Norwegian Medical Products Agency

CDDF
Cancer Drug Development Forum
FACILITATE. DEBATE. ACTIVATE. INNOVATE.

Navigating Policy Evolution: Now and Future

5 – 6 October; CDDF-EORTC Multi-Stakeholder Workshop; Brussels

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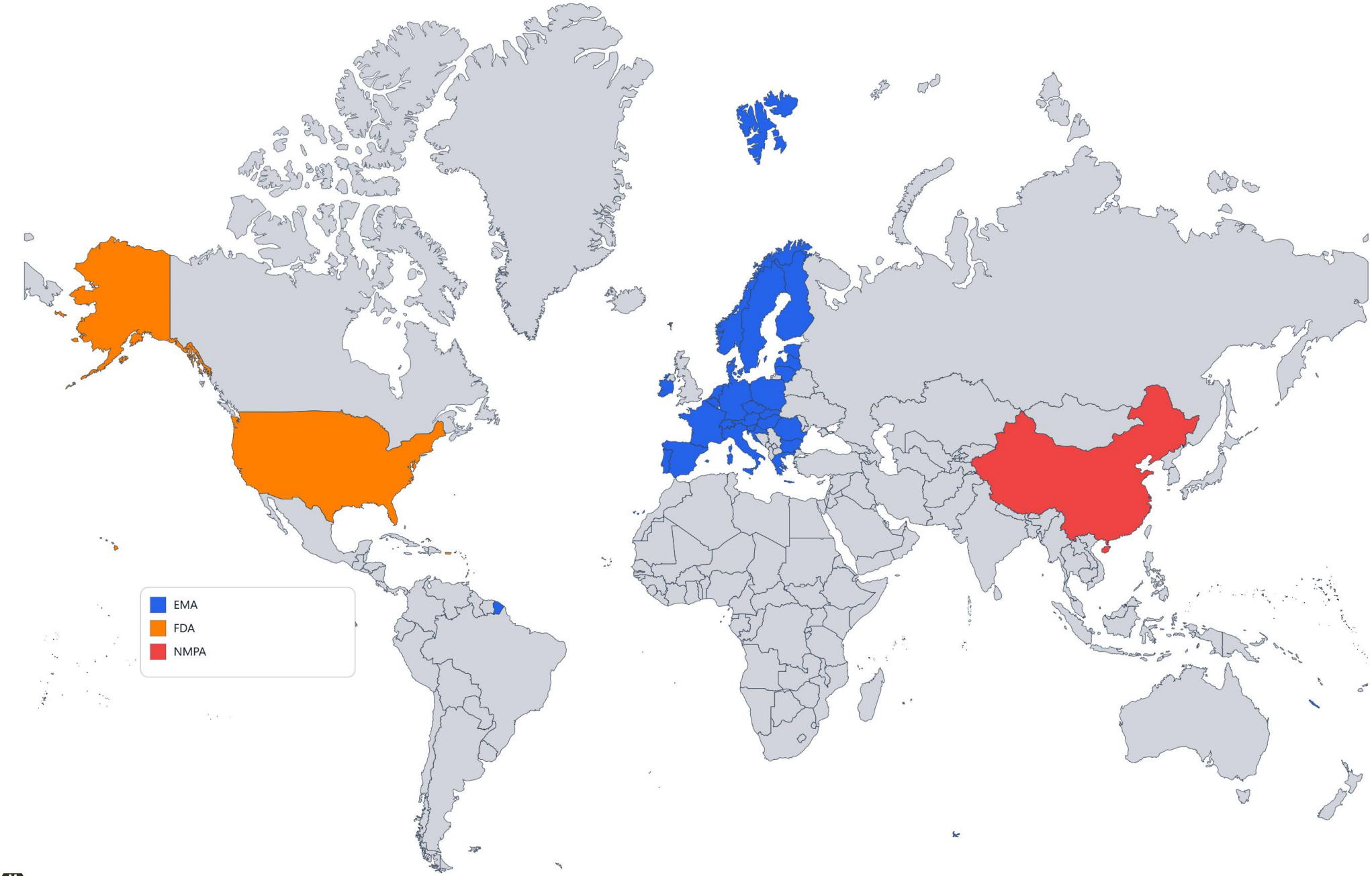
Lead Methodologist in Regulatory and Pharmacoeconomic Statistics

