The EU Pharmaceutical Reform

CoR' Interregional Group on Health and Well-being meeting
6 July 2023, Brussels

European Commission

#HealthUnion

Glossary

- MA - marketing autorisation
- MAA - marketing autorisation application
- ERA - environmental risk assessment
- ASMF - active substance master file
- CP – centralised procedure
- MRP – mutual recognition procedure
- DCP - decentralised procedure
- PhV – pharmacovigilance
- EMA – European Medicines Agency
- PRIME – priority medicinal products
- SoHO – substances of human origin

('SoHO' as defined in the 'SoHO Reg')

- ATMP- advanced therapy medicinal products
- GMO - genetically modified organisms
- CMA – conditional marketing authorization
- comp. use – compassionate use
- TEMA - temporary emergency marketing authorisation
- UMN – unmet medical needs
- HUMN – high unmet medical needs
- HTA – health technology assessment
- P&R – pricing and reimbursement
- PIP – paediatric investigation plan
- PUMA - paediatric-use marketing authorisation
- IPCEI - Important Projects of Common European Interest

57 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

2000
Legislation on medicines for rare diseases

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

2006
Legislation on medicines for children

2007
Regulation on advanced therapy medicines

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

2022
Reform of general pharmaceutical acts packaged with revision of the O/P legislation

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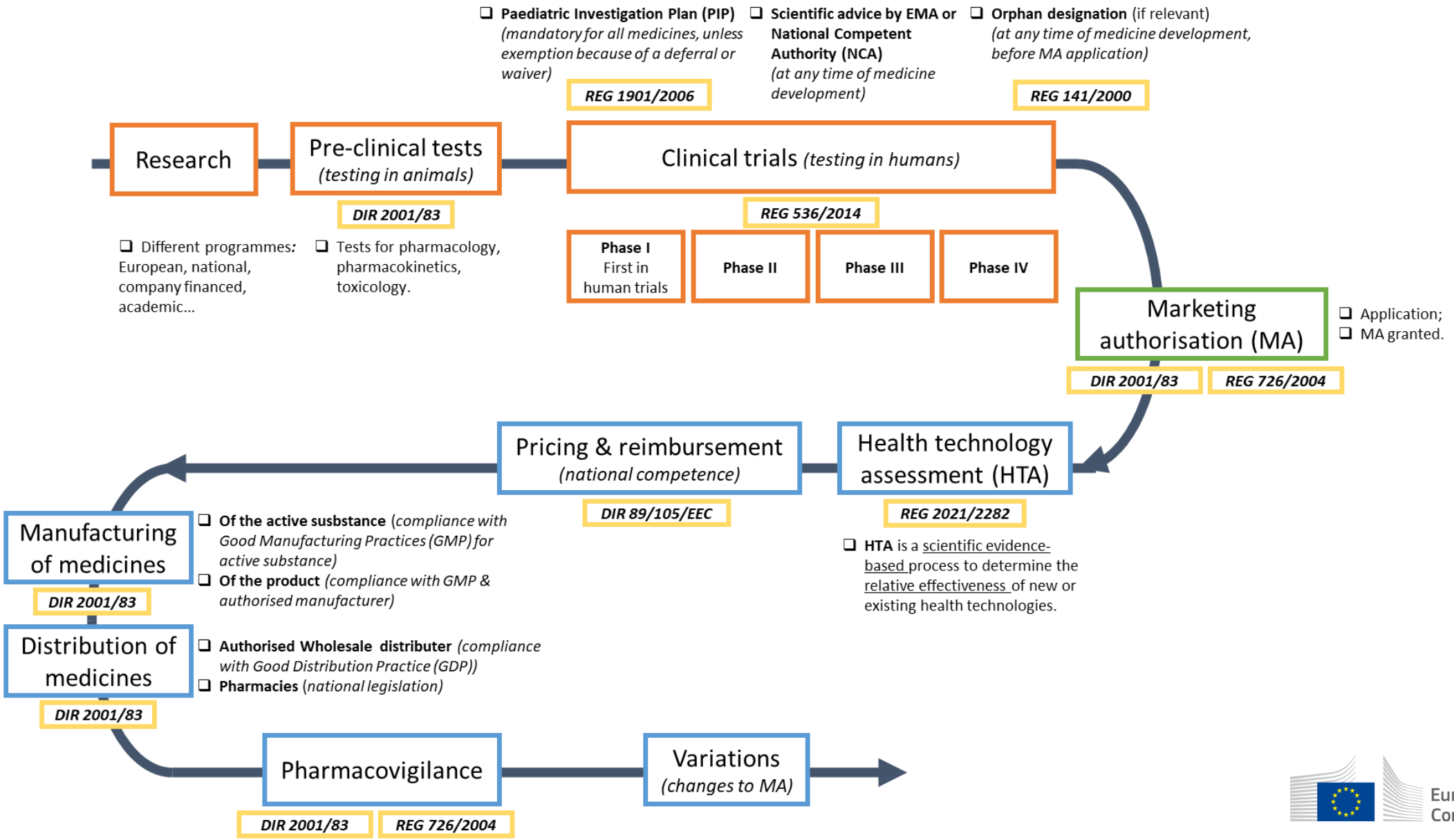
Lifecycle of a medicinal product



