PCWP/HCPWP annual meeting with all eligible organisations 15 November 2022

EUnetHTA 21 & HTA Regulation

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Abbreviations used

See supporting document for more terminology used

COIC = Conflict of Interest Committee

DOI = Declaration of Interest

ECA = EUnetHTA 21 Confidentiality Agreement

EUnetHTA = European Network for Health Technology Assessment

HCP = Healthcare Professional

HTA = Health Technology Assessment

HTAb = Health Technology Assessment bodies

HTAR = Health Technology Assessment Regulation

HTD = Health Technology Developer

JCA = Joint Clinical Assessment

JSC = Joint Scientific Consultation

PICO = Population, Intervention, Comparator(s), Outcomes(s)

An introduction to HTA, EUnetHTA 21 & key products of HTA

Anne Willemsen, ZIN

Regulatory vs. HTA

Regulators (European Medicines Agency) - Authorization focus: Efficacy, Safety, Quality of the drug

- Does technology X work?
- Does the benefit of technology X outweigh the risks? Is technology X of sufficient quality?
- Are there any additional needs for technology X post-licensing?

HTA - Assessment focus: Benefit in comparison to current standard treatment

- How does technology X compare with technology Y?
  - Is technology X better than the current standard we already have (technology Y)?
    - Fewer harms, under what circumstances, for certain patient population?
  - Put in national context - What else needs to be considered? Underlying question: Is it worth paying for?

Situation under the HTA Regulation
For Joint Clinical Assessments (JCA) – pharmaceuticals

What is EUnetHTA 21?

- EUnetHTA 21 is an EC service contract aiming to
  - **Support the implementation of the HTA Regulation (HTAR)**, after its adoption in December 2021
- Consortium of 13 European HTA organisations
- All output is joint output, with all consortium partners around the table

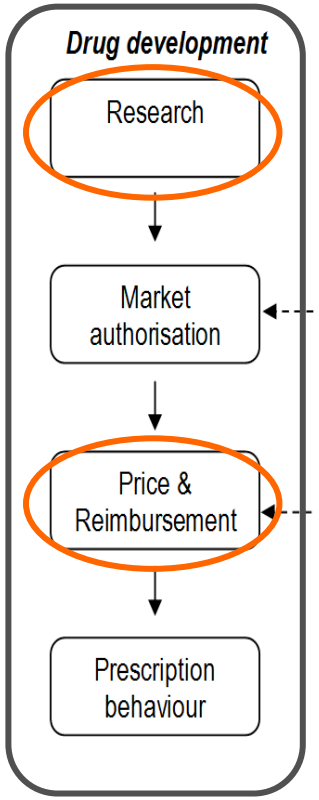
- Focus on developing methodological and transversal guidelines, templates and procedures
  - Also conducting joint products: Joint Scientific Consultation and Joint Clinical Assessment

More info on: https://www.eunethta.eu/jointhtawork/

HTA key products during EUnetHTA 21 and under the HTAR

Joint Scientific Consultation (JSC)	Joint Clinical Assessment (JCA)
DEFINITION	
Scientific advice provided jointly by HTA bodies to manufacturers on the clinical development. Can be in parallel with regulators	Joint HTA reports produced by multiple European Member States, focussing on the clinical domains
AIM	
To generate evidence that satisfies the needs of HTA bodies during their assessment and ultimately facilitates patient access	To avoid duplications of work at the national level, increase consistency and quality of assessments and ultimately facilitate patient access

Lifecycle of a new drug
When would JSC and JCA take place?



