PATIENTS AND HEALTHCARE PROFESSIONALS AS EXTERNAL EXPERTS IN HTA

22 JUNE 2022

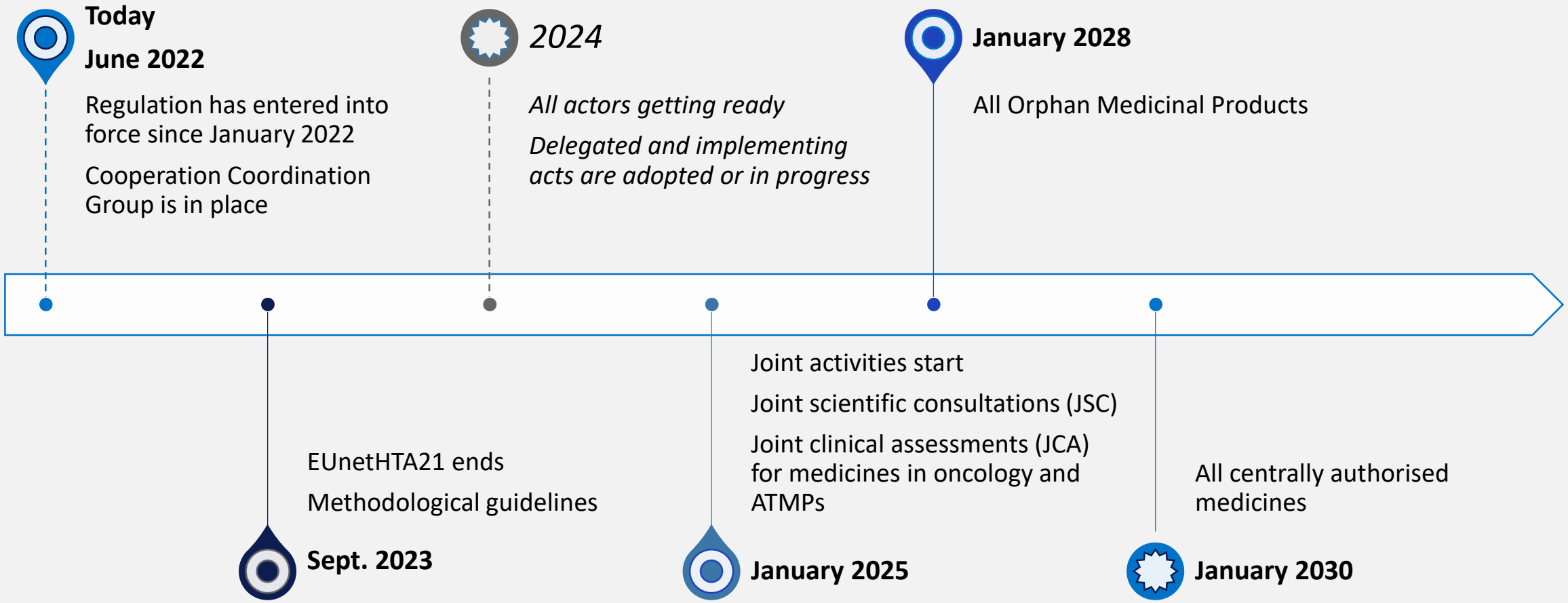
DIRECTOR OF TREATMENT INFORMATION AND ACCESS EURORDIS

PATIENT ADVOCATE SINCE 1988

REPRESENTS EURORDIS AT EMA (PCWP)

AND AT THE FORMER HTA NETWORK

30 months ahead to prepare yourself for:



Different roles envisaged. Organisations: can be members of the stakeholder network, or not

You as an External expert

- Your own views
- Technology related activities
  - Joint Scientific Consultations
  - Joint Clinical Assessments
- Confidentiality +++
- Declaration of interests +++
- Scientific guidelines

Representing your organisation

- More specific to Stakeholder Network
  - Communication
    - Report summaries, awareness of the cooperation
    - Internal procedures, guidelines, reports
- Contribution to Horizon Scanning
  - Technologies of interest in R&D in your field
- Prioritisation / work plan
  - Technologies you think could benefit from a European joint Assessment?
- Assessments
  - Data your organisation can provide
  - Treatment and Care Management guidelines

Joint scientific consultations:

to reduce the risk that inadequate information are submitted at a later stage

P In which population to conduct the research? Which unmet needs, in which **P**atients?

I How can the research questions be answered? Which study design? Which **I**ntervention?

Early phase R&D

C For the research, which would be the most relevant **C**omparator(s)? Their experience with treatments

O What can be measured to demonstrate benefit(s)? Which **O**utcomes, and how to measure them?

