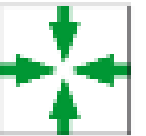


***NEW PHARMA  
LEGISLATION  
(PHARMA PACKAGE):  
PROSPETTIVE  
CLINICI E PAZIENTI***

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***NOT PERFECT***

***OPPORTUNITY***



European  
Commission

## Public Health

[Home](#) > [Medicinal products](#) > [A pharmaceutical strategy for Europe](#) > [Reform](#)

# Reform of the EU pharmaceutical legislation

26 April 2023

## PHARMA PACKAGE

- A systemic overhaul of EU pharmaceutical legislation
- First major overhaul of EU pharmaceutical legislation in over 20 years
- New Regulation + New Directives
- Includes revision of key pharmaceutical legislation (e.g. Orphan framework, EC No 141/2000)

### Regulation (Regolamento)

È direttamente applicabile in tutti gli Stati membri.

Non richiede una legge nazionale di attuazione fissati.

Garantisce un'applicazione uniforme in tutta l'UE.

Esempio: Regolamento Orphan Drug (EC No 141/2000).

### Directive (Direttiva)

Deve essere recepita nella legislazione nazionale.

Gli Stati membri devono modificare o adottare norme nazionali per raggiungere gli obiettivi fissati.

Lascia agli Stati una certa flessibilità sulle modalità di attuazione.

Esempio: Direttiva 2001/83/EC sui medicinali per uso umano.

**Regulation  
Directive**

**= same rules everywhere in Europe  
= common objectives, but  
implementation through national  
legislation**

# ***WHY REFORM?***

## **STRUCTURAL CHALLENGES IN EU PHARMA SYSTEM:**

### **MAIN DRIVERS**

- Unequal access
- Innovation gap vs US/China
- Unmet medical needs

### **ADDITIONAL DRIVERS**

- Shortages
- Sustainability
- Digitalisation

**REDUCED EU COMPETITIVENESS  
IN RESEARCH & DEVELOPMENT INVESTMENT**

# ***OBJECTIVES***

## **KEY TENSION: INNOVATION VS ACCESS**

- **Boost innovation & EU competitiveness**
- **Improve access to medicines across Member States**
- **Address unmet medical needs**
- **Strengthen supply security & reduce shortages**
- **Modernise regulatory processes (digital & data-driven)**
- **Enhance environmental sustainability**

Brussels, 24 February 2026  
(OR. en)

6366/26

LIMITE

SAN 90  
PHARM 14  
MI 127  
COMPET 189  
VETER 21  
ENV 130  
RECH 66  
CODEC 236  
PI 21

NOTE

|          |                                                                                                                                                                                                                                                      |
|----------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| From:    | General Secretariat of the Council                                                                                                                                                                                                                   |
| To:      | Permanent Representatives Committee                                                                                                                                                                                                                  |
| Subject: | Regulation laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency<br>- Analysis of the final compromise text with a view to agreement |

Delegations will find in Annex the final compromise text of the Regulation laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency.

- June 2025 — Council agrees its negotiating position (general approach / mandate)
- Dec 2025 — Council and EU Parliament reached an agreement on the final text (trilogue)
- 2026 — Formal adoption + legal-linguistic revision
- 2026 — Publication in Official Journal (EUR-Lex)
- 2026–2028 — Entry into force & phased implementation

# Implementation timeline

The adopted acts of the **new pharmaceutical legislation** are expected to enter into force in 2026.

The following two years, until 2028, will serve as a **transition period**. In this time interval, all EU Member States will need to update their national laws to align with the new rules.

In addition, the European Commission, EMA and EU Member States will provide **implementation guidance** to stakeholders.

## Timeline

- December 2025  
Political agreement on new pharmaceutical legislation
- 2026  
Adopted acts of the new pharmaceutical legislation enter into force
- 2026-2028  
Transition phase
  - EU Member States update their national laws to reflect the new rules
  - European Commission adopts implementing and delegated acts to help implement the new rules
  - EMA and national competent authorities develop implementation guidance and adapt their procedures and IT systems
- 2028  
New pharmaceutical legislation becomes applicable

# ***REGULATION: MAIN CHANGES***

## **REGULATORY CHANGES**

- **EMA**
- **Real World Evidence (RWE)**
- **Accelerated pathways**
- **Digitalisation**

## **INCENTIVE CHANGES**

- **Exclusivity**
- **Access-linked incentives**
- **Shortages**

# ***REGULATION: MAIN CHANGES***

## **EMA REGULATORY OPERATING SYSTEM**

principalmente una riforma degli incentivi regolatori,  
non una riforma dei sistemi sanitari europei

incide molto su innovazione, esclusività e supply chain,  
molto meno su prezzi e accesso reale dei pazienti,  
che continuano a dipendere soprattutto  
dalle decisioni nazionali di HTA e reimbursement

