National Institute for Public Health
and the Environment
*Ministry of Health, Welfare and Sport*

# Minds Open –

## Sustainability of the European regulatory system for medicinal products

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# Challenges to the regulatory system

Changing healthcare demands

Affordable prices

possibilities for innovation

timely availability on the market

political agenda setting

resources necessary to comply with all requirements

patients access

Stakeholders interaction

pharmacovigilance

Politica ebate

social debate


?

**Is the current system sustainable for the future?**

# Aim

- Identifying **areas of special interest** by analysing **potential vulnerabilities** (that demand closer attention) in the regulatory system, with a special focus on the themes **innovation**, **availability** of (new) pharmaceuticals, **safety & efficacy**, and **costs**.

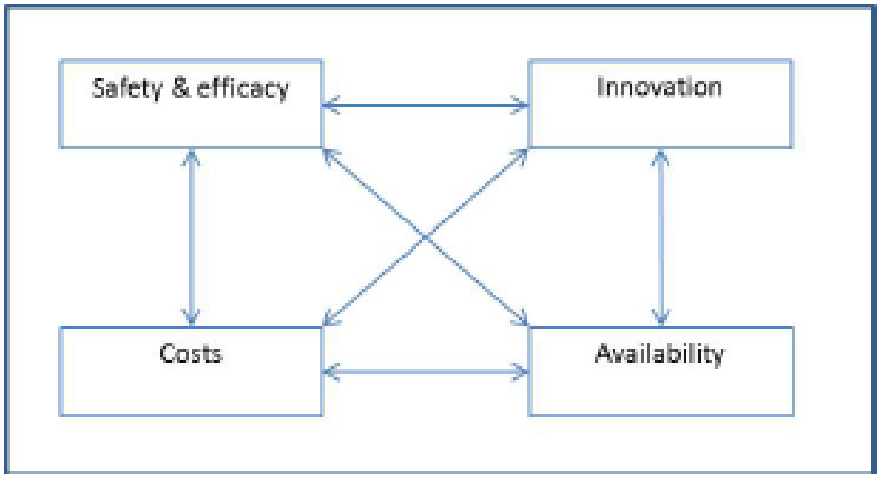

**Figure 1.1 The drug development pathway from discovery to product launch and post-market monitoring.** All areas in green are within the scope of this research.

# Methods

Four main activities:

1. inventory of the **pharmaceutical regulatory history**
2. **literature review** (n=118)
3. semi-structured **interviewing** of **national key experts** (n=9)
4. identification of **illustrative cases**

# Structured findings




- Current issues
- Potential causes (internal and external to the system)
- Government reactions

# Safety & Efficacy

- Regulatory system performs well ...however...


Varying perceptions

↓

Increasing public attention to incidents

↓

'cautious regulator' problem

# Safety & Efficacy - bottlenecks & reactions

Examples of bottlenecks identified in our study:

- Randomized clinical trials:
  - Number of patients is insufficient to evaluate safety (and long term efficacy)
  - Type of patients is not representative of users in daily practice
  - Transparency of trial results not optimal

Examples of reactions:

- RCTs -> more emphasis on post marketing phase
- Communication and transparency – lack of transparency regarding decisions → legal requirements for transparency, including pharmacovigilance issues
- Emphasizing benefit-risk balance

# Innovation

- Decline in number of newly marketed chemical or biological entities (1992 – 2011)
- Overall proportion of innovative medicines is lower
- Increase in investments of pharmaceutical industry BUT decline in output
- Mismatch between innovation and neglected conditions; focus on 3 main therapeutic areas (oncology, infectious diseases and blood disorders)

# Innovation – bottlenecks & reactions

Examples of bottlenecks identified in our study

- Development of new medicinal products
  - Increasing requirements hamper innovation and increases costs
  - No focus on unmet medical need
  - Failing development strategies and/or immature applications

Examples of reactions:

- Development of new medicinal products
  - No focus on unmet medical needs → regulation for orphan medicinal products
  - Failing development strategies → increased interaction with regulators by early dialogue and scientific advice

# Costs

- Increase in costs of research and development. Total R&D investments in Europe*
  - 2010: 28 billion euros
  - 1990: 8 billion euros

*Source: EFPIA. The Pharmaceutical Industry in Figures. Key data 2012. European Federation of Pharmaceutical Industries and Associations, 2013

# Costs – bottlenecks & reactions

Examples of bottlenecks identified in our study

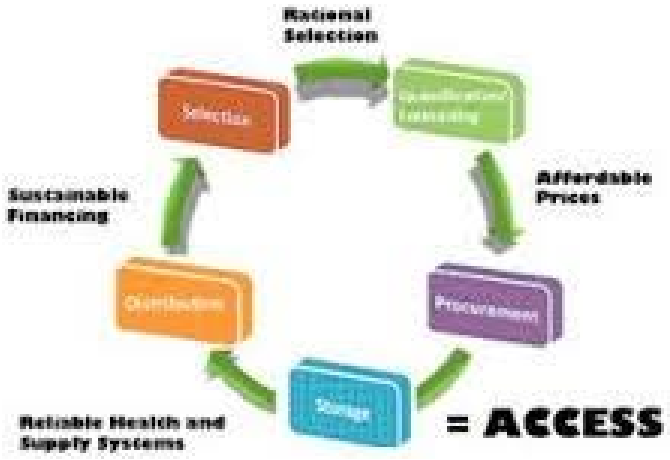
- Growing amount of regulatory guidelines – longer development times
- Additional (divergent) requirements for reimbursement
- Uncertainty of reimbursement
- Development strategies pharmaceutical industry
- Lack of transparency in compilation of prices

Examples of reactions

- Divergent requirements for market access and reimbursement → increase interaction between health technology assessment bodies and regulatory authorities
- Questioning the cost-effectiveness of regulations

# Availability

- Complex issue – many stakeholders play an important role
- Both regulatory system factors and external factors linked to availability



# Availability – bottlenecks and reactions

Examples of bottlenecks identified in our study

- Availability of existing medicinal products
  - No guarenteed supply
  - Uncertainty regarding reimbursement of compassionate use products
- Increased safety requirements
- European versus national procedures

Examples of reactions

- Steering innovation → e.g. priority medicines, IMI
- Fast tracking access → conditional approval and exceptional circumstances
- Extrapolation → learning studies and medicine classes

# Conclusions

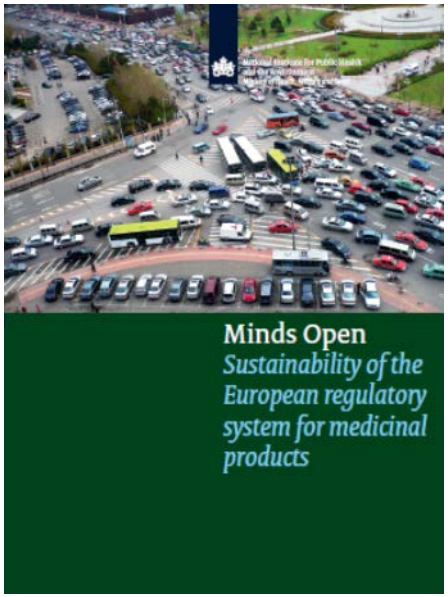
A mixture of factors, internal and external to the system, give reasons to believe that the current system will not be sustainable in future.

Future changes to the regulatory system seem necessary to meet the needs of current/future challenges

Growing need to **rethink the balance**, taking into consideration a.o.:

- risks perceived by society and patients
- continuous dialogue including all stakeholders
- Interrelatedness between themes

# THANK YOU

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