What clinical evidence do we need for HTA?

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CONFLICTS OF INTEREST

I work with Scottish Medicines Consortium but also advise pharma companies on HTA plans
RETHINK PHASE III, OR REWORK FOR HTA?

Work backwards from the Committee saying 'yes'

THE S DRAW K CAB PRINCIPLE
To get a committee to say yes you need:

- A **cost-effectiveness** case that convinces the committee (e.g. a ‘base case’ cost per QALY without too many holes!)
- Exploration of **uncertainty** that convinces the committee very poor cost-effectiveness is unlikely
- But also ‘**buy-in**’ from the clinicians around the committee table
  - Unlikely to be specialists
  - Look to patient-orientated outcomes
  - Look for chunky (or non-trivial?) differences
- Do they need a head-to-head RCT to do this?

**HTA committee members …**

- Generally prefer head-to-head RCTs
- However they rarely get them and have to learn about the various techniques used in HTA
  - Modelling to address evidence synthesis
  - Indirect comparisons to address ‘the wrong comparator’ in the RCT
  - Extrapolation to address inadequate RCT follow-up
  - Allowance for crossover (e.g. RPSFT)
- So don’t stereotype them
Do we use a new stats technique?

‘Jobbing clinician’ reaction

- Don’t know this – look to independent stats advisor
- How does it compare to what my common sense tells me?
- How much difference does it make to cost per QALY?

Implication

- Does not limit choice of technique
- Affects how it is used e.g. only in sensitivity analysis
- Definitely influences how it is presented

Which is fine if they’re calling the shots ...

A LOT OF INDUSTRY THINKING IS DEDICATED TO THE PROFESSIONAL REVIEWERS
**A new stats technique in HTA**

**Hinders**
- Being used for the 1st time
- Technical explanation (especially equations!)
- Used in conjunction with other statistical ‘patches’ on the data
- Pivotal factor in effectiveness and cost-effectiveness

**Helps**
- Preferably reviewed by NICE DSU or similar
- ‘Buy-in’ from committee’s trusted statistician
- Intuitive explanation backed up by pictures
- Used to make conservative claims about effect size

**Some issues**

- Do you agree HTA ‘patches’ are more likely than big changes in RCT design at Phase 3?

- Companies do think about the audience for HTA but do they do it in the way I have described?

- When people feel suspicious of complicated statistical ‘patches’ should we educate them up or dumb down the statistics message?
Question

1. Does your organization design drug development programs to meet the needs of regulators and payers? What would you say are the key success/limiting factors for doing this well?

Question

2. Does your organization encourage or impede effective collaboration cross-functionally when designing drug development programs? What would you say are the key success/limiting factors for collaborating cross-functionally?
Question

3. Are all the relevant stakeholders involved in drug development programs also involved in the review/preparation of payer submission documents? What would you say are the key success/limiting factors for doing this well?

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4. From your experience, what needs to change to ensure drug development programs integrate reimbursement requirements?
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