The New EU Pharmaceutical Strategy Implications for all stakeholders

CRT EMA Workshop "Unmet Medical Needs"

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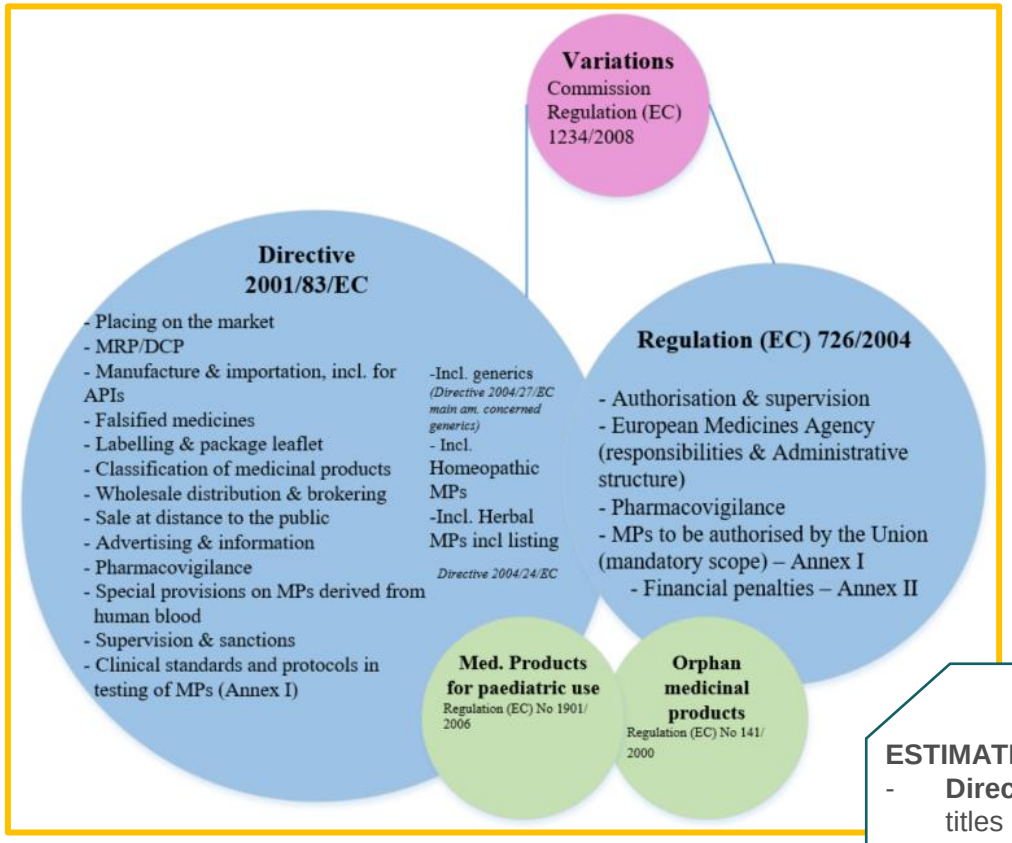
Structure of the pharmaceutical revision



