

When Do We Have Enough Evidence?

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***NICE Conference: The future of drug evaluation in the
UK: a debate***

The context

Launch

Market access

Routine use

Regulatory trials

- comparators?
- which patients?

Limited head-to-head

Some safety

Phase IV

- comparative?
- randomised?

Knowledge about use

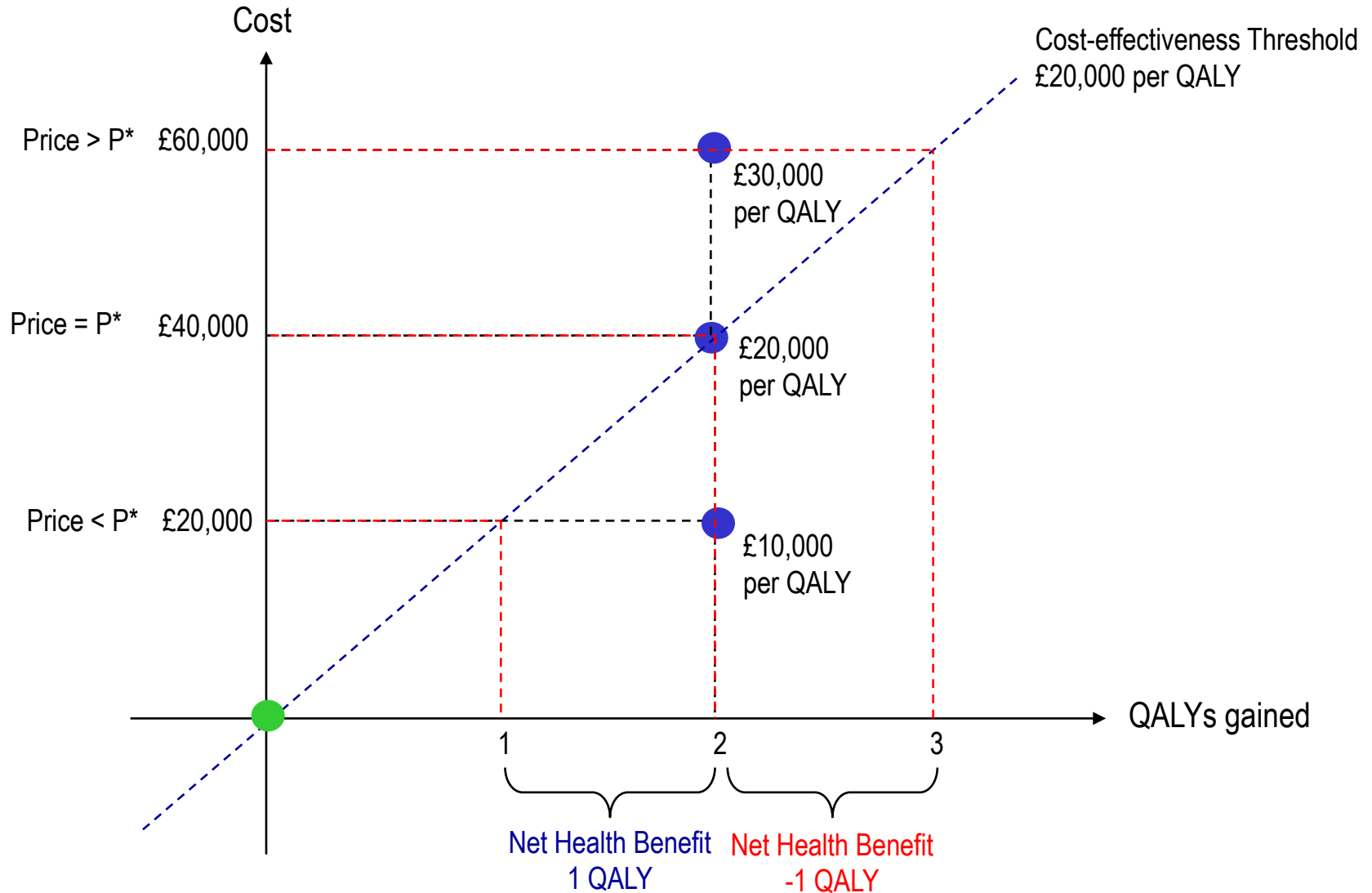
