Cost-Effectiveness Analyses in the UK - Lessons from the National Institute for Clinical Excellence

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Background

- Brief overview of NICE
- Methods issues with formal cost-effectiveness analysis for decision making
- Dealing with uncertainty

The National Institute for Health and Clinical Excellence (NICE)

- Following election of Labour government 1997
- Prolonged controversy about ‘post code prescribing’ in the UK National Health Service
- Wish to ‘de-politicize’ decisions about which technologies to cover in NHS
- Desire to use best available methods to address difficult questions

The NICE process

Overview

The NICE process

Selection

- Focus on pharmaceuticals but not exclusively
- Not all new technologies selected
- Separate committee identifies priorities against criteria:
  - High clinical need
  - Potential for significant health gain
  - Potential for significant cost impact
  - Potential to free up resources
- Some freedom to suggest priorities
- Room for dialogue between NICE and manufacturer
- New collaborative arrangements around ‘scoping’

The NICE process

Assessment – independent report

- Undertaken by academic groups (mainly 6 contracted to NICE), typically over a period of 6 months
- 3 key elements of the review:
  - systematic review of clinical and economic evidence
  - cost-effectiveness analysis
  - critical review of sponsor (manufacturer) submission(s)
- TAR team invited to participate in appraisal committee meeting, but not decision making
- All documents (and economic model) made available to consultees

The NICE process

Assessment – consultee submissions

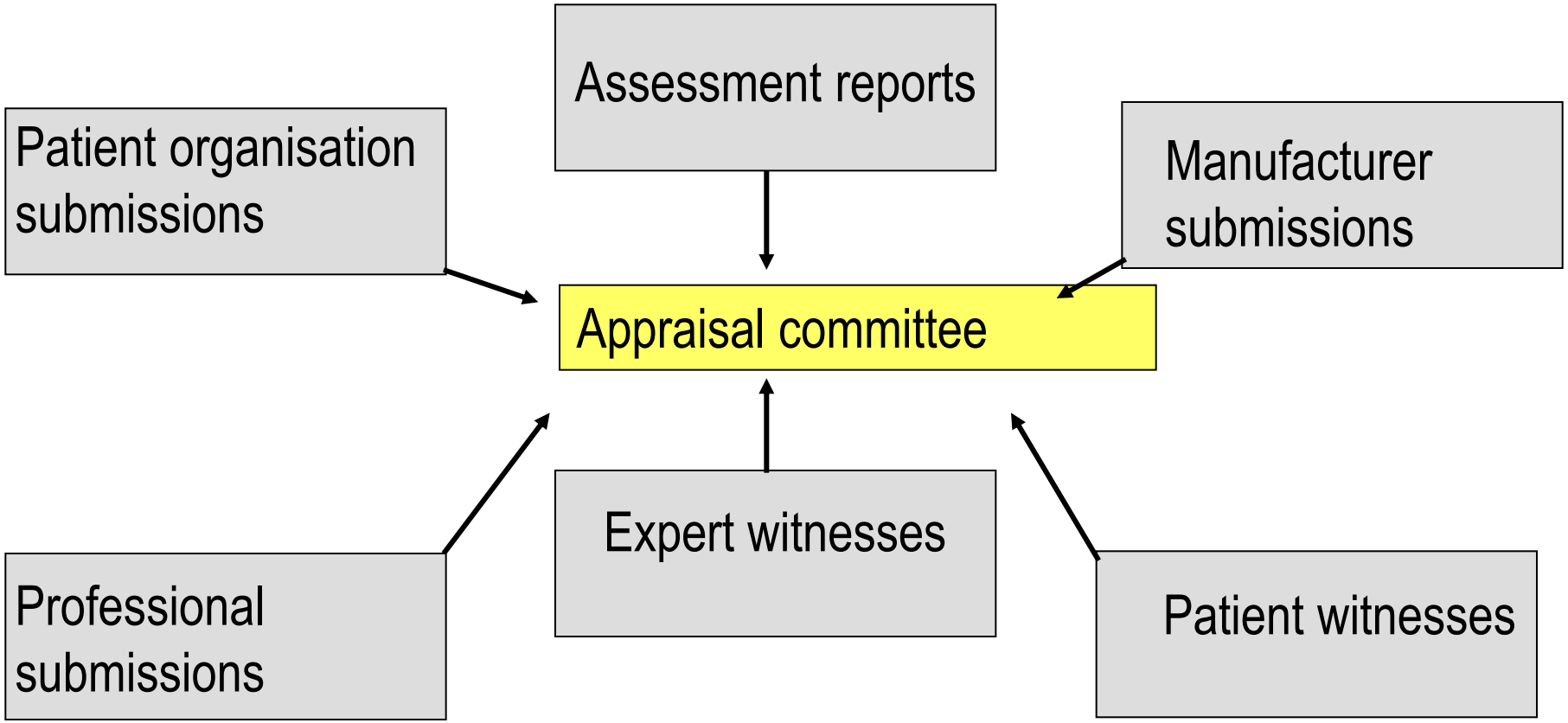
- Most important ones from manufacturers
- Key contribution to appraisal process:
  - provision of unpublished data
  - development of own model to synthesise evidence
- Attention paid to explaining discrepancies between company and TAR analyses
- Some collaboration between academic team and company in developing models
- Debate about the decision often centres around model
- Guidance on methods currently being updated (see www.nice.org.uk)