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-parts package – April 2023

Chapeau communication

New Regulation

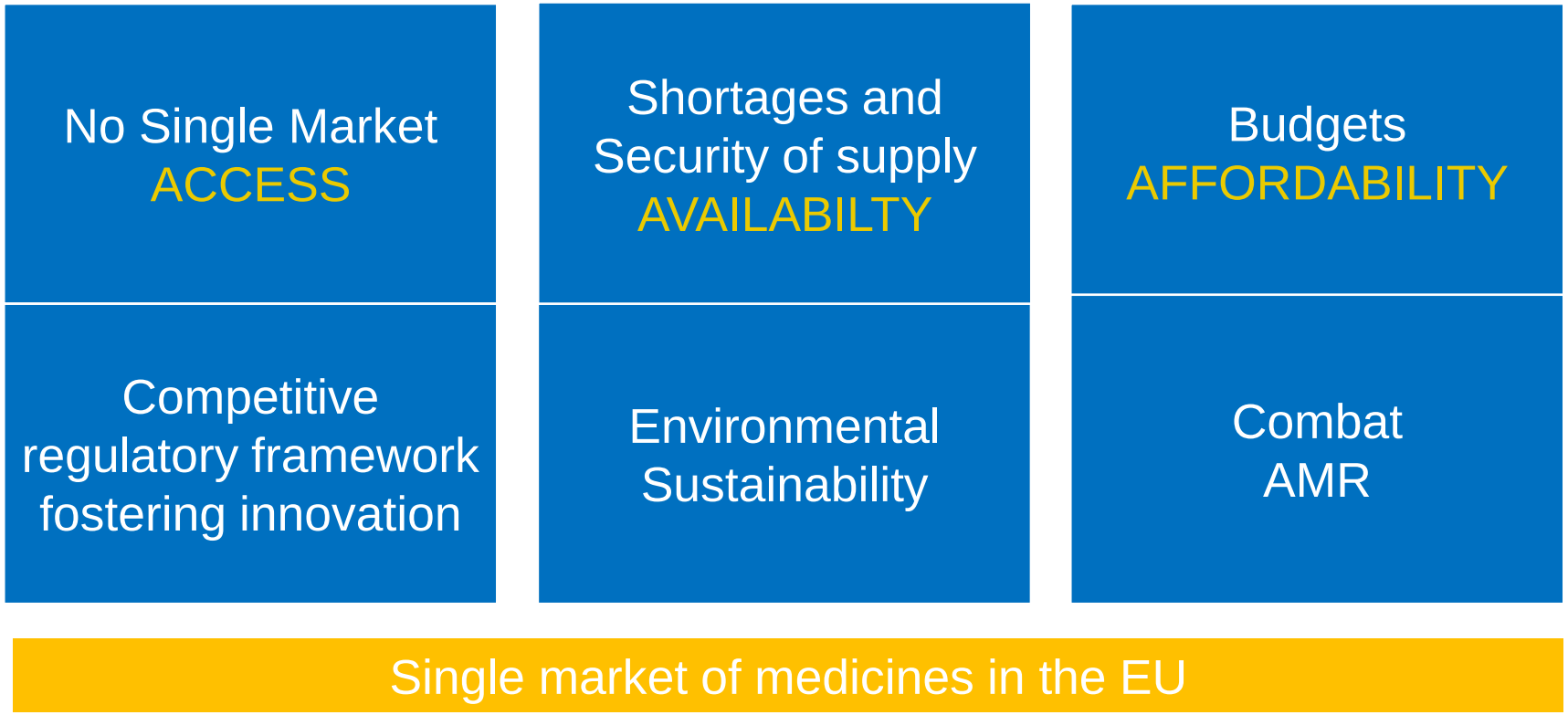
- Specific rules for the most innovative medicines such as orphans, antimicrobials
- Rules on shortages and security of supply
- EMA governance

New Directive

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

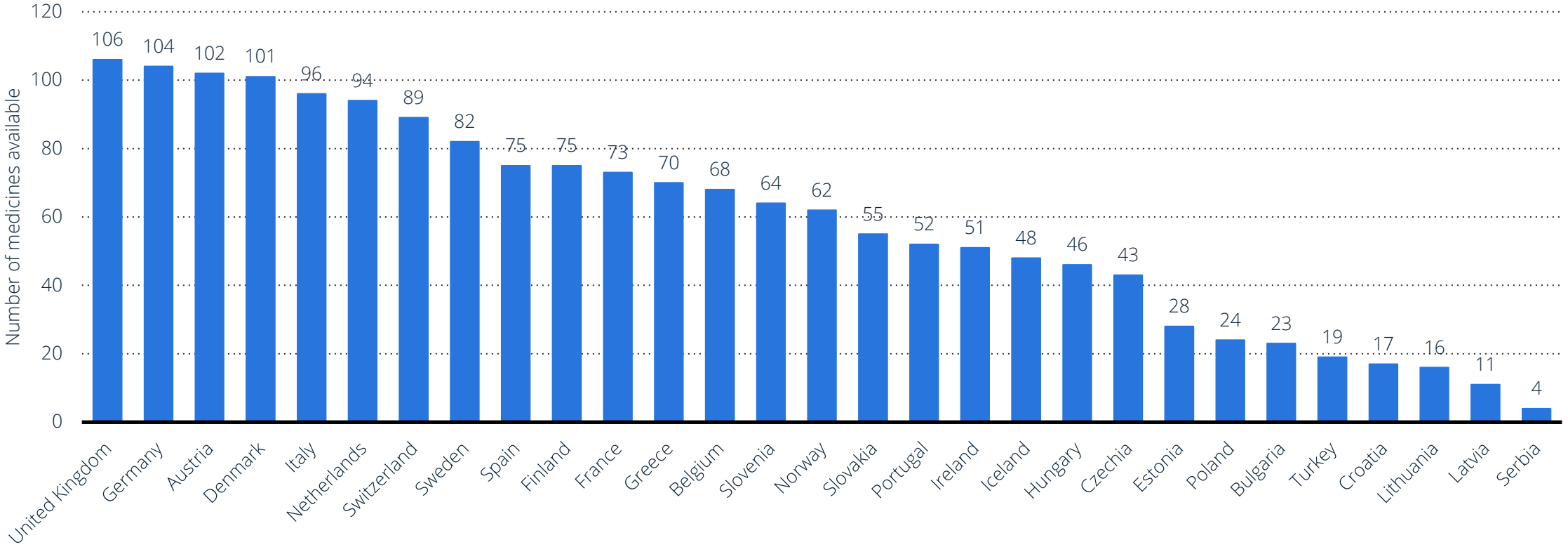
Council Recommendation on AMR

6 Key political objectives



Access to medicines

Number of medicines approved by the EMA between 2015-17 available to patients in Europe as of 2018, by country



Note(s): Europe; 2017

Further information regarding this statistic can be found on page 8.