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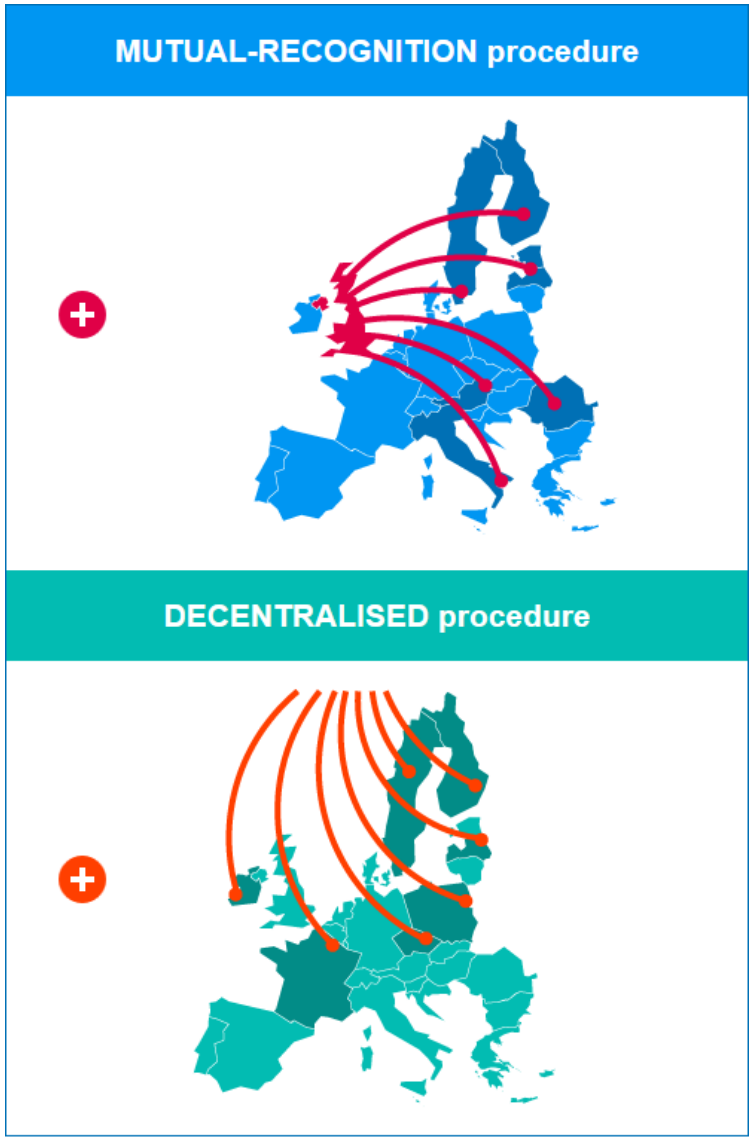

