



# The Pharmaceutical Package

A once in a generation reform

# A reminder: what's in the Pharmaceutical Package?



## THE PHARMACEUTICAL SECTOR

€37 billion contributed to research investment (in 2019)

800 000 direct jobs

€110 billion trade surplus

Each year more than 60 new medicines authorised at EU level

**Scientific excellence:** the European Medicines Agency and national medicines regulators have **thousands of experts from across Europe**, offering scientific advice of the highest quality

## WHERE WE ARE

More than 12 million people infected with COVID-19 in Europe

Increasing pharmaceutical budgets, already accounting for 20-30% of hospital expenditures

Medicines not always available or affordable

Still unmet needs

Antimicrobial resistance responsible for about 33 000 deaths yearly

95% of rare diseases are without treatment option

- More than 50 different legislative and non-legislative actions are proposed
- Review of:
  - The 2001 Pharmaceutical Directive (2001/83)
  - The 2004 EMA Regulation (746/2004)
  - The 2000 Orphan Drugs Regulation (141/2000)
  - The 2006 Paediatric Regulation (1901/2006)

**5%**

Only 5% had received a transformative treatment approved for the entire European Union, with 69% of rare disease patients having received only symptomatic treatment for their rare disease

**22%**

22% of people with rare diseases could not get the treatments they needed because it was not available where they live, reflective of the fragmentation of the market across the 27 Member States

# Why is it relevant to us

**1 160** centrally authorised medicines (CAPs) were authorised in the period 2005-2020 including over **200 OMPs**

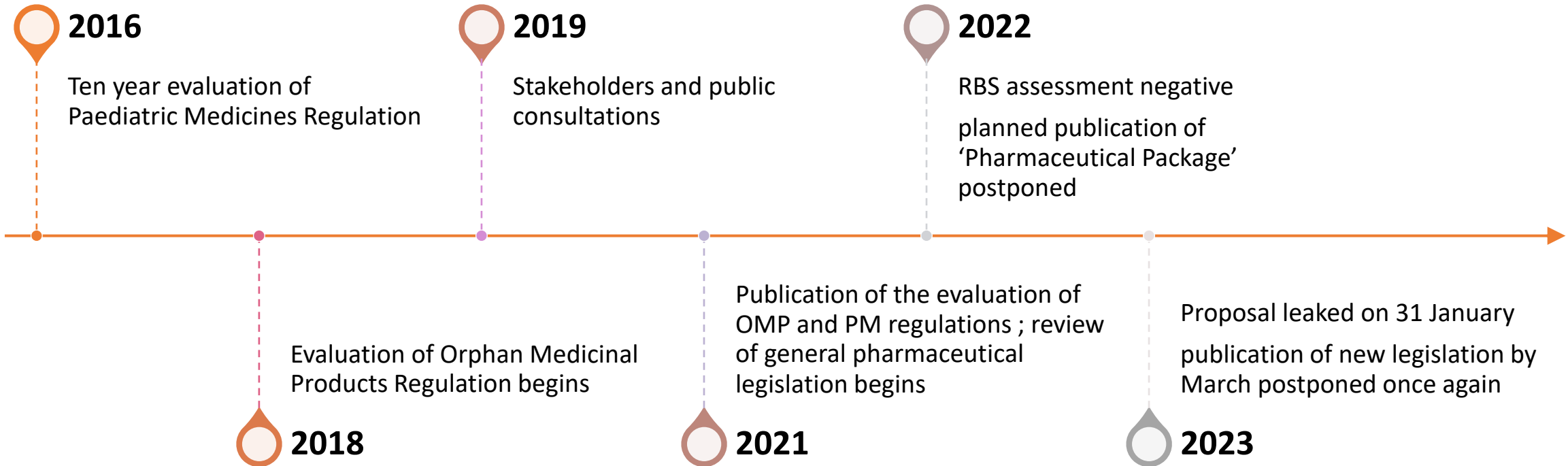
- Medicinal products developed by means of one of the following biotechnological processes:
  - recombinant nucleic acid technology;
  - controlled expression of genes coding for biologically active proteins in prokaryotes and eukaryotes including transformed mammalian cells.
- **Advanced therapy medicinal products**
- Medicinal products for human use containing an active substance which on 20 May 2004 was not authorised in the Union
- Medicinal products that are designated as **orphan medicinal products** pursuant to this Regulation.
- Medicinal products authorised in accordance with a **paediatric use marketing authorisation**.
- **Priority antimicrobials**

