Economic Evaluation to Support Decision Making: Recent Developments

*Mark Sculpher, PhD*

Professor of Health Economics
University of York, UK

vfa-Symposium 7th April 2009
Benefit- and Cost-Benefit-Analysis in Germany
Outline

• Challenges facing economic evaluation for decision making
• Informed by recent developments at NICE
  – The role of the QALY to inform decisions
  – Are all QALYs equal?
  – The appropriate cost-effectiveness threshold
  – The role of decision models
Measuring health benefits

What should the health metric look like?

• Need to be generic?
  – Decisions across diseases and clinical specialties
  – Need to be able to compare health gain with health opportunity costs

• A role for disease-specific measures of health?
  – Ring-fenced budgets
  – No effects of technologies outside the disease of interest

• Need to combine different dimensions of health
  – Length of life
  – Health-related quality of life

• QALYs accepted by many systems, recommended by fewer
Why the QALY as a generic measure of individual health?

- Some empirical work to suggest QALYs imperfectly reflect individual preferences
- Little empirical work in the context of HTA informing real decisions
- Alternative measures developed but rarely applied (e.g. healthy-year equivalent)
- QALY legitimate to inform decisions
  - Widely used in empirical studies
  - Is (or should be) transparent
  - Strengths and weaknesses understood
  - Experience in alternative formal measures limited
  - Further research essential
Interpersonal comparisons of health gain

“A QALY is a QALY is a QALY”

- Severity of baseline prognosis
- Lifetime health experience
- Non health-related disadvantage
- End of life
- Degree of ‘blame’

Generally known
Generally unknown
Inter-personal comparison of health
The analytic approach

- Concept of an ‘equity weighted’ QALY or a measure of the social value of health
- Literature exists
  - Methods of elicitation
  - Surveys of public preferences
  - Methods to augment/replace QALYs
- Limited use in applied studies
- What characteristics of individuals should be taken into account and who should select these?
- How should these characteristics be weighted/valued and by whom?
Inter-personal comparison of health
The deliberative approach approach

- Unweighted QALY gains in analysis do not mean these remain unweighted in decision making
- Range of factors which could be taken into account other than cost per QALY gained
  - Inadequacy of QALY
  - Characteristics of gainers and losers
  - Innovative nature of the product
  - Sufficiency of evidence
NICE’s ‘end of life’ guidelines

Details of guidelines at end of life

• In contexts where benefits are not adequately captured in Reference Case and ICER>£30,000

• Specific (key) criteria:
  – Life expectancy less than 24 months
  – Good evidence that treatment extends life by at least 3 months

• Further analysis:
  – Is the treatment cost-effective when terminal stage of disease valued as good health?
  – What additional weight needs to be given to the QALY gained to make it cost-effective?

• Follow-up data collection likely

• Relates to small populations
Determining a cost-effectiveness threshold

- Incremental cost per additional unit of benefit (e.g. QALY)
- Comparison of two alternatives:
  - Cost A – Cost B / QALYs A – QALYs B
- The additional cost of achieving one extra unit of benefit
- When is this incremental cost-effectiveness ratio worth paying?
  - Need to compare with the cost-effectiveness threshold
What can the threshold represent?

- Opportunity cost given a fixed budget
- Public’s willingness to pay
  - Effectively determines aggregate expenditure (budget)
- Other:
  - Past decisions – may be wrong!
  - Administrative rule – legitimate?
Threshold with a fixed budget

Basing the threshold on past decisions

Figure 5. Probabilistic cost-effectiveness thresholds for NICE decisions

A societal willingness to pay

- A number of empirical studies on ‘social valuation’ of health against consumption
  - Revealed preference
  - Stated preference: contingent valuation, conjoint methods
- Some studies estimating social value of the QALY
- Could be used to compare with an ICER when no budget constraint
- If budget constraint, then these values do not replace the threshold
  - Health gained and health displaced valued in same way
  - Still need a threshold reflecting the value of what is displaced
Value of health from other sectors

