The EU Pharmaceutical Reform - orphan and paediatric medicines

EURORDIS Open Academy
29 September 2023

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European Commission

#HealthUnion

EU Pharmaceutical Reform

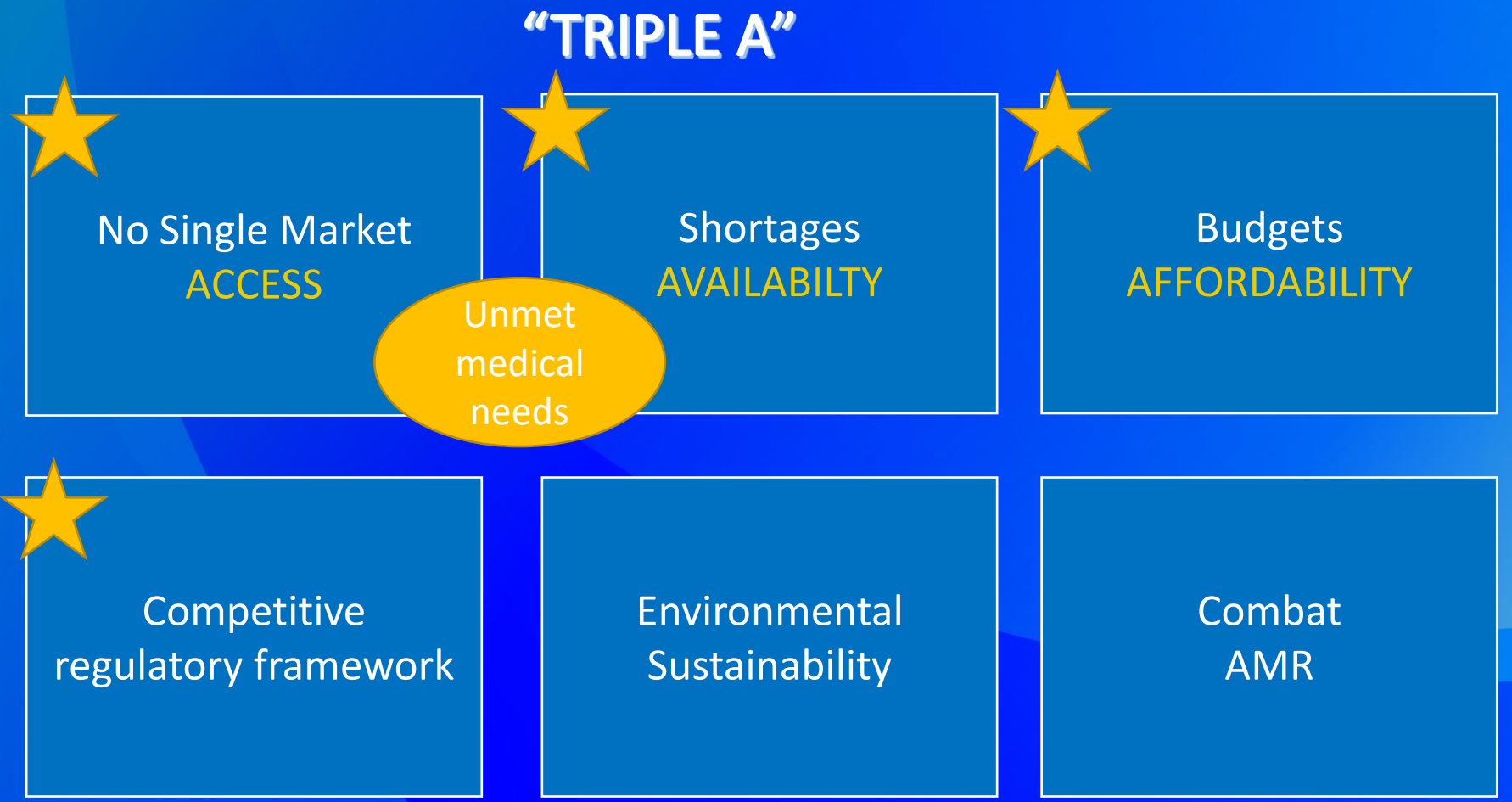
Builds on the **Pharmaceutical Strategy** for Europe (2020)

