

# 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.

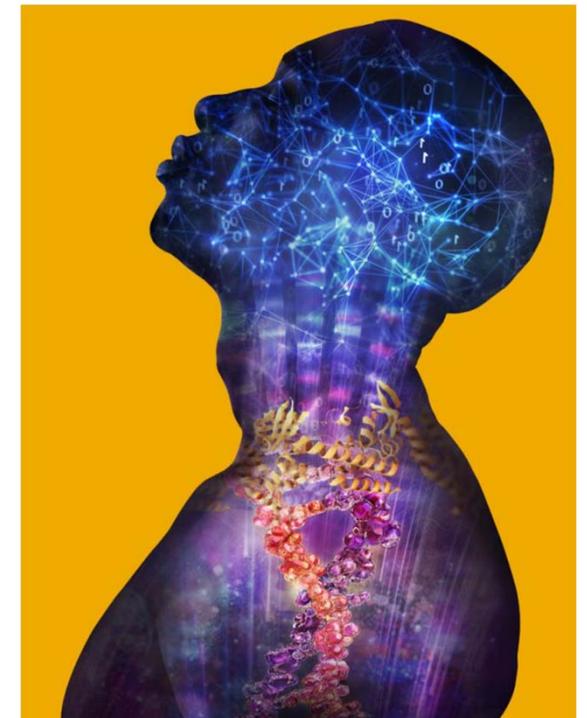
They can improve **accessibility and communication**.

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)



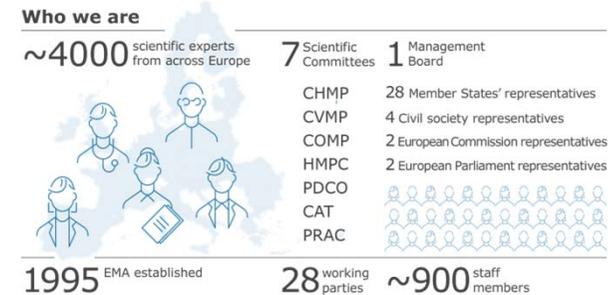
1. **Optimising the EMA committee structure** to speed up the regulatory approval.
2. **Improved Decision-Making:** Faster EMA assessment-to-approval timelines.
3. Simplifying processes and **bringing together different areas of expertise** to better regulatory science.



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

\*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