I.e, experiential knowledge of living with the disease, social consequences, healthcare pathways, …

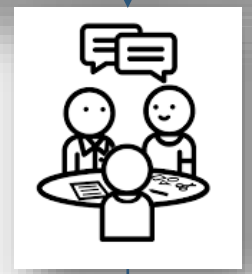
Joint Consultation

Request: HTA only? All? Some? Or with EMA?

Briefing Book with questions from developer

Internal discussions and clarifications

Draft answers / key issues



Minutes / written answers

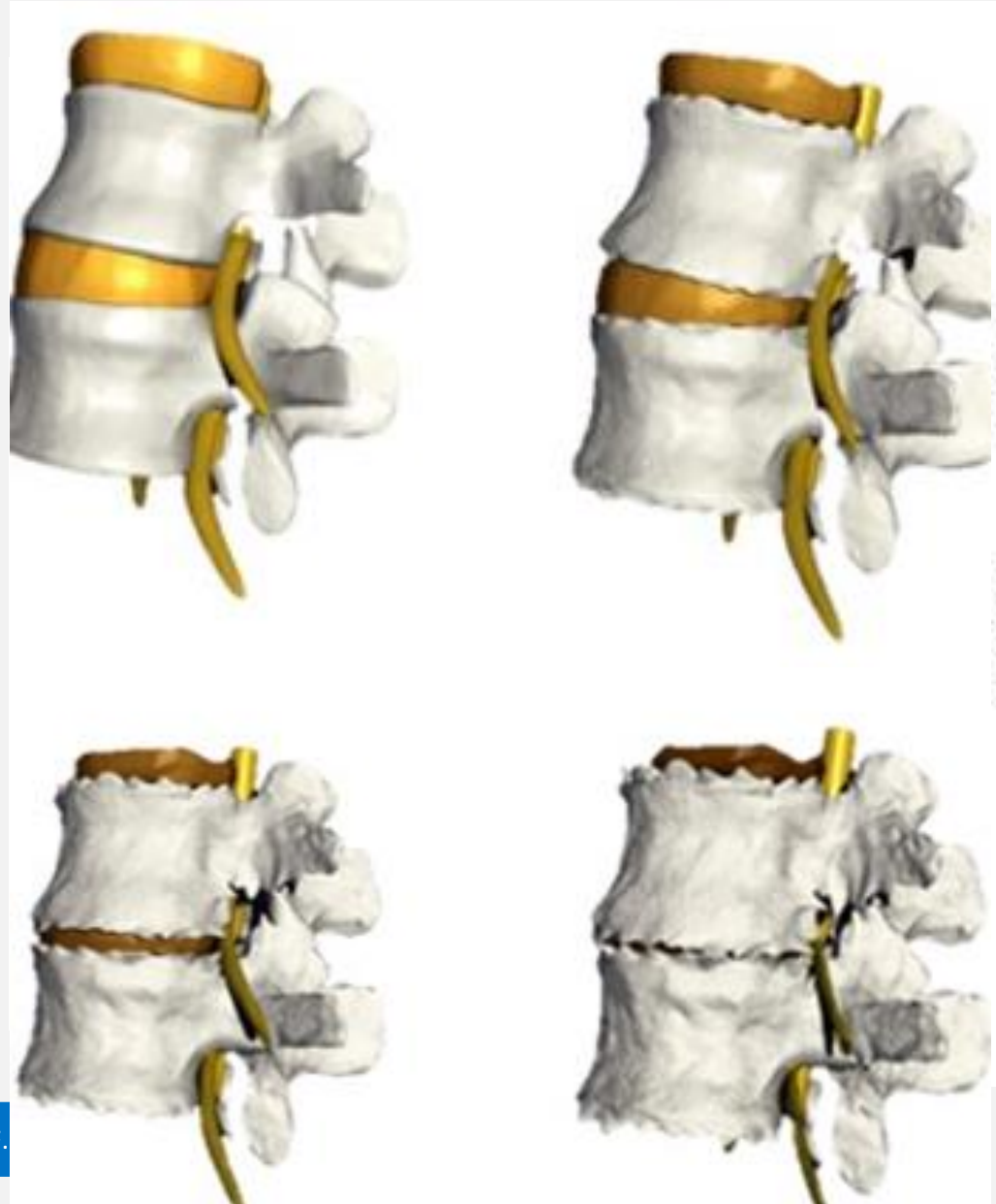
Advice

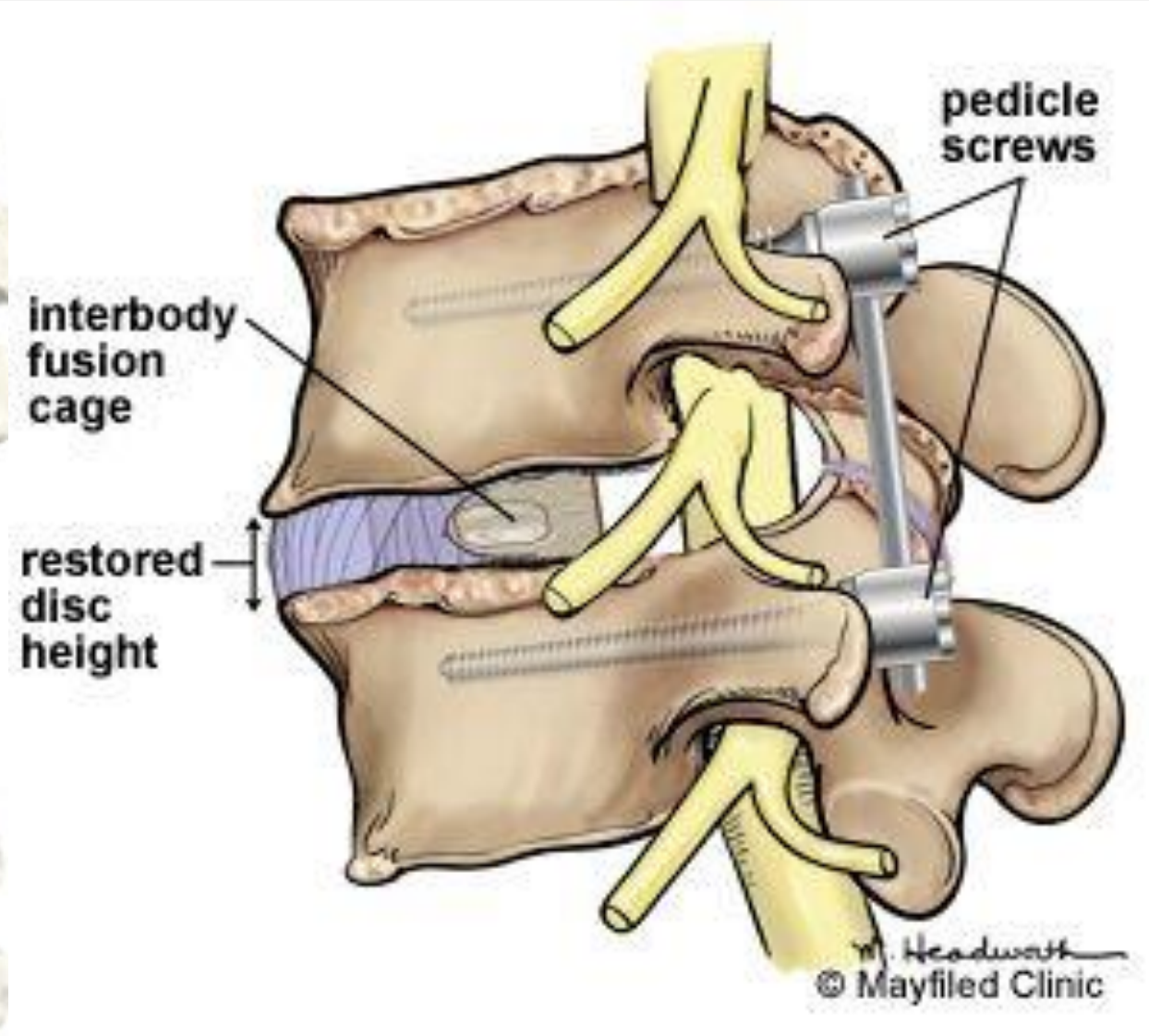
- 45 days? 90 days?
- "To comment on draft report"
- When do external experts come in?
- Which documents?

Technology placed on market

- How do you prepare?
  - As an individual expert?
  - As a member of an organisation?

