TRANSLATING INNOVATION INTO ACCESS FOR ATMPs

STRENGTHENING

THE EUROPEAN INNOVATION ECOSYSTEM

3rd EU-Innovation Network multi-stakeholder meeting

ROME, 15 NOVEMBER 2024

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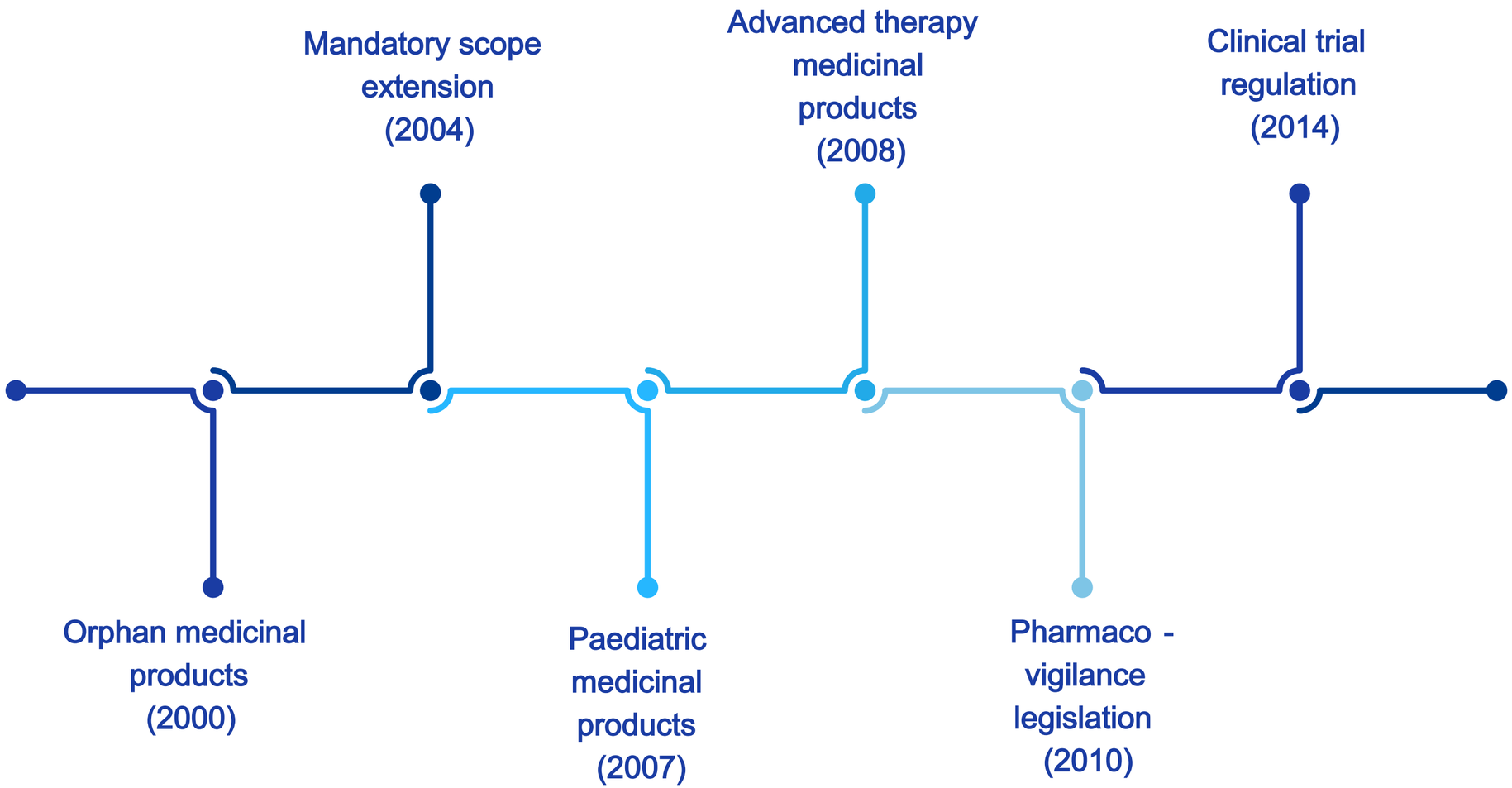
Origins

"It is necessary to establish a centralised community authorisation procedure for technologically advanced medicinal product in particular those derived from EU biotech industry."

