The Pharmaceutical Strategy in Europe, unmet needs and orphan regulation

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EU Pharmaceutical strategy

Ensure access and affordability of medicines for patients and health systems sustainability

Ensuring access and availability addressing shortages

Enabling sustainable innovation

Succeeding on the global level

European Commission

Flagships of the pharmaceutical strategy

Ensure access and affordability of medicines for patients and health systems sustainability

Unmet needs

- Boost **novel antibiotics** - 2021
- Restrict and optimise the **use** of antimicrobial medicines (2021)
- Support medicines for **children and rare diseases** (2022)
- Collaboration on unmet needs, **evidence generation, HTA** (2021)

Accessibility

- Revise the **system of incentives and obligations** in legislation to support innovation, access and the affordability of medicines (2022)
- Improve access to **generic and biosimilar medicines** (2022)

Affordability

- Address in legislation the **market effects** impacting on affordability (2022)
- Develop **mutual learning and best-practice exchange** on pricing, payment and procurement policies (2021-2024)

Flagships of the pharmaceutical strategy

Enabling sustainable innovation

Fertile environment

- Optimise the **supplementary protection certificates system** (2022)
- Legislative proposal on **European Health Data Space** (2021)
- **Interoperable data access infrastructure** to facilitate secure cross-border analysis of health data (2021-2025)
- Support **public-private and public-public partnerships** (2021)

Innovation and digital transformation

- Adapt legislation to **cutting-edge products, scientific developments** and transformations (2022)
- **Enhance dialogue** among regulatory and other relevant authorities (2021)
- Take forward the use of **HPC and AI** (2021-2022)
- Establish the secure federated access to 10 million **genomes** (2025)

Flexible regulatory system

- **Simplification and streamlining** of approval procedures and flexibility for timely adaptation (2022)
- **Optimise the lifecycle management of medicines** more efficient and adapted to digitalisation (2021-2023)

Flagships of the pharmaceutical strategy

Ensuring access and availability addressing shortages

Secure the supply

- Revise the legislation to **enhance security of supply and address shortages** (2022)
- Launch a structured dialogue to **identify vulnerabilities** in the global supply chain (2021)
- Ensure **increased transparency of the industry** on the supply chains (2021)

High quality, safe and environmentally sustainable

- revise manufacturing and supply provisions in the legislation to **ensure environmental sustainability, quality and preparedness** (2022)
- revise the legislation to strengthen **environmental risk assessment** requirements and conditions of use (2022)

Crisis response mechanisms

- Proposal for an **EU Health Emergency Response Authority** (2021)

Flagships of the pharmaceutical strategy

Succeeding on the global level

Work with the EMA and the network of national regulators, to promote **regulatory convergence** to ensure access to safe, effective high-quality and affordable medicinal products globally (ongoing)

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

Objectives of the revision

- To foster research and development of medicines for rare diseases in areas of unmet need and in better alignment with patient needs
- To ensure availability and timely access of patients to orphan medicines
- To ensure legislation to be fit to embrace technological and scientific advances
- To provide effective and efficient procedures, for assessment and authorisation

Impact assessment study

- **To test the impact of various (novel) incentives on the development of products addressing UMN in rare diseases:**
  - **Increased scientific support by EMA and priority assessment** for products addressing UMN in rare diseases (PRIME-like)
  - **Post-authorisation rewards and incentives (extended market exclusivity; possible novel incentives)** specially dedicated to medicines addressing unmet needs for rare diseases' patients.

Next steps revision of the orphan and paediatric legislation

- Impact Assessment
  - Public consultation
  - Targeted consultations
  - interviews
- Legal Proposal(s) (Q1 2022)

Revision of the pharmaceutical legislation

- Roadmap inception impact assessment (Q1/2 2021)
- Evaluation and impact assessment
- Public consultations (Q2/3 2021)
- Other consultation activities (Q2/3 2021)
- Adoption of proposal(s) (Q4 2022)

Thank you

European Commission