A new technology for back pain / discopathy

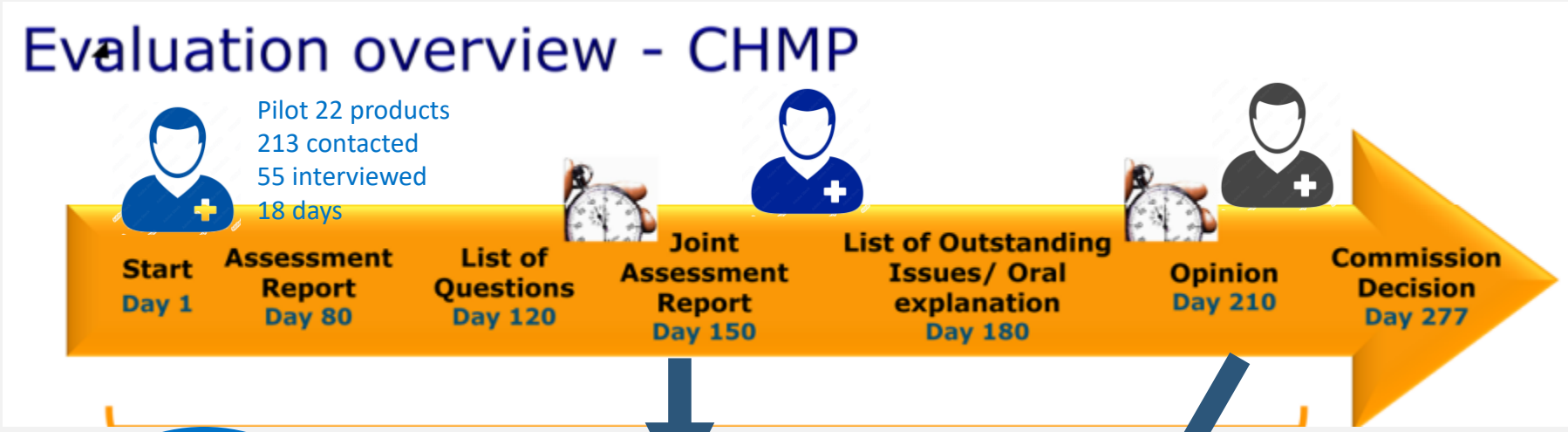




Joint Clinical Assessments

Timelines of medicine evaluation at EMA CHMP Committee for Human Medicinal Products

Pre-submission meeting
Data to be submitted? Other committees?



Scoping

Of all possible questions to inform on the relative effectiveness of the technology which are the most relevant?

P I C O

Patients & Clinicians Input

Data submitted

By technology developer

1st draft

Exchange of data with CHMP

Patients & Clinicians comments

2nd draft

Author and co-author discuss with subgroup members

Patients & Clinicians comments

Report

Published simultaneously with the EMA Product Information /Decision on the Marketing authorisation ± 1 or 2 months

Patients & Clinicians comments

F. Houy[illegible] irector | EURORDIS

What does it mean for Member States?
(how the system is meant to work)

Industry submits a dossier for a EU HTA

EU HTA Report (Joint Clinical Assessment)

EUROPEAN LEVEL

NATIONAL LEVEL

Conclusion on benefit and use in the population

Recommendation on reimbursement

National HTA (final) Report

MEMBER STATES ARE OBLIGED TO

1. **Annexe the EU Report** in National HTA Report
2. **Share their final national HTA report** with the EU Cooperation, **within 30 days** from its completion
3. **Inform about how they used the EU Report (**EC will publish an overview)
4. **National level can't request any evidence already submitted at EU level** (and the developer cannot submit evidence already submitted)

(if **later in time** they request/receive **new evidence at national level** in the scope of the published EU Report) **MS must share all with the EU cooperation**

Stages of Expert involvement

Find experts

Call: information –> Disseminate the call and select the right people

Prepare experts

Explain the procedure –> administrative tasks (Conflicts of interest, confidentiality…)

Involve experts

Achieve the interaction (meetings / questionnaires / interviews / focus groups / citizens' jury / aggregated data…)

Elaborate Input

Adjustment and insertion in reports – Follow-up

Acknowledge Input

Feedback – Measurement - Visibility

Trainings for patients and for healthcare professionals

Not exhaustive

DIU Clinical trials in Rare Diseases

Universities of Lille, Dijon, Lyon

For patients' advocates and healthcare professionals

105 hours

Clinical research, R&D, evaluation and regulation of medicines

E-learning and onsite

HTADS International Continuing Education

https://www.umit-tirol.at/page.cfm?vpath=departments/public_health/htads-continuing-education-program/introduction-to-health-economics-and-hta

Modeling Approaches for HTA 3 days
50 hours

Introduction to Health Economics and Health Technology Assessment 3 days
50 hours

EUPATI Patient Expert Training Programme

https://learning.eupati.eu

27 modules, + 8 days = **75 hours**

Clinical development, regulatory affairs, HTA

E-learning and onsite

LSE Principles of HTA

https://www.lse.ac.uk/resources/calendar/courseGuides/HP/2021_HP4D2E.htm?from_serp=1

20 hours

Lectures and seminars: half a unit

Part of a 2-year programme

EURORDIS Summer School Rare Diseases

https://openacademy.eurordis.org/summerschool/

For patients' advocates & academics

23 hours e-learning + 5 days = **57 hours**

Clinical development, regulatory affairs, HTA, pharmacovigilance

So, when do you prepare ?

