The Value of Reference Case Methods for Resource Allocation Decision Making

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Outline

- Two perspectives:
 - Model evaluator
 - Decision maker (NICE)
- NICE and its Reference Case
- The value of <u>additional</u> disease-specific standardisation



National Institute of Health and Clinical Excellence (NICE)

- Technology appraisal relevant to this discussion
- Considers 20-30 technologies each year, mainly new and mainly pharmaceutical
- Offers guidance to NHS their effectiveness and costeffectiveness
- Decisions greatly informed by manufacturers' and assessment group models
- Between 1 and 5 models per appraisal



The NICE Reference Case

- Revised methods guidelines in 2004
- Range of motivating factors for Reference Case:
 - The nature of NICE's decisions
 - Consistency between appraisals
 - Consistency within appraisals
- Reference Case is prescriptive and generic
- But no intention to thwart methods development or innovative techniques
- Reference case \neq standardisation



NICE Reference Case

Element of health technology assessment	Reference case	Section providing details
Defining the decision problem	The scope developed by the Institute	5.3.2
Comparator	Alternative therapies routinely used in the NHS	5.3.2
Perspective on costs	NHS and PSS	5.3.3
Perspective on outcomes	All health effects on individuals	5.3.3
Type of economic evaluation	Cost-effectiveness analysis	5.3.4
Synthesis of evidence on outcomes	Based on a systematic review	5.4.1
Measure of health benefits	Quality-adjusted life years (QALYs)	5.5
Description of health states for calculation of QALYs	Health states described using a standardised and validated generic instrument	5.5
Method of preference elicitation for health state valuation	Choice-based method, for example, time trade-off, standard gamble (not rating scale)	5.5
Source of preference data	Representative sample of the public	5.5
Discount rate	An annual rate of 3.5% on both costs and health effects	5.7.2
Equity position	An additional QALY has the same weight regardless of the other characteristics of the individuals receiving the health benefit	5.9.7

Source: National Institute for Clinical Excellence (NICE). *Guide to the Methods of Technology Appraisal*. London: NICE, 2004.



Areas of potential tension between diseasespecific and generic Reference Cases

- Measures of health outcomes
 - QALYs vs. disease specific
- Choice of comparators
 - Need to incorporate all relevant comparators
 - Actual choice needs clinical knowledge and experience



What the generic Reference Case misses

- Considerable variation between alternative models for a given appraisal
- Choice of comparators
- Characterising heterogeneity at baseline
 - Severity
 - Risk status
- Measure of treatment response
 - May define who stays on treatment
- Measures to characterise prognosis
 - Typically determines key costs
 - Typically determines QoL weights
- Measures of treatment effect



The example of psoriatic arthritis

Sources of heterogeneity

Measures of treatment response

Little specific

PsARC Joint count (ACR20)

Characterisation of prognosis

Treatment effects

HAQ Joint count

Differences in PsARC Differences in HAQ Differences in joint count

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Conclusions

- For cross-disease decision making, value in a <u>generic</u> Reference Case
- Major potential role for disease/technology-specific references cases
- Ultimate example: the 'agreed single model'
 - Used for all interventions in a given disease area
 - Structure and core parameterisation publicly available
 - Will be updated over time
- But getting consensus is usually not easy!