New Single Technology Assessments

- Concern about delay in giving guidance
- From 2006, a new process for ‘some’ drug technologies
- All evidence and analysis comes from a single manufacturer
- Assessment team provides a critical review of submission – no independent analysis
- Decision making similar although burden of proof now more firmly with manufacturer

The NICE process

Appraisal



The NICE process

Decisions

- Unconditional reimbursement
- Reimbursement conditional on future research
- Reimbursement conditional on particular patient characteristics
- Unconditional refusal to reimburse
- Opportunity for appeal
- Decisions are reviewed in future

The impact of cost-effectiveness on NICE decisions

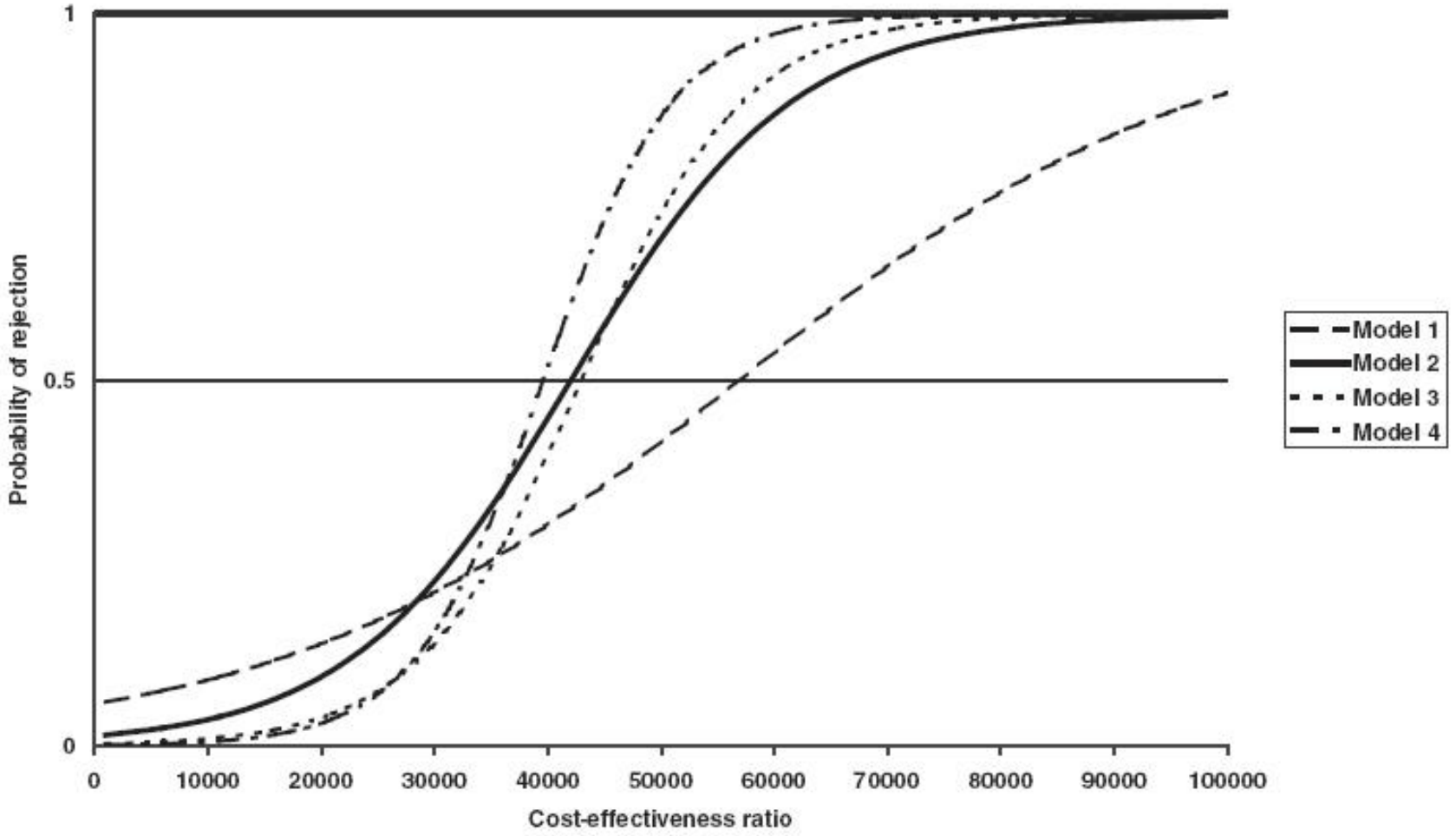


Figure 5. Probabilistic cost-effectiveness thresholds for NICE decisions

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

NICE's preferred methodology – the Reference Case

Element of health technology assessment	Reference case	Section providing details
Defining the decision problem	The scope developed by the Institute	5.3.2
Comparator	Alternative therapies routinely used in the NHS	5.3.2
Perspective on costs	NHS and PSS	5.3.3
Perspective on outcomes	All health effects on individuals	5.3.3
Type of economic evaluation	Cost-effectiveness analysis	5.3.4
Synthesis of evidence on outcomes	Based on a systematic review	5.4.1
