Making decisions about health care: methods for estimating the benefits from investments in health

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Overview

• National Institute for Health and Care Excellence (NICE)
  – Evolving role

• Principles of making decisions
  – Net health effects of a technology
  – Informing pricing decisions

• What methods are required?
  – Measure of health
  – Comparison of relevant alternatives
  – Using all relevant evidence
  – Representing uncertainty
  – What threshold for cost-effectiveness should be applied?

• Results of NICE appraisal
The evolving role of NICE

Source: M Goodall NICE
Why economic analysis?

- **Price > P* £60,000**
- **Price = P* £40,000**
- **Price < P* £20,000**

**Cost-effectiveness Threshold £20,000 per QALY**

**Net Health Benefit**
- **Net Health Benefit 1 QALY**
- **Net Health Benefit -1 QALY**
Price, value and innovation

$P^* = \text{VBP on average for } Q^*$

Value of the innovation = $P^*. Q^*$
All value is appropriated by manufacturer
Price, guidance and volume

\[ P^* = \text{VBP on average for } Q^* \]
Price, guidance and volume

<table>
<thead>
<tr>
<th>Choose</th>
<th>Coverage</th>
<th>Revenue</th>
<th>Net Benefit</th>
</tr>
</thead>
<tbody>
<tr>
<td>P1</td>
<td>S1</td>
<td>P1.Q1</td>
<td>0</td>
</tr>
<tr>
<td>P2</td>
<td>S1+S2</td>
<td>P2.Q2</td>
<td>A</td>
</tr>
<tr>
<td>P3</td>
<td>S1+S2+S3</td>
<td>P3.Q*</td>
<td>A+B</td>
</tr>
</tbody>
</table>

The diagram illustrates the relationship between price, coverage, revenue, and net benefit. The table above provides the data for three different options (P1, P2, P3) with their respective coverage (S1, S1+S2, S1+S2+S3) and the resulting revenue and net benefit.
What methods are required?

• Need to estimate
  – Health effects and costs
  – For each of the alternatives available
  – To treat specific target (sub) population(s)
  – Using all relevant evidence
  – Over the period when costs and benefits will differ
  – Represent uncertainty in the estimates

• What cost-effectiveness threshold should be applied?
## NICE Methods – reference case

<table>
<thead>
<tr>
<th>Element of health technology assessment</th>
<th>Reference case</th>
<th>Section details</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Defining the decision problem</strong></td>
<td>The scope developed by NICE</td>
<td>5.1.4</td>
</tr>
<tr>
<td>**Comparator(s)</td>
<td>As listed in the scope developed by NICE</td>
<td>2.2.4 to 5.1.14</td>
</tr>
<tr>
<td><strong>Perspective on outcomes</strong></td>
<td>All direct health effects, whether for patients or, when relevant, carers</td>
<td>5.1.7, 5.1.15</td>
</tr>
<tr>
<td><strong>Perspective on costs</strong></td>
<td>NHS and PSS</td>
<td>5.1.9</td>
</tr>
<tr>
<td><strong>Type of economic evaluation</strong></td>
<td>Cost–utility analysis with fully incremental analysis</td>
<td>5.1.11 to 5.1.15</td>
</tr>
<tr>
<td><strong>Time horizon</strong></td>
<td>Long enough to reflect all important differences in costs or outcomes between the technologies being compared</td>
<td>5.1.15</td>
</tr>
<tr>
<td><strong>Synthesis of evidence on health effects</strong></td>
<td>Based on systematic review</td>
<td>5.2</td>
</tr>
<tr>
<td><strong>Measuring and valuing health effects</strong></td>
<td>Health effects should be expressed in QALYs. The EQ-5D is the preferred measure of health-related quality of life in</td>
<td>5.3.1</td>
</tr>
</tbody>
</table>

| Source of data for measurement of health-related quality of life | Reported directly by patients and/or carers                                   | 5.3.3           |
| Source of preference data for valuation of changes in health-related quality of life | Representative sample of the UK population                                  | 5.3.4           |
| Equity considerations | An additional QALY has the same weight regardless of the other characteristics of the individuals receiving the health benefit | 5.4.1           |
| Evidence on resource use and costs | Costs should relate to NHS and PSS resources and should be valued using the prices relevant to the NHS and PSS | 5.5.1           |
| Discounting | The same annual rate for both costs and health effects (currently 3.5%) | 5.6.1           |