EORTC
European Organisation for Research and Treatment of Cancer
The future of cancer therapy


EMA
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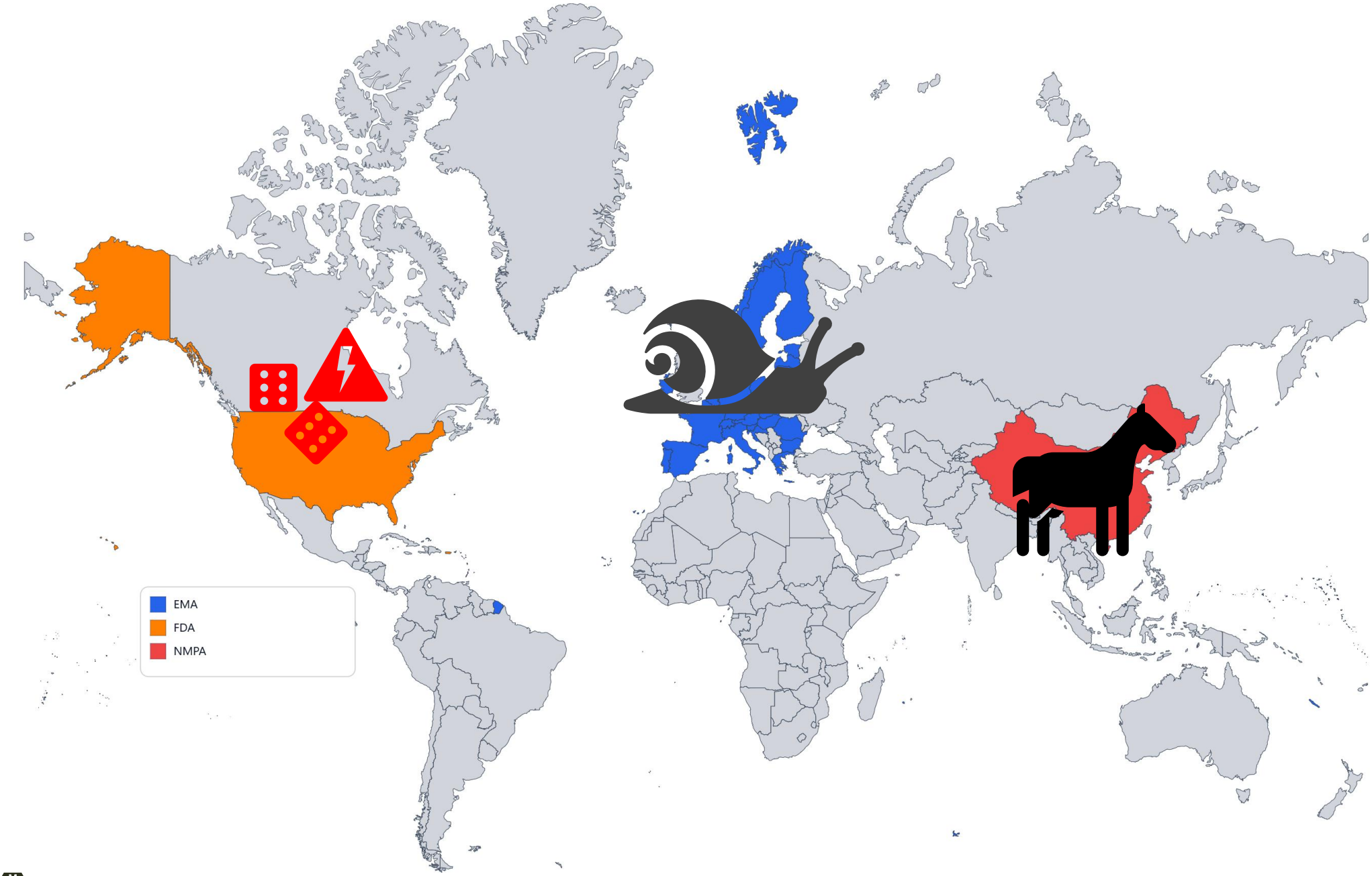
EMA
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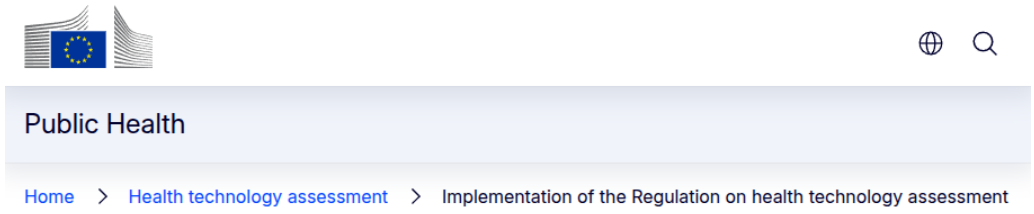
What is happening globally

