EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

New regulations for the centralised procedure: Impact on the approval process and interactions with HTA

26th DGRA Annual Congress, 20 June 2024

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Disclaimer

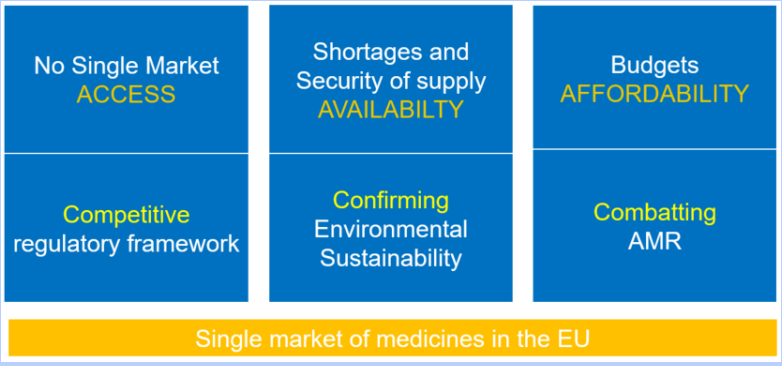
The presenter does not have any conflict of interests.

Topics of today's talk

- Agile framework with enhanced pre-authorisation support
- Proposed reform of the scientific committee structure
- Interactions with other decision makers, particularly HTA bodies

The broader context:

The six key objectives of the EU Pharmaceutical Reform



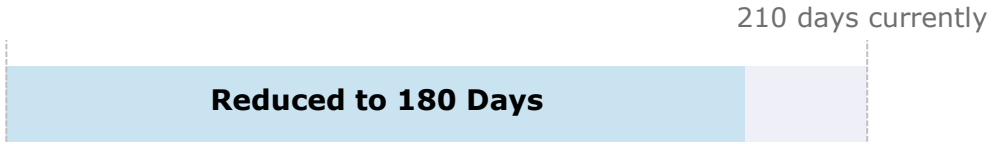
Headlines on procedural simplification

- Reduction of assessment and **approval time** from 277 days to 226 days
- Optimising **EMA's structure** and simplifying **regulatory procedures**
- Better use of **data and digitalisation**
- **Enhanced support** to developers

The current presentation reflects the revision to EU pharmaceutical legislation proposed by the EC.

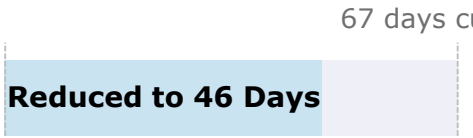
Changes to CHMP review timeline

Standard marketing authorisation application



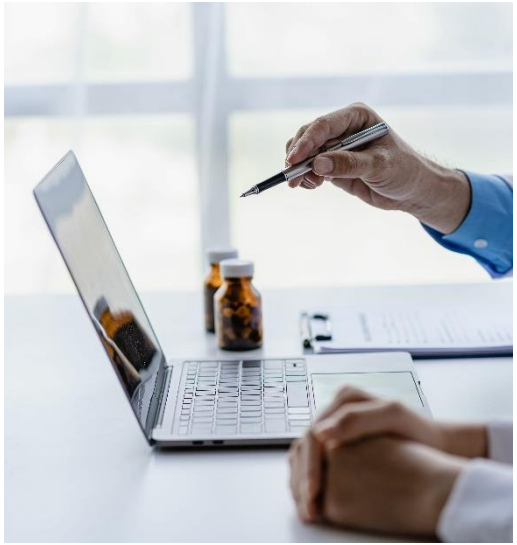
Accelerated assessment remains at **150 days**

European Commission decision-making process



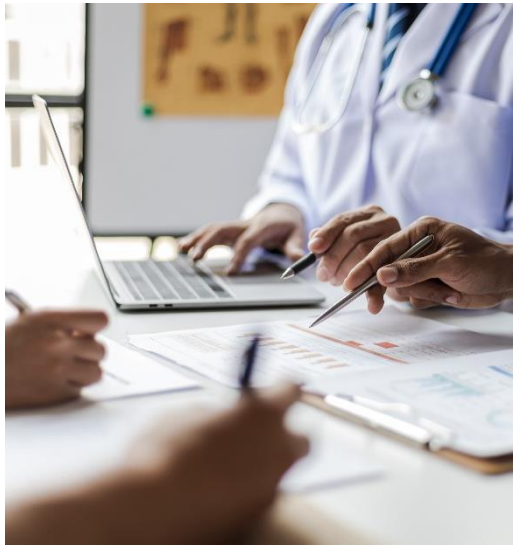
Reinforcing existing tools for developers

