Nice Guidelines For Economic Evaluation: Methodological Developments and Remaining Uncertainties

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Economic evaluation for NICE – the Reference Case

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Background

• Technology Appraisal at NICE issues guidance on around 20 technologies per year
• Range of medical technologies selected
• Economic evaluation plays a major role in decision
  – Manufacturer submission
  – Independent technology assessment review
• Initial methods guidance published in 2001
• Focussed largely on economic evaluation methods
• Very general guidance – little prescriptive material
• 2002 House of Commons Select Committee on Health report recommended updated guidance by end-2003
Outline

• Guiding principles
• Reference case
Guiding principles
Objectives and constraints

- Objective of maximising some measure of health gain
- Budget constraint: NHS/PSS resources
  - Implies particular cost perspective
- Long-term time horizon
- Clear implications for defining the decision problem (relevant to UK practice)
- Other constraints difficult to operationalise
  - Fixed budget
  - Equity
  - ‘Political’ constraints
Guiding principles

Need for comparability

• Both within and between submissions
• Generic measure of health gain
  – Comparison within and between submissions
  – Quantifying opportunity costs
Guiding principles

Need for synthesis and modelling

- Inevitable need to draw evidence from number of sources
- Need for systematic methods to identify evidence on all parameters
- Large demand on evidence synthesis methods
- Need appropriate analytical framework – statistical or decision analytic model
  - Framework for synthesis
  - Characterise uncertainty
Guiding principles
Requirements for evidence

• Recognition of different types of data needed to estimate cost-effectiveness

• Evidence required to estimate parameters will differ
  – RCT evidence needed to estimate relative treatment effect
  – Not essential for natural history, cost and quality of life parameters

• Imperfections in evidence base inevitable
  – Imprecise estimates
  – Not on appropriate patients

• Need to use all available data, be explicit about limitations and modelling assumptions, and quantify additional uncertainty
The concept of the reference case

- Concept supported by Washington Panel in 1990s
- Range of uncertainties in (economic) evaluation methods – values and technical
- But need for consistency in approach for decision making
- Reference case defines the methods which should be used in one particular analysis
- Does not preclude other additional analyses
- When reference case not possible, submissions still encouraged, absence of reference case justified and additional uncertainty should be quantified
### Summary of reference case requirements (1)

<table>
<thead>
<tr>
<th>Element</th>
<th>Reference case</th>
</tr>
</thead>
<tbody>
<tr>
<td>Defining the decision problem</td>
<td>Consistent with NICE’s scope</td>
</tr>
<tr>
<td>Perspective on costs</td>
<td>NHS and PSS</td>
</tr>
<tr>
<td>Perspective on outcomes</td>
<td>All health effects on individuals</td>
</tr>
<tr>
<td>Type of study</td>
<td>Cost-effectiveness analysis</td>
</tr>
</tbody>
</table>
## Summary of reference case requirements (2)

<table>
<thead>
<tr>
<th>Element</th>
<th>Reference case</th>
</tr>
</thead>
<tbody>
<tr>
<td>Synthesis of outcome evidence</td>
<td>Systematic review</td>
</tr>
<tr>
<td>Measure of health benefits</td>
<td>QALYs</td>
</tr>
<tr>
<td>Health state descriptions</td>
<td>Validated generic measure</td>
</tr>
<tr>
<td>Method of preference elicitation</td>
<td>Choice-based</td>
</tr>
</tbody>
</table>
### Summary of reference case requirements (3)

<table>
<thead>
<tr>
<th>Element</th>
<th>Reference case</th>
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</thead>
<tbody>
<tr>
<td>Source of preference data</td>
<td>Sample of public</td>
</tr>
<tr>
<td>Discount rate</td>
<td>Annual rate of 3.5% on costs and health effects</td>
</tr>
<tr>
<td>Equity</td>
<td>QALY given the same weight for all recipients</td>
</tr>
<tr>
<td>Dealing with parameter uncertainty</td>
<td>Probabilistic methods</td>
</tr>
</tbody>
</table>
Two areas of controversy

- Probabilistic sensitivity analysis
- Measurement and valuation of health