Source(s): IQVIA; ID 1011132

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Access to medicines

Current challenges:

Access is not timely and differs across Member States:

90% variance between Northern and Western European countries and Southern and Eastern European countries

Average waiting time across the EU is from 4 months to 29 months

Proposed solutions:

Incentives for innovation and access:

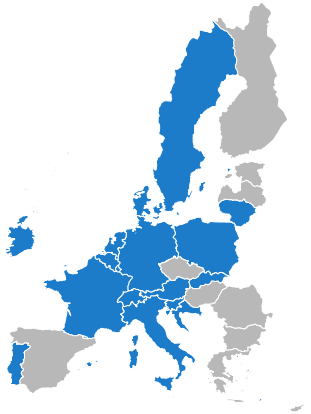
Targeted approach vs current “one-size-fits-all” unconditional data protection and market exclusivity (for orphans)

Earlier market entry of generic and biosimilar medicines

- Faster authorisation
- Pre-authorisation support

Market launch conditions

- Launch in all Member States where the marketing authorisation is valid (CP and DCP)



- **Actual placing** on the market and continuous supply for the needs of the patients in each MS (incl. presentations, quantities)
- **MS has 4+1 options**:
  - Positive/negative confirmation of actual supply;
  - Waiver;
  - Tacit;
  - [or] positive pricing and reimbursement decisions (based on Transparency Directive)

Availability – shortages and security of supply

Shortages: Multiple root causes

Quality and manufacturing issues

Commercial reasons, incl. market withdrawals, and unexpected increases in demand

EU dependency on non-EU countries for medicines for supply of certain pharmaceutical ingredients.

Current challenges

Growing concern for **all EU countries**

- **Critical shortages** of medicines; current examples thrombolytics, antibiotics
- Security of supply of **critical medicines**

Ad hoc processes for dealing with **critical shortages**

Proposed solutions

Improved **coordination, monitoring and management** of shortages, in particular critical shortages (MS and EMA); **Earlier** and **harmonised notification** of shortages and withdrawals (industry)

Shortage Prevention Plans

Union list of critical medicines

Stronger coordinating role for **EMA &** more powers for **MS** and **Commission**

Outside pharma package

- Other **Commission initiatives**, including the work of **HERA**
- **Joint Action** on shortages
- **IPCEI** in the area of health
- **National measures** e.g. State aid
- **EMA mandate extension** (Regulation (EU) 2022/123)

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)

How will the proposal foster innovation?

Incentivizing innovation by future proofing legislation

- Regulatory **sandbox** to test new innovative therapies
  - Not possible to develop the product/technology in compliance to the leg. requirements due to its characteristics, AND
  - These characteristics contribute to its safety, efficacy/ major therapeutic advantage patient treatment.
  - EMA issue recommendation, Comm set up
  - It is a temporary tool, derogations from legislation, sandbox plan
  - Only for products at early phases dvlp
  - MA limited to the duration of the sandbox

Incentivizing innovation by future proofing legislation

- **Adapted frameworks** with specific regulatory requirements tailored to the characteristics of certain novel medicines
  - For products listed in Annex (phages)
  - Similar condition than sandboxes
  - It is “permanent”
  - Derogations limited to what strictly necessary

Incentivizing innovation by future proofing legislation

- Scientific advice by EMA for UMN products
  - When needed, consultation of other authorities/bodies
- PRIME
  - UMN
  - HUMN orphans
  - Major interest public health
  - Antimicrobial (new class, new MoA, new active adressing multidrug resistant organisms)
  - In case of cross borders health threats
- Parallel scientific advice
  - Scientific advice at the same time as joint scientific consultations by MS coordination group on HTA
  - Possible and with medical devices experts.

Incentivizing innovation by future proofing legislation

- Possibility for EMA to review **data in phases**, as they become available (rolling or phased review)
  - Exceptional therapeutic advantages
  - Following advice by ChMP

Incentivizing innovation by future proofing legislation

- Introduction of possibility for a scientific recommendation decision on **regulatory status** of a medicinal product under development
- Facilitate use of **real-world evidence**, and of **health data** for regulatory purposes
- Stepwise PIP

Unmet medical needs

All rare diseases-orphan medicines automatically considered UMN

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

Next steps

- Council:
- European Parliament

Thank you

Slide 4 picture source: Getty images

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