- **Scientific advice** extended to include medical device authorities/expert panels, health technology assessment bodies (HTAs), payers, etc.
- **PRIority MEdicines (PRIME) scheme:** embedded in legislation (as enhanced scientific and regulatory support)
- **Support for SMEs and non-for-profit entities** (regulatory, procedural and administrative support and reduction, deferral or waivers of fees)



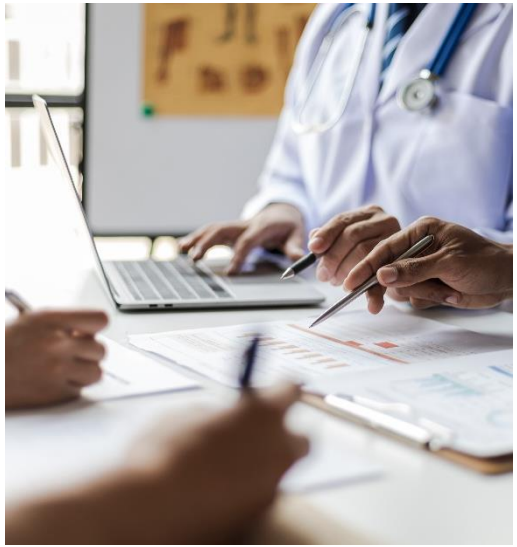
New regulatory tools for certain medicines (1/2)

- **Regulatory sandbox**: EMA and Member States to set up environment to test adapted, waived or deferred requirements for products that provide major advantage to patients and cannot be developed in full compliance with current rules (e.g. bacteriophages)
- **Phased review**: for products that offer exceptional therapeutic advantage in areas of high unmet medical need



New regulatory tools for certain medicines (2/2)

- **Repurposing**: CHMP can give a scientific opinion on **adding a new indication** to authorised medicines (for indications that fulfil an unmet medical need / are of major interest to public health)
- **Temporary Emergency Marketing Authorisation (TEMA)**: set up at EU level for public health emergencies where there is a major interest in developing and authorising safe and effective medicines as quickly as possible



Other key changes

- **Predictability of marketing authorisation application**: applicants to agree submission date with the Agency
- **Premature applications**: stop at validation or terminate at Day 90 if the application has critical deficiencies or if the data are not sufficiently mature.
- **No renewals**: abolished (but can be maintained in exceptional circumstances, e.g. if requested by CHMP)
- **Simplification**: Waiver of risk management plans (RMPs) for generics and biosimilars, abolishing marketing authorisation renewals in most cases
- **Digitalisation**: mandatory submission of electronic marketing authorisation applications in a common format, electronic product information

Changes to EMA scientific committees

- **Reduced number** of committees: human medicines committee (CHMP), safety committee (PRAC) and veterinary medicines committee (CVMP) to remain
- **Extended expertise** in CHMP and PRAC core membership
- Complemented by **stronger scientific support** from network experts (pool of experts, working parties)
- CHMP to set up a **scientific advice** working party & other working parties with multi-disciplinary expertise
- Strengthened **voice of patients** and healthcare professionals in CHMP and PRAC
- Possibility to have **public hearings** in CHMP
- **EMA responsible** for orphan designations and PIP applications/deferrals/waivers

EMA's human medicines committee (CHMP)

	Current composition	Proposed composition*
	1 member per Member State + 1 alternate per Member State	1 member per Member State + 1 alternate per Member State
	Up to 5 co-opted members based on their specific scientific competence	Up to 5 co-opted members based on their specific scientific competence
Stakeholder representatives		
Healthcare professional rep.	— no healthcare professional representatives	**4 members** + **1 alternate**
Patient representatives	— no patient representatives	**4 members** + **4 alternates**

EMA's human medicines committee (PRAC)