## 2- Delivering on 6 priority areas

**By 2030, EURORDIS will have made contributions to the goals of (Based on the Foresight Study Rare 2030):**

- Earlier, faster and more accurate **diagnosis** – goal of diagnosis within 6 months
- High-quality national and European **healthcare pathways**, including cross-border healthcare – a goal of improving survival by 3 years on average over 10 years and reducing the mortality of children under 5 years of age by one third
- **Integrated medical and social care** with a holistic life-long approach and inclusion in society – a goal of reducing the social, psychological and economic burden by one third
- **Research and knowledge development** that are innovative and led by the needs of people living with a rare disease
- Optimised **data and health digital technologies** for the benefit of people living with a rare disease and society at large
- Development and availability, accessibility, affordability of treatments, particularly transformative or curative **therapies** – goal of 1000 new therapies within 10 years

# Where are we now? A long road so far





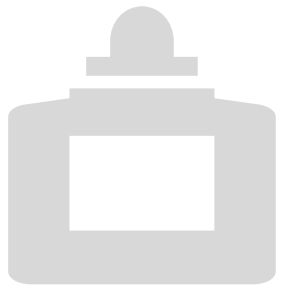
## What happened on 26 April?

- One 'chapeau' communication
- A new Directive and a new Regulation
- Council Recommendation on Antimicrobial Resistance

# Objectives of the proposed reform



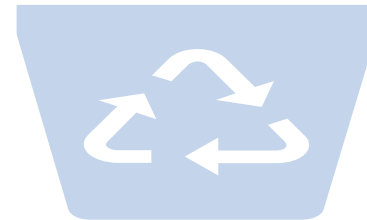
Make sure all patients across the EU have **timely and equitable access** to safe, effective, and affordable medicines.



Enhance **security of supply** and ensure medicines are always available to patients, regardless of where they live in the EU.



Offer an **attractive, innovation- and competitiveness friendly environment** for research, development, and production of medicines in Europe.



Make medicines more **environmentally** sustainable.



Address **antimicrobial resistance (AMR)** through a One Health approach, encompassing human health, animal health and the environment



# In a nutshell – key changes introduced

All in one regulation and one directive:

1. Laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing rules governing the European Medicines Agency ('The Regulation')
2. Union code relating to medicinal products for human use ('the Directive')



Introduction of the concept of Unmet Medical Need / High Unmet Medical Need



Modulation of incentives & conditionalities linked to access



Radical changes in the EMA structure, codification of PRIME & shortening assessment times



Patient representation at CHMP & consultation on guidelines



Addressing shortages, AMR and environmental impact

# Introduction of UMN / HUMN concept

- **Unmet Medical Need (UMN)** if at least one of its indications relates to a life threatening or severely debilitating condition and the following conditions are met:
- **No medicinal product** is authorised in the EU, or where an authorised medicinal product exists, it does not offer a satisfactory method of diagnosis, prevention or treatment of the disease because of remaining high morbidity or mortality in the relevant patient population, and
- The use of the medicinal product results in a **meaningful reduction** in disease morbidity or mortality for the relevant patient population.

***All rare diseases  
– orphan  
medicinal  
products are de  
facto deemed to  
be addressing an  
unmet medical  
need***

An orphan medicinal product shall be considered as addressing a **high unmet medical** need where it fulfils the following requirements:

- (a) there is no medicinal product authorised in the Union for such condition or where, despite medicinal products being authorised for such condition in the Union, the applicant demonstrates that the orphan medicinal product, **in addition to having a significant benefit, will bring exceptional therapeutic advancement;**
- (b) the use of the orphan medicinal product results in a **meaningful reduction** in disease morbidity or mortality for the relevant patient population.