Principle of recognition of an already existing national marketing authorisation by one or more Member States

Application for the marketing authorisation submitted simultaneously in several Member States, one chosen as the Reference Member State

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Responsibilities shared between EU and Member States

EU (harmonised) general pharmaceutical legislation

- Centralised authorisation procedure
- Inspections of manufacturing sites
- Pharmacovigilance

Decentralised procedure and mutual recognition procedure to authorise medicines in MS

By EU-level standards

EC and EMA through a network of MS experts and National Competent Authorities

Strictly MS competence outside the scope of the pharmaceutical legislation!

- Organisation and delivery of health services and medical care
- Setting of prices for medicinal products or their inclusion in the scope of national health insurance schemes

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#EUPharmaStrategy

- Adopted in November 2020
- Ambitious long-term agenda in the field of pharmaceutical policy
- Objective: creating a future proof regulatory framework and at supporting industry in promoting research and technologies that actually reach patients in order to fulfil their therapeutic needs

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A 4-part package

Chapeau communication

New Regulation

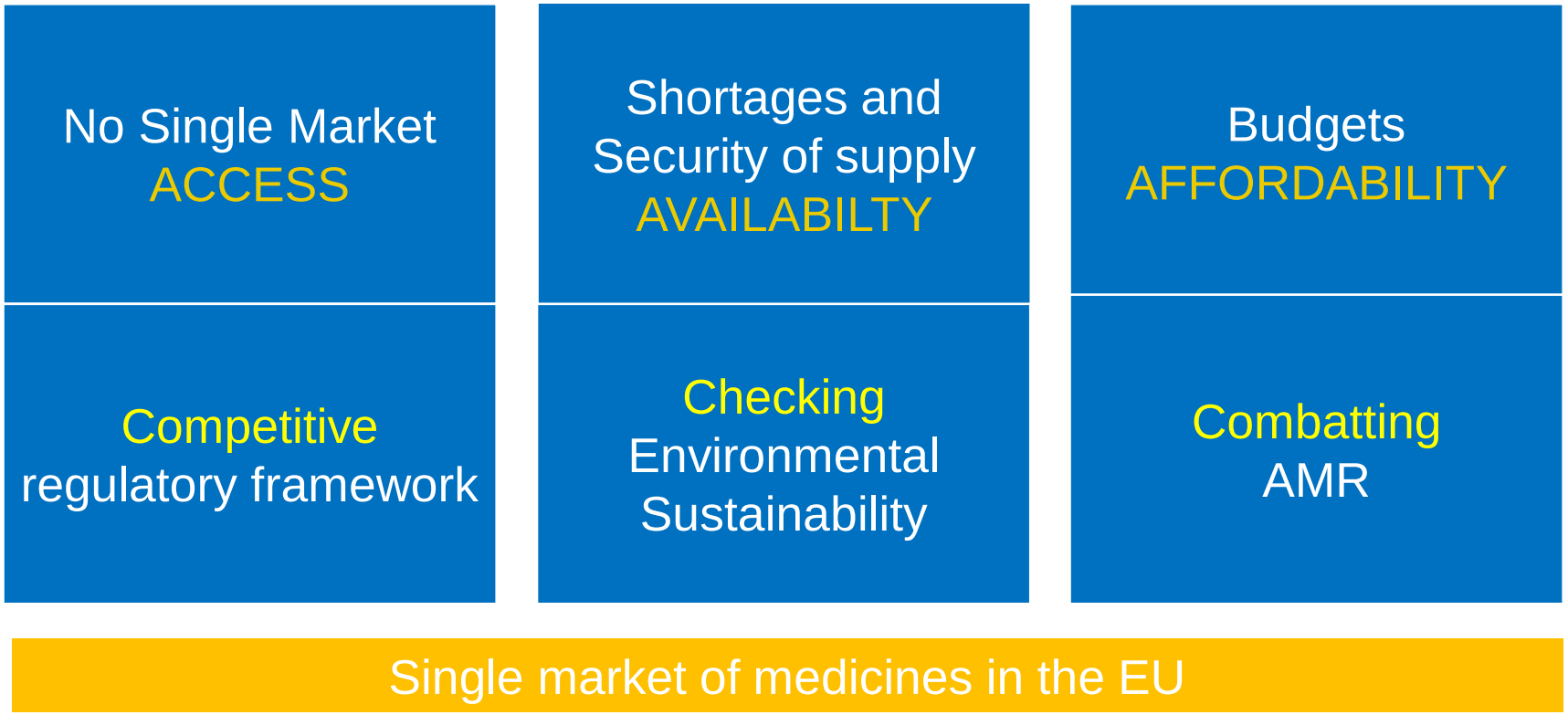
- Specific rules for the most innovative medicines such as orphans, antimicrobials
- Rules on shortages and security of supply
- EMA governance

