The EU Pharmaceutical reform for medicines for rare diseases. Unmet medical needs

Rare Diseases in the EU: Joint Action shaping the future of ERNs; JARDIN kick-off meeting
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A 4-part package – 26 April 2023

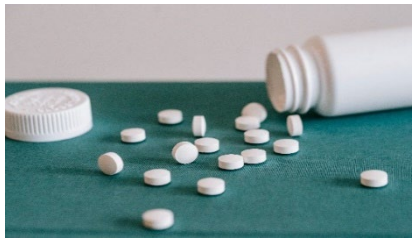
Chapeau communication

New Regulation

- Specific rules for the most innovative medicines such as orphans, antimicrobials
- Rules on shortages and security of supply
- EMA governance

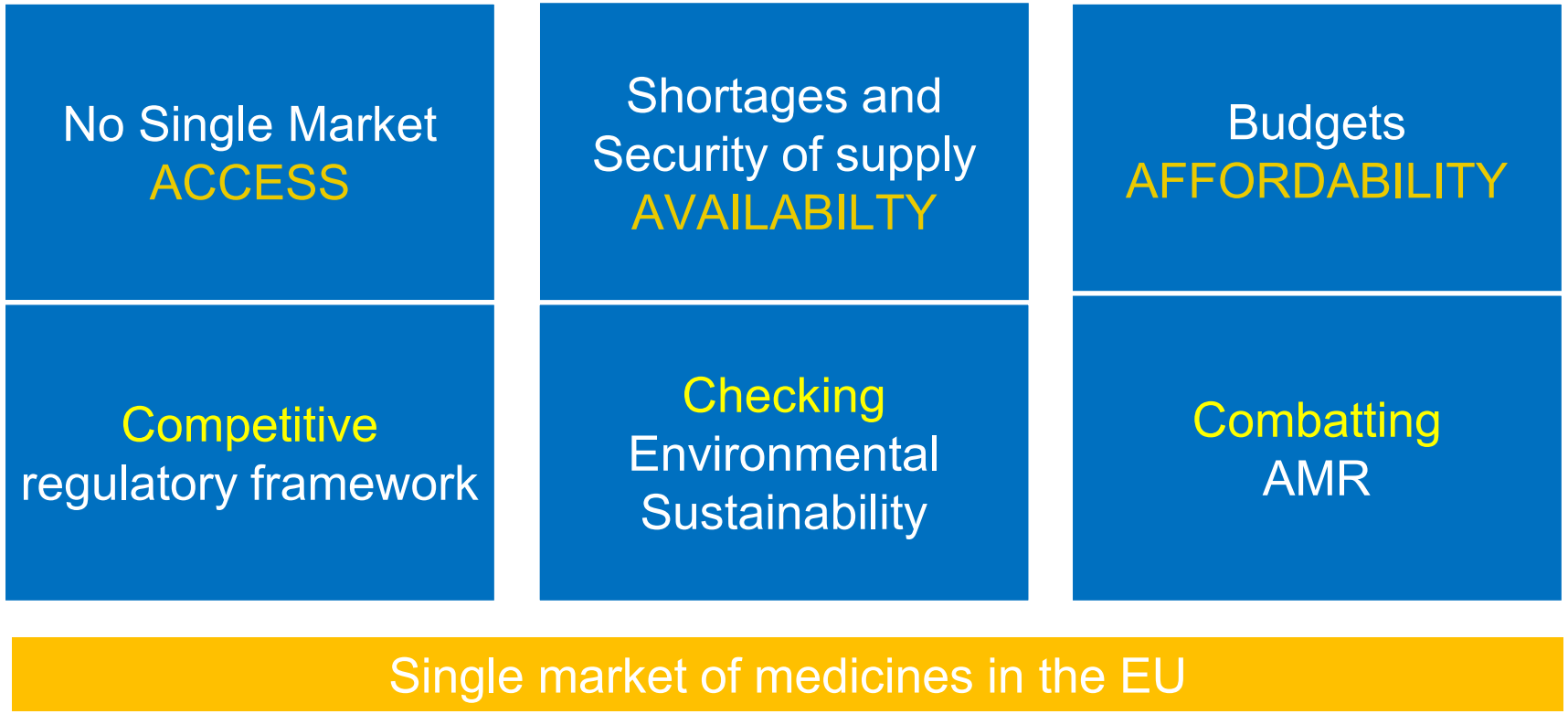
New Directive

- Placing on the market of all medicines
- Authorisation and labelling requirements
- Strong incentives for access



Council Recommendation on AMR

6 Key political objectives



Objectives of revision for orphan medicines

- 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

Unmet medical needs

ALL ORPHANS address Unmet Medical Needs

Indication criterion: Therapeutic indication must relate to a *life threatening [*OR] *severely debilitating* condition

Comparison to authorised medicines

- No medicine is authorised in the EU

[OR]

- A medicine is authorised in the EU but disease is associated with remaining high morbidity / mortality

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).