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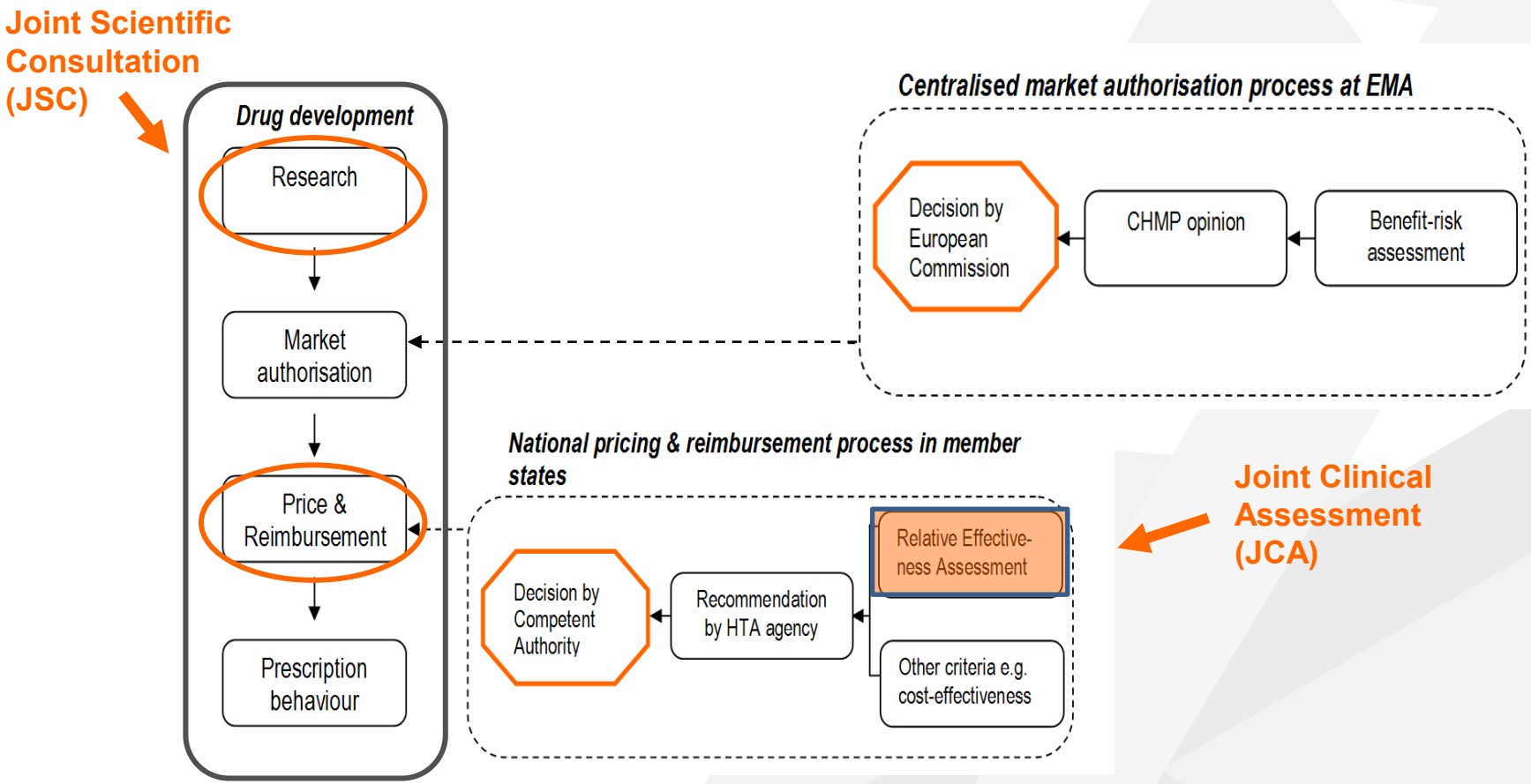


Continuous collaboration between EMA and EUnetHTA 21

- EMA colleagues reviewed the EUnetHTA 21 guidance on patient and clinical expert involvement
- Discussions between EMA colleagues and EUnetHTA 21 on:
  - Experience on resources for stakeholder management
  - Experience of setting up and maintaining an expert database
  - Process for identification of experts
  - Experiences on data sharing with experts and relevant confidentiality procedures
  - Experiences on incorporating expert input into regulatory and HTA activities
- EUnetHTA 21 exchanges with EMA for JCA (and JSC) to support the expert selection process
- Participate in training sessions or round tables

