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

<table>
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<tr>
<th>Sources of heterogeneity</th>
<th>Little specific</th>
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<tr>
<td>Measures of treatment response</td>
<td>PsARC&lt;br&gt; Joint count&lt;br&gt; (ACR20)</td>
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<tr>
<td>Characterisation of prognosis</td>
<td>HAQ&lt;br&gt; Joint count</td>
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<tr>
<td>Treatment effects</td>
<td>Differences in PsARC&lt;br&gt; Differences in HAQ&lt;br&gt; Differences in joint count</td>
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Conclusions

• For cross-disease decision making, value in a generic 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!