New HTA Regulation: key elements and next steps

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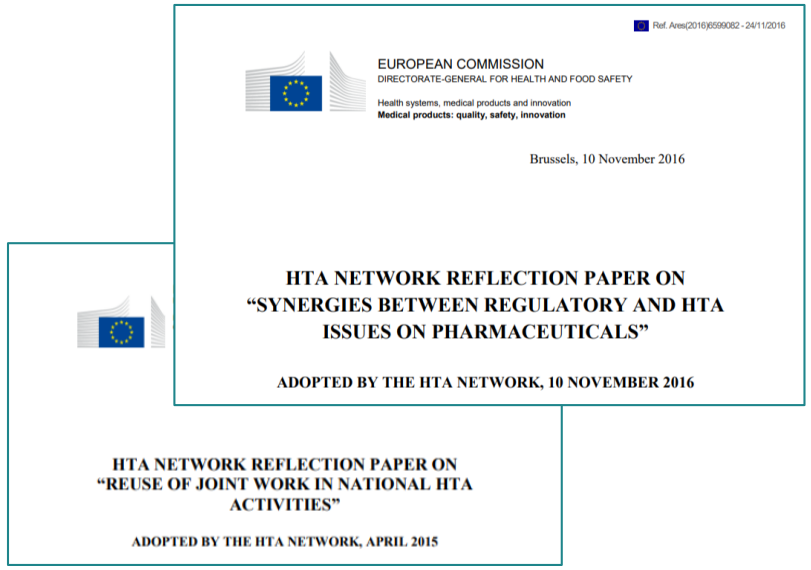
Strengthening EU HTA cooperation



JA1 (2010 – 2012)
JA2 (2012 – 2015)
JA3 (2016 – 2021)

Cooperation EUnetHTA-EMA
(2 work plans 2012-2015; 2017-2021)

HTA Regulation

European Commission

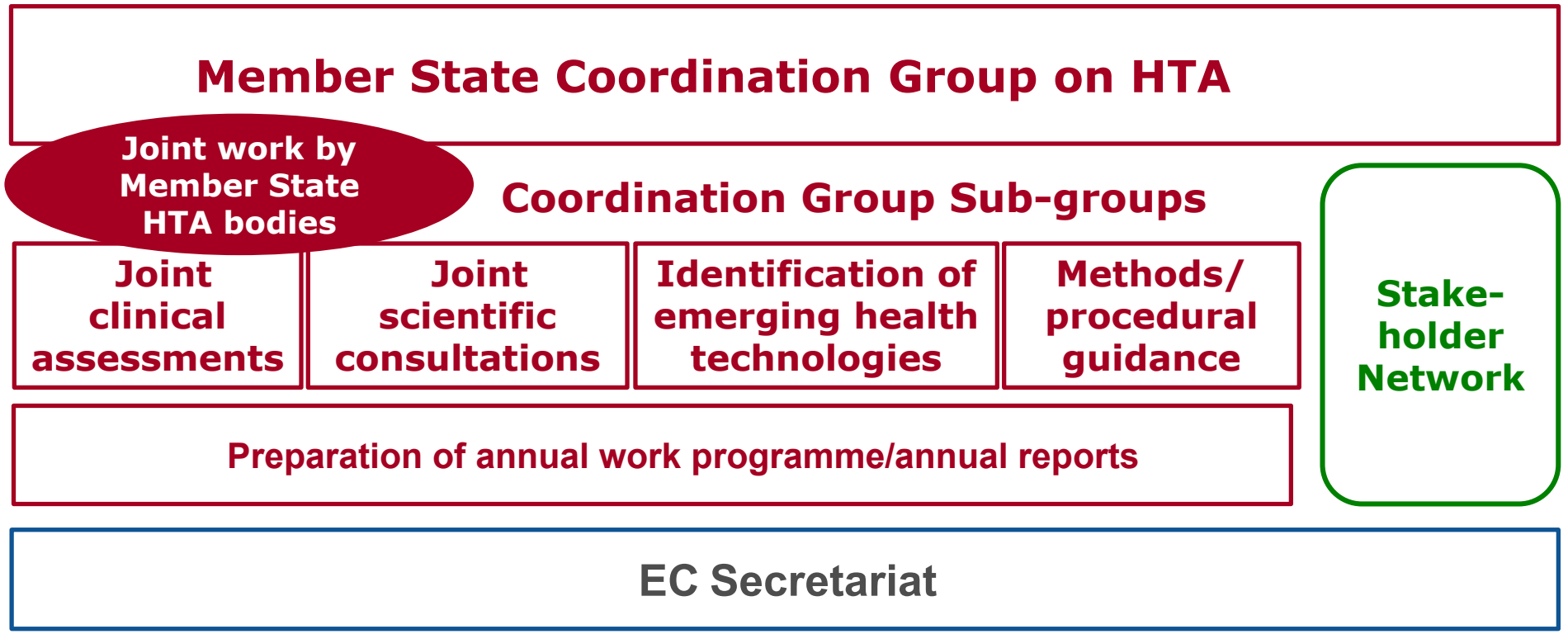
HTA Regulation
Key principles

- **Joint work** on common **scientific, clinical aspects** of HTA
- Joint work **driven by Member State HTA bodies**
- 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**
- **Progressive implementation**

