MANAGING UNCERTAINTY OVER THE LIFE CYCLE OF DRUG DEVELOPMENT AND USE:
INTRODUCING ADAPTABILITY AND FLEXIBILITY IN REGULATION OF PHARMACEUTICALS
- INTRODUCTION

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Risk-management in health systems

• Risks to health in regulation of health products

• Risks to public budgets in adoption/coverage of new technologies

• Balancing risk to patient privacy and risk to public health
1. Regulation of health products under pressure for change

*Market entry regulation is under pressure for change*

- New technologies (small populations, higher uncertainty, products which are half-medicine/half-medical device, etc).
- It is insufficient for the medical devices.
- Possibility to manage risks *after* marketing authorisation.

*Clinical trials need to be harmonised and streamlined*

- OECD recommendations on the governance of CT issued in 2013: improve consistency of national regulations and their interpretation; introduce a “proportionate regulatory approach”; enhancing the protection of trial participants.
- Initiative at the European level (EC proposal in 2012) and in the United States to simplify and improve regulation of CTs.

*Regulatory science needs to be modernised*

- Encourage dialog with innovators, who often know better than regulators about scientific developments – without ignoring the risk of regulatory capture
- Increase transparency in decision-making process and open acknowledgement of ethical concerns and local values, generate greater legitimacy for the public.
Is there a pattern in attitudes to risk?

VS.

**REQUIREMENTS & PROCESS**
Converging but US (FDA) more demanding for biosimilars
Generalised PROMS in Europe, symptom specific in US

**SPEED**
US (FDA) approves cancer drugs more quickly than Europe (EMA)...

**OUTCOMES (for oncology drugs)**
50% end up with same conclusion
30% differences in labelling (2\textsuperscript{nd} agency more restrictive)
20% approved by one, rejected by other.
Convergence ahead?

- Involvement of patients in decision-making to determine the degree of risk they are willing to take.
- Towards life-cycle management of products
- Progressive licensing framework (Canada), adaptive licensing (Europe): aims to ensure continuous re-evaluation of risks and benefits of medicines
2. Managing financial risks for payers

- New products with very high prices, more often to treat small population (orphan drugs or "indications"; personalised medicines) but not always (new drug for Hepatitis C)

- Risks for payers:
  - Paying for drugs that do not worth it (diverting scarce resources from more cost-effective care);
  - Threatening health systems sustainability in medium and long term
  - To be balanced with the risk to not make effective drugs available and affordable to patients.
Policies to address financial risks for health care payers

**Value based pricing**: price or coverage determined by reference to the additional cost of obtaining additional clinical benefits (health technology assessment)

**Managed entry agreements to mitigate**

1) Uncertainty in risks and/or effectiveness: Coverage with Evidence Development

2) Uncertainty in cost-effectiveness: performance-based agreement where companies have to pay rebates when «claimed benefits» are not observed in real life

3) Budget impact: volume-price agreements or dose capping

- Hundreds of agreements have been signed in European countries, Australia
Unprecedented opportunities to monitor safety, effectiveness and comparative effectiveness of treatments and medical products, through:

- Electronic health records
- Registries (disease- or specialty-based) – Italy, Finland, Sweden

➢ Need to balance costs and benefits of data collection and processing health data
➢ Need to balance individual rights to privacy with collective rights to safe and effective health care and to high performing health systems
Trade-off between safety/performance of health systems and privacy

Risk for Individual Data Privacy

No Risk

- No data
- No data sharing
- No data linkage

No monitoring
No research

High Risk

Best practices in data sharing, linkage and analysis:
- protection of individuals’ privacy
- Regular programmes of monitoring and research

Data use with Weak privacy protection practices

Data use with weak privacy protection practices and incentives to misuse the data

Risk for Patient Safety & Health System

High Risk

Low Risk
Very little linkage and quality monitoring beyond hospital data & cancer registries

- Mortality data
- Hospital in-patient data
- Cancer registry data
- Prescription medicines data
- Mental hospital in-patient data
- Population census or registry data
- Population health survey data
- Primary care data
- Formal long-term care data
- Patient experiences survey data

19 Countries

- National dataset available
- Contains a unique patient number that could be used for linkages
- Is used regularly for linkages to monitor health care quality

Source: OECD HCQI Country Survey, 2011/12
Thank you

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