- **The EU has legislated; the US is mid-cycle.** Europe has locked in its once-in-a-generation reform (Pharma Package) and a functioning supranational HTA layer (JCA), while the US awaits the 2027 user-fee reauthorisation with the IRA contested in court and partially modified by OBBBA.
- **Payer-regulatory convergence is now operational, not theoretical.** Since January 2025, EU clinical development must be planned around JCA PICO scoping in parallel with EMA dossier preparation — directly relevant to your launch-planning and evidence-strategy work.
- **China has completed the transition from regulatory follower to counterparty**, now exporting pipeline rather than only importing standards — which is precisely why the US investment-restriction law and Treasury rules have become a new legal frontier for drug development.
- **Intellectual-property/exclusivity architecture diverges**: the EU held RDP at 8+1 years under industry criticism, while the US expanded orphan exclusions from negotiation via OBBBA — opposite directions, both defensible under competing affordability-versus-innovation logics.
- China now accounts for approximately **20% of global drug development**, and in 2024, **31% of major pharmaceutical companies' innovative pipeline assets originated via in-licensing from China**

What is happening in Europe

- Regulation on health technology assessment (Regulation (EU) 2021/2282 of the European Parliament and of the Council)

- Revision of the Pharmaceutical legislati (Council Directive 65/65/ECC)



Public Health

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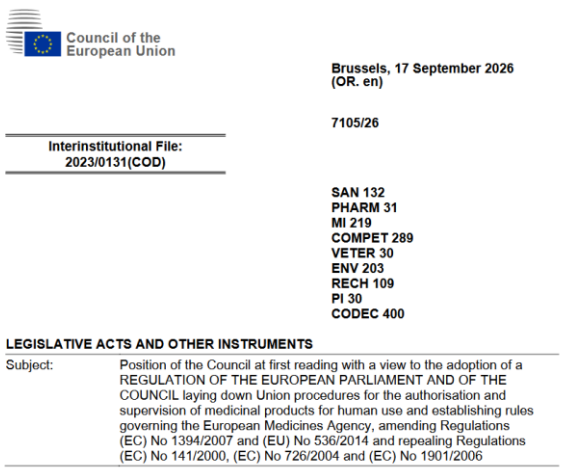
Implementation of the Regulation on health technology assessment

The Regulation (EU) 2021/2282 on health technology assessment (HTAR) entered into force on 11 January 2022 and applies from 12 January 2025.



Council of the European Union

Brussels, 17 September 2026
(OR. en)

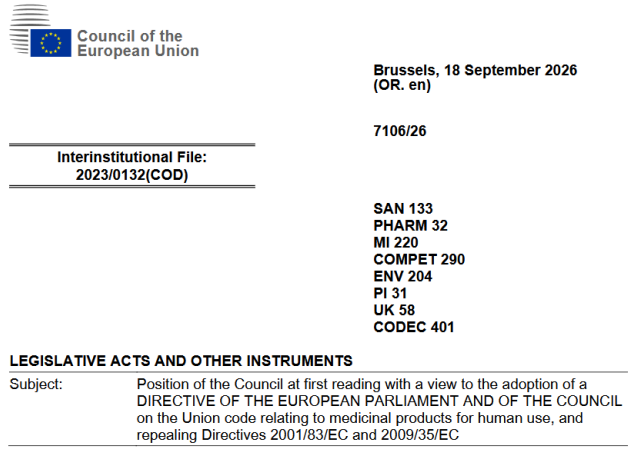
7105/26

Interinstitutional File:
2023/0131(COD)