New Directive

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

Council Recommendation on AMR

6 Key political objectives



Impact Assessment

- Commission published two impact assessments supporting the reform:
  - Impact assessment related to changes of the general pharmaceutical legislation
  - Impact assessment related to changes of the orphan/paediatric legislation
- The impacts assessments considered several policy options and includes a granular analysis of multiple elements supporting the policy interventions
- The impact assessments were supported by two independent studies and stakeholder consultations

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:

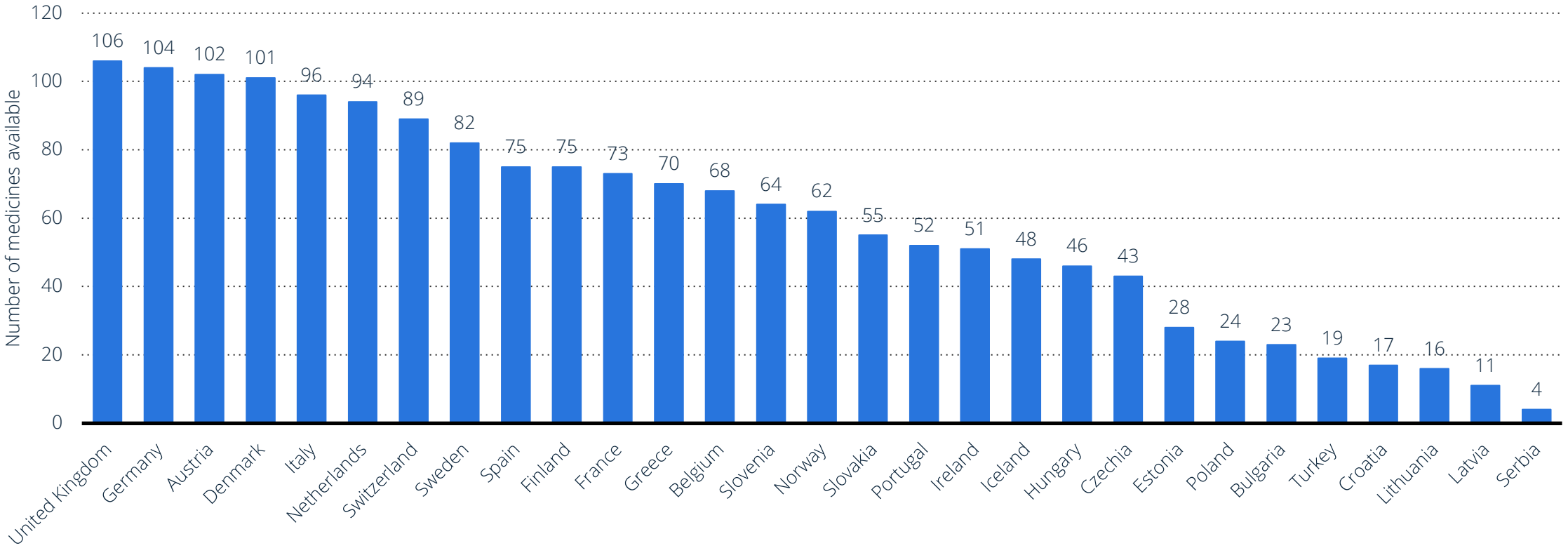
Targeted approach vs current “one-size-fits-all” unconditional data protection and market exclusivity (for orphans)

