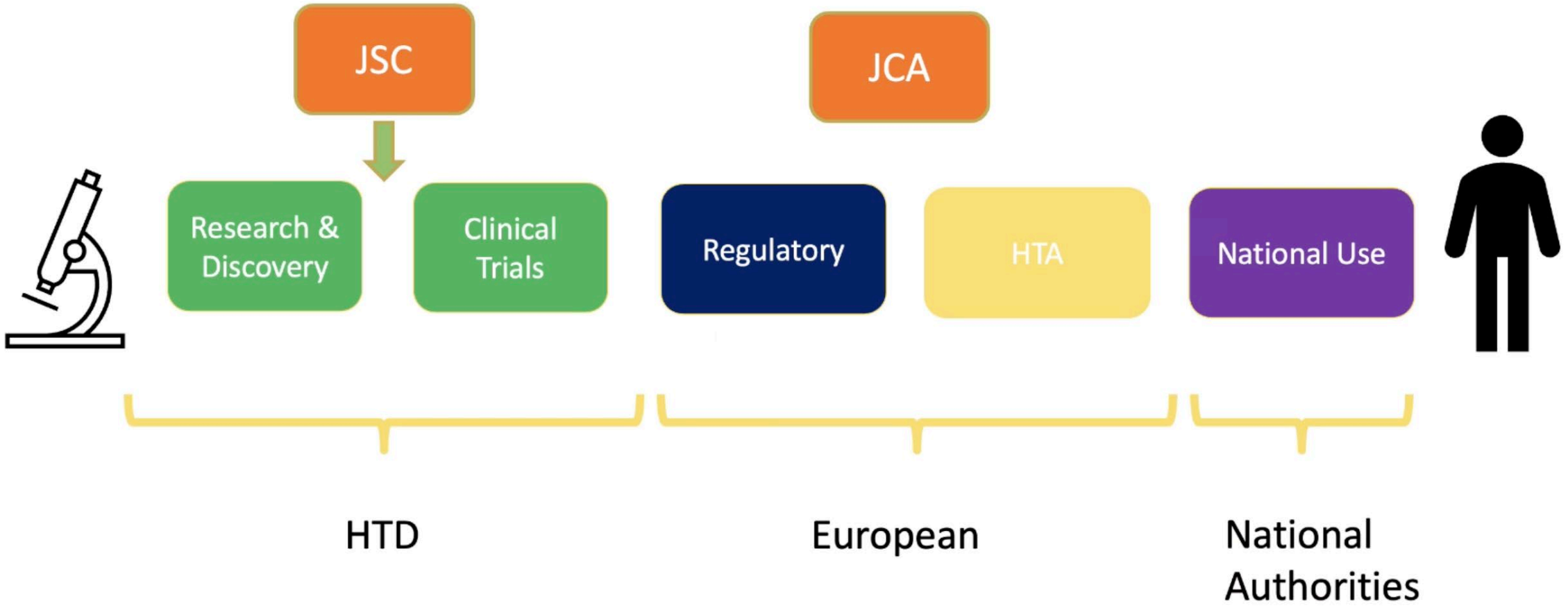
- Joint work on common scientific, clinical aspects of HTA
- Driven by Member State HTA bodies
- Ensure high quality, timelines and transparency
- Ensure use of joint work in national HTA processes
- Member States remain responsible for:
  - Drawing conclusions on added value for their health system
  - Taking decisions on pricing & reimbursement
- Addresses stakeholders' engagement in joint work
- Progressive implementation



4 areas of joint work

- **Joint Clinical Assessments**
  - Compilation of comparative clinical evidence with an analysis of the degree of certainty of the available data
  - In accordance with an assessment scope (PICOs)
  - Based on the scientific aspects of the clinical domains of HTA
- **Joint Scientific Consultations**
  - Offers recommendations to HTDs on their development plans for at an early stage of the development where the clinical studies and clinical investigations are still in the planning stage
  - Discussions are structured around PICO and health economic assessment (optional)
- Emerging health technologies (horizon scanning)
- Voluntary cooperation

JSC and JCA


JSC
JCA
Research & Discovery
Clinical Trials
Regulatory
HTA
National Use
HTD
European
National Authorities

A quick word on the HTA assessment scope

- The basis of a HTA is a set of **defined research questions** that are to be answered by the assessment and that together define the assessment scope.
  - **P** – Population
  - **I** – Intervention
  - **C** – Comparator
  - **O** – Outcome
- Translation of national policy questions into research questions
- Opportunity for each MS to identify and provide their national needs
- E.g. Refractive laser surgery for people with vision conditions

PICO	Description
Population	**People** with **vision conditions** (e.g., myopia, astigmatism, presbyopia)
Intervention	**Refractive laser surgery**
Comparator	**Conventional vision technologies** (e.g., prescription glasses, contact lenses)
Outcomes	**Clinical benefits** (e.g., visual acuity, QoL, patient satisfaction) and **harms** (adverse events) **Other domains**: **Organisational** (e.g., implementation considerations) and **Social** (e.g., values and preferences of patients and physicians) **Recommendations** from **evidence-based guidelines**

Patient Engagement in EU HTA – What to expect?

