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

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BETTER POLICIES FOR BETTER LIVES

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 (2nd 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

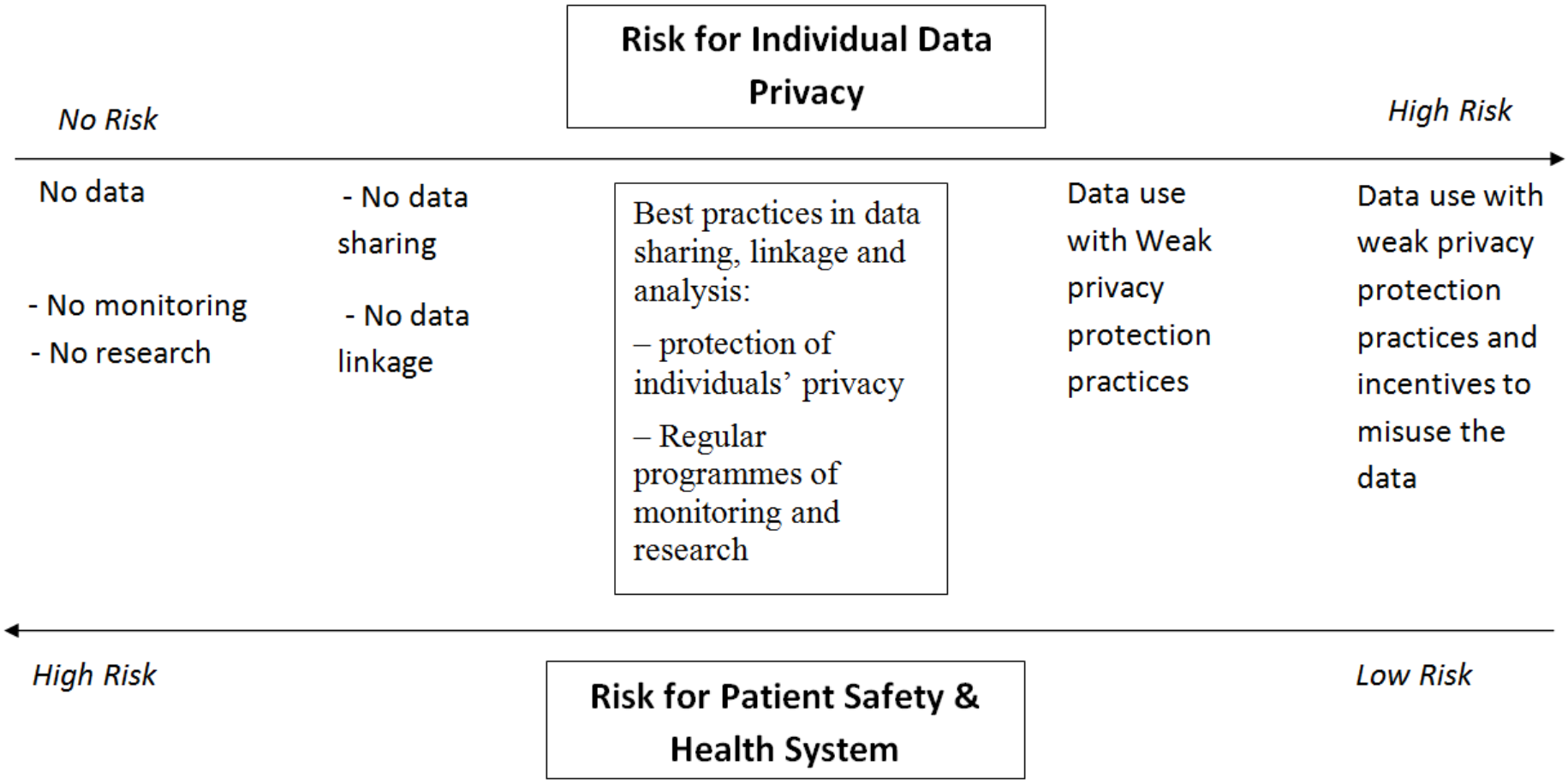
3 Risks and attitudes to data

Unprecedented opportunities to monitor safety, effectiveness and comparative effectiveness of treatments and medical products, through:

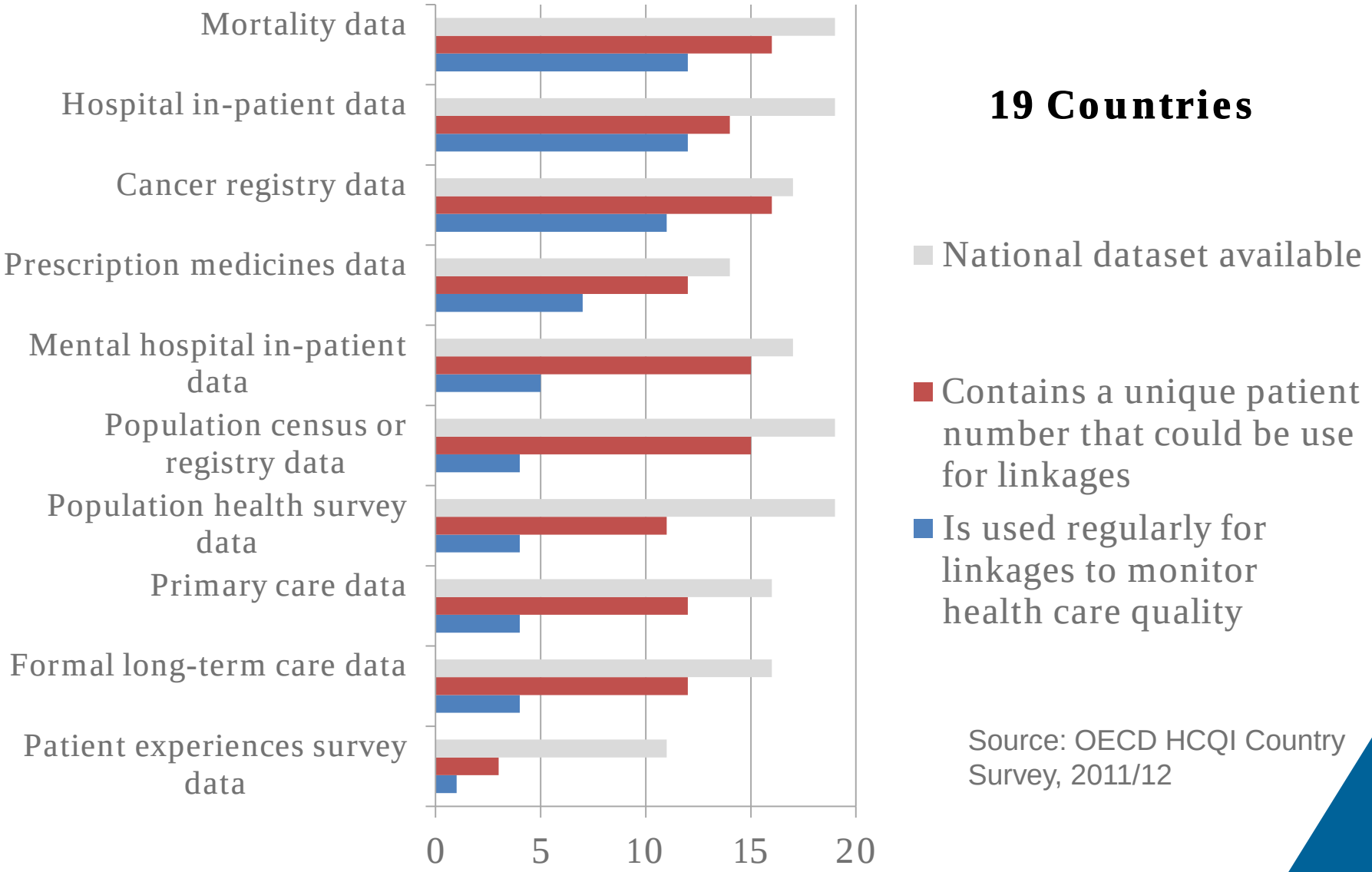
- Electronic health records
- Registries (disease- or specialty-based) – Italy, Finland, Sweden

- 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



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



Source: OECD HCQI Country Survey, 2011/12

Thank you

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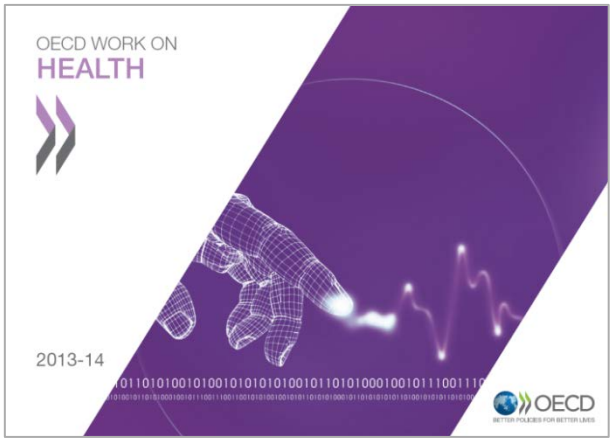

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