

CONFERÊNCIA ANUAL DO INFARMED, I.P.



Futuro inovador dos medicamentos e das tecnologias da saúde:
Estratégias regulamentares, digitalização e integração

Innovative future of medicines and health technologies:
Regulatory strategies, digitalisation and integration

Novas Estratégias Regulamentares New Regulatory Strategies

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Sustainable and Resilient Global Health

CONTEXT:

- **Ageing population** + general rise in chronic conditions, multi-morbidity
- Need interventions in many areas to address **unmet medical needs**
- Low **patient engagement**
- **Health inequalities** across Europe
- *Ad hoc* introduction of **digital tools**: low uptake, data silos



Digital Transition

CHALLENGES:

“Digital solutions can radically change the way **health and care services** are delivered - and help them respond better to crises like COVID-19.

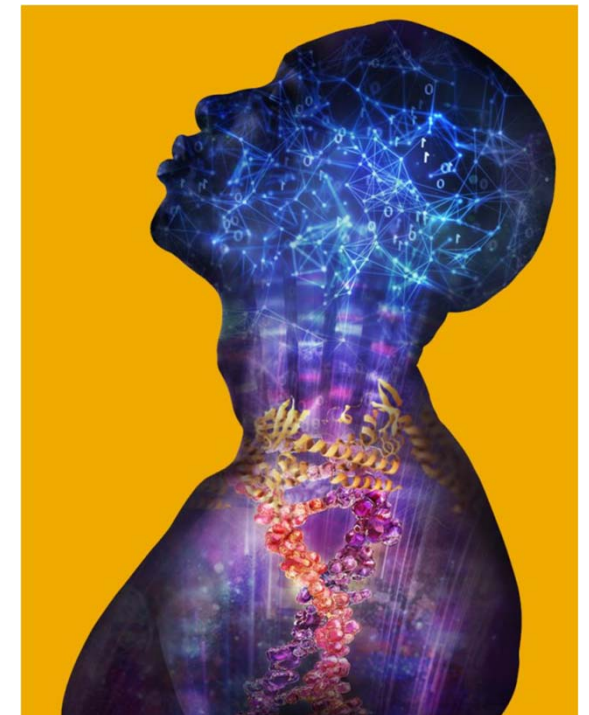
They can improve **accessibility and communication**.

They can **empower citizens**, enabling them to actively participate in the management and monitoring of their own health.

They bring health professionals together to make more efficient **use of knowledge and resources**.

And they allow better use of **health data** in research and innovation, enabling stronger and more resilient health and social care systems .

And as we embrace the possibilities of a digital future, we must always keep the **human being in its centre.**”



Digital Transition

KEY ACTIONS AND LEGISLATION:



- European Health Union / lesson learnt COVID-19
- Digital funding mainstreamed across Multiannual Financial Framework
- EU Health Data Space
- Ethical, human-centric EU approach to AI & robotics, guidance on apps
- eHealth Digital Services Infrastructure – MyHealth@EU
- European Reference Networks, ‘1+ million Genomes Initiative’
- General Data Protection Regulation, ePrivacy, cybersecurity
- Digital Services Act
- **EU Pharmaceutical Strategy**

Building a future-proof Regulatory System

POLITICO

Building a future-proof regulatory system

Simpler, faster, better – let's not miss the opportunity to revamp the EU pharmaceutical regulatory framework to transform lives.

SHARE



“There is a risk this becomes a missed opportunity to future-proof the regulatory framework as an innovation enabler, keeping pace with the latest scientific and technological developments that benefit patients.”

SEPTEMBER 26, 2024 5:00 AM CET

*With the rapid and wide-ranging advances in **digital health, artificial intelligence and precision medicines**, the **revision of the pharmaceutical legislation** is a once-in-a-generation opportunity to build a future-proof regulatory system that truly supports patient needs and the timely adoption of medical innovation.*

HOW?

<https://www.politico.eu/sponsored-content/building-a-future-proof-regulatory-system/>

One Regulatory Road to Innovation



To drive an **agile, competitive** and **world-class regulatory system in Europe and beyond** that embraces advances in science, technology and medicines, accelerating access to innovative healthcare solutions and optimized patient outcomes.

Non-legislative:

Regulatory Road to Innovation

- Dynamic regulatory assessment
- Drug device combinations & Biomarker validation
- Digitalisation across product lifecycle
- Real world evidence

Legislative:

Revision of the Pharmaceutical Legislation

- Enable swifter, expertise-driven decision making at EMA
- Optimal use of expedited pathways
- Expand the role of the EMA in the assessment combination products
- Allow the replacement of the PIL with an electronic PIL

Short-Mid-term Objectives

Long-term Objectives

Fonte: EFPIA

Enhancing the Regulatory Framework (1/5)



1. **Optimising the EMA committee structure** to speed up the regulatory approval.
2. **Improved Decision-Making:** Faster EMA assessment-to-approval timelines.
3. Simplifying processes and **bringing together different areas of expertise** to better regulatory science.

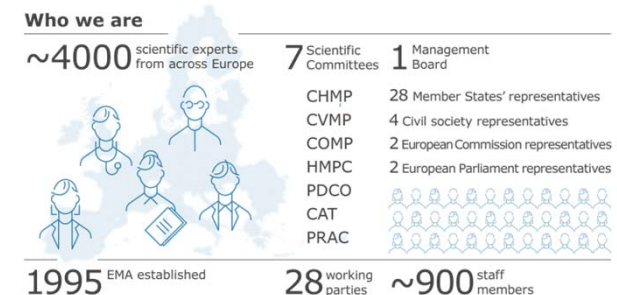


- **EMA Reorganization:**
 - **Reduction of primary committees** from 7 to 2 (**CHMP** and **PRAC**), supported by specialized advisory.
 - **Faster assessment timelines:**

Regular review from 210 to 180 days

Accelerated review from 150 to 120 days

EC decision from 67 to 30 days



- **Support for Orphan Medicines:** Centralized EMA decision-making for orphan drugs to expedite approvals.

