Pharma reform medicines for children

Enpr-EMA

Amsterdam, 10 October 2023

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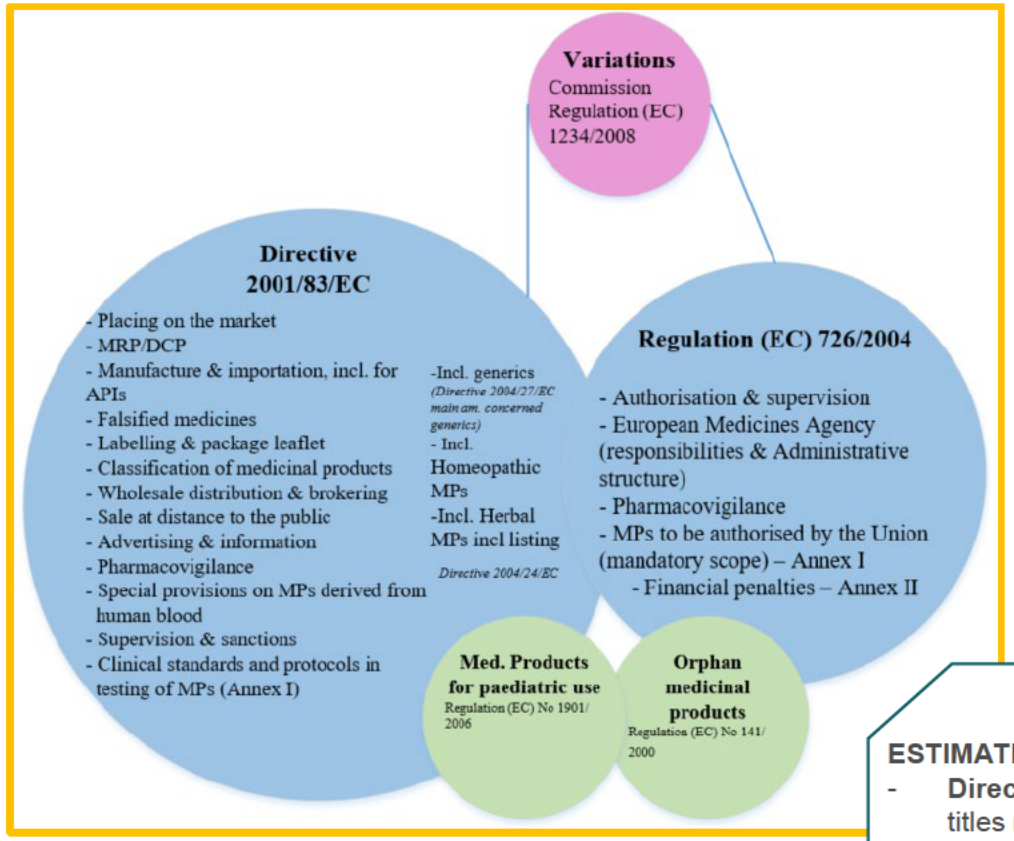
European Commission – DG SANTE

Structure of the revision



ESTIMATION

- **Directive 2001/83**: 13/14 titles revised – about 60-70% of 191 articles concerned.
- **Regulation 726/2004**: 4 titles revised – about 70% of the more than 65 articles concerned.

Areas not changed in substance

- Homeopathic medicines
- Herbal medicines (exception: herbal committee)
- Falsified medicines
- Sale at distance to the public

Areas with minimum intervention

- Pharmacovigilance
- Wholesale distribution
- Advertising
- Clinical standards and protocols in testing

European Commission

A 4-part package

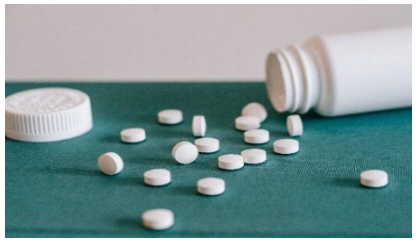
Chapeau communication

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

58 years of EU pharmaceuticals regulation

SAFETY – EFFICACY - QUALITY

Thalidomide disaster exemplifies the need for EVIDENCE-BASED AUTHORISATION

1965
1st EC legislation: medicines need to be authorised before being placed on the market

1995
Centralised, EU-wide procedure for authorisation – creation of the EMA

2004
Last major revision – extending scope of centralised procedure, simplification

2007
Regulation on advanced therapy medicines

2006
Legislation on medicines for children

2002
Legislation on medicines for rare diseases

2010
New EU Pharmacovigilance rules: better prevention, detection and assessment of adverse reactions, direct patient reporting of adverse events

2011
Legislation against falsified medicines

2020
Pharmaceutical strategy for Europe: addresses long standing challenges, learnings from COVID-19

2023
Revision of general pharmaceutical acts packaged with revision of the O/P legislation

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Responsibilities shared between EU and Member States

- Centralised authorisation procedure
- Inspections of manufacturing sites
- Pharmacovigiliance
- …..