- The HTAR establishes quality standards for the joint work
  - It requires the systematic and timely participation of patient experts in the procedures, especially in the main activities, such as JSCs and JCAs.
- Patients are expected to share their expertise with the condition through a questionnaire, in written form or during an online interview.
- For instance, they may be asked to provide inputs on
  - The impact of the disease in daily life,
  - If different patients are affected differently,
  - Which treatments are currently used and what are their limitations and benefits,
  - If different people respond differently to these treatments,
  - If patients think specific subgroups of patients need special consideration,
  - On which effect will they decide if the medicine is working for them,
  - If they should take it, what are their expectations for a new treatment
  - And other questions that will help experts assess the medicine.

Patient Engagement in EU HTA – where is engagement happening?

- **Scientific consultation**
  - To minimise the risks that inadequate information from clinical trials are submitted at a later stage
- **Scoping (PICO)**
  - Which domains/topics/questions should be answered?
  - Which target (P)opulation? Which (I)ntervention? Which (C)omparator? Which relevant (O)utcomes to consider?
- **Clinical assessment**
  - Answers related to questions important to impact of disease, experience with currently available interventions, expectations of/requirements for new health technologies under assessment, and additional information which the patient and/or caregiver believed would be helpful to the HTA researchers
- **Comments on draft reports**

Patient Engagement in EU HTA – How are patients recruited?

- To identify experts, the European Commission (EC) relies on:
  - The EMA and Orphanet databases
  - The HTA Stakeholder Network (of which EURORDIS is a member), ERNs, and National contact points
- For each procedure, these actors and database provide contact details of patients to the EC
- Once the EC receives the patient's contact details, the patients need to fill out a Declaration of Interests form and a resume (CV) on the HTA IT Platform
- Patients are selected by the relevant subgroup (JSC/JCA) to take part in a procedure
- Patients are contacted by the Brussels Centre for Collaboration in Health (BCCH for administrative support (e.g. signing confidentiality agreement)
- Patients gain access to all necessary information to contribute to JSC/JCA
- Personal data of patients involved remain will remain confidential

Conflict of Interest

- Participants must be free of conflict of interest (CoI)
- Examples of what constitutes a CoI
  - Executive position in a health technology developer in the past 5 years
  - Reimbursement above EUR 1 000 from one health technology developer over the past 3 years
  - Shares or other intellectual property rights
  - Principal investigator or investigator over the past 3 years
- Annex II of Implementing Regulation 2024/2745 provides the list of what may constitute a conflict of interest
- If no patient free of CoI can be found to participate in joint work (cf rare diseases), the EC might be flexible

Barriers to patient engagement at national level (in CEE countries)

- Applicable more broadly than solely for CEE countries
- Must be differentiated between the perspectives of 1. HTA bodies and payers, and 2. patients and patient communities
- HTA bodies and payers

HTAR

  - Limited willingness to involve patients (lack of understanding of added value, lack of trust in objectivity and relevance, PE not mandatory in local HTA guidelines)
  - Conflict of interest and confidentiality (fear of COI due to PO funding, fear of violation of confidentiality by representatives)
  - Finding the “right” representative (lack of support tools, lack of understanding of patient roles, lack of potential representativeness)
  - Lack of resources and experience/training/skills, etc

- Patients and patient communities
  - Lack of understanding (basic knowledge in HTA, regulatory process, medical language)
  - Lack of knowledge and guidance of evidence-based advocacy (lack of experience in searching/interpreting results, no guidance to support activities)
  - Lack of resources (compensation, time, financial constrains)
  - Lack of ethical guidance for representativeness (no clear rules on how to represent a community)

Expertise, experience, knowledge

- As patients, the EC considers you are experts in your field
- No need to be an expert an HTA, data, clinical trials (etc) to participate in joint work
- But, should you want to increase your skills and knowledge to feel better prepared, training opportunities on HTA exist

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The HTA task-force – composition

- 12 members maximum (currently 8) from member organisations for a 3-year mandate (new mandate coming up)
  - Proficient in English
  - Training (e.g. open Academy)
  - Experience/expertise in regulatory/HTA
  - Available for 6 to 8 meetings
- Commitment to:
  - Participate actively in the work, incl. regular meetings
  - Propose ways to improve the transparency of the provision of information Contribute to the development and implementation of new Community Legislation and guidance in health technology assessment
  - Participate in the collection of information on published HTA reports

