New HTA Regulation: key elements and next steps

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CAT Industry Interested Parties Meeting, 26 October 2021
Strengthening EU HTA cooperation

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

Member State Coordination Group on HTA

Joint work by Member State HTA bodies

Coordination Group Sub-groups

Joint clinical assessments
Joint scientific consultations
Identification of emerging health technologies
Methods/procedural guidance

Preparation of annual work programme/annual reports

Stakeholder Network

EC Secretariat
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
- **Date of Application**: Dec. 2024
- **Full Joint Clinical Assessment scope**: Dec. 2029

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

**5 years**
- Joint Scientific Consultation (JSC)
- Stepwise build-up of Joint Clinical Assessment (JCA) scope for medicines:
  - From 2024: cancer medicines, ATMPs
  - From 2027: orphan drugs
Current content of national HTA reports

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Non-clinical domains
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)

**COMPLEMENTARY CLINICAL ANALYSES (IF NEEDED)**
- 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;)

**NON-CLINICAL ASSESSMENTS**
- E.g. economic, organisational, ethical aspects

**DRAWING CONCLUSIONS (“APPRAISAL”)**
- 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 adrenoleukodystrophy (CALD)
  Exchange of information with EMA: CHMP report + webinar with CAT
  Report accessible at [https://www.eunethta.eu/ptja17/](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)