Systematic extension of stimulus to innovation



Advantages of Clinical Trials (CTs) in Europe

1

Access to a **world class Clinical Research Community** with extensive experience in conducting high quality CTs.

2

An **evolving regulatory environment** that facilitates clinical trial conduct

3

Policies that promote research into orphan drugs and rare diseases – support for SMEs

4

Ability to obtain **scientific advice on innovative CT design** and innovative evidence generation

Approx. **4000** clinical trials of medicines are authorised annually in the EU.

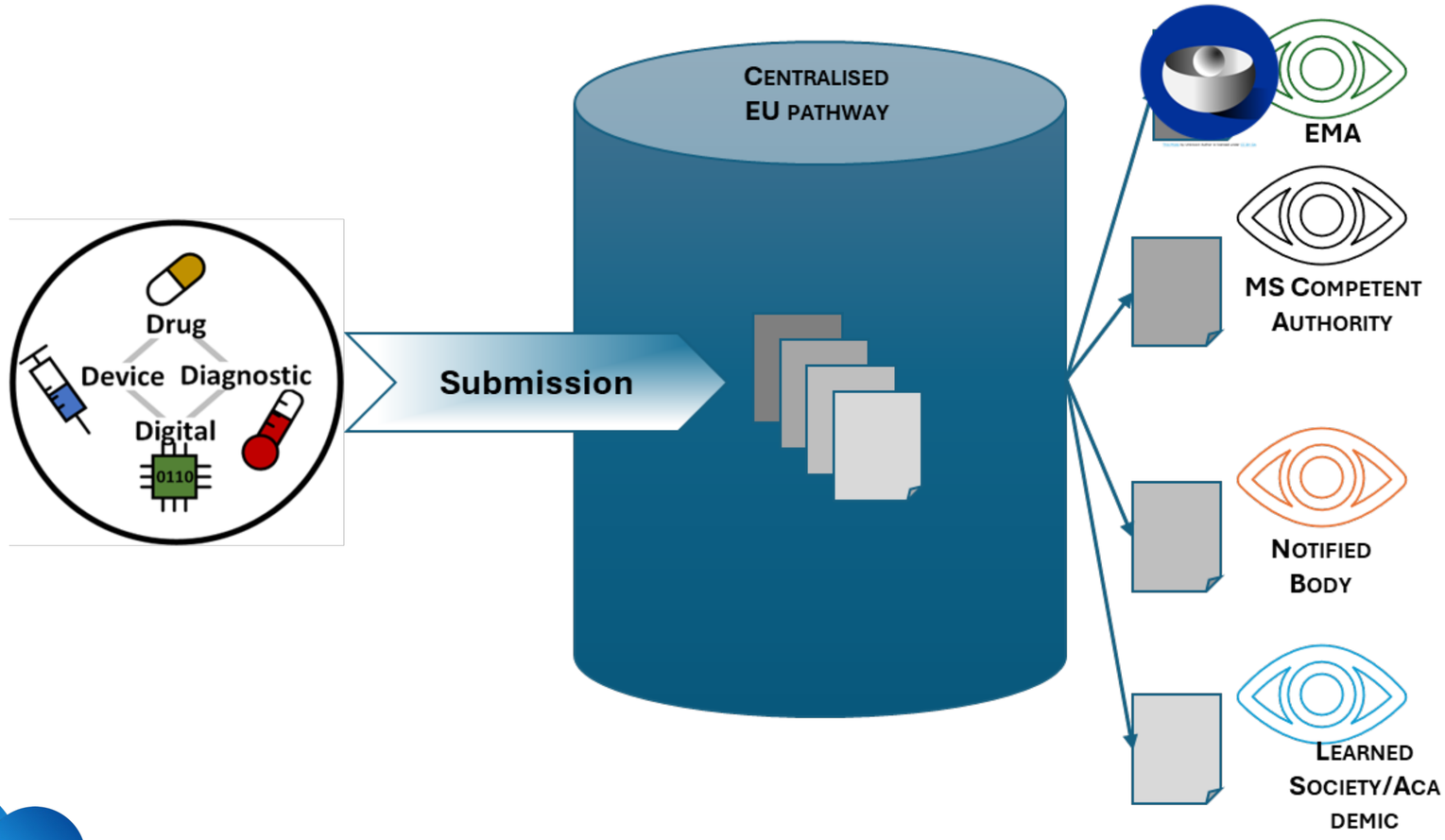
The EU is home to approx. 1.8 million **practising physicians** ,2.8 million **practising nursing professionals** and 450 000 **practising pharmacists** .