SAN 132
PHARM 31
MI 219
COMPET 289
VETER 30
ENV 203
RECH 109
PI 30
CODEC 400

LEGISLATIVE ACTS AND OTHER INSTRUMENTS

Subject: Position of the Council at first reading with a view to the adoption of a REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency, amending Regulations (EC) No 1394/2007 and (EU) No 536/2014 and repealing Regulations (EC) No 141/2000, (EC) No 726/2004 and (EC) No 1901/2006



Council of the European Union

Brussels, 18 September 2026
(OR. en)

7106/26

Interinstitutional File:
2023/0132(COD)

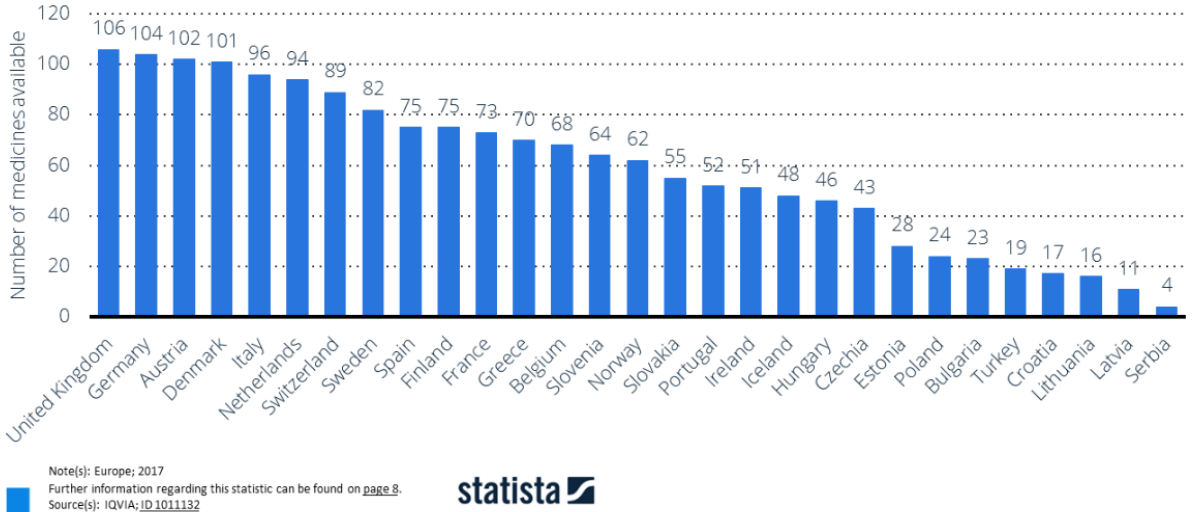
SAN 133
PHARM 32
MI 220
COMPET 290
ENV 204
PI 31
UK 58
CODEC 401