Supports EU citizens and industry

Addresses **long-standing challenges and public emergencies**

Marks a **European Health Union milestone**

6 Key political objectives

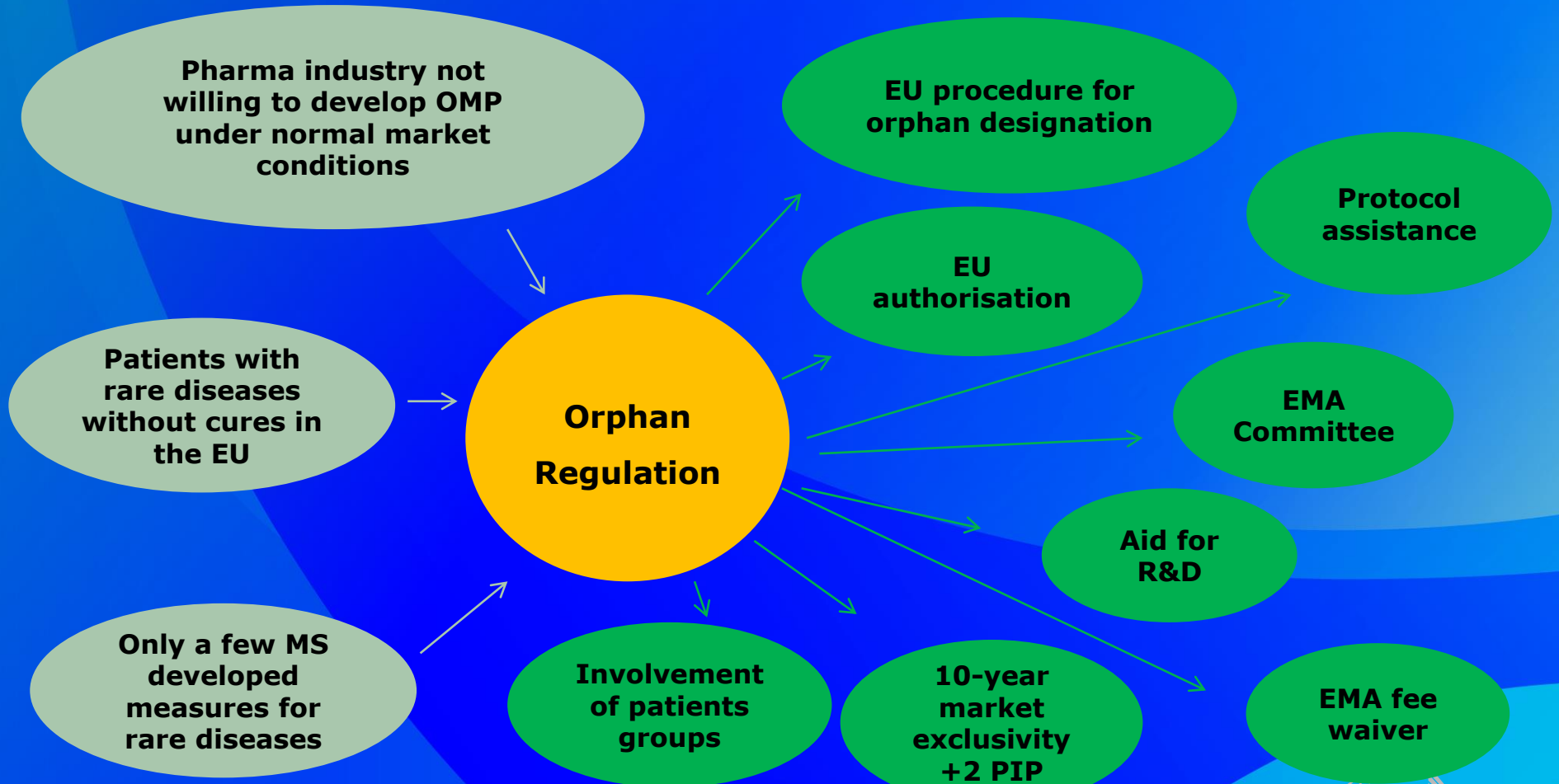


Single market of medicines in the EU

Orphan medicines

Problems

Tools



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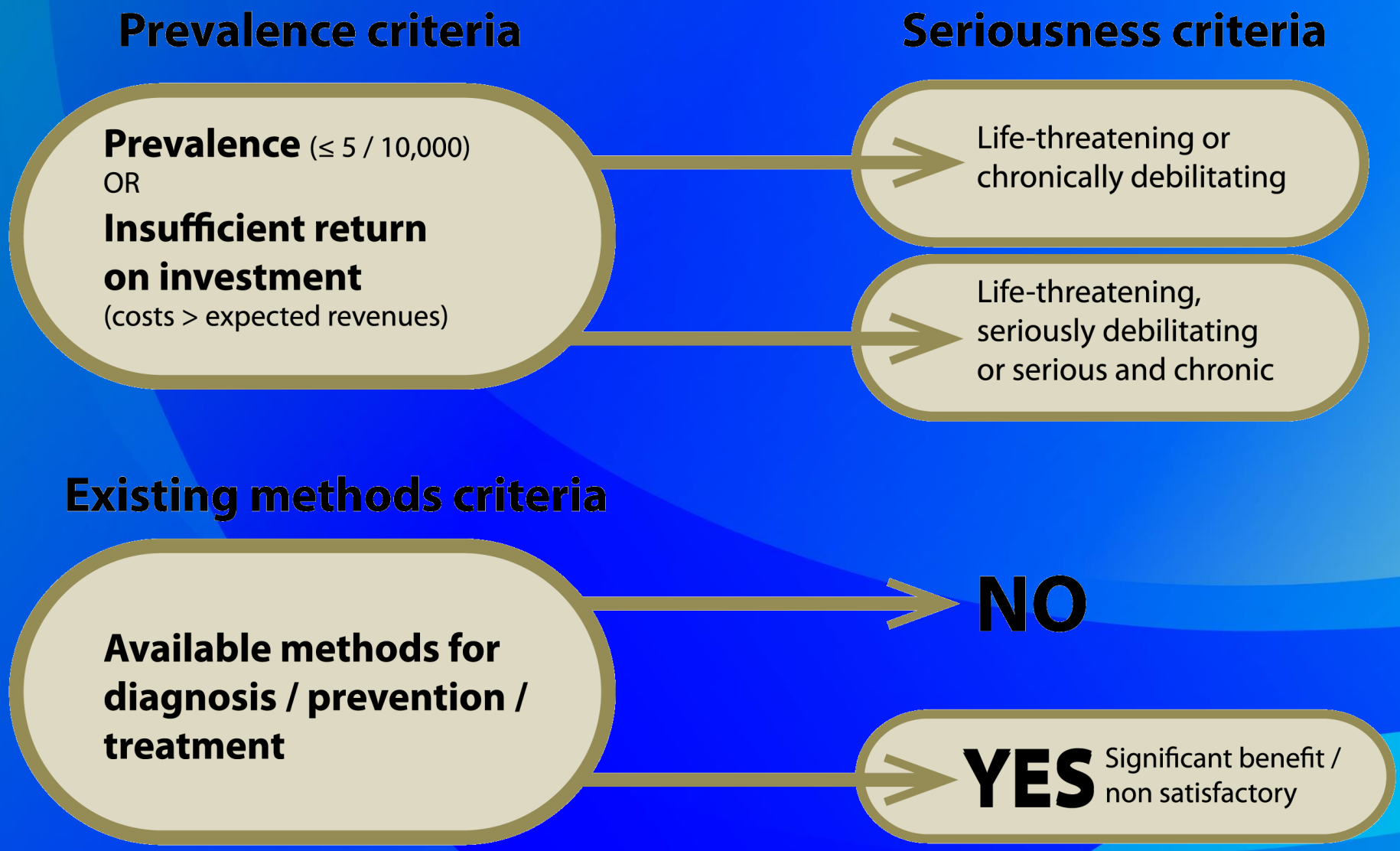

Prevalence criteria
Seriousness criteria
Prevalence (≤ 5 / 10,000)
OR
Insufficient return on investment
(costs > expected revenues)
Life-threatening or chronically debilitating
Life-threatening, seriously debilitating or serious and chronic
Existing methods criteria
Available methods for diagnosis / prevention / treatment
NO
YES
Significant benefit / non satisfactory