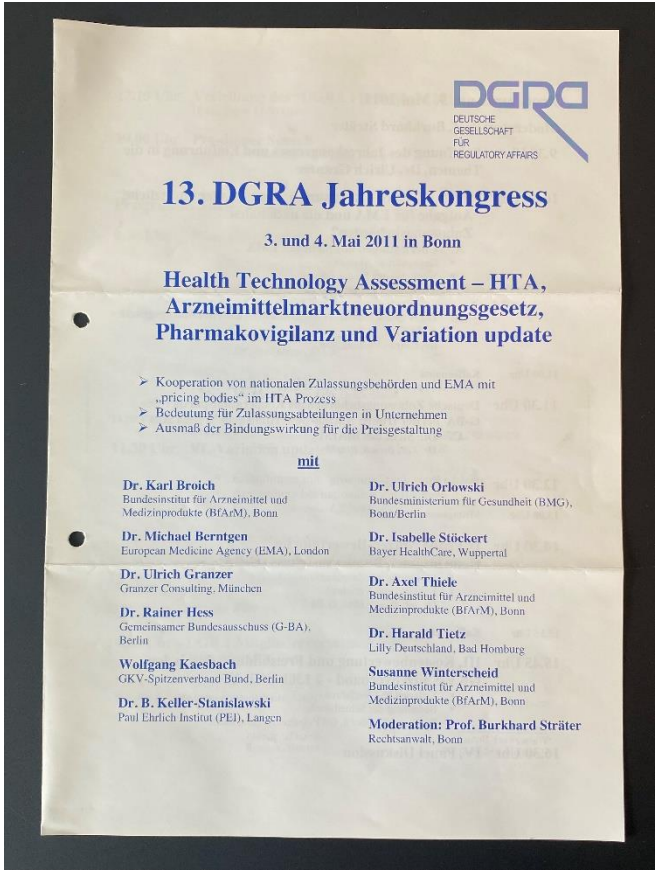
	Current composition	Proposed composition*
	1 member per Member State + 1 alternate per Member State	1 member per Member State + 1 alternate per Member State
	6 members appointed by the European Commission with complementary expertise including clinical pharmacology and pharmacoepidemiology	6 members appointed by the European Commission with complementary expertise including clinical pharmacology and pharmacoepidemiology
Stakeholder representatives		
Healthcare professional rep.	1 member + 1 alternate	**2 members** + **2 alternates**
Patient representatives	1 member + 1 alternate	**2 members** + **2 alternates**

Going back in time when we looked at the future …

Focus at the DGRA Annual Congress in **2011** was on the interaction between regulatory review and health technology assessment that now becomes reality:

- Through areas of information exchange under the new HTA Regulation
- Proposed consultation process as part of the EU Pharmaceutical Reform

-> Article 162 "in particular guidelines on **unmet medical needs** and the **design of clinical trials**, other studies and the **generation of evidence along the life cycle** of medicinal products."



DGRA
DEUTSCHE GESELLSCHAFT FÜR REGULATORY AFFAIRS

13. DGRA Jahreskongress

3. und 4. Mai 2011 in Bonn

Health Technology Assessment – HTA, Arzneimittelmarktneuordnungsgesetz, Pharmakovigilanz und Variation update

- Kooperation von nationalen Zulassungsbehörden und EMA mit „pricing bodies" im HTA Prozess
- Bedeutung für Zulassungsabteilungen in Unternehmen
- Ausmaß der Bindungswirkung für die Preisgestaltung

mit

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Rechtsanwalt, Bonn

HTA Regulation – Key principles

- Joint work on common scientific, clinical aspects
- High-quality, timely scientific reports
- Better evidence-base, efficiency, non-duplication

- Joint work driven by Member States' HTA bodies
- Ensure use in national HTA processes

- Improved transparency and inclusiveness

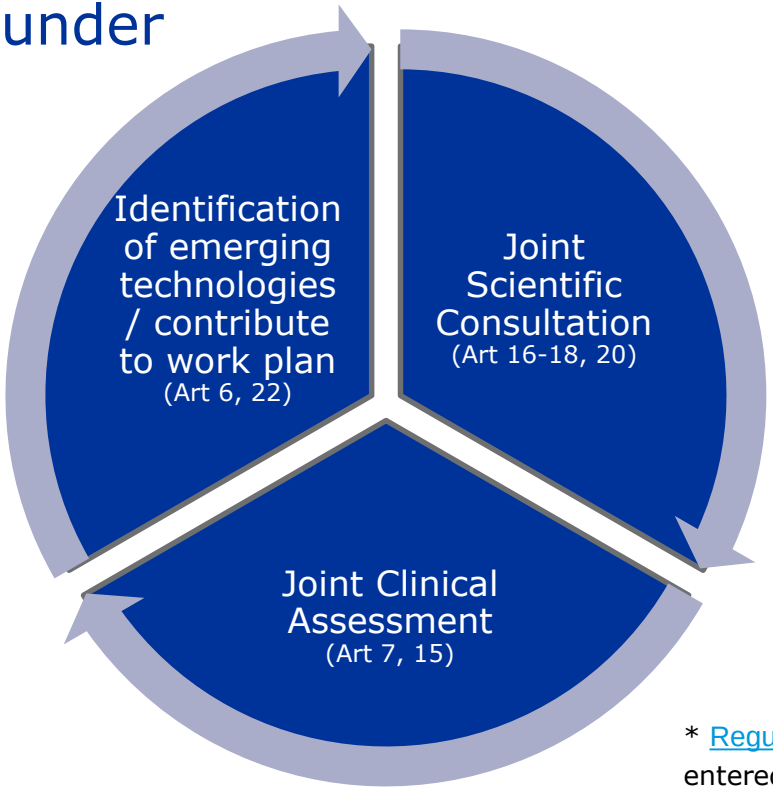
- Recognised value of regulatory/HTA collaboration along the medicines lifecycle to create synergies