Measure of health benefits	Quality-adjusted life years (QALYs)	5.5
Description of health states for calculation of QALYs	Health states described using a standardised and validated generic instrument	5.5
Method of preference elicitation for health state valuation	Choice-based method, for example, time trade-off, standard gamble (not rating scale)	5.5
Source of preference data	Representative sample of the public	5.5
Discount rate	An annual rate of 3.5% on both costs and health effects	5.7.2
Equity position	An additional QALY has the same weight regardless of the other characteristics of the individuals receiving the health benefit	5.9.7

Source: National Institute for Clinical Excellence (NICE). *Guide to the Methods of Technology Appraisal*. London: NICE, 2004.

Methods issues with NICE

Comparators

- Getting the question right is the most fundamental methods issue:
  - Population/sub-populations
  - Comparators
- Importance of clear scope
- Constraint of license
- Agreed in advance before manufacturer and independent submissions

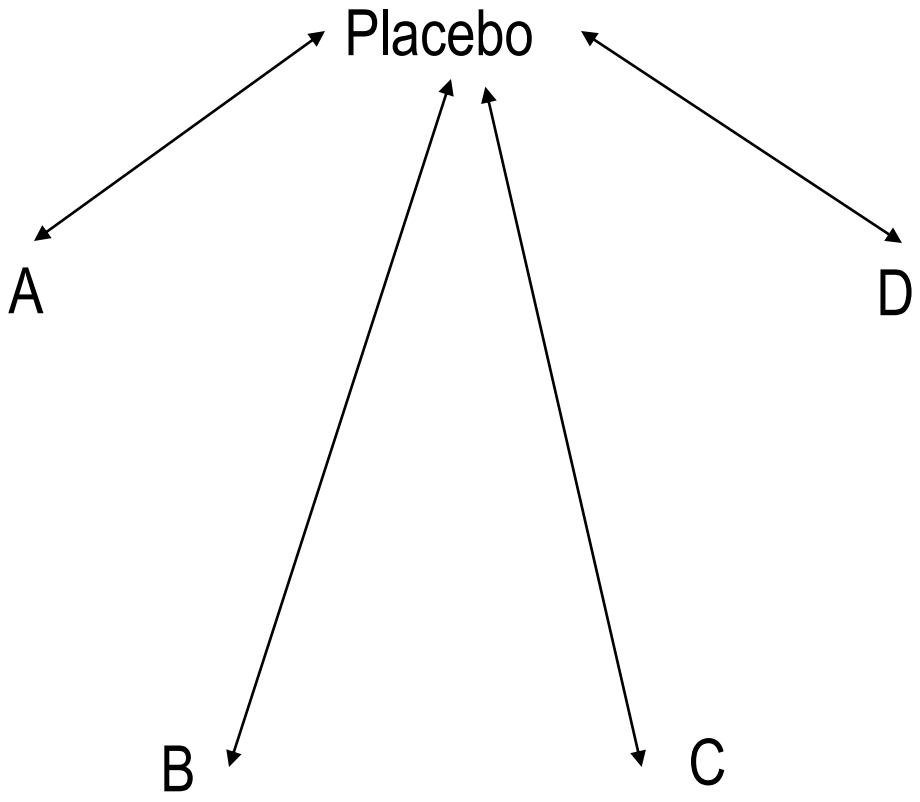
Methods issues with NICE

Evidence synthesis

- How often will an economic evaluation be based on a single trial?
  - Limited comparators, no other evidence
- For more comparators, head-to-head trials unlikely
- Likely need to handle indirect and mixed treatment comparisons