# An effective incentives framework for innovation, access and addressing unmet medical needs

## General Pharmaceutical Legislation

- Minimum period of **regulatory protection** for innovative medicines will be 8 years, which includes 6 years of data protection and 2 years of market protection.
- Companies can benefit from additional periods of regulatory data protection if they **launch the medicine in all Member States (+2 years)** or if they develop a medicinal product addressing unmet medical needs (+6 months) or conduct comparative clinical trials (+6 months).
- An additional year of data protection can be granted for a new therapeutic indication.

## Paediatric Medicines

- These will also apply to **paediatric medicines**. In addition, medicines which have conducted the paediatric development plan agreed with EMA will continue to receive an extension of **6 months of their SPC**.
- **Rules on paediatric development plans will be adapted** to further stimulate research and development of medicines for diseases that affect only children.

## Orphan Medicinal Products

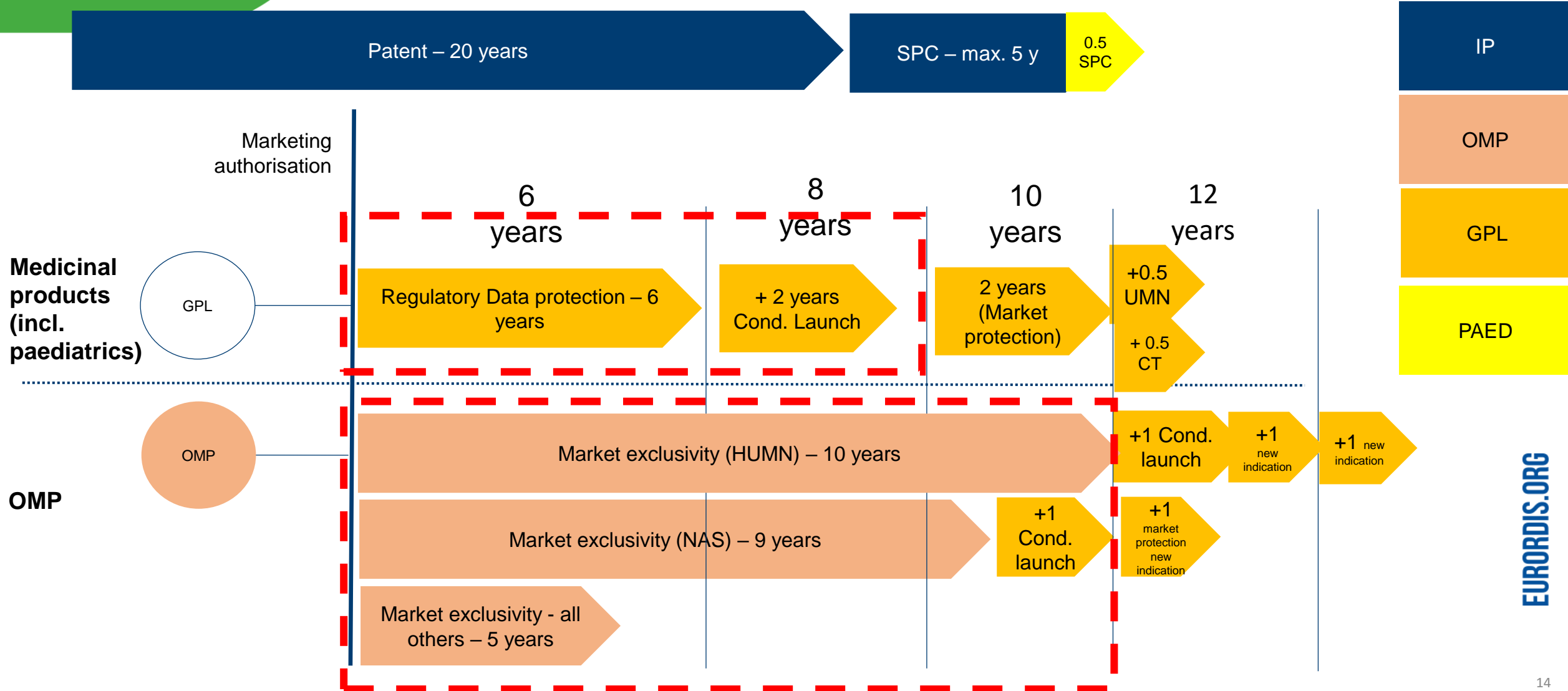
- The standard duration of market exclusivity for **orphan medicines** will be **9 years**.
- Companies can benefit from additional periods of market exclusivity if they address a **high unmet medical need (+1 year)**, **launch the medicine in all Member States (+1 year)**, or **develop new therapeutic indications for an already authorised orphan medicine (up to 2 extra years)**.