Accelerating Clinical Trials in the EU (ACT EU)

ACT EU is a joint initiative of HMA/European Commission/EMA to **transform the EU clinical research environment** in support of medical innovation and better patient outcomes.

- **Builds on the momentum** of the Clinical Trials Regulation and CTIS
- **Driven by** the Network Strategy to 2025, the Regulatory Science Strategy and the EU Pharmaceutical Strategy

Vision of a future integrated pathway for complex health care solutions

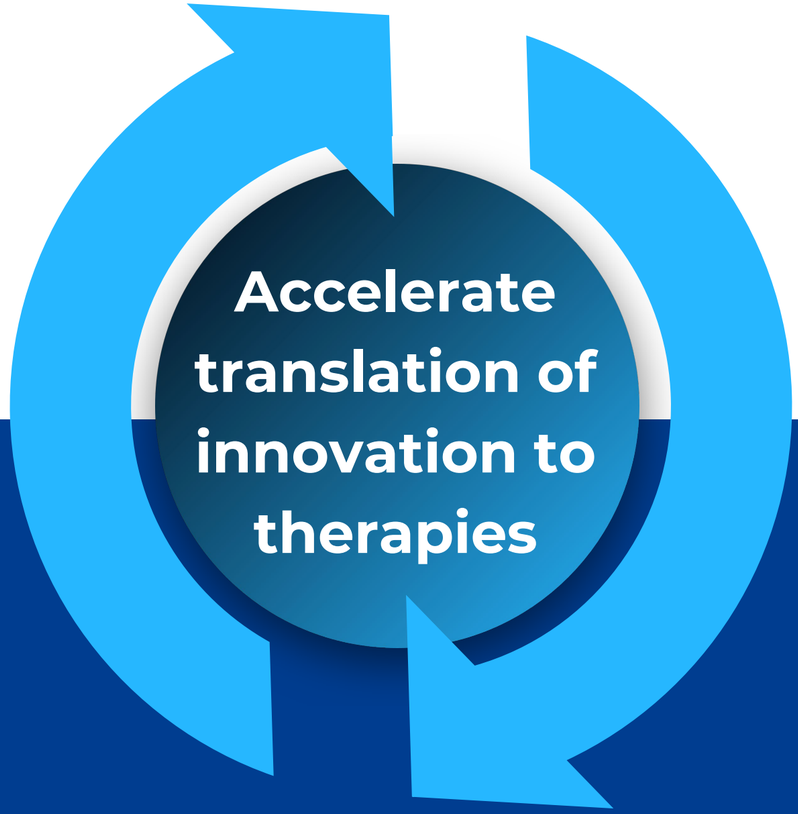


MA decision / EU certification
(SA/MAA; Conformity assessment / CE marking)

Integrated pathway can offer support to applicants in development, borderline aspects, assessment and lifecycle stages

EU strategies on fostering an innovative development ecosystem

EMRN STRATEGY TO 2028



EMRN STRATEGY TO 2028

1 **Promote integration Science & Technology medicines development**

Accelerate translation of innovation to therapies

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2 Foster generation of high quality impactful evidence