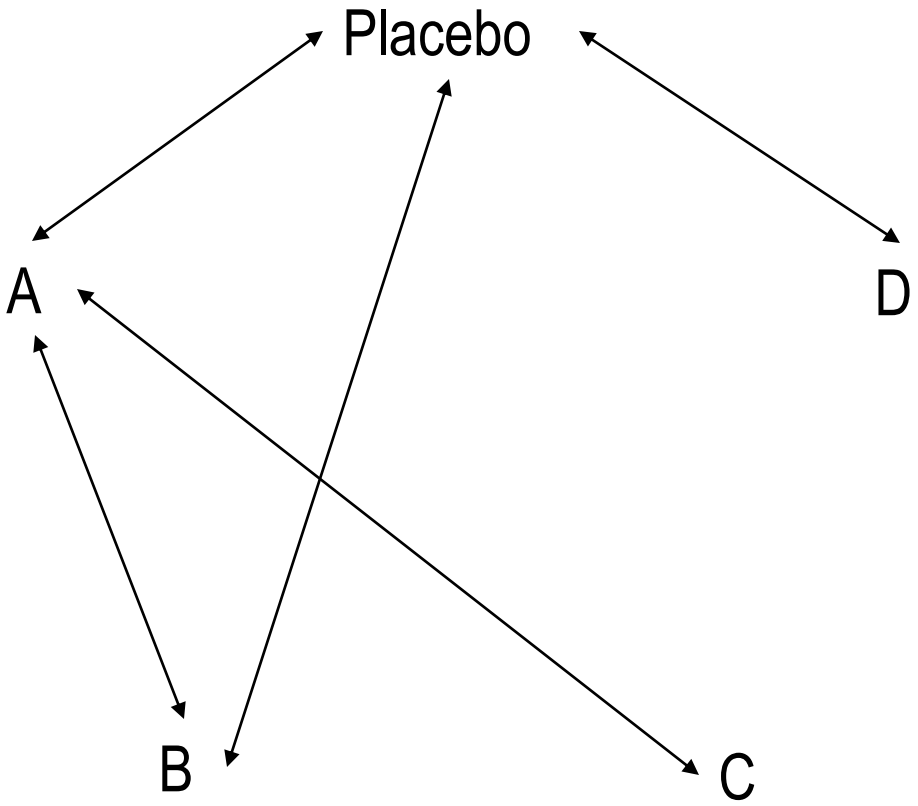
Methods issues with NICE

Synthesising evidence – indirect comparison



Methods issues with NICE

Synthesising evidence – mixed treatment comparison



Methods issues with NICE

Dealing with uncertainty

- Evidence on relevant costs and effects typically imprecise
- Range of other uncertainties
- Is there a role for statistical inference?
- Key issue is decision uncertainty – probability of a technology/intervention being cost-effective
- But how is this used in decision making?

Case study – Glycoprotein IIb/IIIa antagonists in acute coronary syndrome

Strategy 1: GPA as part of initial medical management [7 trials]

Strategy 2: GPA in patients with planned percutaneous coronary interventions (PCIs) [1 trial]

Strategy 3: GPA as adjunct to PCI [10 trials]