- The value of a statistical life is used in the UK to inform transport investment decisions.
- Also considered by other sectors (e.g., environment).
- These values are based on contingent valuation exercises.
- In principle could be generalised to QALYs.
- Tend to imply a higher valuation of health than NICE.
- Suggestion that government should strive to fund sectors to achieve this value:
  - Other sectors have objectives other than health gain.
  - Budgets reflect government valuation of other objectives.
## The role of modelling to support decisions

### Contrasting paradigms

<table>
<thead>
<tr>
<th>Measurement</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Testing hypotheses about individual parameters</td>
</tr>
<tr>
<td>• Relatively few parameters of interest</td>
</tr>
<tr>
<td>• Primary role for trials</td>
</tr>
<tr>
<td>• Focus on parameter uncertainty</td>
</tr>
</tbody>
</table>

≠

<table>
<thead>
<tr>
<th>Decision making</th>
</tr>
</thead>
<tbody>
<tr>
<td>• What do we do now based on all sources of current knowledge?</td>
</tr>
<tr>
<td>• Decisions cannot be avoided</td>
</tr>
<tr>
<td>• A decision is always taken under conditions of uncertainty</td>
</tr>
<tr>
<td>• Decision making involves synthesis</td>
</tr>
<tr>
<td>• Can be based on implicit or explicit analysis</td>
</tr>
</tbody>
</table>
## Limitations of trials as vehicles for decision making

<table>
<thead>
<tr>
<th>Trial limitations</th>
<th>Modelling responses</th>
</tr>
</thead>
<tbody>
<tr>
<td>Inappropriate or partial comparisons</td>
<td>Indirect and mixed treatment comparison</td>
</tr>
<tr>
<td>More than one trial</td>
<td>Meta-analysis</td>
</tr>
<tr>
<td>Partial measurement</td>
<td>Synthesis of alternative types of evidence</td>
</tr>
<tr>
<td>Unrepresentative practice</td>
<td>Distinguish baseline risks from treatment effects</td>
</tr>
<tr>
<td>Intermediate outcomes</td>
<td>Model links to final outcomes (e.g. QALYs) using non-trial sources</td>
</tr>
<tr>
<td>Limited follow-up</td>
<td>Extrapolation modelling using alternative scenarios</td>
</tr>
</tbody>
</table>
Cost-effectiveness of EVAR in aortic aneurysms – the EVAR1 trial

Relative clinical effect

<table>
<thead>
<tr>
<th></th>
<th>EVAR (n=543)</th>
<th>Open repair (n=539)</th>
<th>Hazard ratio from Cox regression model (95% CI; p)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Crude</td>
<td>Primary adjusted</td>
<td>Secondary adjusted†</td>
</tr>
<tr>
<td>Aneurysm-related deaths†</td>
<td>19 (0.31–0.96; 0.04)</td>
<td>0.55</td>
<td>0.51 (0.29–0.92; 0.02)</td>
</tr>
<tr>
<td>Deaths from all causes</td>
<td>100 (0.69–1.18; 0.46)</td>
<td>0.90</td>
<td>0.88 (0.67–1.16; 0.36)</td>
</tr>
</tbody>
</table>

*Adjusted for age, sex, FEV₁, AAA diameter, log (creatinine), and statin use. †Adjusted for variables in primary adjustment plus BMI, smoking, systolic blood pressure, and serum cholesterol. ‡Deaths within 30 days of surgery for AAA plus deaths with underlying cause given as ICD10 codes (T13–19).