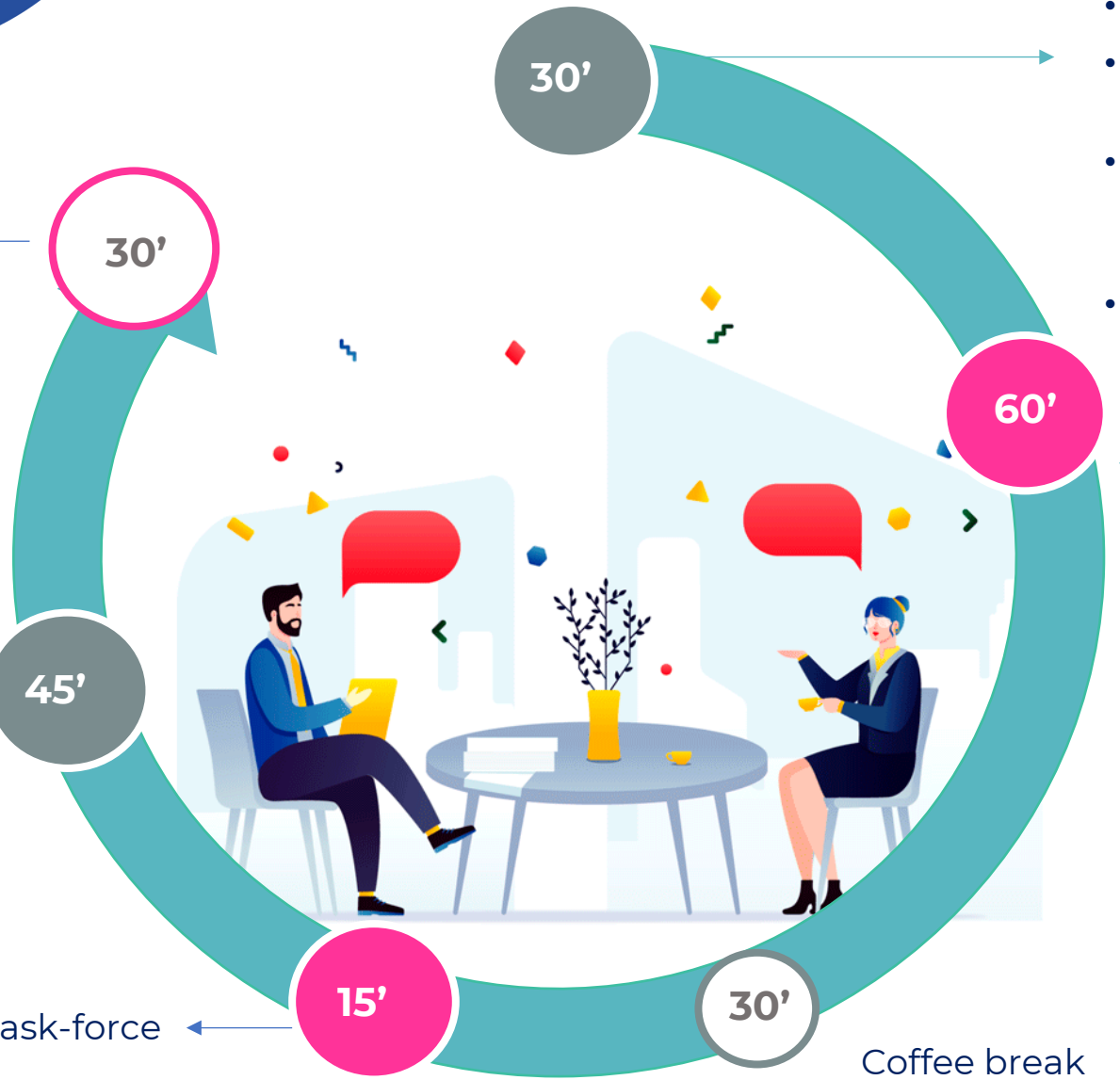
The HTA task-force – tasks

- Advises EURORDIS on all aspects regarding HTA policies and procedures
- Informs EURORDIS on how health technologies are assessed at the national level, how patients are involved
- Raises awareness on HTA
- Contributes to consultations
- Contributes to EURORDIS' positions
- Analyses and contributes to HTA activities (mapping of HTA activities, guidelines development, horizon scanning, early dialogues, parallel EMA/HTA scientific advice, etc)

The HTA task-force – examples of activities

- Contributions to responses to consultations on EU Implementing Acts (HTAR)
- Discussions around PICOs at European and national level
- Participations in specific parts of projects (HTx, EUCAPA)
- Participation at events (ERTC, EMM, external events, etc)
- Advice on product-specific cases
- Discussion around hospital exemption and pricing/reimbursement policies
- Etc.

