Safeguarding public health

MHRA

Health Technology Assessment:

Impact on Regulators

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Themes of this overview

- Objectives of regulation, HTA and appraisal
- Scope for efficiencies and synergies between them

Medical products are regulated in different ways in different jurisdictions:

Pharmaceuticals
Medical Devices
Diagnostics
Advanced therapies

HTA includes a wider range of interventions:

All of the above plus e.g. procedures, screening, service delivery, immunisation programmes…..

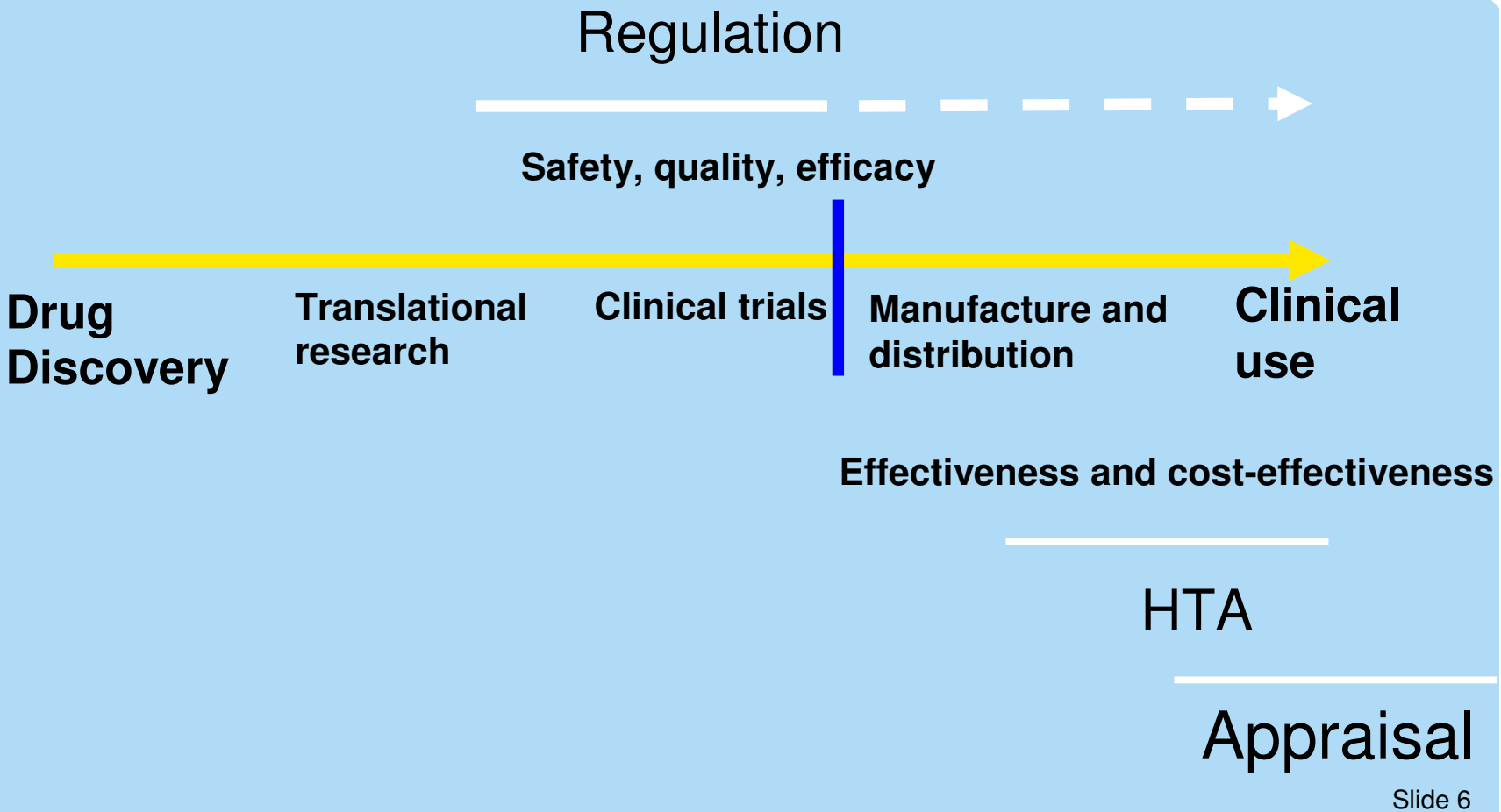
EU Regulatory Network

"A unique economic and political partnership between 27 democratic European countries"