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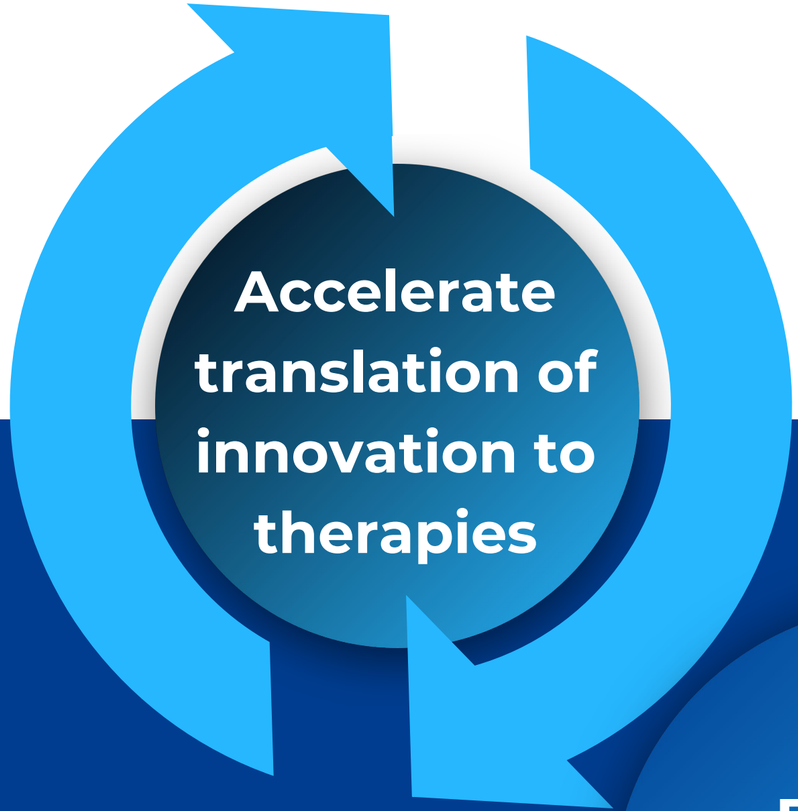
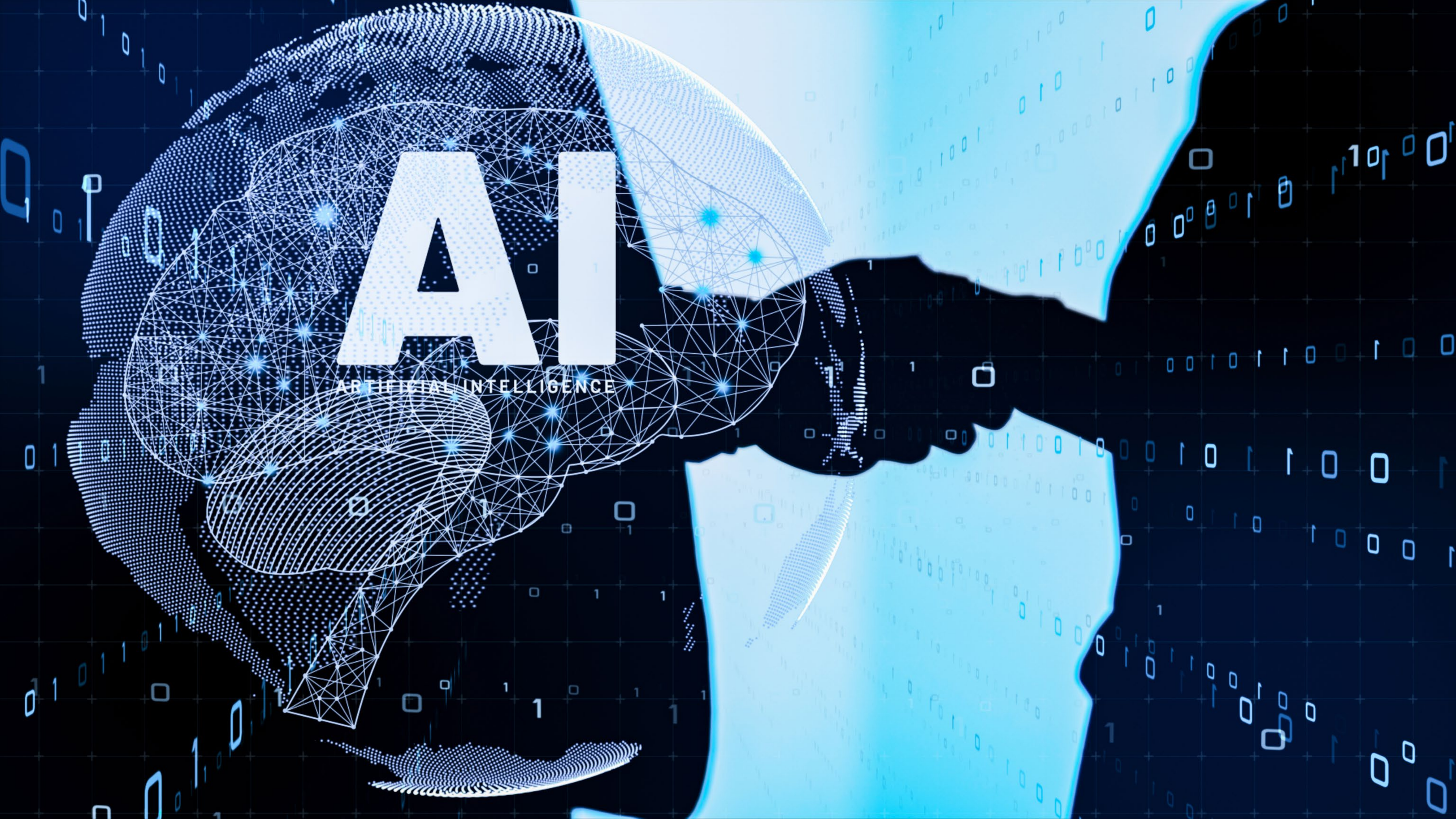