Table 1: Aneurysm-related and all-cause mortality (intention-to-treat analysis)

Cost-effectiveness of EVAR in aortic aneurysms – the EVAR1 trial

Procedural costs

<table>
<thead>
<tr>
<th></th>
<th>EVAR (n=543)</th>
<th>Open repair (n=539)</th>
<th>Mean difference</th>
<th>SE of difference</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Primary hospital admission</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Main procedure</td>
<td>7569</td>
<td>2811</td>
<td>4757</td>
<td>108</td>
</tr>
<tr>
<td>Hospital stay</td>
<td>3015</td>
<td>6304</td>
<td>-3290</td>
<td>568</td>
</tr>
<tr>
<td>Other</td>
<td>235</td>
<td>89</td>
<td>146</td>
<td>34</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td>10819</td>
<td>9204</td>
<td>1613</td>
<td>607</td>
</tr>
<tr>
<td><strong>Secondary procedures, adverse events, scans</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Secondary AAA procedures</td>
<td>1056</td>
<td>200</td>
<td>856</td>
<td>227</td>
</tr>
<tr>
<td>Other adverse events</td>
<td>294</td>
<td>359</td>
<td>-65</td>
<td>169</td>
</tr>
<tr>
<td>Outpatients/CT scan/ultrasound scan*</td>
<td>1089</td>
<td>182</td>
<td>907</td>
<td>37</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td>2439</td>
<td>741</td>
<td>1698</td>
<td>631</td>
</tr>
<tr>
<td>Total cost including 4-year follow up</td>
<td>13258</td>
<td>9945</td>
<td>3313</td>
<td>690</td>
</tr>
</tbody>
</table>

*Average number of outpatient follow-up appointments, CT and ultrasound scans estimated from a survey of trial centres.

Table 6: Estimated costs (UK£) over 4 years follow-up based on intention to treat

Cost-effectiveness of EVAR in aortic aneurysms - need for modelling

Modelling the long-term cost-effectiveness of endovascular or open repair for abdominal aortic aneurysm

D. M. Epstein¹, M. J. Sculpher¹, A. Manca¹, J. Michaels², S. G. Thompson³, L. C. Brown⁴, J. T. Powell⁴, M. J. Buxton⁵ and R. M. Greenhalgh⁴

¹Centre for Health Economics, University of York, York, ²Sheffield Vascular Institute, Sheffield, ³Medical Research Council Biostatistics Unit, Institute of Public Health, University of Cambridge, Cambridge, ⁴Department of Vascular Surgery, Imperial College, London, and ⁵Brunel University, Uxbridge, UK

Correspondence to: Mr D. M. Epstein, Centre for Health Economics, University of York, York YO10 5DD, UK
(e-mail: dme2@york.ac.uk)

<table>
<thead>
<tr>
<th></th>
<th>Cost (£)</th>
<th>QALYs</th>
</tr>
</thead>
<tbody>
<tr>
<td>EVAR</td>
<td>15,823 (14,606, 17,418)</td>
<td>5.050 (3.685, 6.172)</td>
</tr>
<tr>
<td>Open repair</td>
<td>12,065 (10,358, 14,144)</td>
<td>5.070 (3.754, 6.123)</td>
</tr>
<tr>
<td>Difference</td>
<td>3,758 (2,439, 5,183)</td>
<td>-0.020 (-0.189, 0.165)</td>
</tr>
</tbody>
</table>
Cost-effectiveness of EVAR in aortic aneurysms

Non-trial evidence

• Need for modelling to estimate long-term cost-effectiveness

• Use of non-trial evidence on
  – Non-AAA mortality - general population
  – Non-AAA mortality – additional risk in AAA population
  – ‘Frailty’ effect
  – Risks by sub-group
  – Costs and quality of life associated with longer term effects
Is there an acceptance of modelling?

- Position on modelling varies internationally
- Few systems unequivocally reject models
- Less widely seen as a ‘trial versus model’ dichotomy
- A decisions involved assumptions and judgements, models can make these explicit
- Importance of quantifying uncertainty
Thanks...

http://www.york.ac.uk/inst/che/staff/sculpher.htm

Centre for Health Economics’ short courses:
http://www.york.ac.uk/inst/che/training/index.htm#short