Economic Evaluation to Support Decision Making: Recent Developments

Mark Sculpher, PhD

Professor of Health Economics
University of York, UK

vfa-Symposium 21st April 2009
Benefit- and Cost-Benefit-Analysis in Germany

THE UNIVERSITY of York

CHE
Centre For Health Economics

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

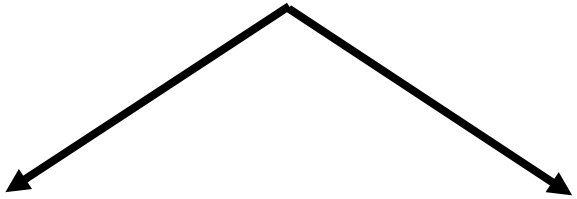
Why the QALY as a generic measure of individual health?

- 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'



Those that gain health	Those that lose health
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

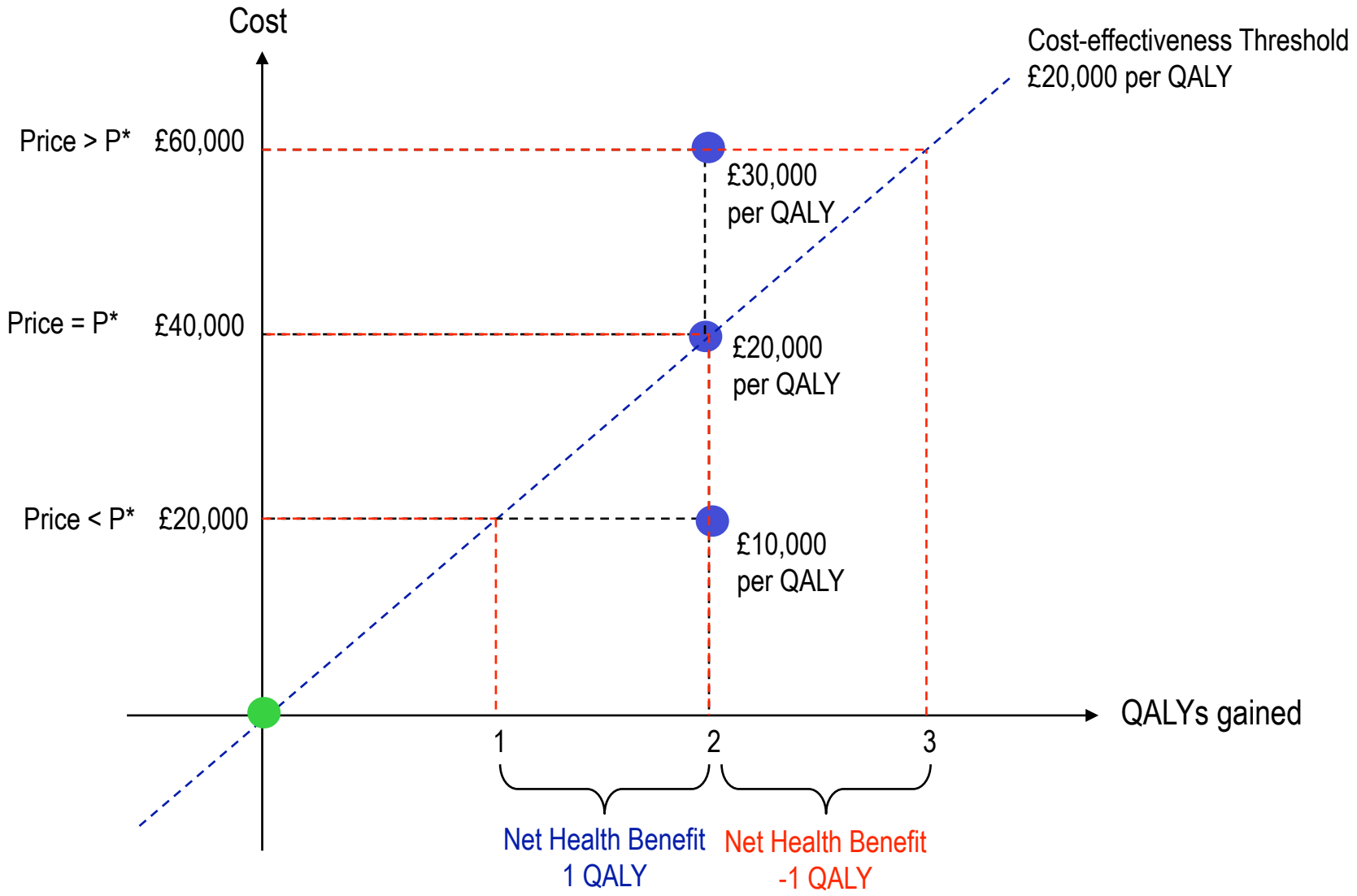
- Incremental cost per additional unit of benefit (e.g. QALY)
- Comparison of two alternatives:

  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



Claxton *et al. British Medical Journal* 2008;336:251-4.