- 500 million population
- Common legislative frameworks for medicines, medical devices, blood, advanced therapies, clinical trials etc.
- Diverse societies, health care systems, constitutions and legal traditions

Regulation, HTA and Appraisal

The Development Pathway of a Medicine



Objectives of regulation

Regulation uses science and law to promote public health on the basis of:

- Quality
- Safety
- Efficacy (in RCTs, against placebo +/- active comparator)

From a public health perspective:

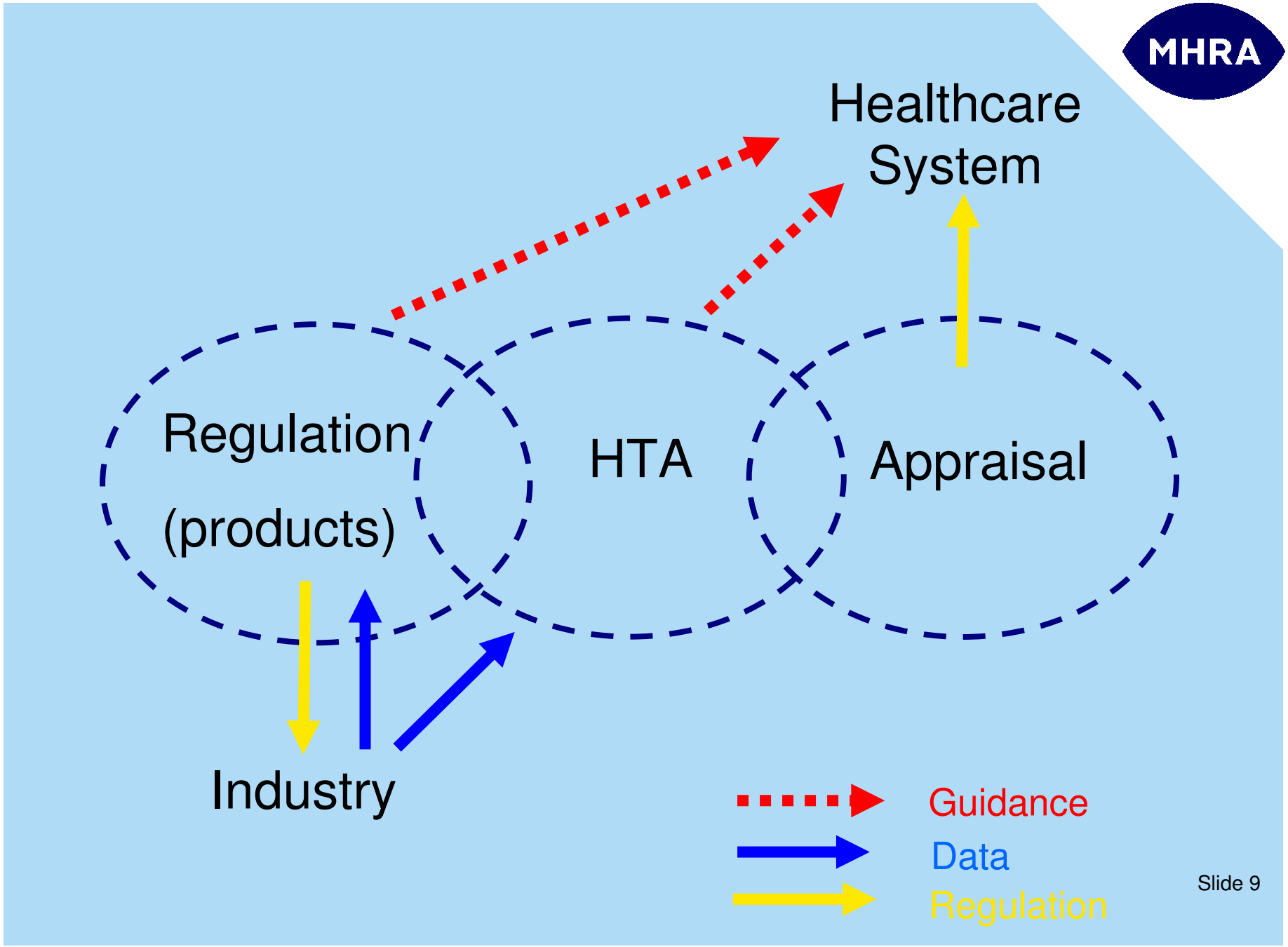
- The benefit-risk relationship in a defined target population, i.e. effectiveness and 'safeness' under conditions of use.
- Supporting innovation