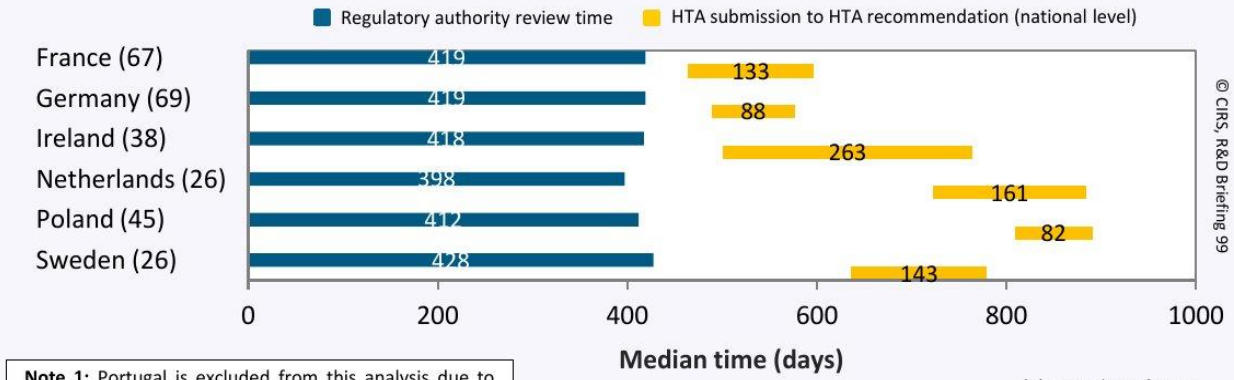
LEGISLATIVE ACTS AND OTHER INSTRUMENTS

Subject: Position of the Council at first reading with a view to the adoption of a DIRECTIVE OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL on the Union code relating to medicinal products for human use, and repealing Directives 2001/83/EC and 2009/35/EC

Why is this happening?

Number of medicines approved by the EMA between 2015-17 available to patients in Europe as of 2018, by country