NICE, National Institute for Health and Care Excellence; NHS, National Health Service personal social services; QALYs, quality-adjusted life years; EQ-5D, standardised for use as a measure of health outcome.
Need a measure of health

• Compare the effects of the alternatives available
  – Different multiple effects (length, quality of life, side effects)
  – Effects on subsequent disease

• Consistency and accountability in how decisions are made

• Comparison with health displaced
  – Across a range of different disease areas

• Generic/general description of health (states)

• Weights relative to full health
  – Reflecting community preferences
  – How much life expectancy give up to return to full health
Costs restricted to NHS and PSS

• Primary purpose of the NHS is to improve health
• Cost and benefits outside NHS
  – Costs of care borne by patients and families
  – Impact on the wider economy (net productivity)
• Cant be treated in the same way as NHS costs
  – Benefits cant be used to offer health care
  – Cost don't displace health
• Potentially socially divisive (e.g., age discrimination)
  – Why include some and not others
• Would need assess displaced wider effects
  – Danger that reduce health and reduce net wider benefits
Comparison of alternatives

- Not restricted to comparators in licensing trial
- Not restricted to current clinical practice
- Not restricted to licensed use
Comparison of alternatives

- Not restricted to comparators in trials
- Not restricted to current clinical practice
- Not restricted to licensed use
Comparison of alternatives

- Not restricted to comparators in trials
- Not restricted to current clinical practice
- Not restricted to licensed use

ICER = £80,178

ICER = £551,900

ICER = £18,783
Synthesis of relevant evidence

• Meta-analysis
• Indirect and mixed treatment comparisons

Three trials, each making a pair-wise comparison

<table>
<thead>
<tr>
<th>Trial ID</th>
<th>Alternative interventions for advanced ovarian cancer</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Paclitaxel (Pac)</td>
</tr>
<tr>
<td>039</td>
<td>53.0 (n=114)</td>
</tr>
<tr>
<td>30-49</td>
<td>-</td>
</tr>
<tr>
<td>30-57</td>
<td>56.3 (n=108)</td>
</tr>
</tbody>
</table>

Median weeks survival (number of patients analyzed)

• Options?
  – Separate pair-wise analysis
  – Indirect treatment comparison (Top as a common comparator)
  – Mixed treatment comparison using all three trials
Reflecting uncertainty in estimates of costs and effects

Clinical effect

Disease Progression

QALY

Costs

Model Structure

<table>
<thead>
<tr>
<th>Treatment A</th>
<th>QALY</th>
<th>Cost</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>1</td>
<td>£10,000</td>
</tr>
<tr>
<td></td>
<td>0</td>
<td>£5,000</td>
</tr>
<tr>
<td></td>
<td>2</td>
<td>£15,000</td>
</tr>
<tr>
<td></td>
<td>1</td>
<td>£10,000</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Treatment B</th>
<th>QALY</th>
<th>Cost</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>2</td>
<td>£30,000</td>
</tr>
<tr>
<td></td>
<td>3</td>
<td>£20,000</td>
</tr>
<tr>
<td></td>
<td>4</td>
<td>£40,000</td>
</tr>
<tr>
<td></td>
<td>3</td>
<td>£30,000</td>
</tr>
</tbody>
</table>
What threshold should be applied?

- NICE threshold range (unchanged since 2004)
- Implied from past decisions

Explicit reference to:
- Certainty
- HRQoL adequately captured?
- Innovative nature
- Social value judgment

An increasingly strong case for ‘special circumstances’ (e.g., EoL)

Probably cost effective

Cost per QALY (£’000)
Results of NICE appraisal (2007 – Sept 2013)

Source: NICE
But what should the threshold be?

• NICE threshold is too high?
  – NICE guidance is doing more harm than good
    • more health is lost than gained
  – Paying too much for new drugs
  – Incentivising wasteful investments

• NICE threshold is too low?
  – Patients unnecessarily denied access
  – Paying too little for new drugs
  – Not sufficiently rewarding valuable innovation

• How should we account for other considerations?
  – e.g., burden of illness and wider social benefits