When procedure starts: how to anticipate and to start identifying patients early? How much time to explain the role of the external experts, what is expected from them, how to best contribute?

Upstream

- Existing training programmes: 20 to 105 hours or more
- Discuss your unmet needs, treatment guidelines
- Engage in Patient Preferences Elicitations, Community Advisory Boards (CABs), patient/disease registries
- Familiarise with Patient Reported Outcomes Measures / Patient Relevant Outcomes for their disease (PROMs)
- Familiarise with PICO and with methods used in HTA
- Compare standard of care in different countries, discuss the limits and the benefits of different technologies
- Organise a database of patients / clinicians (different stages of the disease, ensuring gender, language, geographic diversification etc., as well as various levels of expertise)

Dowstream

- Either the Cooperation secretariat or an HTA body to initiate the procedure?
- Dependent on when developer requests a Joint Consultation, or when developer submits an application to EMA
- For medical devices?
- Short time to identify and mentor the expert (if not already familiarised with HTA)
- Just a few hours – days to explain procedure and prepare (administrative work and documents)

Open questions

To engage in HTA Cooperation

How can we build EU processes from national HTA experiences and feedback from experts?

How to train more external expert/organisations on HTA in general, and on EU procedure(s)?

Organisations: contact database, awareness raising, collecting experiences?

Methods to identify / involve clinicians or patients (organisations' membership list, social networks, ERNs, clinics in Pubmed...)?

THANK YOU

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Further reading
Achievement of the HTx Project

Potential barriers of patient involvement in Health Technology Assessment in Central and Eastern European countries
Frontiers in Public Health
Dimitrova M, Jakab I, Mitkova ZE, Kamusheva MS, Tachkov K, Nemeth B, Zemplényi A, Dawoud D, Delnoij D, Houÿez F, Kaló F
https://www.frontiersin.org/articles/10.3389/fpubh.2022.922708/abstract

In press – accepted 13 June

HTA Regulation: the structure

- Work plan
- Annual Report
- Validates the work

Stakeholder Network

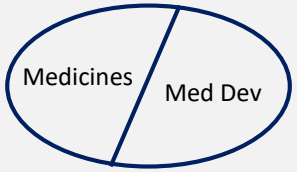
Patient Organisations
Health Professionals
Consumer organisations
Health NGOs
Industry Associations

Article 29

> Sub-Groups

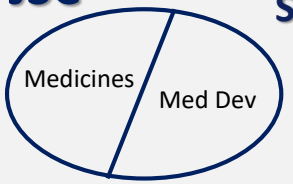
Obligations Art 10 & 13

JCA



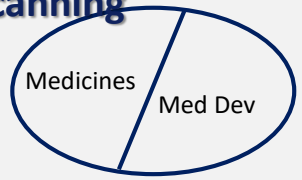
JCA Report / Summary Report

JSC



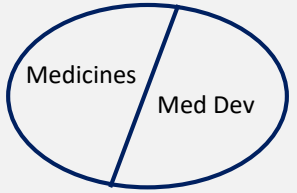
Advice

Horizon Scanning



Report

Methods



Guidelines

Others

?

Voluntary Cooperation

Secretariat

HEALTH

Art. 15 & 28

- *Support the Coordination Group and Sub-Groups*
- *Ensure interactions with Stakeholders, EU Agencies, Developers*
- *Enforce procedural rules and Involvement of experts*

JCA: Joint Clinical Assessment

JSC: Joint Scientific Consultation

HTA Regulation 2021/2282: the structure

Chapter I - Art. 1-6

General Provisions

The brain of the system: Coordination Group

Chapter II - Art. 7-23

Joint Work

The HTA activities to be fulfilled by the Member States in/as European Cooperation

Section 1 - Art. 7-15

Joint Clinical Assessment

Section 2 - Art. 16-21

Joint Scientific Consultation

Section 3 - Art. 22

Emerging Health Technologies

Section 4 - Art. 23

Voluntary Cooperation

Chapter III - Art. 24-26

General Rules for Joint Clinical Assessment

Rules others than process and obligations

Chapter IV - Art. 27-31

Support Framework

Funding, **Stakeholders,** EC role and rules to be implemented

Chapter V - Art. 31-36

Final Provisions

To enforce the law