1 Promote integration Science & Technology medicines development

2 Foster generation of high quality impactful evidence

3 Promote stakeholders' co-operation


AI
ARTIFICIAL INTELLIGENCE


HMA
Heads of Medicines Agencies
Multi-annual AI workplan
2023-2028
HMA-EMA Big Data Steering Group
– NOVEMBER 2023
europa.eu


FDA
ADMINISTRATION
Using Artificial Intelligence
& Machine Learning
in the Development of
Drug & Biological Products
Discussion Paper and Request for Feedback


Health
Technology
Assessment
#HealthUnion
European
Commission

EC Pharmaceutical system problem statement

- **Medical needs** of patients are not sufficiently met (incl. for patients with rare diseases and of children)
- **Affordability** of medicinal products is a challenge for health systems
- Patients have **unequal access** to medicinal products across the EU. Shortages of medicinal products are an increasing problem in the EU
- The pharmaceutical product lifecycle can have negative impacts on the **environment**

The regulatory system does not sufficiently cater for **innovation** and in some instances creates unnecessary **administrative burden**

Incentives for innovation

1	2	3
Targeted data exclusivity approach with 8 years of unconditional data protection	Pre-authorisation support Faster authorisation	Earlier market entry of generic and biosimilar medicines

- Additional indication(s)
- Comparative clinical trials
- (High) Unmet Medical Need
- Market launch

Streamlined regulatory framework

Pre-authorization:

support to promising medicines to accelerate development and attract investments + regulatory sandboxes option

Faster authorisation:

180 days standard review
150 days accelerated review

Regulatory efficiency / Lower regulatory burden:

simplified procedures, more agile and flexible expertise, better use of data and digitisation, ePI

Repurposing

data analysis of evidence from non-for-profit entities

Development support

1

Scientific Advice extended to involve other bodies e.g., Medical Devices authorities/expert panels, HTAs, payers, SoHO, Clinical Trials Coordination Group (CTCG)

2

PRIME codified in legislation as “enhanced scientific and regulatory support for priority medicines”

3

Phased review of complete data packages: only for products that offer an exceptional therapeutic advancement in areas of (High) Unmet Medical Needs

4

Regulatory sandbox environment to test adapted, waived or deferred requirements for products that provide major advantage to patients

Novel types of applications

1

Platform Marketing Authorisation

2

Combination pack

3

Temporary Emergency Marketing Authorisation

4

Conditional MA

4

Hospital Exemption for ATMPs

EC Review Tries to Advance

1

Attract pharmaceutical R&D by providing a future-proof, stable legal framework and a favourable regulatory environment

2

Boost regulatory support for the development of promising medicines

3

Provide a more targeted incentives framework for innovation with a focus on patient access and addressing unmet medical needs

4

Boost innovation and EU competitiveness through an efficient and simplified regulatory framework

THANK YOU

FOR YOUR ATTENTION