Saturday Morning Workshop - Volunteer engagement across the medicine's lifecycle and healthcare (Part 2)



- Introduction and aims of the session
- What is health technology assessment (HTA) and what is it used for?
- The HTA Regulation and patient engagement under the European framework
- The EURORDIS HTA task-force

- Case studies on HTA presented by volunteers

Coffee break

- Community advisory boards (CABs)

- The DITA Task-force

- Q&A and wrap-up

Further resources

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

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Patients' perspectives are important to identify preferences, estimate values and appreciate unmet medical needs in the process of research and development and subsequent assessment of new health technologies. Patient and public involvement in health technology assessment (HTA) is essential in understanding and assessing wider implications of coverage and reimbursement decisions for patients, their relatives, caregivers, and the general population. There are two approaches to incorporating the patients' voice in HTA, preferably used in a mix. In the first one, patients, caregivers and/or their representatives directly participate at discussions in different stages of the HTA process, often at the same table with other stakeholders. Secondly, patient involvement activities can be supported by evidence on patient value and experience collected directly from patients, caregivers and/or their representatives often by patient groups Patient involvement practices, however, are limited in Central and Eastern European (CEE) countries without clear methodology or regulatory mechanisms to guide patient involvement in the HTA process. This poses the question of transferability of practices used in other countries, and might call for the development of new CEE-specific guidelines and methods. In this study we aim to map potential barriers of patient involvement in HTA in countries of the CEE region.

Keywords: patient engagement, health technology assessment (HTA), barrier, central and eastern EU countries, potential

INTRODUCTION

Patients' perspectives are important to identify preferences, estimate values and appreciate unmet medical needs in the process of research and development and subsequent assessment of new health technologies (1). Health technology assessment (HTA) is a multidisciplinary process that uses explicit methods to determine the value of a health technology from different dimensions. Such health technology can be a medical test, device, medicine, vaccine, medical procedure, program,

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Recommendations to overcome barriers to the use of artificial intelligence-driven evidence in health technology assessment

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Background: Artificial intelligence (AI) has attracted much attention because of its enormous potential in healthcare, but uptake has been slow. There are substantial barriers that challenge health technology assessment (HTA) professionals to use AI-generated evidence for decision-making from large real-world databases (e.g., based on claims data). As part of the European Commission-funded HTx H2020 (Next Generation Health Technology Assessment) project, we aimed to put forward recommendations to support healthcare decision-makers in integrating AI into the HTA processes. The barriers, addressed by the paper, are particularly focusing on Central and Eastern European (CEE) countries, where the implementation of HTA and access to health databases lag behind Western European countries.

Methods: We constructed a survey to rank the barriers to using AI for HTA purposes, completed by respondents from CEE jurisdictions with expertise in HTA. Using the results, two members of the HTx consortium from CEE developed recommendations on the most critical barriers. Then these recommendations

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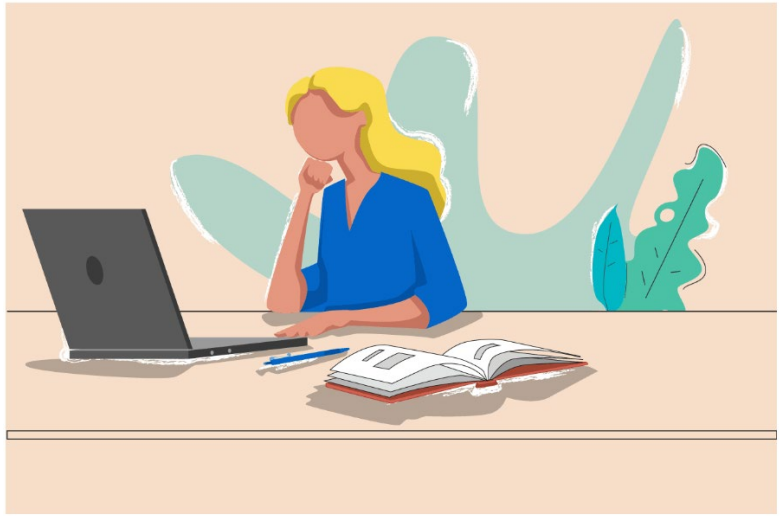
Project Trainings

Trainings > Introductory Training

Introductory Training

Length: 2 hours · Modality: Online



HTx Patient Toolbox

Facing challenges in HTA

Policies and Real-World Data (RWD)

Evolving challenges for HTA agencies for COVID-19 technologies

Choosing the best treatment for you

Developing prediction models

Predicting risks of complication

Using the right PROMs for you

Using the PROMs app

Using the PROMs SELECT app