Evaluation results

- More orphan medicines, available faster and to more Member States
- Development for the rarest diseases
- Increased use of orphan incentives = important for the development of orphan products
- Impossible to achieve similar results without EU level action

Summary of problems found in evaluation

- Insufficient development in areas of greatest unmet medical needs
  - ➢95 % rare diseases no treatment option
  - ➢‘One-size-fits-all’ incentives and rewards <-> unmet needs
- Availability and accessibility varies across MS
  - ➢No link between incentive and placing on market (orphans)
  - ➢Limited generic competition after expiry of exclusivity periods
- Scientific and technological developments cannot be fully exploited
  - ➢Instruments in legislation not adequate for advances in science: biomarkers and personalised medicine
- Certain procedures inefficient and burdensome

Specific objectives for orphans

- **Promote innovation** for rare diseases in particular in areas of **high unmet medical need**
- Create a **more balanced system** for pharmaceuticals in the EU that promotes affordability for health systems while rewarding innovation
- Ensure timely **patient access** to orphan medicines in all Member States
- Reduce the regulatory burden and provide a flexible regulatory framework

Addressing UMN of rare disease patients

Orphan criteria at designation stage

A life-threatening or chronically debilitating condition:

(a) affecting not more than five in 10 000 persons in the Union;

(b) there exists no satisfactory method authorised in the Union or, where such method exists, that the medicinal product would be of **significant benefit** to those affected by that condition.

What is new?

- No insufficient return on investment criterion
- If prevalence not possible – other criteria set for certain conditions
- Commission Notice on significant benefit encoded in an Implementing Regulation

High unmet medical needs

only medicines for rare diseases (orphan medicines)

Established use products excluded

Indication criterion: therapeutic indication must relate to a *life threatening* [OR] *chronically debilitating condition* – criterion of the definition of the orphan medicinal product

Comparison to authorised medicines:

- *No medicine is authorised* in the EU

[OR]

- A medicine *is authorised* in the EU but it will bring **exceptional therapeutic advancement (more than 'significant benefit')**

Effect criterion: Use of the medicine results in *meaningful reduction in disease morbidity / mortality* for the relevant patient population

EMA to set *scientific guidelines* for the application of the article + consultation process of downstream actors and stakeholders (HTA/P&R bodies (possibility to include patients, industry, others).

Addressing UMN of rare disease patients and access

Market exclusivity

(a) 9 years for orphan medicinal products other than those referred to in points (b) and (c);

(b) 10 years for orphan medicinal products addressing a high unmet medical need

(c) 5 years for orphan medicinal products which have been authorised in accordance with Article 13 of revised Directive 2001/83/EC (well established use).

What is new?

