Recently updated methods guidelines: Discussion

Mark Sculpher

Centre for Health Economics

University of York, UK
Purpose

• Comparing and contrasting guidelines
• Reflections on NICE’s methods and 2007 update
Nature of the decision making organisation and its decisions

- Which technologies
  - Pharmaceuticals only
  - Includes others (e.g. devices, procedures)

- Is there clarity about the system’s objective function?

- Where does the budget constraint fall
  - Is there one in the system?
  - Does the decision maker hold the budget?
  - Is the decision maker an agent for the system?
  - How is the budget constraint reflected in decisions?

- How limited is a pharmaceutical’s license?
Key differences between methods guidelines

Definition of the decision problem

• Licensed indication vs routine use

• Specifying comparators
  – ‘Standard practice’ (many)
  – All feasible options
  – Big analytical implications

• Definition of relevant sub-groups – at what stage?
# Variability in methods guidelines

**Choice of comparator (n=27)**

<table>
<thead>
<tr>
<th>Comparator</th>
<th>Frequency</th>
</tr>
</thead>
<tbody>
<tr>
<td>Most commonly used</td>
<td>8</td>
</tr>
<tr>
<td>Existing, most effective or minimum practice</td>
<td>2</td>
</tr>
<tr>
<td>Existing or most effective</td>
<td>1</td>
</tr>
<tr>
<td>Justify</td>
<td>1</td>
</tr>
<tr>
<td>Existing and no treatment</td>
<td>1</td>
</tr>
<tr>
<td>Most common, least costly, no treatment</td>
<td>2</td>
</tr>
<tr>
<td>Most common, least costly, no treatment, most effective</td>
<td>1</td>
</tr>
<tr>
<td>Most common, least costly, most effective</td>
<td>2</td>
</tr>
<tr>
<td>Most likely to be displaced</td>
<td>1</td>
</tr>
<tr>
<td>Most efficient, most effective, do nothing</td>
<td>2</td>
</tr>
<tr>
<td>All relevant comparators</td>
<td>2</td>
</tr>
<tr>
<td>Most effective and no treatment</td>
<td>1</td>
</tr>
<tr>
<td>Not clear/specific</td>
<td>3</td>
</tr>
</tbody>
</table>

Key differences between methods guidelines

Role of modelling

• Prominent view: models necessary when trial data inadequate

• Alternative view: models always needed for decision making:
  – Include all appropriate comparators
  – Synthesise all relevant evidence
  – Appropriate time horizon (often lifetime)
  – Fully quantify decision uncertainty
Key differences between methods guidelines

Role of QALYs

- Emerging consistency
- Reflects needs of decision making
- Limitations clear – are there any feasible alternatives
- Quest for increasing consistency in QALYs
- Are other outcomes ever appropriate?
Key differences between methods guidelines

Importance of uncertainty

• Analysis of uncertainty has always featured in guidelines (albeit with variation)
• Increasing prominence of probabilistic sensitivity analysis
• Experience of NICE – how should uncertainty be used in making decisions?
  – Role of conventional inference
  – Concept of decision uncertainty
  – Irreversabilities and sunk costs
  – Linking decisions to the needs of future research
## Variability in methods guidelines

Methods for sensitivity analysis (n=27)

<table>
<thead>
<tr>
<th>Method Description</th>
<th>Count</th>
</tr>
</thead>
<tbody>
<tr>
<td>Need to state and justify</td>
<td>3</td>
</tr>
<tr>
<td>Not stated/not specific</td>
<td>10</td>
</tr>
<tr>
<td>Probabilistic sensitivity analysis (PSA)</td>
<td>3</td>
</tr>
<tr>
<td>One-way, multi-way</td>
<td>1</td>
</tr>
<tr>
<td>One-way, two-way</td>
<td>2</td>
</tr>
<tr>
<td>Multi-way (of most important)</td>
<td>1</td>
</tr>
<tr>
<td>One-way, multi-way and PSA</td>
<td>5</td>
</tr>
<tr>
<td>One-way, multi-way and worst-best scenario</td>
<td>1</td>
</tr>
<tr>
<td>One-way with tornado diagram</td>
<td>1</td>
</tr>
</tbody>
</table>

Tam TY, Smith MD. Pharmacoeconomic guidelines around the world. ISPOR Connections 2004;10(4):5.
NICE methods update 2007

Methods workshops

- Indirect and mixed treatment comparisons
- Heterogeneity and sub-groups
- Costs
- Utilities
- Uncertainty
- Diagnostics
Thank you!

Email: mjs23@york.ac.uk

www.york.ac.uk/inst/che/research/teehta.htm