Improving access to medicines while promoting pharmaceutical innovation

Presentation of the study prepared at request of the Panel for the Future of Science and Technology (STOA)

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Introduction and objectives

- Health as a **fundamental right**, and equality in access to medicines is crucial

- Two conditions:
  - **Innovation**: ability to develop new products that are more effective than the existing ones
  - **Access**: in terms of prices and availability

- In the current framework, challenging to find a **balance** between the two conditions
  - Innovation driven by market size (less likely when small expected returns, even if high value to society)
  - Access not always granted, even if strong public investments

- ***Objective of the study***: evaluating the impact of regulatory mechanisms and alternative frameworks on innovation and access

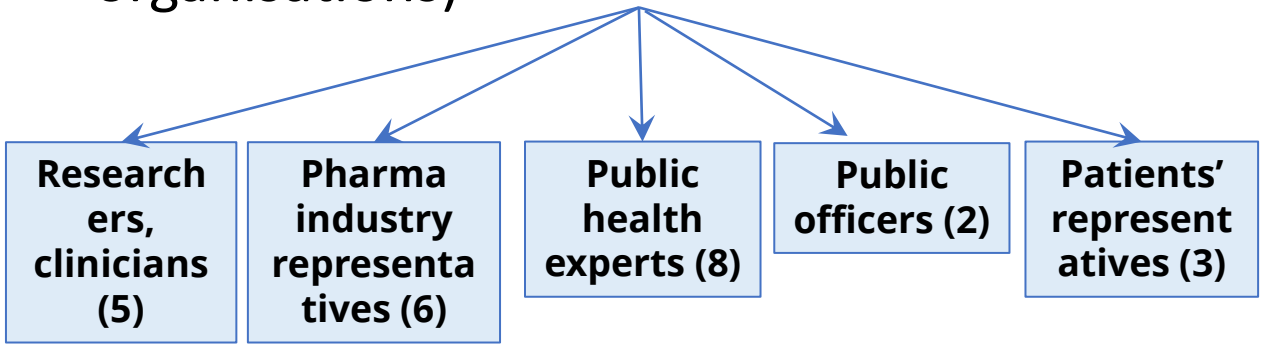
Methodology

Literature review

- 195 scientific articles
- 39 reports
- 3 books
- statistical sources

Semi-structured interviews to expert stakeholders

- 24 respondents (from 23 different organisations)



- Hurdles to innovation, role of incentives, alternative frameworks that may be implemented, comments on the proposal for a new EU pharma legislation

Report drafting

- Evidence triangulation
- Critical reviewers
- Policy options design
- Interactions with Scientific Foresight Unit (STOA)

Summary of results: exclusivities

	Impact on:			
	innovation	**direction of R&D (e.g. UMN)**	**access**	**predictability for generics, biosimilars, competitors**
Patents	Prevailing view: positive	Very limited (market-based incentive)	Negative (high prices from limited competition)	Negative (strategic behaviour)
SPCs	Controversial	Very limited (market-based incentive)	Negative (high prices from limited competition)	Negative (differences among countries)
Data exclusivity	Positive but limited	Limited (market-based incentive)	Negative (barely relevant if shorter than market protection)	Negative (strategic behaviour)
Market protection	Positive (in absence of patents)	Very limited (market-based incentive)	Negative (high prices from limited competition)	Negative (strategic behaviour)
Market exclusivity	Positive	Very limited (market-based incentive): weak incentives for ultra-rare	Negative (high prices from limited competition)	Negative (strategic behaviour)

- ✓ Widely adopted and analysed
- ✓ No upfront payment from the healthcare system required
- × Currently struggle to find a balance between stimulus to innovation and access

Summary of results: vouchers

	Impact on:			
	innovation	**direction of R&D (e.g. UMN)**	**access**	**predictability for generics, biosimilars, competitors**
TEVs	Potentially positive (never implemented)	Positive (incentive delinked from market size)	Null in the market of targeted product; negative in the market where it is used	Negative in the market where it is used (provisions to limit this drawback need to be included)
PRVs	Controversial	Positive (incentive delinked from market size)	Null in the market of targeted product; positive but limited in the market where it is used	Null

× Limited use so far (PRVs: UMN; TEVs: never implemented)
× Access to the incentivised product is not guaranteed: access conditions need to be defined
× TEVs: cost unknown in advance
× Size of the reward decoupled from the value of the innovation
✓ No upfront payment from the healthcare system required

Summary of results: ex-ante commitment and push incentives

	Impact on:			
	innovation	**direction of R&D (e.g. UMN)**	**access**	**predictability for generics, biosimilars, competitors**
APAs	Positive (reduced market risk for manufacturers)	Positive (incentive delinked from market size)	Positive (provided amounts are appropriately defined)	Positive
SMs	Positive (reduced market risk for manufacturers)	Positive (incentive delinked from market size)	Positive	Positive
Innovation prizes	Potentially positive (with limited evidence)	Positive (incentive delinked from market size)	Positive (if patents are replaced)	Positive (if patents are replaced)
Tax credits	Positive (reduced costs for manufacturers)	Limited (weak incentives for ultra-rare)	Null	Null

- × Ex-ante commitment (APAs, SMs, prizes):
  - × limited use so far
  - × need to define ex-ante the criteria for receiving the incentive
  - × difficulty in setting the value ex-ante
- × Tax credits: not implementable at the EU level (Member States retain control over fiscal policies)
- × Upfront payment from the healthcare system required