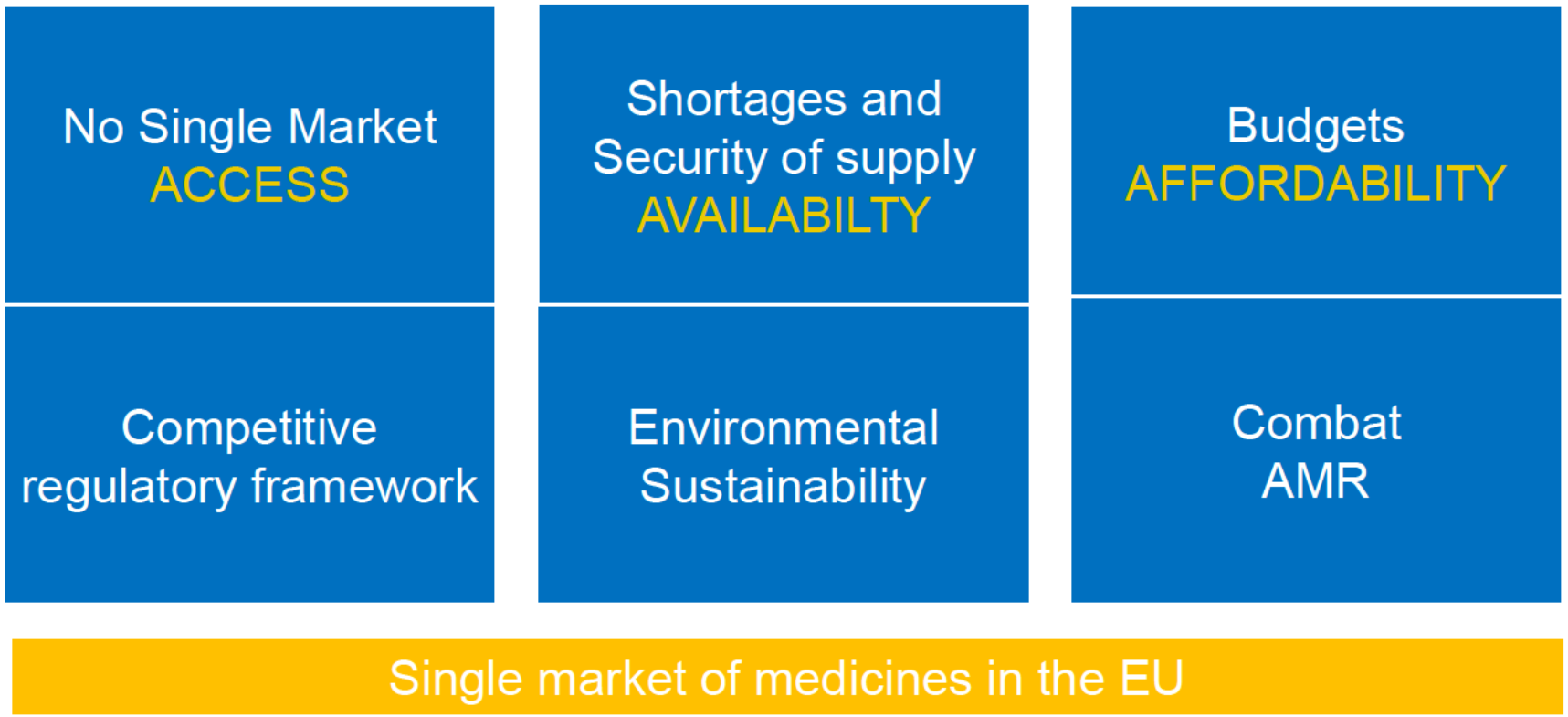
EMA and network of National Competent Authorities

By EU-level standards

- Decentralised procedure and mutual recognition procedure to authorise medicines in MS
- Organisation and delivery of health services and medical care
- P&R for medicinal products or their inclusion in the scope of national health insurance schemes

Strictly MS competence!

6 Key political objectives



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Revision of the paediatric provisions

- Obligations – rewards structure maintained;
- Submissions before the initiation of safety and efficacy studies;
- Step-wise PIP;
- Adapted key binding elements for step wise PIP, paediatric only products, PUMA.

Revision of the paediatric provisions

- Mandatory PIP on the base of the mechanism of action of a MP (same therapeutic area);
- Temporary waiver from PIP obligation during public health emergencies for medicines relevant for the public health emergency;
- Cap to the length of deferrals.

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.

Medicines for rare diseases

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

Medicines for rare diseases

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

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).