EUnetHTA 21 process for involvement of patients and clinical experts

Stephanie Said, G-BA

Value of patient and clinical expert/stakeholder input in HTA

Patient experts	Clinical experts
Aspects on the disease/condition and (available) therapies ➢ What is expected from new treatments? ➢ What are the limitations of current treatments?	Aspects on the disease/condition and (available) therapies ➢ Long term effects of the disease ➢ Most common standard of care in EU
Information on outcomes ➢ Information about signs and symptoms that have the greatest impact on the functional and psychological aspects of life	Information on outcomes ➢ Help defining relevant safety, efficacy and patient-centered outcomes
Other information relevant to patients ➢ For example, ethical and/or social issues	Information on Intervention ➢ Contextual factors which may affect the safety and/or effectiveness of the intervention ➢ Position of intervention in the current treatment landscape
Expert input helps to contextualize the data for the JSC and JCA	

Who can get involved?
Based on requirements from the HTA Regulation

As external experts

- Patients (or close carers) affected by the disease or condition
- Clinical experts
  - Speaking on their own behalf
  - Able to describe the situation of patients beyond the borders of their own Member State

- Must complete the Declaration of Interest (DOI) form and the EUnetHTA 21 Confidentiality Agreement (ECA)
  - The documents are reviewed by the EUnetHTA 21 Conflict of Interest Committee (COIC)
  - Reviewed on a case-by-case basis and exceptions to the guidance may occur

More information can be found here:

https://www.eunethta.eu/coic/

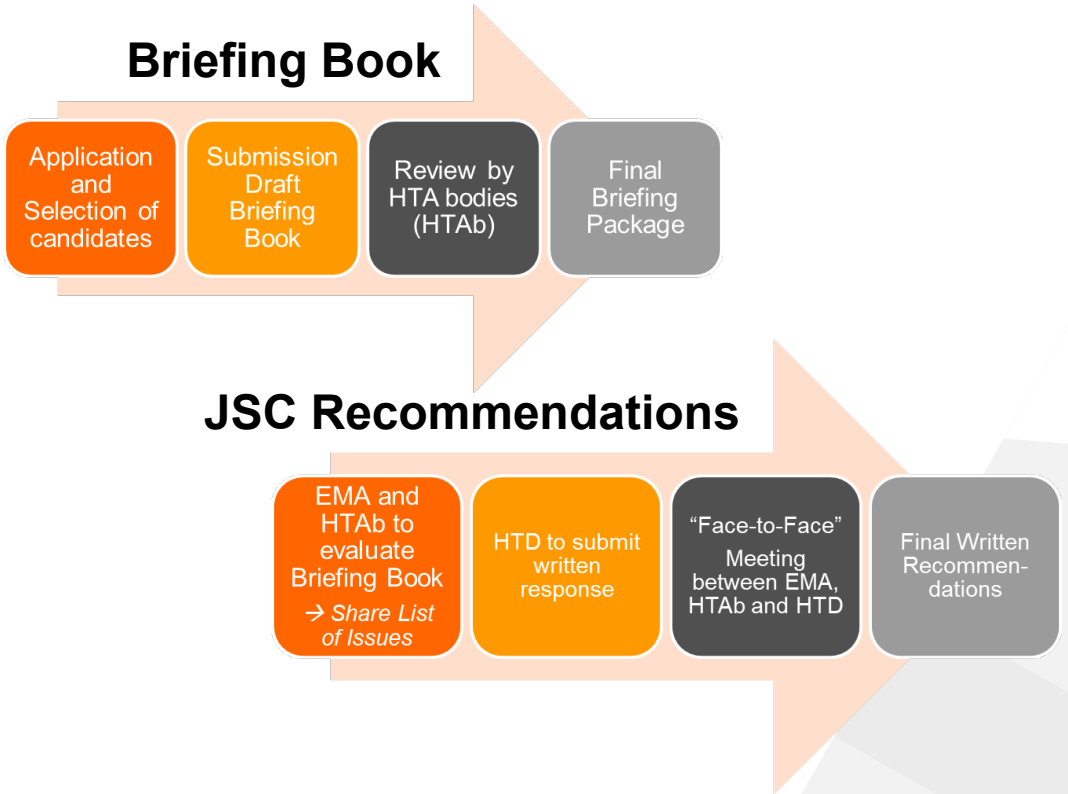
Who can get involved?
Based on requirements from the HTA Regulation