Basing the threshold on past decisions

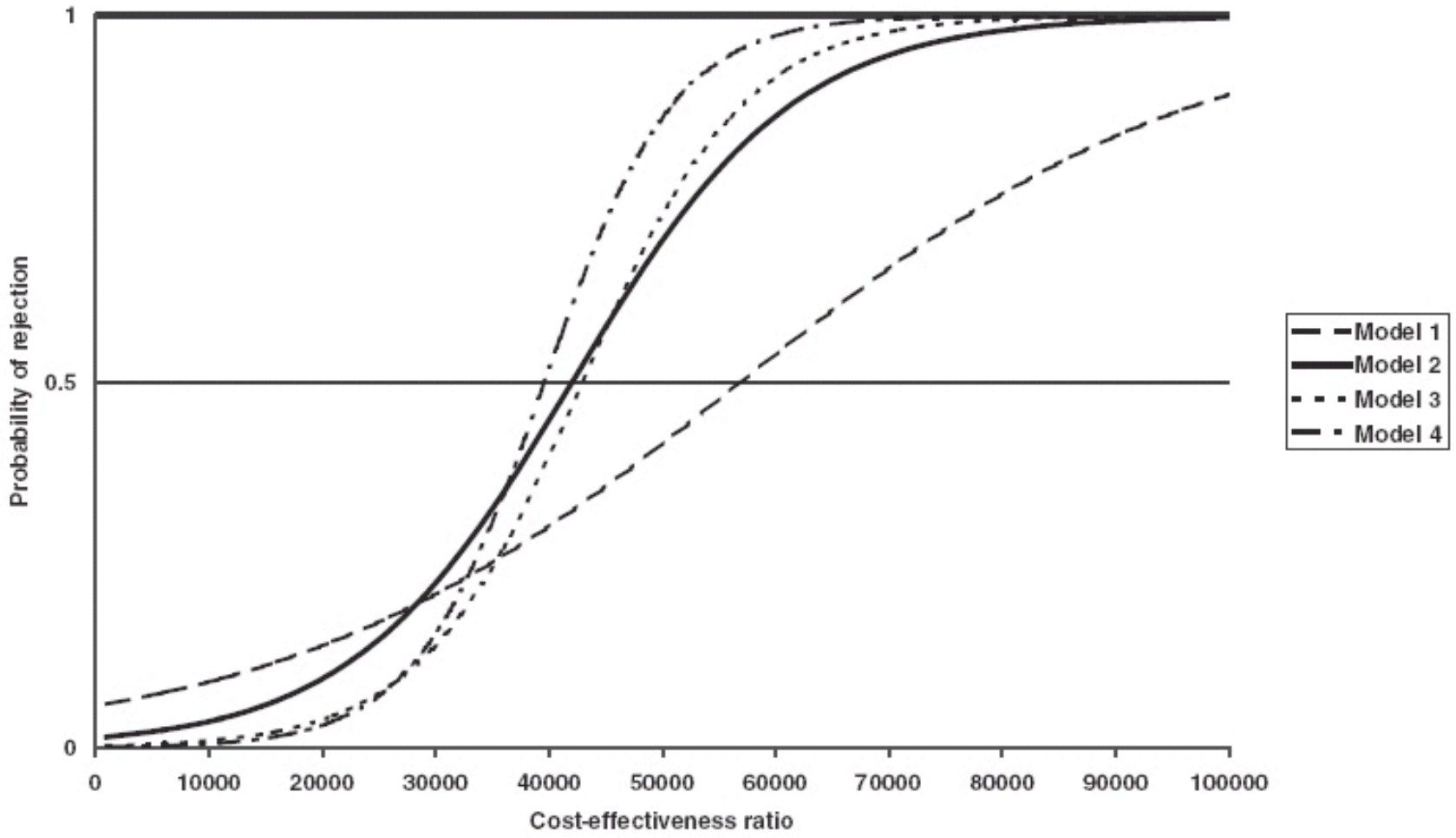


Figure 5. Probabilistic cost-effectiveness thresholds for NICE decisions

Source: Devlin N, Parkin D. *Health Economics* 2004;13:437-52.

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

Measurement

- Testing hypotheses about individual parameters
- Relatively few parameters of interest
- Primary role for trials
- Focus on parameter uncertainty

≠

Decision making

- What do we do now based on all sources of current knowledge?
- Decisions cannot be avoided
- A decision is always taken under conditions of uncertainty
- Decision making involves synthesis
- Can be based on implicit or explicit analysis

Limitations of trials as vehicles for decision making

Trial limitations	Modelling responses
Inappropriate or partial comparisons	Indirect and mixed treatment comparison
More than one trial	Meta-analysis
Partial measurement	Synthesis of alternative types of evidence
Unrepresentative practice	Distinguish baseline risks from treatment effects
Intermediate outcomes	Model links to final outcomes (e.g. QALYs) using non-trial sources
Limited follow-up	Extrapolation modelling using alternative scenarios

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

Relative clinical effect

	EVAR (n=543)	Open repair (n=539)	Hazard ratio from Cox regression model (95% CI; p)		
			Crude	Primary adjusted*	Secondary adjusted†
Aneurysm-related deaths‡	19	34	0·55 (0·31–0·96; 0·04)	0·55 (0·31–0·96; 0·04)	0·51 (0·29–0·92; 0·02)
Deaths from all causes	100	109	0·90 (0·69–1·18; 0·46)	0·90 (0·69–1·19; 0·46)	0·88 (0·67–1·16; 0·36)

*Adjusted for age, sex, FEV_1, 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 I713–19.

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

EVAR Trial Participants, *Lancet* 2005;365: 2179-2186

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

Procedural costs

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

*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

EVAR Trial Participants, *Lancet* 2005;365: 2179-2186

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. Epstein[1], M. J. Sculpher[1], A. Manca[1], J. Michaels[2], S. G. Thompson[3], L. C. Brown[4], J. T. Powell[4], M. J. Buxton[5] and R. M. Greenhalgh[4]

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

	Cost (£)	QALYs
EVAR	15 823 (14 606, 17 418)	5·050 (3·685, 6·172)
Open repair	12 065 (10 358, 14 144)	5·070 (3·754, 6·123)
Difference	3758 (2439, 5183)	–0·020 (–0·189, 0·165)

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