Objectives of HTA

- Effectiveness and cost-effectiveness under normal conditions of use

Objectives of appraisal (coverage decision)

- Maximising health gain from finite resources, taking account of HTA but also priorities, needs, skills, resources in a specific local context


MHRA
Healthcare System
Regulation (products)
HTA
Appraisal
Industry
Guidance
Data
Regulation
Slide 9

Interaction between regulation and HTA

1. A shared requirement for effectiveness data
2. A shared interest in minimising the cost of information
3. Need for compatibility of guidance given to healthcare professionals and to patients.

Effectiveness (and 'safeness')

- Relates to health benefit obtained under actual conditions of use

- Can differ substantially from efficacy (and safety) against placebo under optimised conditions of contrast in an RCT
    - Actual dose, duration and frequency of use
    - Patient characteristics
    - Co-prescription, co-morbidity

Examples: COX-2 inhibitors; sibutramine

A Comparison of Cost Effectiveness Using Data from Randomized Trials or Actual Clinical Practice: Selective Cox-2 Inhibitors as an Example

Tjeerd-Pieter van Staa[1,2*], Hubert G. Leufkens[2], Bill Zhang[1], Liam Smeeth[3]

PLoS Med 6(12): e1000194. doi:10.1371/journal.pmed.1000194

1 General Practice Research Database, London, United Kingdom
2 Utrecht Institute for Pharmaceutical Sciences, Utrecht University, Utrecht, The Netherlands
3 London School of Hygiene & Tropical Medicine, London, United Kingdom

Mean cost per case avoided: COX-2 inhibitors ($US, thousands)

	Mean Cost (95% CI)
Event probabilities from GPRD	52 (39-73)
Event probabilities from RCTs:	
• VIGOR	8 (6-10)
• CLASS	10 (8-13)
• Rofecoxib meta-analysis	14 (11-19)
• Celecoxib meta-analysis	21 (17-27)
• Etoricoxib meta-analysis	14 (12-20)

Measuring effectiveness

1. Large conventional trials with wide inclusion criteria (and enrolment) to maximise external validity and with clinically relevant outcome measures

2. Randomisation within normal care

3. Epidemiological analysis of comprehensive clinical data sets

Each of these also has the power to assess risk-benefit

Scope for efficiencies and synergies between regulation and HTA

1. Joint work between industry, regulators and HTA/appraisal bodies on product development planning

2. Scrutinising the cost-efficiency of research regulation

3. Moving R&D resources towards effectiveness studies as soon as possible in product development

4. Exploitation of health informatics to capture analysable data on outcomes under usual conditions of use

5. Coherence of guidance provided to patients, professionals and payers

6. Advocating the value of innovation for advancing the quality of health care

Conclusions

- Have considered regulator/HTA interactions only in respect of pharmaceuticals
- Regulation and HTA are distinct and different functions
- Their main point of overlap is the estimation of effectiveness
- Containing the cost (and thus the feasibility) of product development must be a public health priority

"HTA is global, appraisal is local"

John Eisenberg