Main areas of joint work

- **Joint clinical assessments** (medicines, medical devices)
- **Joint scientific consultations**
  (advice to health technology developers on clinical study design;
  parallel HTA-EMA advice for medicines)
- **Identification of emerging health technologies**
  ("horizon scanning")
- **Voluntary cooperation in other areas**
  (e.g. on other health technologies or non-clinical HTA aspects)

Organisational framework



Involvement of external experts and stakeholder organisations

- **External experts** (patients, clinical and other relevant experts):

  *Provide input based on their specialised expertise (e.g. therapeutic area) during **Joint Clinical Assessments** and **Joint Scientific Consultations***

- **Stakeholder organisations** (e.g. healthcare professional organisations, insurers/payers, industry associations, patient groups, scientific societies):

  - Provide input on horizontal and strategic issues
  - Regular meetings between Stakeholder Network and Coordination Group

Joint Clinical Assessments: medicines

- **Centrally authorised new medicines**:

  - Step 1 (3 years): cancer medicines, ATMPs

  - Step 2 (2 years): orphan drugs

  - Step 3: full scope

- For those medicines that have already undergone a JCA:

  extensions of therapeutic indication (variations)

HTA Regulation: implementation timeline

Entry into force

Dec. 2021

3 years

Date of Application

Dec. 2024

5 years

Full Joint Clinical Assessment scope

Dec. 2029

- **Setting up the Coordination Group (EC)**
- **Setting up Stakeholder Network (EC)**
- **Drafting implementing and delegated acts (EC)**
- **Drafting guidance documents (CG)**

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SERVICE CONTRACT

Joint Scientific Consultation (JSC)
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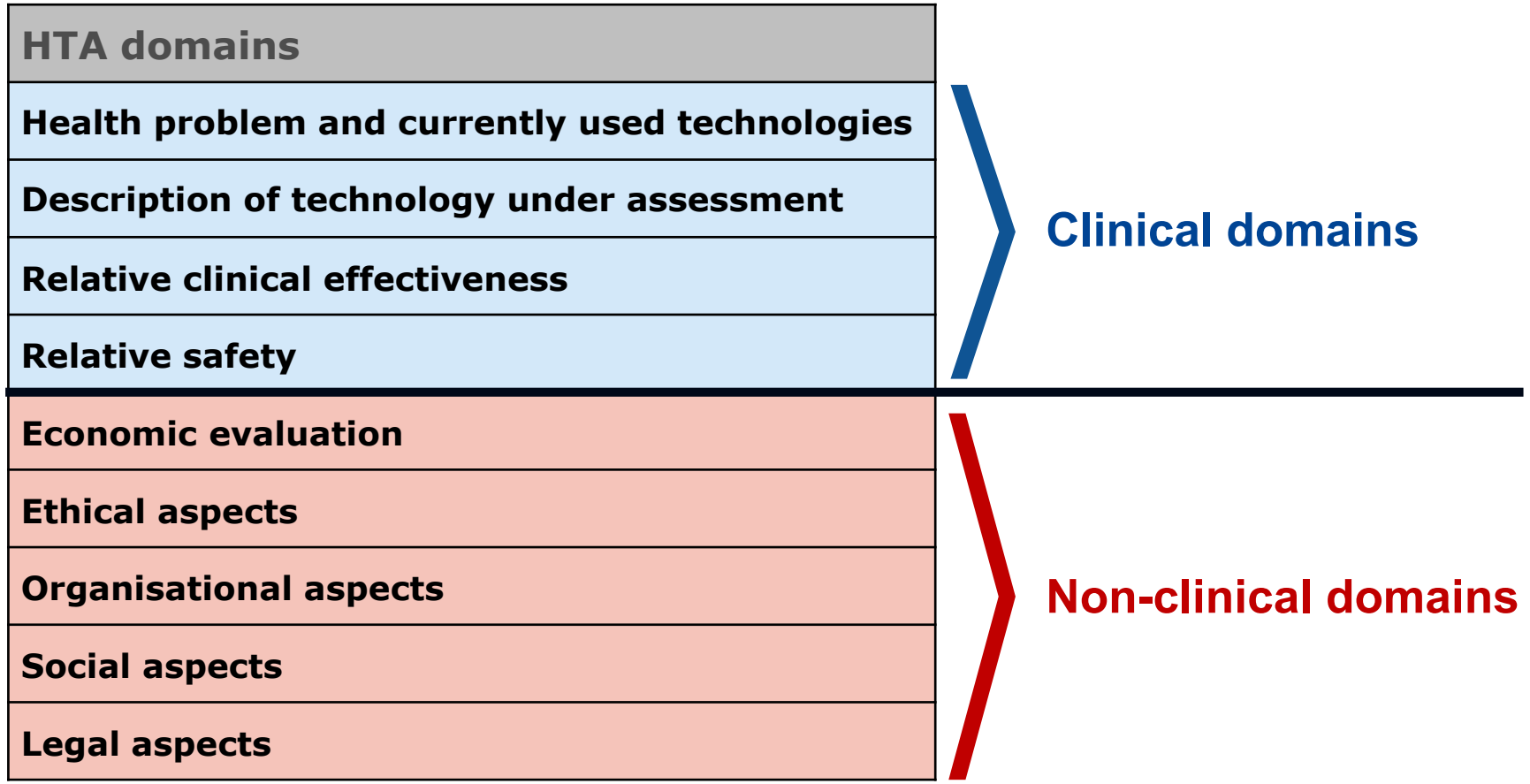
Stepwise build-up of Joint Clinical Assessment (JCA) scope for medicines:
- From 2024: cancer medicines, ATMPs
- From 2027: orphan drugs