Safety data increases

Limited evidence

Potential for more evidence

Limits to gaining more evidence

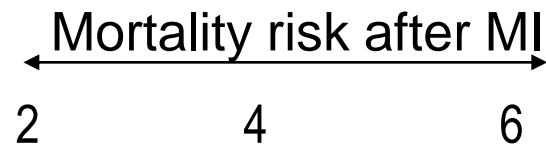
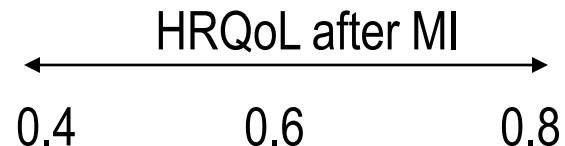
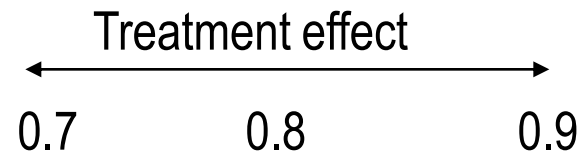
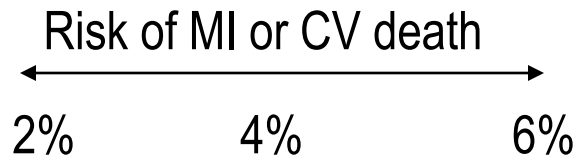
The basic appraisal decision



Uncertainty matters

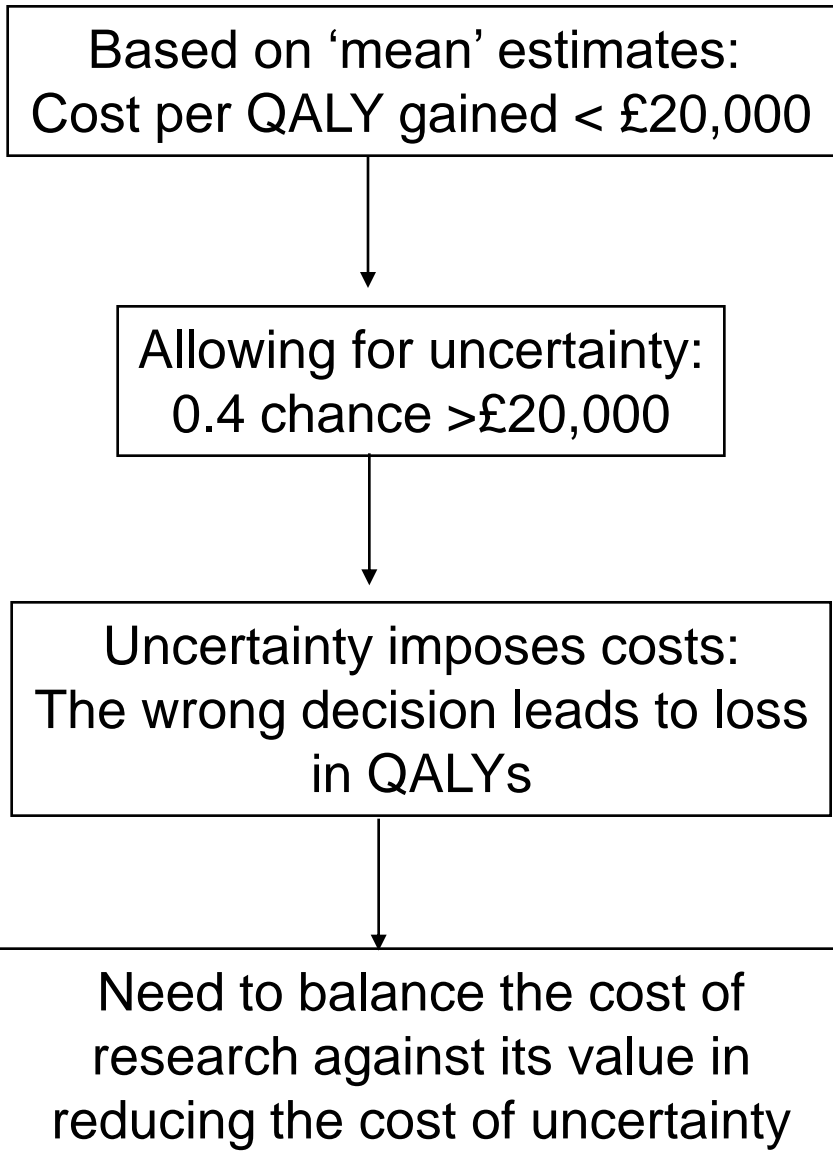
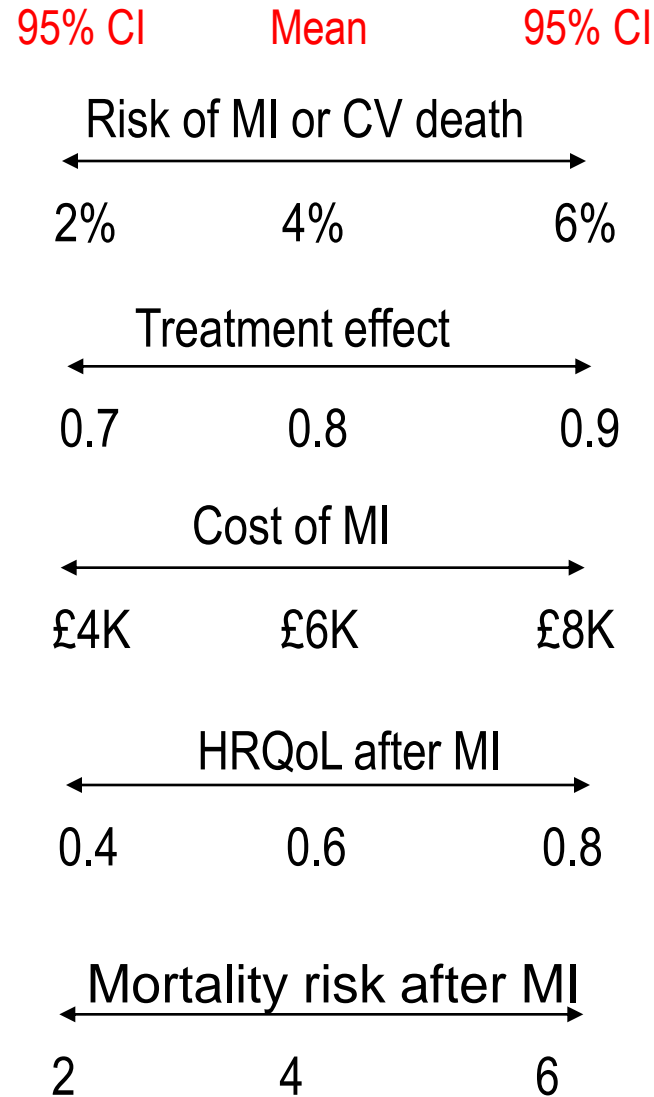
The evidence