Summary of results: public oriented approaches

	Impact on:			
	innovation	**direction of R&D (e.g. UMN)**	**access**	**predictability for generics, biosimilars, competitors**
Open science framework	Positive	Positive (no profit objectives)	Positive	Positive
PPPs	Positive	Positive (dedicated effort)	Positive (many product-development PPPs focus on this aspect)	Positive (most PPPs adopt an open science approach)
Public R&D infrastructures	Positive	Positive (dedicated effort)	Positive	Positive

- ✓ Exploitation of synergies and complementarities
- × Limited use so far (UMN)
- × Public R&D infrastructure: long-term implementation and large upfront payment from the public sector required
- × PPPs: coordination issues

PO0 Current regulatory framework

- Central role of exclusivities (patents, SPCs, market & data protection, market exclusivity)
- UMN: mainly addressed with exclusivity extensions (orphan, paediatrics)
- IPRs: constraints upstream (TRIPs) and heterogeneity downstream (SPCs at national level)
- MSs are responsible for pricing and reimbursement decisions

ADVANTAGES	DISADVANTAGES
• Innovations with important impacts on patient outcomes • Attention toward rare diseases (orphan legislation) • Incentive to invest in promising projects • Limited EU coordination needed	• Lack of clarity due to overlapping tools • National level decisions create uncertainty and disparities in access • Negative impact on access (some very high prices) • Relevance of UMN • Weak incentives for private firms to address future emergencies • More incremental than disruptive innovation

PO1 Strengthening EU coordination in IPR and procurement

- Adding «unitary SPC» to «unitary patent»
- EU procurement authority + EU pharmaceutical fund
- Transparent EU price
- Clear rule for MSs contribution to the EU pharmaceutical fund
- Option to opt out for MSs

ADVANTAGES	DISADVANTAGES
• Better access and availability • More homogeneous (across MSs) availability • Shorter time to launch for the industry • Reduced transaction costs • Greater transparency on prices	• Need to establish an EU procurement authority (or extra competences to an existing one) and financial mechanism • Need to define each MS contribution to the fund • Need to reach wide consensus

PO2 Adjusting current incentives to limit extra-rents

- Objective: reduce the risk of overpaying for R&D
- Tool: adjustment of length of exclusivities to account for e.g.:
  - Profits made
  - Public funding received throughout the R&D process

ADVANTAGES	DISADVANTAGES
• Saving public financial resources to reinvest in R&D (e.g. to address UMN) • Greater transparency on the use of public funds • Enhanced patient access	• Scarce feasibility of estimating profitability, or public funds received, at product level • Reduced incentive to improve efficiency for the industry (profit caps) • Difficulty in defining a fair level of profits

PO3: Redesigning incentives

- Incentives mainly based on patents and SPCs. Reduction of length of data and market protection
- Support of studies to investigate new indications: extended length of market protection
- New tools (in addition to the existing ones) for ultra-rare diseases and antimicrobials: SMs

ADVANTAGES	DISADVANTAGES

• Explicit targeting of (high) UMN
• Antimicrobials: mechanism for appropriateness (e.g. price to be paid to the fund possibly increasing in quantity)
• SMs:
  • De-linkage of revenues from volumes
  • Reduced risk of shortages
  • Reduced uncertainty for the industry
	• Risk of lower incentives to R&D investment (reduction of market and data protection)
	• Difficulty in setting the SM value ex-ante
	• Need to find an agreement on the rules defining national contributions to subscription payments

PO4 European infrastructure for pharmaceutical R&D

- More active role of the public sector throughout the whole R&D and production process (in-house or outsourcing)
- Focus on areas where the private sector is under-investing relative to public health needs (UMN, emergencies)
- Engagement in independent superiority trials and repurposing studies
- Socially responsible IP approach (open science, or non-exclusive licensing)

ADVANTAGES	DISADVANTAGES
• Better alignment between public health needs and R&D investments • Attention to repurposing and superiority trials • Enhanced access to innovation • Better knowledge integration • Transparency on R&D costs	• Long-term implementation • Large upfront payment from the public sector required

PO5 A comprehensive approach

- Combination of PO1, PO3 and PO4:
  - Greater EU coordination on IPR and procurement
  - Redesigned incentives (reduced length + new incentives)
  - European infrastructure for pharmaceutical R&D

ADVANTAGES	DISADVANTAGES

• Benefits of PO1 + PO3 + PO4
• Synergies, e.g.:
  • Easier to implement SMs through the EU fund
  • Effects of redesigned incentives while implementing the R&D infrastructure
  • More efficient allocation of R&D priorities between private and public
  • Weaker incentives for firms compensated by earlier market access and lower market access costs
	• Need to reach a wide consensus among MSs (EU procurement authority and financial mechanism)

Conclusions

- **Several issues** characterise the pharmaceutical sector and its regulation:
  - Access
  - Industry's R&D priorities only partially aligned with public health goals (UMN, preparedness,...)
  - More incremental than disruptive innovation
  - Huge public investments and privatisation of returns

- A **reform** is required

- **Five policy options**. The most ambitious involves a comprehensive approach:
  - Strengthening EU coordination in IPR and procurement
  - Redesigning incentives:
    - reduction in exclusivities' lengths
    - new incentives specific to (high) UMN and delinked from market size
  - Public R&D infrastructure active throughout the whole R&D and production process in specific areas

THANK YOU

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