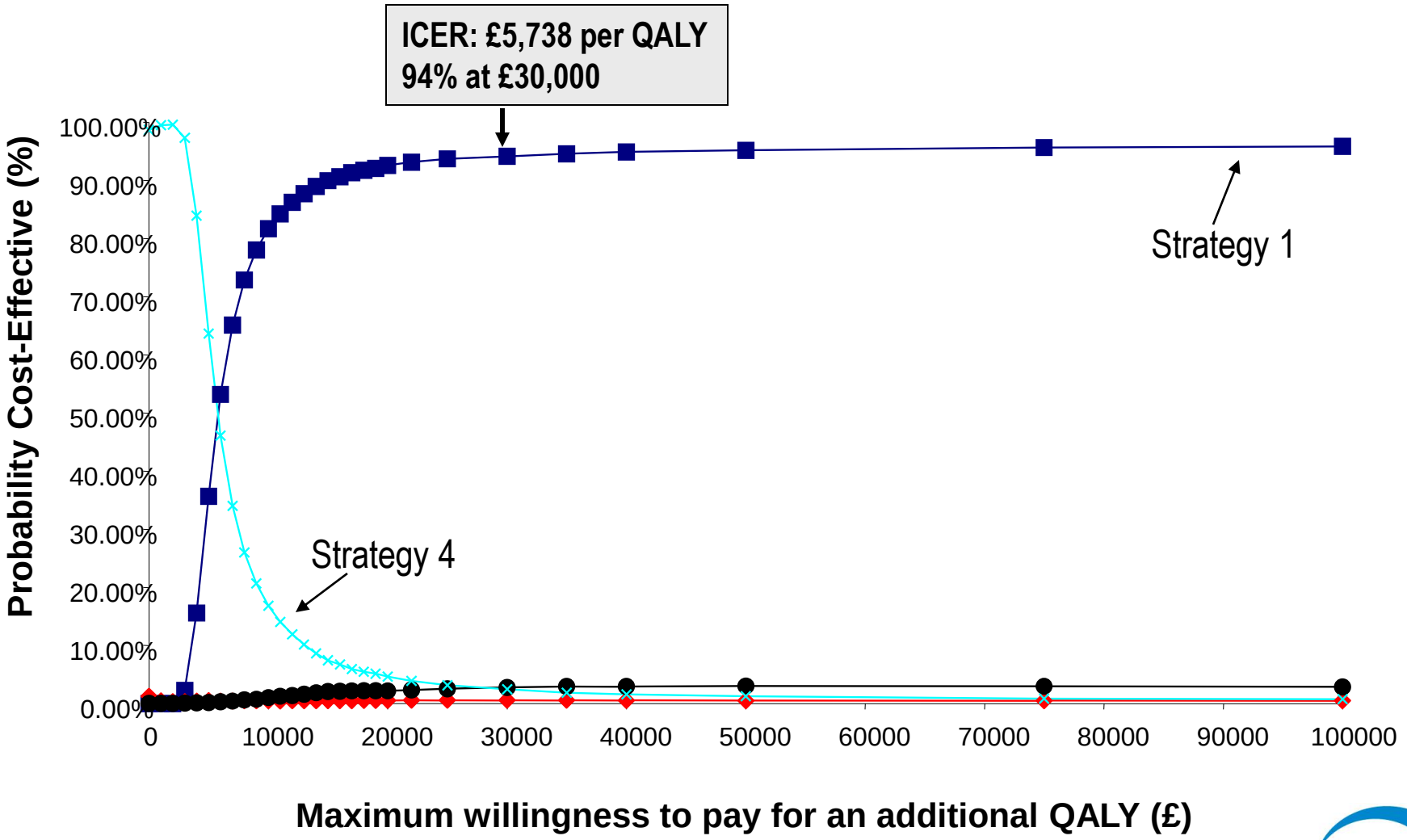
Strategy 4: No use of GPA

Palmer, S *et al.* *International Journal of Cardiology*, **100**: 229-240.

Limitations with GPA trials

Trial characteristic	Modelling method
Extensive trial evidence on treatment effect	Random effects meta-analysis of relative risks
Partial comparison	Pooled relative risks from trials applied to common baseline risks
Non-UK case-mix and clinical practice	UK-specific baseline risks from observational study. Relationship between baseline risks & treatment effect explored with meta-regression
No resource use data	Resource use data from UK observational study attached to clinical events
Short-term time horizon	Extrapolation from 6 months based on Markov model populated from UK observational study

Decision uncertainty



Two decisions for new technologies

Is the technology cost-effective based on existing evidence?

Is additional research cost-effective?	Yes	No
Yes	Adopt Demand additional evidence Revisit decision	Do not adopt Demand additional evidence Revisit decision
No	Adopt Do not demand extra evidence Review decision if other evidence emerges	Do not adopt Do not demand extra evidence Review decision if other evidence emerges

Should we demand additional research?