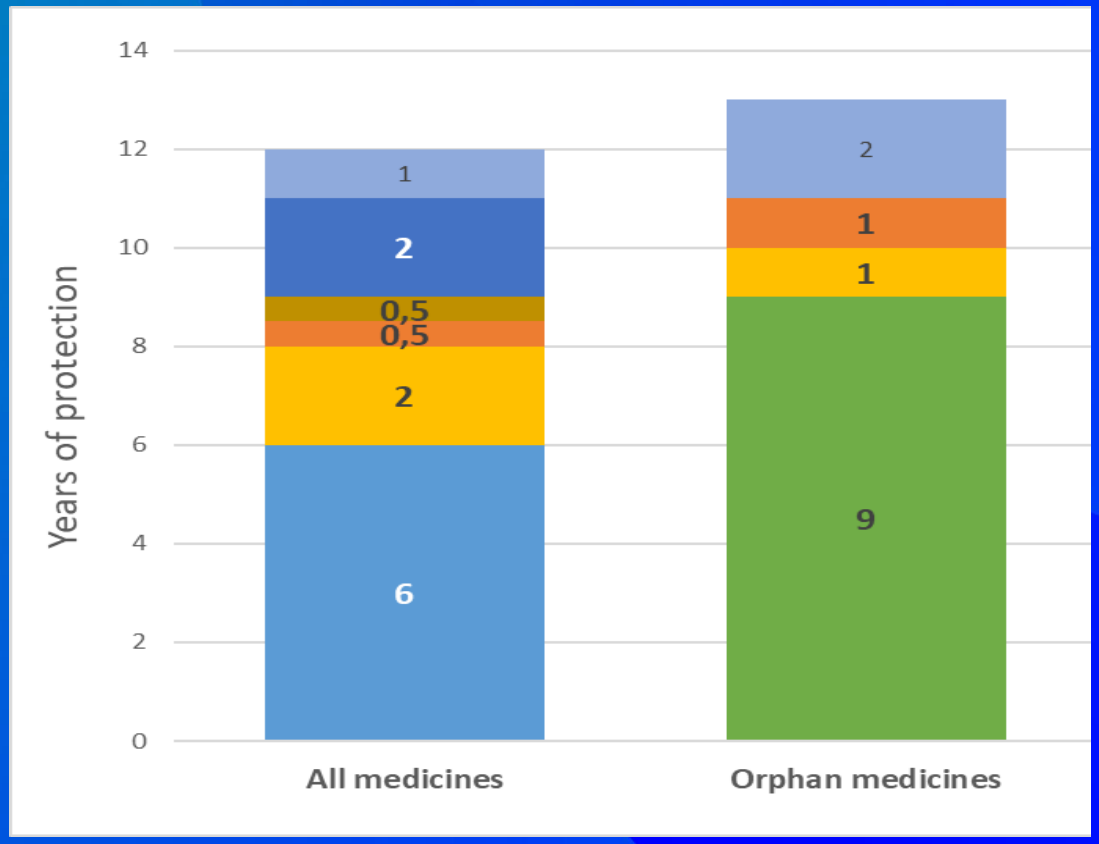
No one-size-fits-all incentive

Where a MAH holds more than one orphan marketing authorisation for the same active substance, those authorisations shall not benefit from separate market exclusivity periods

Modulation of incentives- proposed changes for medicines for rare diseases (orphan medicines)

Modulation of data protection

Modulation of market exclusivity



max 12 years protection

max 13 years protection for orphan medicines

A streamlined regulatory framework

- EMA granting designations
- EMA structure simplified
- Pre-authorisation support for promising medicines
- Faster EMA assessment and EC authorisation

Affordability

Current challenges:

- Pricing, reimbursement and procurement of medicines is a **national** competence
- High prices endanger national health systems' sustainability **& restrict patient access**
- Lack of **transparency of public funding** is a growing issue
- Lack **of streamlined coordination** among national authorities

Proposed solutions:

- **Earlier market entry of generics/biosimilars** to increase competition and reduce prices
- Increased **transparency on public contribution** to R&D
- Comparative **Clinical Trials** to support national decisions on pricing
- Further support for **information exchange** between Member States (cooperation on pricing, reimbursement and payment policies)

Paediatric medicines

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The Paediatric Regulation

Results achieved:

- More clinical trials involving children;
- More and more trails and paediatric clinical developments completed;

- More medicines authorised for children or containing information on their use in children.

The Paediatric Regulation (since 2006)

- Main instrument: obligation for pharmaceutical companies study all products in children (waivers possible) following a clinical development plan (PIP) agreed with EMA

- Rewards.

Evaluation of the Paediatric Regulation

- PIP procedure adequate in all situations?
  - Waivers - products which may be useful for children?
  - Long deferrals.
- Development steered by adult needs;
- Some therapeutic areas still limited development;
- Differential availability in the various EU MS;
- Rewards, complex to obtain.

Revision of the paediatric provisions

- Obligations to agree and conduct paediatric clinical studies (PIP) – rewards structure maintained;
- Step-wise and adapted PIP;
- Mandatory PIP on the base of the mechanism of action of a MP (same therapeutic area);
- Cap to the length of deferrals (extendible).

Revision of the paediatric provisions

- Possibility for NGO to submit data for repurposing of medicine;
- 6 months SPC extension following PIP completion also for orphan medicines;
- EMA reorganization.

Revision of the paediatric provisions

- Increased transparency on PIP conducted for discontinued medicines;
- Multi-stakeholders discussions about prioritisation of paediatric R&D in a pre-competitive environment.

Thank you for your attention

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