## Others

- New therapeutic uses of established medicines (**repurposing**) can benefit from a **four year data protection** period.
- **Non-profit entities** will be able to submit to EMA evidence supporting new therapeutic indications addressing unmet medical needs for already authorised medicines



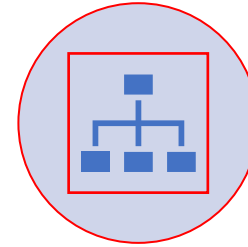
# What changes



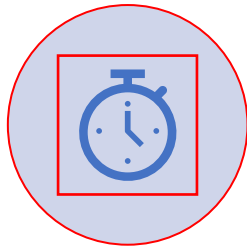
# Regulatory support and simplification measures to reduce regulatory burden



**Strengthening the early regulatory support by EMA, particularly for promising medicines under development for unmet medical needs.**



**Optimising EMA's structure (e.g. fewer scientific committees), with a focus on expertise and capacity-building within the network of competent authorities.**



**Reducing the assessment time by EMA from 210 days (in practice, on average 400 days) today to 180 days and the time for the Commission to authorise the medicine from 67 to 12 46 days.**



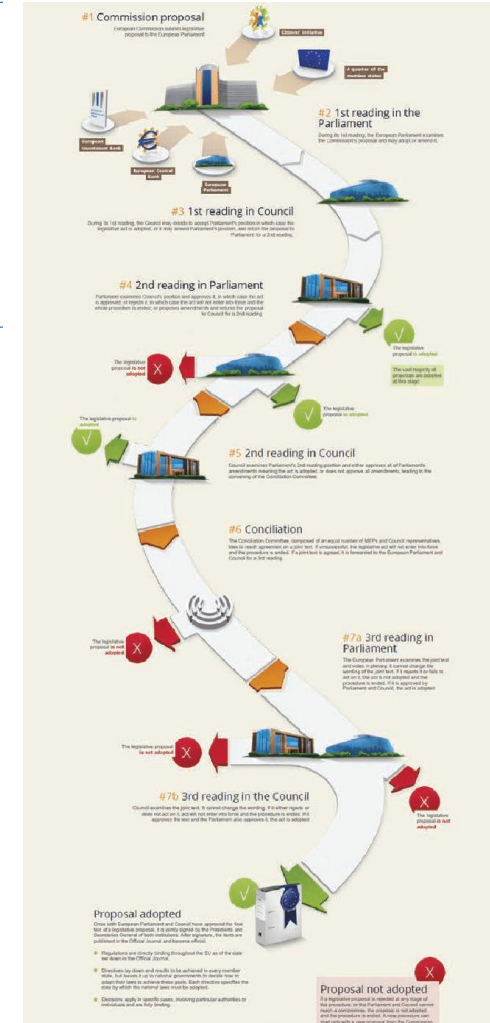
**Products addressing unmet medical needs and bringing major contributions to public health needs could benefit from an accelerated procedure and be assessed in 150 days.**



**Patient participation in CHMP extended but not to all process in a structured way**

# What's next

- **Ordinary legislative procedure** begins
- European Parliament will try to complete first reading by April 2023 (unlikely)
- Member States will take their time given other legislative files (EHDS, SoHO, EMA fees)
- Unlikely to have agreement before 2025 at the earliest
- EUROPEAN ELECTIONS!