European Commission

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*

Comparison to authorised medicines:

- *No medicine is authorised* in the EU

[OR]

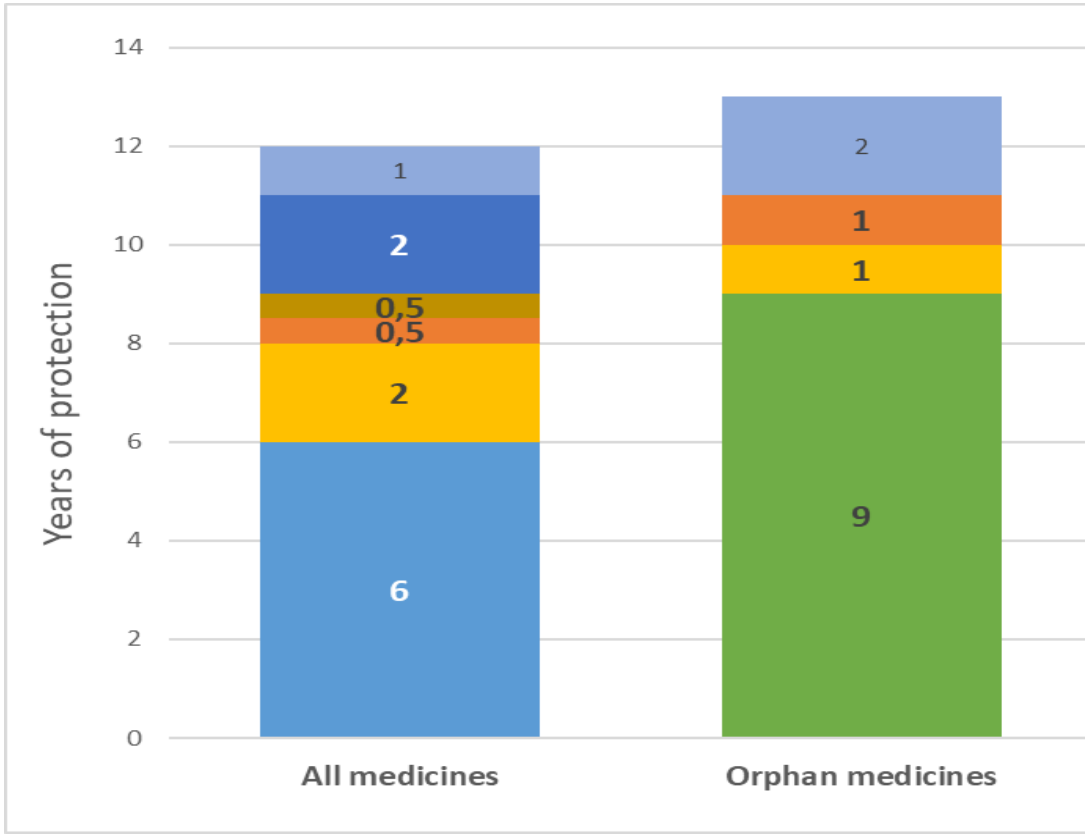
- A medicine *is authorised* in the EU but it will bring exceptional therapeutic advancement (more than 'significant benefit')

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).

European Commission

Modulation of incentives - proposed changes for medicines for rare diseases



12 years max

13 years max

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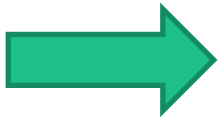
CRITERIA FOR ORPHAN PRODUCTS

- *Currently:* **prevalence** (the number of persons affected by a condition at a specified instant in time in the EU – not more than 5 in 10,000 persons);
- ***Change:*** Possibility of setting specific criteria for certain conditions by a delegated act on a recommendation of EMA => may allow for **incidence** criterion



Flexible regulatory framework

- **Deleting the "sufficient return on investment" criterion**

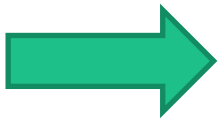
- **No review of the criteria for market exclusivity**, incl. compliance with orphan criteria, after six years from granting of marketing authorisation

ORPHAN DESIGNATION PROCEDURE

- *Currently:* **designation as an orphan product and the Register** under the responsibility of the **COM**
- ***Change****:* all under responsibility of **EMA**

- *Currently:* **no cap** of duration of designation => overpopulating of Register, unclear picture of orphans under development in the EU, may be unclear for competitors

- ***Change****:* **7-year cap** on designation; after which => removal from the Register; loss of benefits linked to the designated orphan status



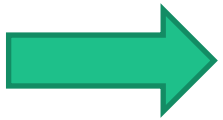
Reduction of regulatory burden by procedural improvements

GLOBAL MARKET EXCLUSIVITY + prolongation for new indications

- *Currently:* each new indication benefits from a new period of market exclusivity => **evergreening** (10 years + 10 years + ….of market exclusivity (no similar products can be granted marketing authorisation, unless proved clinical superiority/consent/insufficient supply)

- ***Change**:* **No separate market exclusivity** when a marketing authorisation holder holds more than one orphan marketing authorisation for the same active substance

- ***Change:*** **Prolongation of ME for new indications:** The 9 (default) or 10 (HUMN) years' ME may be extended by 12 months twice, if new indication(s) for a different orphan condition.



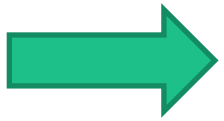
Faster entry similar products, incl. of generics/biosimilars

Faster entry of generics

Currently: no marketing authorisation application can be accepted for a similar medicinal product during the 10 years ME of the original orphan medicine

Change 1: Assessment of an application for a marketing authorization of a similar product possible if the remainder of market exclusivity of the reference product less than 2 years, which allows entry on the market at day-1 after expiration of ME

Change 2: Market exclusivity of a similar product to the reference medicinal product (for which ME expired) will not prevent granting an authorization to the generic/biosimilar to this reference product



Faster entry of similar products, including generics/biosimilars

Thank you

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