Earlier market entry of generic and biosimilar medicines

- Faster authorisation
- Pre-authorisation support

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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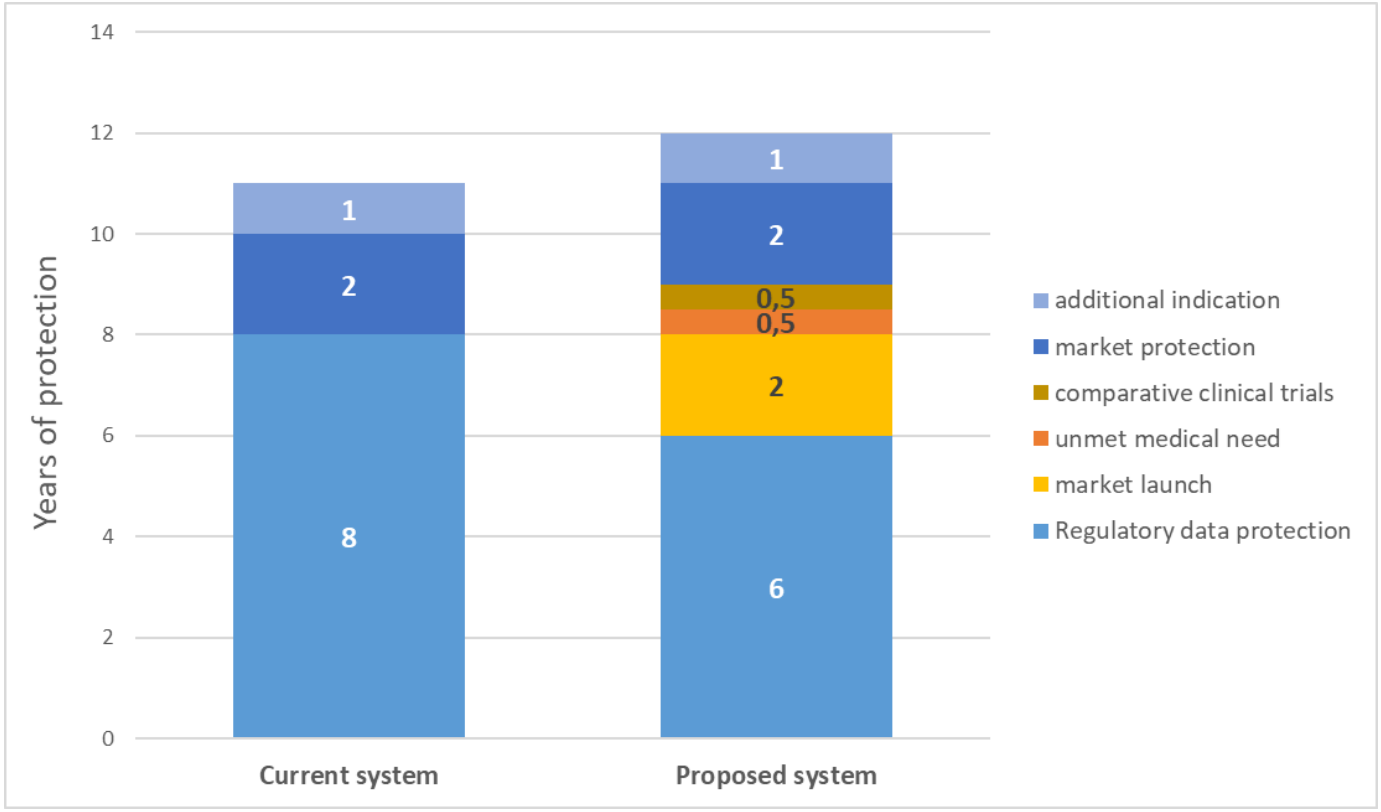
Modulation for the majority of innovative medicines