**PHARMA PACKAGE =  
REGULATORY REFORM,  
NOT HEALTHCARE SYSTEM REFORM**

### ***PRIMA:***

- Valutazione standard EMA ~210 giorni (+clock stops)
- Accelerated approval usato ma limitato e poco strutturato

### ***DOPO:***

- Valutazione EMA standard da ~210 a 180 giorni
- Rafforzato il supporto regolatorio per farmaci che rispondono a elevati bisogni medici insoddisfatti
- Maggiore utilizzo di strumenti regolatori accelerati e adattativi

## ***WHAT CHANGES:***

### **MODULAR EXCLUSIVITY**

Protezione regolatoria dei dati  
da 8+2+1 anni  
a un sistema modulare  
(base + bonus legati a specifici obiettivi)

### **LAUNCH OBLIGATION**

### **ACCESS INCENTIVES**

Se un'azienda non lancia il farmaco in più  
Stati membri può perdere parte del beneficio  
regolatorio

### ***PRIMA:***

- RWE usata “caso per caso”
- Soprattutto post-authorization

### ***DOPO:***

- RWE diventa parte integrata del ciclo regolatorio
- Usata anche per supportare:

Autorizzazione iniziale

Estensioni di indicazione

Rivalutazione della sicurezza

***PRIMA:***

- Gestione delle carenze prevalentemente a livello nazionale

***DOPO:***

- Monitoraggio/coordinamento a livello UE attraverso l'EMA

# ARTICLE 048

1. *An entity not engaged in an economic activity ('A not-for-profit entity') may submit to the Agency or to a competent authority of the Member State substantive non-clinical or clinical evidence for a new therapeutic indication that is expected to fulfil an unmet medical need.*

*The Agency may, at the request of a Member State, the Commission, or on its own initiative and on the basis of all available evidence, including any additional evidence that may be submitted by the MAHs for the medicinal products concerned, make a scientific evaluation of the benefit-risk of the use of a medicinal product with a new therapeutic indication that concerns an unmet medical need.*

*The Agency shall draw up guidance on the consultation process.*

*The opinion of the Agency shall be made publicly available, and the competent authorities of the Member States and the MAH shall be informed.*

## ARTICLE 048

2. *In cases where the opinion is favourable and the new therapeutic indication addresses an unmet medical need, on the request of the Agency, the MAHs of the medicinal products concerned shall submit a variation to update the product information with the new therapeutic indication.*

# KEY POINTS

- Potentially one of the most relevant changes for academic oncology.
- This applies to **on-patent and off-patent drugs** – the originator companies were pushing to make this apply only to off-patent drugs
- **No strict definition of ‘unmet medical need’** – clearly if there is no specifically approved treatment for a disease there is an unmet need – but what if there is already a drug approved?
- Benefit/risk assessment is still ‘business as usual’ – there is **no change in the standard of evidence, and therefore weighted in favor of more common cancers**
- The MAHs will be instructed by the regulators to add the new indication to the label – **what if the company disagrees?**
- All focus now on the implementation phase

# ***OPEN IMPLEMENTATION QUESTIONS***

- Can the well-established use pathway still apply here?
- Which organisations will take on the task of creating the dossier to present to the regulators with data supporting a new indication?
- How will the regulators deal with the increased demands that arise from article 48?
- Will existing pricing mechanisms – which can penalise companies adding new indications to generic medicines – have to change?
- How will the companies react if they do not agree with the new indication?
- How will liability change if the regulators instructs companies?

# ***DIRECTIVE: MAIN CHANGES***

**The Directive focuses on  
how Member States organise  
access, supply  
& public health obligations**

## ***DIRECTIVE: MAIN CHANGES***

- Availability across Member States
- Supply obligations
- AMR measures
- Environmental sustainability
- Pharmacovigilance
- Transparency

**MOST PROVISIONS  
REQUIRE  
NATIONAL IMPLEMENTATION**

## ***KEY CONCERNS***

**UNPREDICTABILITY**

**COMPLEXITY**

**FRAMEWORK STILL  
BETTER SUITED FOR  
COMMON THAN  
RARE DISEASE**

**LIMITED IMPACT ON  
REAL ACCESS**

# ***KEY CONCERNS***

**Mancata soluzione  
all'accesso diseguale**

le procedure di pricing restano nazionali  
tempi di rimborso restano nazionali;  
i budget restano nazionali

**IL PACKAGE RISCHIA DI INCIDERE  
RELATIVAMENTE POCO SULLA VERA  
CAUSA DELLA DISUGUAGLIANZA**

WORKING *ALONGSIDE* WITH THE REGULATORY AGENCIES

# THANK YOU!

**Pan Pantziarka**

Anticancer Fund

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