European Commission

Current content of national HTA reports



HTA Regulation: JCA in national HTA processes

JOINT CLINICAL ASSESSMENT (JCA) = CLINICAL HTA ASPECTS

- **Assessment scope agreed jointly** (incl. patient population/subgroups, comparators and health outcomes relevant for different Member States)
- Contains a **scientific analysis of clinical effects** observed in clinical studies (incl. on mortality, disease symptoms, adverse events, health-related quality of life), along with a discussion of **scientific uncertainties** (strengths/weaknesses of the underlying evidence: e.g. limitations of clinical study designs, reliability of outcome measurement tools, statistical analyses)

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COMPLEMENTARY CLINICAL ANALYSES (IF NEEDED) — NATIONAL

- e.g. **of more context-specific data not analysed in the JCA** (e.g. data on national disease epidemiology; data from a national patient registry reflecting the specific healthcare context;)

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NON-CLINICAL ASSESSMENTS — NATIONAL

- E.g. economic, organisational, ethical aspects

DRAWING CONCLUSIONS ("APPRAISAL") — NATIONAL

- Taking into account the **JCA**, any **complementary clinical analyses**, and any **non-clinical assessments**
- Consideration of any **additional criteria in accordance with the national HTA framework** (e.g. rarity of disease, severity of disease, lack of alternative interventions)

Conclusions on added value (e.g. no/minor/major added clinical value, more/less cost-effective than comparator) in the context of the national healthcare system

Expected benefits of the HTA Regulation

Member State decision-makers

- ✓ **High quality**, timely scientific **reports** (pooling of HTA resources/expertise; better evidence base for HTA across EU)
- ✓ Supports **evidence-based decision-making** at national level

Patients

- ✓ **Improved transparency** and engagement in the HTA process for EU patients
- ✓ Contribute to improved availability of **technologies with added value** for patients across the EU (due to more timely, evidence-based decision-making)

Industry

- ✓ **Clearer, more coherent clinical evidence requirements** across the EU
- ✓ **More efficient clinical evidence** generation and **submission** (EU-level JCA dossier)

HTA cooperation experience with ATMPs

Joint Actions EUnetHTA (ended June 2021):

- **Piloting of Joint Clinical Assessments**
  1 Joint Clinical Assessment conducted on an ATMP:
  Elivaldogene autotemcel (eli-cel) for the treatment of cerebral adrenoleukodystryophy (CALD)

  Exchange of information with EMA: CHMP report + webinar with CAT

  Report accessible at https://www.eunethta.eu/ptja17/

- **Piloting of Joint Scientific Consultations**
  11 Joint Scientific Consultations conducted on ATMPs
  (10 of which were conducted as parallel scientific advice procedures together with EMA)

HTA Regulation
Next steps (to be confirmed)

- December 2021 – expected date of adoption
- Q1 2022 – call for Member States to nominate their representatives for the Coordination Group
- Mid-2022 – first meeting of the Coordination Group
- Q4 2022 – launch of the procedure for setting up the Stakeholder Network

- EUnetHTA21 – EMA work plan → work in progress

Thank you

Joint Clinical Assessments: medical devices

- **Medical devices (class IIb and III)**
  with scientific opinion under the clinical evaluation consultation procedure (Regulation (EU) 2017/745)
- **In vitro diagnostic medical devices (class D)**
  with expert panel view under the clinical evaluation consultation procedure (Regulation (EU) 2017/746)
- **Selection by Coordination Group** (from within above outer scope):
  based on defined criteria (e.g. unmet medical needs, impact on public health)