Designing drug development to optimize HTA decisions

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Designing drug development to optimize Health Technology Assessment (HTA) decisions

- Healthcare paradigm
- HTA
- Issues for statisticians in drug development

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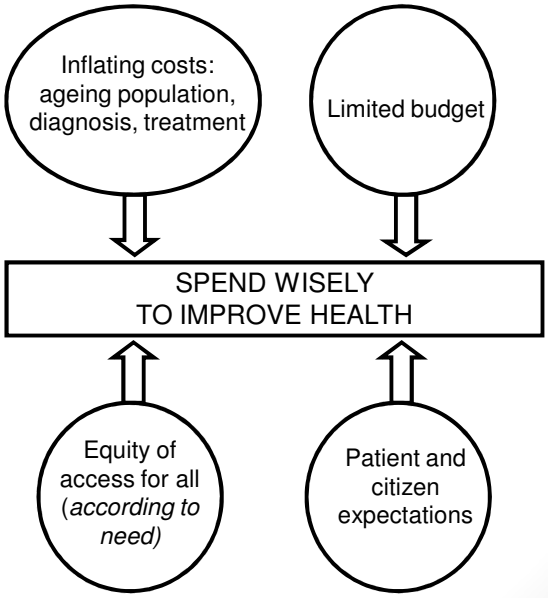
Current healthcare paradigm



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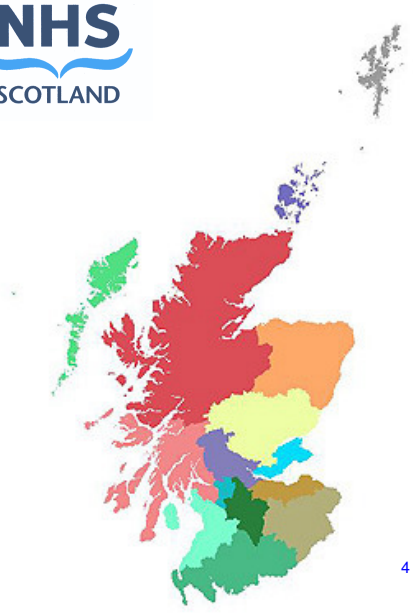
- Population = 5.2 million, budget ~£11 billion
- Taxation based health system, no co-payments
- 14 health boards - payers/providers providing primary, community, acute care
- Challenges – financial austerity, ageing population, expensive new treatments/devices
- ~12% spent on prescribing in primary care
- Drug prices set by UK, devices negotiated through Scottish and local procurement

NHS SCOTLAND



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Health Technology Assessment (www.eunethta.net)

HTA is a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to use of a health technology* in a systematic, transparent, unbiased, robust manner

It aims to inform policy at national, regional or hospital level.

*screening , vaccines, diagnostics, medicines, devices, education, rehabilitation….

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EUnetHTA: European network for HTA

- Improve cross-border collaboration/avoid duplication
- Establish effective, sustainable European HTA network
- Create tools to
  - Support relative effectiveness assessments (in collaboration with EMA)
  - Produce a 'core' model for HTA that creates consistency and allows key data to be shared (Piloted with Industry)
  - Share knowledge of studies underway to collect extra evidence for HTAs

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EUnetHTA HTA Core Model

1. Health problem
2. Technical description of technology
3. Safety
4. Clinical Effectiveness
5. Costs and economic evaluation
6. Ethical analysis
7. Organisational aspects
8. Social aspects
9. Legal aspects

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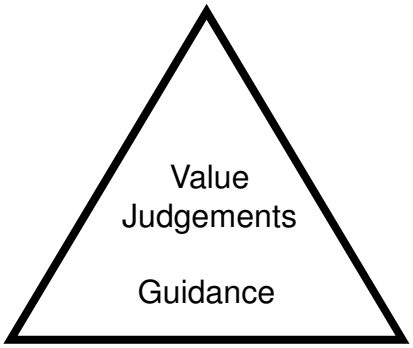
HTA: Evidence based decision-making

Scientific publications and submissions
(including evidence from a range of sources)



Needs and preferences of patients & carers

Health professionals' experience

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Clinical Effectiveness
(Relative effectiveness/Comparative Effectiveness)

- Evaluation of benefit/risk in a standard clinical setting
  - no upper age restrictions, concurrent medical conditions, polypharmacy
  - compared to best standard care (BSC)
- Measuring outcomes that demonstrate added clinical value
  - long-term – survival, delayed progression
  - QOL (being able to dress, walk, work…)
- Network meta-analysis (NMA) when no trials against BSC

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Cost effectiveness
(Economic evaluation)

Economic evaluation has been defined as

*' the **comparative** analysis of alternative courses of action in terms of both their **costs** and **consequences**'* (Drummond & McGuire, 2001)

An evolving art: modelling what happens to patients in the long-term, in the light of major uncertainties

Guidelines from NICE, SMC, PBAC, CADTH…

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Issues in Economic Evaluation

- Obtaining robust outcomes relating to quality of life (QOL) and utilities
- Timescale of evaluation
  - want to show long term benefits to offset costs
  - is rationale for extrapolation reasonable?
- Determining costs of treatment associated with technology and comparator over a long time horizon
- Uncertainty and its impact need to be quantified - Probabilistic Sensitivity Analyses

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Issues for drug development

- Pragmatic study designs
- Outcomes that demonstrate added clinical value
- Duration of treatment
  - Reassessment, how?
  - Continue responders
- Duration of effect – extrapolation…
- National studies of resource utilisation
- Identify patients that benefit most

- *Rules of regulation don't' apply*
- *Creating an evidence base for HTA*

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Scientific Advice

- Individual Agencies alone and in collaboration with national regulatory agency
- EMA in collaboration with EUnetHTA
- Tapestry
- Green Park Collaborative

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Statistical potential?

- Providing experience from the clinical development programme to explain the value of the product
- Building the NMA and economic model
- Phase III trials designed to collect evidence required for HTA
- Critically appraising model inputs
- Assessing uncertainty
- Patient Access/Evidence Generation Schemes Post Marketing

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