As stakeholders

- Patient organisations or Healthcare professional organisations
  - Associations and professional actors
  - Who represent the general interest of groups affected by EUnetHTA 21 and the HTA Regulation

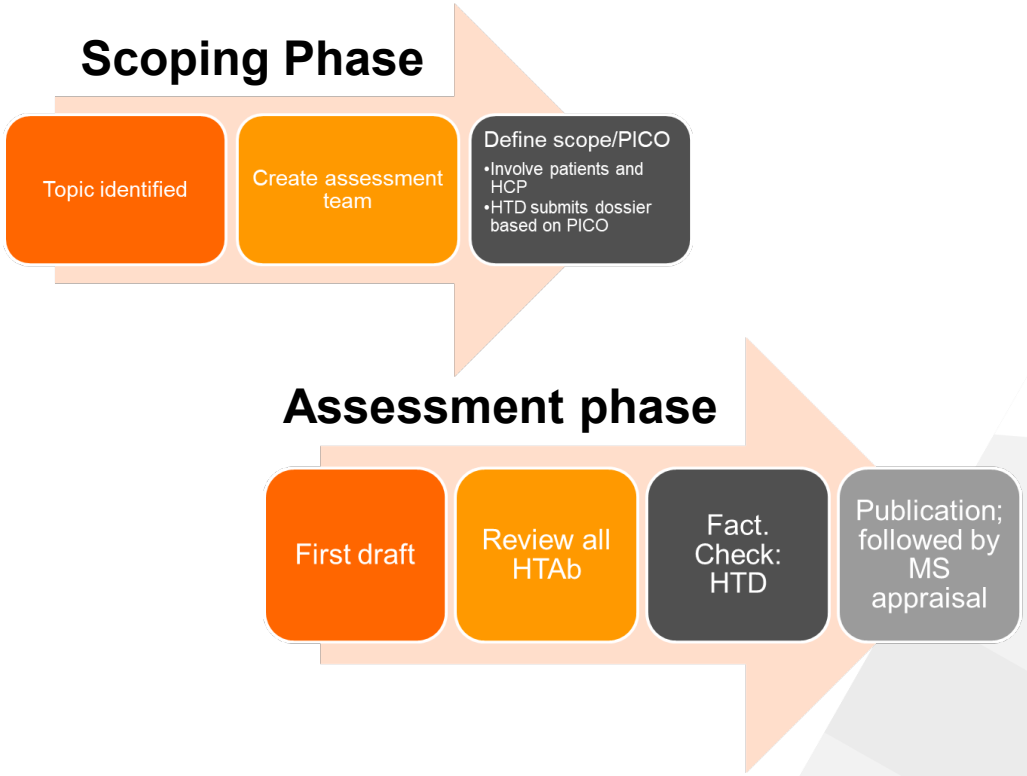
- A Declaration of Interest (DOI) form is not required for stakeholder organisations
  - However, they must provide information on their organisation's funding
  - Includes public and private funding
  - As part of any stakeholder submission

When are patients & clinical experts involved in EUnetHTA 21?
Joint Scientific Consultation (JSC) process



- Interview or written statement before List of Issues
  - At the latest **before the Face-to-Face meeting**
- Review and provide feedback on List of Issues
- Participation in Face-to-Face meeting
- Final Written Recommendations to be shared with patient external experts

When are patients & clinical experts involved in the EUnetHTA 21?
Joint Clinical Assessment (JCA) process



- When defining the PICO
  - By means of online questionnaire
  - Interviews with external experts may be conducted
  - External experts may participate in the PICO consolidation meeting
- During the assessment phase they answer questions the Assessor and Co-Assessor may have
- National procedure is not in remit of HTA Regulation
  - But the EUnetHTA 21 guidance highlights where this could take place