# Key players in the European Parliament

## ENVI COMMITTEE

### Directive

- Rapporteur: Pernille Weiss (EPP, DK)
- Shadows:
  - Monika Benova (S&D, SK)
  - Veronique Trillet Lenoir (Renew, FR)



*ITRE and IMCO (and BUDG and CONT) Committees will also be involved in the drafting of the EP position*

### Regulation

- Rapporteur: Tiemo Wolken (S&D, DE)
- Shadows:
  - Tomislav Sokol (EPP, HR)
  - Frederique Ries (Renew, BE)



# It is time to engage at national level

Road to 2025



- Alignment on **core messages**, whilst respecting local realities and equity principle
- Identify **key decision makers** at national level
- Coordination **Brussels – capitals** is key
- **Sweden, Spain, Belgium, Hungary & Denmark** will have this large file on their agenda
- But none of you is excluded



- **Equity** principle for RD remains includes, as for the prevalence threshold
- Attention to **access** issues is given and **modulation** of incentives is included
- **Introduction of regulatory innovation** (e.g. codification of PRIME, rolling review, sandboxes etc.)

- Changes proposed are **not sufficient** to create a better ecosystem
- Many clarifications still needed including, on definitions of significant benefits, UNM / HUMN and on **patient engagement**
- **Access conditionalities** do not take into account reality at Member State level

***Not sufficiently ambitious to create a truly European single market for pharmaceuticals***

# Five overarching principles for a responsible evolution of the RD incentives framework



To **transform** the European Research & Development for the rare disease ecosystem building upon advances of the past 20 years, for the next 20 years



To situate **Europe as a global leader in research, development and access**, through a regulation that is attractive for developers, and competitive globally.



To define a **model that is centered on the unmet needs** of people living with a rare disease, and includes **patient participation** in its establishment and implementation.



To establish a **European pathway**, from development to access, to ensure innovation coupled with **affordability** and to gain that crucial **strategic autonomy** in research and development.



To ensure **convergence** and **coherence** between different relevant legislation

# Concrete proposals: to lead and show a new way



01. Maintain the prevalence threshold to leave no disease behind, while including an incidence threshold  
(incidence of less than 6 individuals per 100, 000 a year)



02. Encourage structured early dialogue in a multi-stakeholder format to address unmet needs at the right time point: a process rather than criteria



03. Introduce an "Orphan Drug Development Plan" to guide the development of new treatments with the continuous input of experts



04. A modulation of incentives, rewarding earliest dialogue and favouring areas with no therapeutic options



# Concrete proposals for a responsible evolution of the RD incentives framework: to lead and show a new way



o5. Maintain Market Exclusivity as an incentive, to ensure global competitiveness



o6. Conditional significant benefit until the conversion into full Marketing Authorisation



o7. Strengthen the responsibilities and functioning of the Committee for Orphan Medicinal Products (COMP), while reporting to CHMP



o8. Include PRIME within the revision of the OMP Regulation or within the wider Pharmaceutical Package as it applies beyond rare diseases



# With a focused narrative



## HUG

- **The European Commission is proposing measures that many rare disease patients and their families will welcome**, from more rapid regulatory pathways for new products to more targeted incentives for companies to develop desperately needed medicines



## PIVOT

- Europe has lost major ground to its global competitors over the past couple of decades. Despite a favourable regulatory and incentives framework, **the market remains fragmented and there is a lack of coordinated research infrastructure, investment and access.**



## BEAR

- We need a **structured and seamless pathway for medicines**, from early dialogue, to scientific advice, to marketing approval, to clinical assessment, to health technology assessment, and ultimately to European-level negotiation and procurement

## Next steps

- Draft response to public consultation (draft to be shared 1<sup>st</sup> week of June)
  - Basis of both narrative &
- Collate feedback from our community (e.g. 25 May CNA meeting, but also TAG, DITA, etc.)
- RareOnAir
- Webinar
- Targeted approach to EP

**Change can be scary, but we  
need change**