Enhancing the Regulatory Framework (2/5)



4. **Enhancing the agility and broadening the scope of expedited pathways**, like phased review, PRIME, and conditional marketing approval.

➤ The EU has put in place these tools to fast-track the approval of medicines, **yet their use has been limited.**



- **Adapting regulatory assessments dynamically as new data becomes available:**

| Rolling Reviews | TEMA* | Expansion of the PRIME** |
|---|---|--|
| <ul style="list-style-type: none">○ Continuous submission and real-time assessment of new data to support quicker regulatory updates.○ Proven effective during COVID-19 vaccine approvals. | <ul style="list-style-type: none">○ Enables rapid approval of new drugs and indications in health emergencies.○ Includes a transition to full or conditional marketing authorization (MA/CMA) to ensure continuity post-emergency. | <ul style="list-style-type: none">○ Expanded eligibility for accelerated regulatory pathways to cover unmet medical needs.○ Focus on orphan drugs and life-saving therapies, increasing patient access to innovative treatments. |

*Temporary Emergency Marketing Authorization | **Priority Medicines

Enhancing the Regulatory Framework (3/5)



5. Gradually replacing the paper **patient information leaflets with electronic versions.**

- The COVID-19 and the Ukrainian crisis have shown how important electronic information leaflets are for patients.



- **Building for the Digital Future:**

- A step-wise approach informed by the Digital inclusion principle.
- In complement to the current EMA ePI Pilot, build on existing learning (MSs pilots, COVID19 vaccines, survey from hospital pharmacists):
 - Transition starting with products given by healthcare professionals where patients never see the pack, such in hospitals;
 - Later, it can expand to more products (ambulatory) in countries that are prepared for this change.
- Base future steps on patient centricity:
 - Contents improvement;
 - Look at alternative ways to equip patients and end-users with the most updated product information.

Enhancing the Regulatory Framework (4/5)



6. Addressing the rise of **combination products** (medicines and medical devices), representing 25% of today's pipeline:

- New legal category + clearer rules enabling the full potential of personalised medicine and integrated healthcare



• Creating an integrated evaluation pathway for the assessment of combination products:

Regulatory pathway

- EMA to ensure the possibility for a **parallel advice** with Notified Bodies;
- Long-lasting impact can be achieved through **legislative changes in particular for certain types of drug-device combinations**.

Early-stage validation

- Clear standards on **biomarker validation** & enabling more precise diagnostics and response monitoring.
- **Innovative testing environments** for highly complex products, allowing early-stage validation under controlled conditions.

Enhancing the Regulatory Framework (5/5)



7. **RWD/RWE:** expedite **understanding of disease** and **inform decision making** through the product lifecycle from discovery through to access.



- **Good quality RWD/RWE give insights on the real-life impact of medicines and health care:**
 - RWE has shown its value in assessing effectiveness, efficacy and safety of vaccines/medicines when traditional randomised clinical trials are unethical or impossible to conduct (e.g: COVID-19 treatments and vaccines).
 - RWD/RWE framework with **clear principles for data quality and interoperability, access, analysis and regulatory acceptance.**
 - A key aspect will be to support the joint advice with HTA bodies to ensure their readiness to accept alternative evidence generation for market access decisions.

Evidence planning for an early access decision



Ensuring **faster, more informed decisions**,
aligning with the pharma industry's commitment
to delivering innovative, patient-centered
healthcare solutions



REGULATIONS
REGULATION (EU) 2021/2282 OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
of 15 December 2021
on health technology assessment and amending Directive 2011/24/EU

Optimised development plan → Improved access for patients



Simultaneous notification
to EMA and HTA CG



Joint Scientific Consultations in
parallel with Scientific Advice



Interaction between EMA and HTA CG
during procedure - Scientific advice
provided jointly by HTA bodies

A future for regulatory science in the European Union

CHALLENGES

Agile, competitive, world-class regulatory systems:

- Ensure through analysis of gaps and opportunities in regulatory framework

Embraces advances in science and technology:

- Evolve the framework for innovation, addressing evidentiary sources and uses

To promote global harmonization in RWE use, with varied expectations and standards across regions and stakeholders:

- Alignment in data quality and relevance criteria

To accelerate access to innovative healthcare solutions and optimized patient outcomes:

- Elevate multi-stakeholder involvement to focus decisions, support on patients' needs and preferences
- Expand global convergence to deliver European leadership on regulatory standards and practices

Evidence
planning
for an
early
access
decision



REGULATORY SCIENCE STRATEGY

Regulatory science as the foundation:

- Enabling and leveraging research and innovation in regulatory science

Catalysing integration of science and technology in drug development:

- Driving collaborative evidence generation
- Improving the scientific quality of evaluations

Robust data ecosystem that ensures interoperability, quality, and secure data linkage:

- The EU Health Data Space (EHDS) is a key initiative by:
 - Enhancing data governance,
 - Facilitating secondary use of data for research and policymaking.

To achieve a new and active role at the crossroads between science and healthcare:

- Advancing patient-centered access to medicines in partnership with healthcare systems
- Addressing emerging health threats and availability/therapeutic challenges

We All Work For ...



... More and Better Health !

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