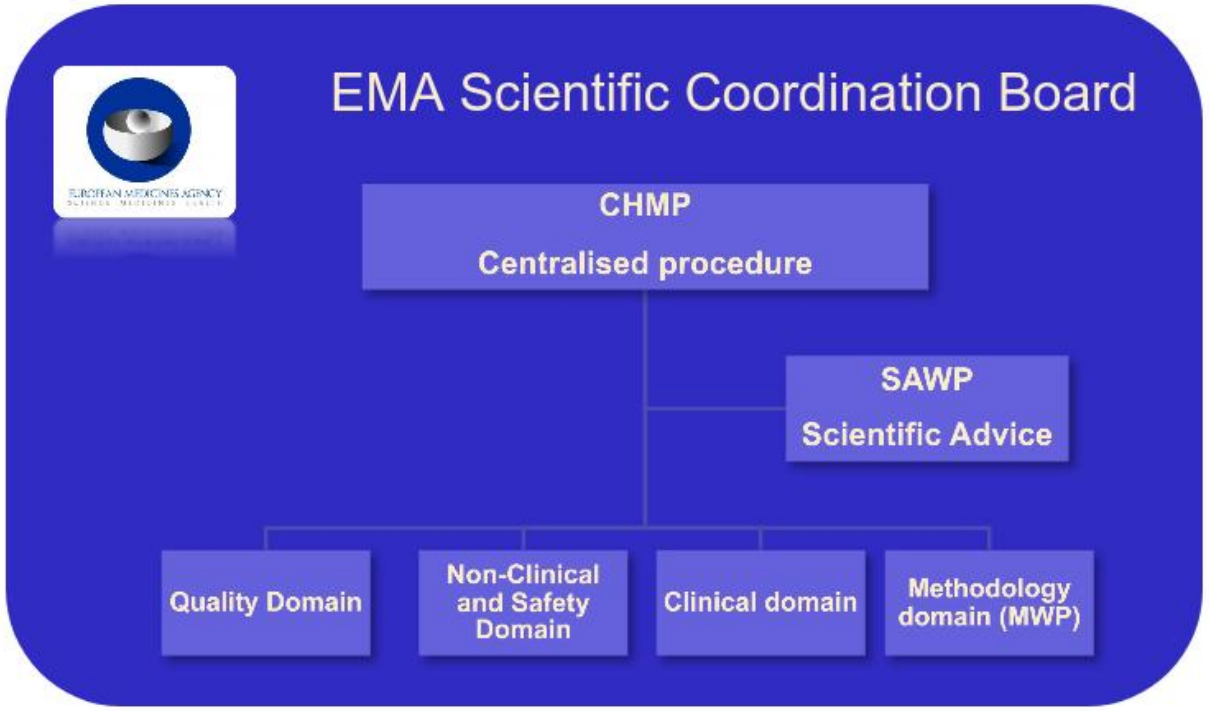


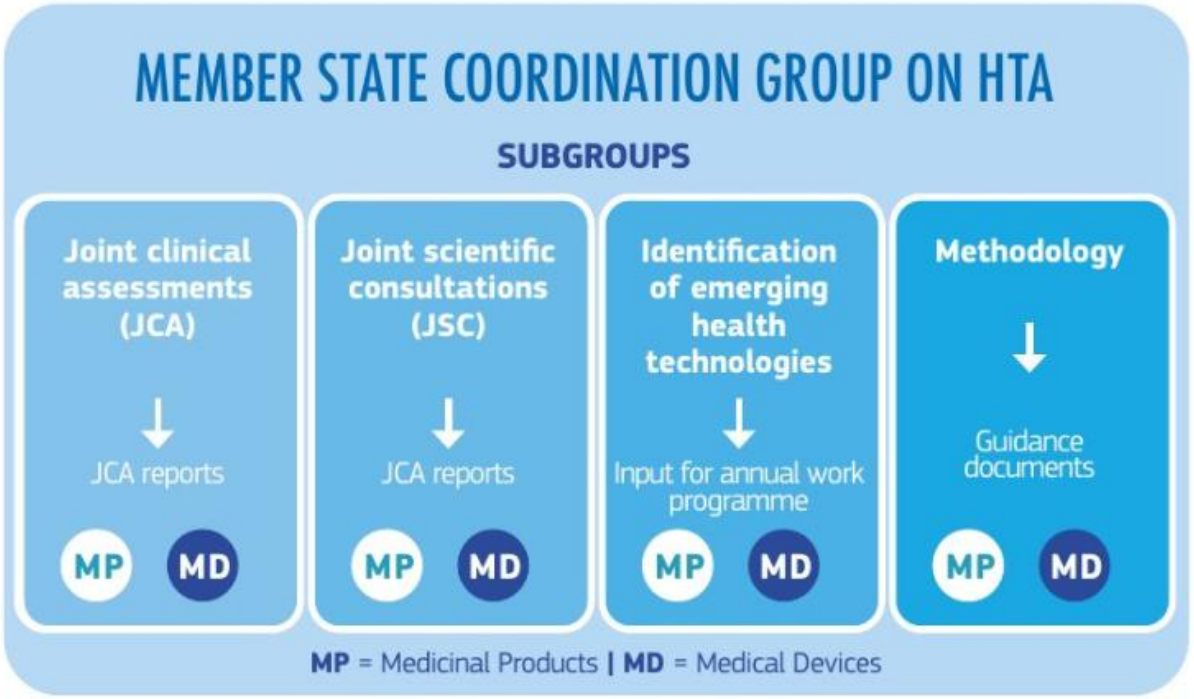
Note 1: Portugal is excluded from this analysis due to unavailable HTA submission dates in the public domain.

(n) = number of NASs

Note 2: For Ireland, both rapid and full reviews are included in this analysis. In addition, the HTA review for Ireland time is calculated as (Rapid review completed - Rapid review commissioned) + (NCPE assessment completed - Full submission received from applicant)

How is this happening in Europe





How is this happening in Europe

EMA CENTRALISED PROCEDURE AND JOINT CLINICAL ASSESSEMENT OCCUR IN PARALLELL

EMA Centralised Procedure	Joint Clinical Assessment
Submission of Application for Marketing Authorisation to EMA	Scoping phase and finalisation of the assessment scope (PICO) 10 days after CHMP adopts its list of questions
Opinion of the Committee for Medicinal Products for Human Use (CHMP)	Dossier submission within 100 days (60 in some situations) after the request and at the latest 45 days before the Opinion by the CHMP
Adoption of the Commission decision granting the Marketing Authorisation (MA)	Finalisation of JCA report by the JCA Subgroup at the latest on the date of the granting of MA
	Endorsement of the JCA report by the Coordination Group no later than 30 days after the granting of a MA

Additional timelines of the JCA procedure are detailed in the Procedural guidance for JCA medicinal products
https://health.ec.europa.eu/publications/procedural-guidance-jca-medicinal-products_en

HOW DOES THE PROCESS WORK?