Regulatory data and market protection today and as proposed



Current system, max 11 years protection

Proposed system, max 12 years protection

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).

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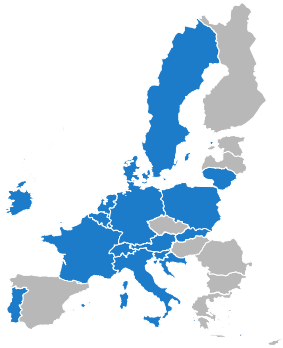
Market launch conditions

- 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 (Transparency Directive)

Availability – shortages of all MPs and 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) (REG Art 116)

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)

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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)

Streamlined and agile regulatory framework catering for innovation

Current challenge:

Longer approvals times than in other regions (US 244 days)

Administrative burden and compliance costs for the industry

The clock stop mechanism

Proposed solutions:

Faster autorisation:
a) 180 days standard procedure
b) 150 days accelerated procedure

Regulatory efficiency:
Improved EMA structure, simplified procedures, better use of data and digitisation, regulatory sandboxes

Pre-authorisation support to promising medicines to accelerate development and attract investments

Lower regulatory burden (especially important for SMEs and not-for-profits)

Environmental sustainability

Current challenge:

Pharmaceuticals in environment can **harm environment and human health**

Presence of antimicrobials in the environment exacerbates AMR

Weak enforcement of current rules

Proposed solutions:

Better enforcement of the current rules on **Environmental Risk Assessment** (part of the application)

Extending ERA to **medicines already on the market before 2005**

Stricter environmental rules for AMR, also covering manufacturing

Electronic leaflet and **electronic submission** of applications (less use of paper)

Combatting AMR

Current challenge:

AMR causes **35000 deaths per year** in the EU.

It amounts to +/-1.5 bn EUR per year in healthcare costs

By 2050, **10 million deaths globally each year**

Current market failure/ Lack of effective antimicrobials

Lack of market incentives

0,5 bln EUR cost of a new antibiotic

AMR toolbox

Measures on prudent use of antimicrobials – prescription, restricted quantities, education etc.

Regulatory incentives with transferable exclusivity vouchers under strict conditions

Financial incentives with **procurement mechanisms** (HERA)

5 Targets, incl on the total **EU consumption of antibiotics for humans** (ECDC) → reduction by 20% by 2030

(Council Recommendation)

AMR voucher

- Additional year of data protection
- Strict conditions (only novel antimicrobials, full transparency of all funding, obligation of supply, max 10 vouchers in 15 years, review after 15 years, etc.)

Focus point – elements of special relevance at regional or local level

- Access on all markets incentive- will increase access to medicines
- Leaflet/e-leaflet – will increase appropriate info of patients
- Inspections for GMP compliance –EU system of supervision of national inspectorates, including a Joint Audit Programme, which builds on structures already informally in place today.
- Decentralised manufacturing - will allow manufacturing very locally, under supervision of qualified person of the central site. This may be needed for novel, personalised products.
- ERA applies to AMR / manufacturing. Some MS are moving back production of antibiotics to the EU. Manufacturers would have to submit an ERA for manufacturing.
- Transparency of public funding. Could be useful for negotiations at local level.
- Availability - more a national issue but of interest locally too.

Thank you

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