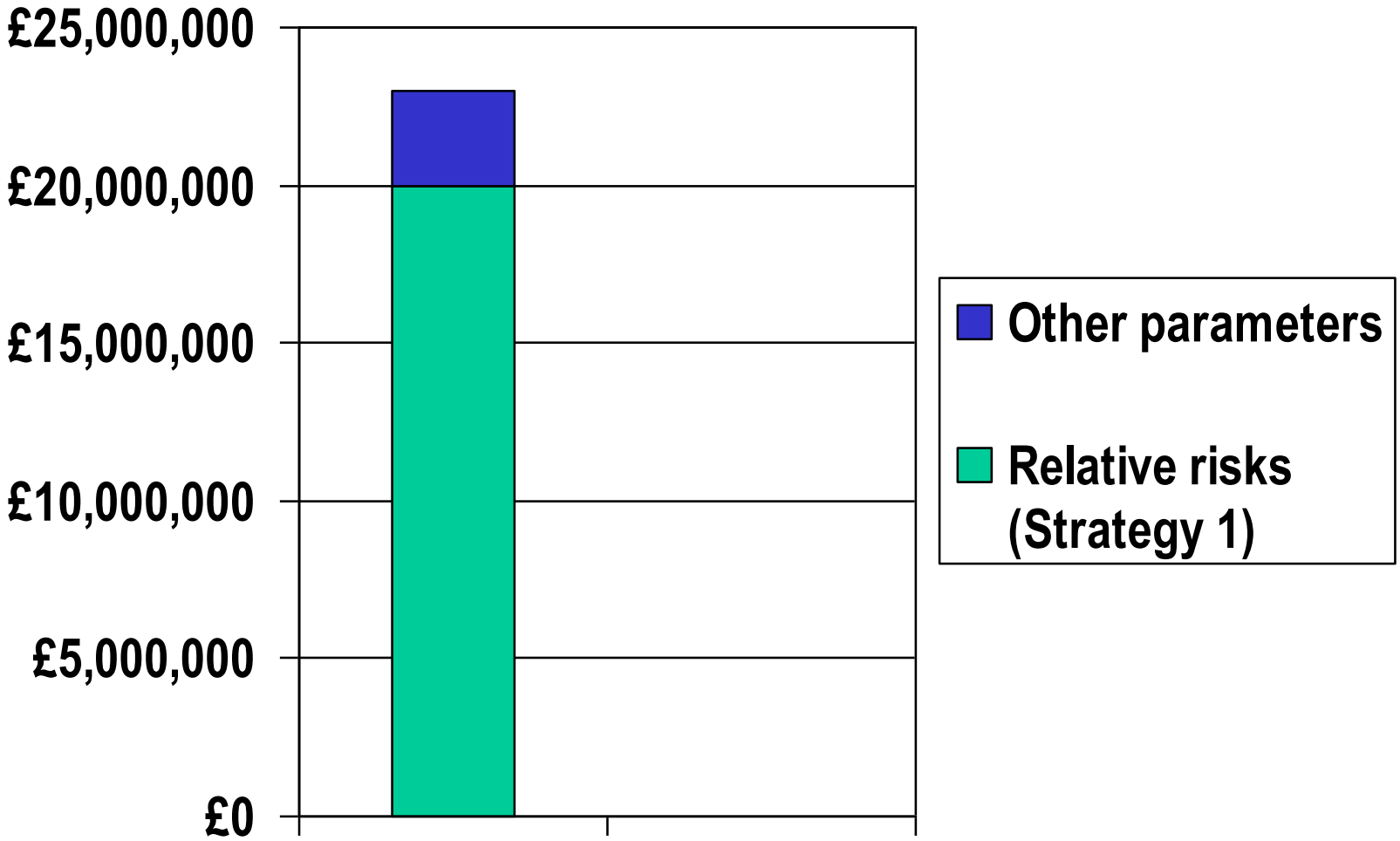
Decision uncertainty	X	Implications of getting it wrong	=	Value of perfect information

- What is the probability of the wrong decision?
- Joint effect of uncertainty in all parameters

What are the implications of a wrong decision in terms of resources and health?

- Sets an upper bound on the value of further research
- Can be calculated overall and for individual parameters
- Calculated per patient and across a population of patients

GPA example: value of information



Assumes research is useful for 10 years and a QALY is valued at £30,000

Philips Z *et al. International Journal of Technology Assessment in Health Care*, vol. 22, pp.379-387.

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When the decision maker does not control research

Balancing two costs

- Decision makers may not control research
- What is appropriate decision when
  - Seems cost-effective on average
  - High decision uncertainty
- Need to balance two costs:
  - If accept: value of research forgone
  - If reject: value to patients and health system