1. START OF JCA PROCESS

1. The **EMA notifies the HTA secretariat** of the receipt of a marketing authorisation application.
2. When submitting a marketing authorisation application to the EMA, the **HTD submits relevant information to the HTA secretariat**.
3. The JCA process formally begins upon the **appointment of assessor and co-assessor** by the JCA subgroup.

2. SCOPING PHASE

4. The assessor and co-assessor draft an **assessment scope proposal** detailing research questions for the JCA. This is known as PICO (Patient or Population Intervention Comparison or Control Outcome).
5. To ensure that the assessment scope reflects the needs of the Member States, the **members of the JCA subgroup are invited to comment** on the suggested scope from their national perspective. In addition, individual experts are invited to provide their input on the assessment scope.
6. The **assessment scope is finalised by the JCA subgroup** and is shared with the HTD in the Commission's initial request for the submission dossier. This is done within **10 days** after the Committee for Medicinal Products for Human Use (CHMP) adopts its list of questions, or at the latest **75 days** after the EMA validation of the marketing authorisation application in accelerated procedures and for variations to the terms of an existing marketing authorisation.
7. The HTD can request an assessment scope explanation meeting with the JCA Subgroup.

3. DOSSIER SUBMISSION

8. The HTD submits a **comprehensive dossier**, including clinical and safety evidence, to the HTA secretariat in digital format within **100 days** of the Commission's initial request (reduced to **60 days** in accelerated procedures and for variations to the terms of an existing marketing authorisation).
9. The assessor and co-assessor can ask the HTD to submit additional information via the HTA secretariat.
10. The HTA secretariat shares all the information received from the HTD with the assessor and co-assessor and the JCA subgroup.

4. DRAFT JCA REPORT

11. The assessor and co-assessor prepare a **draft JCA report and a summary report** with input from the JCA subgroup and the individual experts.
12. The draft JCA and summary reports are **shared with the HTD** to check technical or factual inaccuracies, and to comment on any information the HTD considers to be confidential.
13. The HTD may provide, on their initiative, new relevant information, data, analyses and other evidence to the HTACG via the HTA secretariat.

5. FINALISATION OF JCA REPORT

14. The JCA subgroup **discusses and finalises the draft JCA report** and summary report in a meeting. Individual experts may be invited.
15. The finalised report is submitted to the HTACG for endorsement. The HTACG must endorse the reports no later than **30 days** following the adoption of the Commission Decision granting the marketing authorisation for the medicinal product under assessment.
16. After the Commission's procedural check, the **JCA report and summary report endorsed by the HTACG is published**.

Please note that the Joint Clinical Assessment is to be discontinued, for example, where an application for a marketing authorisation or for a variation to the terms of an existing marketing authorisation is withdrawn, or where the outcome of the centralised procedure is negative.

Don’t try to read too much into the current state of things but…..

- 16 procedures initiated, 4 completed, 4 discontinued
- The 25% discontinuation rate is of concern but needs to be seen in context of the first products chosen to go first….



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Discontinuations

- JCAs can terminate because the underlying MAA is withdrawn or the centralised procedure failed. These reflect pipeline attrition upstream, not process failure.
- Three of the four discontinuations (Catequentinib, Tacquell, the unnamed September case) explicitly cite **non-compliance with Article 9** of the HTA Regulation (the core provision governing dossier completeness, transparency, and methodological robustness).
- Unexplained evidence gaps are considered fundamental flaws in HTA, irrespective of the jurisdiction
  - For Catequentinib and Tacquell **systematic literature review (SLR) failures were identified.** The absence of transparent study identification, no feasibility assessment when evidence was claimed absent, and missing documentation of exclusion criteria.
  - Tacquell's discontinuation was also due to failure to respond to an information request.

Give it time…

- The process is functioning procedurally (reports published on schedule, discontinuation rules applied)
- There are two basic objectives: **reducing duplication** and **improving access speed** => too early to conclude
- The PICO inflation problem hasn't been solved
- The process is front-loaded. The bulk of work is currently on the HTDs and assessor pair (and to a lesser extent the entire HTAR CG during peer review).
- The benefit (national reuse of a single clinical dossier) depends on the degree of uptake in downstream processes (decisions) and that has not yet been observed at scale.

The proof is in the pudding!