95% CI Mean 95% CI



Uncertainty matters

The evidence

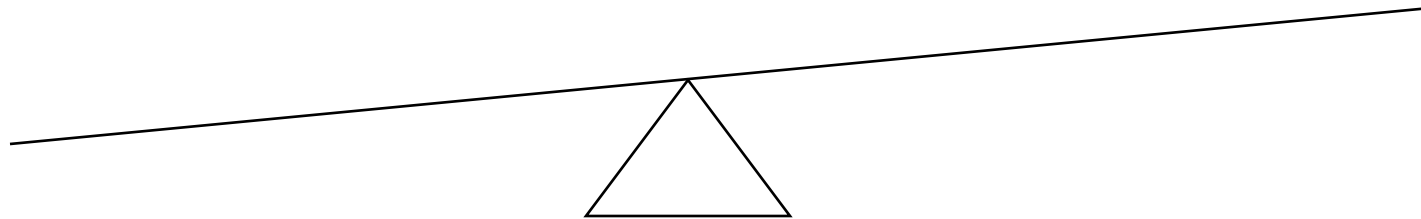


Making decisions under uncertainty

A two-decision world

- Costs of research are too high compared to reduction in cost of uncertainty
- 'External' research is underway and the costs of reversing the decision modest
- Further research will not be disincentivised
- No more research is feasible

- Research of value: costs of research are low compared to reduction in cost of uncertainty
- High costs of reversing a decision following research
- 'Yes' decision likely to disincentivise research



Positive guidance

Negative guidance

Making decisions under uncertainty

More nuanced decisions

Arrangement

Considerations

Only in research

- How different from a 'no' decision?
- Cost per QALY < £20,000
- Clear justification
- Define what research is needed

Patient access schemes

- Reduce effective price of product
- Lowers cost per QALY
- Can reduce the cost of uncertainty

Conditional guidance

- Define what research is needed
- Will research be undertaken by manufacturer?
- What cost to the NHS?
- Cost of reversing the decision

Conditional guidance
(at lower effective price)

- How is effective price lowered?
- Can incentivize research (get premium price)
- Cost of NHS undertaking the research
- Cost of reversing the decision

Bright horizons

- The spirit of Cooksey – collaborative arrangements with NHS
- Better evidence at launch
 - NICE's Scientific Advisory Programme – principal focus on design of Phase 3 trials
- Early cost-effectiveness modelling
 - Quantify likelihood of licence and reimbursement
 - NICE sets clear and transparent framework
 - Can have international influence
 - NHS input of modelling for UK?
 - Social value \neq commercial value: share cost of development

Conclusions

- Appraisal decisions are initially addressed when evidence is inevitably limited
- Evidence has value in terms of health, just like interventions
- Sufficiency of evidence rests of when the cost of undertaking research cannot be justified by the reduction in uncertainty
- Appraisal Committee's decisions influence use of interventions and likelihood of future research
- Need explicit framework for how uncertainty is factored into NICE decisions
- Need for more nuanced decisions to reflect value of additional evidence