- Stepwise implementation

Collaboration with EMA under the HTA Regulation*

Further details through Implementing Acts
to be adopted by the EC (Art 15, 20)

Transversal elements in the Regulation to facilitate such work, supported by the European Commission, include the sharing of confidential information



Note: "Joint" refers to collaborative work amongst HTA bodies

* Regulation (EU) 2021/2282, entered into force in Jan 2022 and applies as of Jan 2025

HTA Regulation Implementation timeline

Adoption

December 2021

Entry into force

January 2022

Preparatory phase for implementation

Date of Application

January 2025

Application phase

Joint Clinical Assessment Full Scope

January 2030

- **Setting up the Coordination Group (HTACG) and its subgroups (EC)**
- **Setting up the Stakeholder Network (EC)**
- **Developing the IT platform (EC)**
- **Drafting implementing and delegated acts (EC)**

Joint Scientific Consultations (JSC)

+

Stepwise build-up of Joint Clinical Assessments (JCA) scope for medicines:

- From Jan. 2025: cancer drugs, ATMPs

(from date of application)

- From Jan. 2028: orphan drugs

(3 years after date of application)

- **Drafting guidance documents (CG)**

eunethta

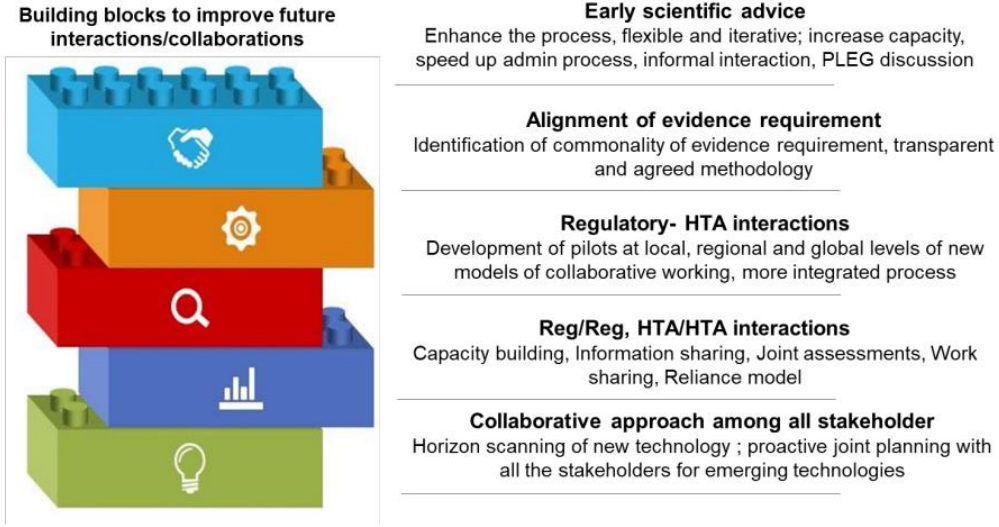
Service contract EUnetHTA21

- **Workplan activities**

EUROPEAN MEDICINES AGENCY

European Commission

Evolving opportunities for interactions and collaborations between regulators, HTAs and payers



Regulatory, HTA and payer interactions and collaborations: optimising their use and outcome success, CIRS workshop 10-11th March 2021 (workshop report)

The EU Pharmaceutical Reform and the new HTA Regulation provide frameworks for enabling such interactions and collaborations