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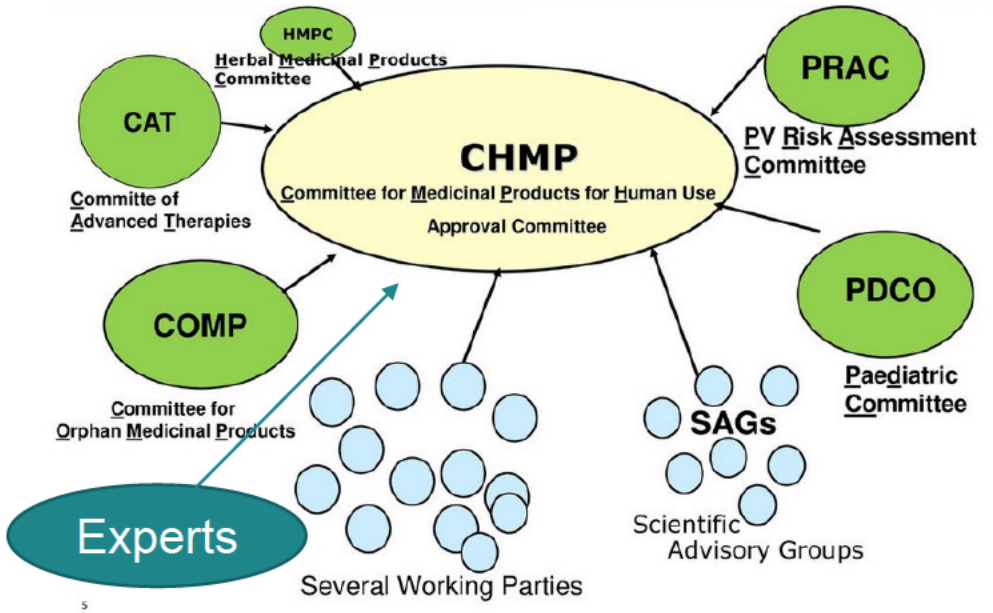
Medicines for rare diseases

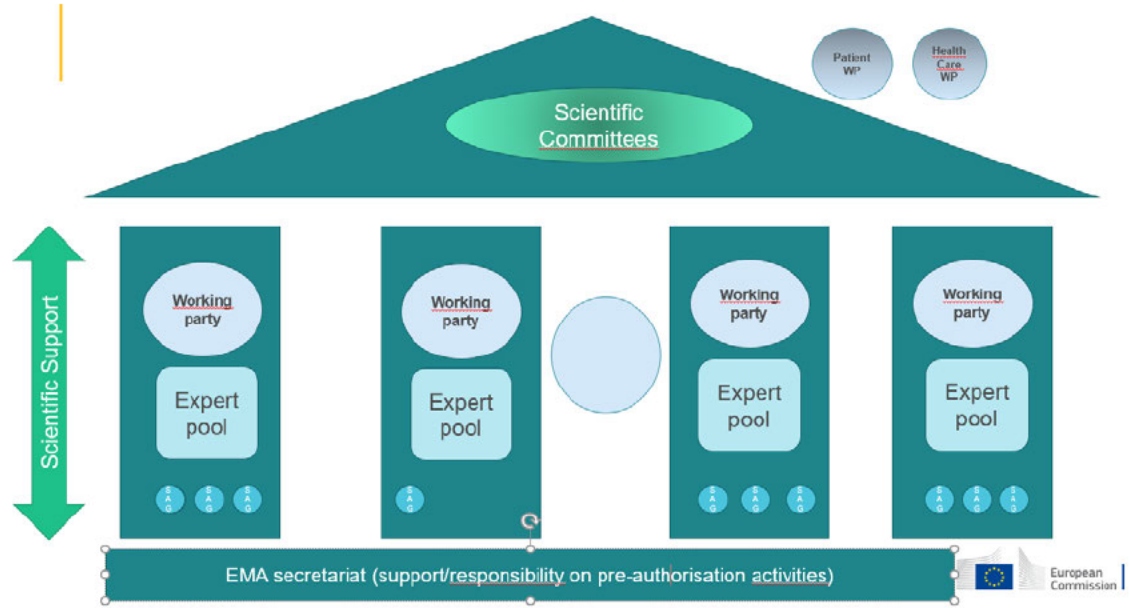
- EMA granting designations
- EMA structure simplified
- Pre-authorisation support for promising medicines
- Faster EMA assessment and EC authorisation

EMA structure – today and tomorrow

Today



Tomorrow



Principles maintained in the future structure	
Full MS representation in EMA committees	Maintained
Rapporteurship	Maintained (no impact on fees)

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EMA structure –tomorrow

Working parties on:

- Quality;
- Methodology;
- non- clinical;
- Clinical;
- Scientific advise;
- ERA;
- Other wp possible.

Composition:

- High level of scientific expertise;
- Majority of members from MS authorities;
- MS not represented can attend the meetings as observers.

Scientific experts

Next steps

- Council:
  - Start of the „reading“ expected under the Spanish Presidency
- European Parliament
- European Economic and Social Committee

Thank you

European Commission