Relevant Documents for expert involvement during EUnetHTA 21

1. **Briefing package/Briefing Book for JSC**
   - Contains all information provided by the company for the consultation (e.g. development plan) and the questions to EMA and HTA organisations

2. **Input template (external experts and stakeholders) for JSC or JCA**
   - Contains questions about the specific disease, e.g. relevant symptoms and current treatment limitations
     - As a written statement of the expert, filled out by the Assessor during an interview or completed online for stakeholders (for JCA only)

3. **Final Written Recommendations for JSC or Assessment Report for JCA**
   - Final output document of the corresponding procedure
     - Contains the recommendations of the HTA (for JSC) or the assessment (for JCA)
     - Includes the full expert (for JSC and JCA) & stakeholder (for JCA) contribution as an annex
     - Patients will not be named, but an anonymized description of the patient(s) will be included. Clinical experts can be named upon approval

Extract from the input template (questionnaire) for patients

- Starts with explanation how to fill the form
  - Each question has a series of prompts for consideration
  - Describe experiences at different stages of the condition/disease
- Questions based on HTAi questionnaire template, including:
  - Background information
  - Funding required for stakeholders
  - Impact of condition
  - Experience with available therapies
  - Expectations for new treatment

Question on funding/ direct and indirect links with industry

Please state for the current year, previous year and 2 years ago the following:

- name of the organisation that provided funding (private and public)
- funding amount in euro's
- percentage of funding of the organisation's annual budget
- link to annual report (if available)

How does <condition> affect a patient's daily life?

Aspects to consider in your response (extract of prompts):

- Aspects of the condition that are most challenging (e.g., symptoms, loss of ability to work, loss of confidence in going out.
- Emotional and psychological impacts such as fear, anxiety, uncertainty, stigma, embarrassment, loneliness/isolation.
- Activities that patients find difficult or are unable to do.
- Aspects of the condition that are the most important to control.
- Support required for daily living (physical or emotional).
- Types of patients who are most affected by the condition (e.g., men/women, children, ethnic groups).
- Challenges in managing this condition when patients also have other medical conditions.

Extract from the input template (questionnaire) for clinical experts

1. P – Population

For JSC only:
How would you define the population to be included in a clinical trial to measure the efficacy of the intervention in the required indication?

For both JSC and JCA:
Please state relevant patient sociodemographic (e.g., age, ethnicity, socioeconomic status) and clinical baseline characteristics (e.g., severity of condition, comorbidities) which may contribute to differences in treatment outcomes or treatment preferences.

What are the relevant eligibility criteria for treatment decisions made by HCPs?

Important to fill the questionnaire, even if no common position exists in the medical community

2. I – Intervention

Are there contextual factors, (e.g., prior, concurrent or subsequent treatments, training on administration, etc.) which may affect the safety and/or effectiveness of the intervention?
Does the specific (professional) experience of the treating HCP or medical staff play a relevant role in the decision to use the intervention?

3. C – Comparator(s)

What is the standard of care in your country? Are you aware of the standard of care most commonly used in Europe?
Are there different treatment options for different patient groups depending on severity, previous treatment, biomarker levels, etc.?

4. O – Outcome

Please define relevant safety, efficacy and patient-centred outcomes (e.g., quality of life) which should be assessed.
What safety and efficacy outcomes are used in clinical practice to dictate inform clinical decisions regarding treatment and how are they measured?

Messages to take home

1. **Regulatory and HTA have different remits and purposes**, but both aim for data generation by the health technology developer that is fit for purpose to make innovative medicines available to patients.

2. **EMA and EUnetHTA 21 collaborate intensively** and (amongst other items for collaboration) share experiences on aspects related to stakeholder and expert involvement in their activities though their processes for involvement may differ.

3. The **contribution of patients and clinical experts is of high value and helps to contextualize on the needs and experiences** of those who are either affected by a particular disease or are dealing with it in their daily lives. We therefore highly encourage and welcome expert participation in all **Joint Scientific Consultations (JSC) and the Joint Clinical Assessments (JCA)**.

THANK YOU!
Any questions?

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Back-up

EUnetHTA HTA Core Model®