VALUING A CURE: ARE NEW APPROACHES NEEDED?

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Complexity in evaluation

- 'Standard'
- System disruption
- Few patients
- Fragile intellectual property
- Non-health effects
- Claims for high social value
- Evidential uncertainty
- Affordability & upfront costs
Complexity in evaluation

- System disruption
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Cost-effectiveness and affordability

Cost

Price = P* £40,000

£20,000 per QALY

QALYs gained

2
Cost-effectiveness and affordability

Health opportunity cost: 1 QALY per £20,000 (Budget impact 1)

QALYs gained

Cost

Price = P* £40,000

£20,000 per QALY

2 QALYs gained

2 QALYs opportunity cost

Cost-effectiveness and affordability

Health opportunity cost: 1 QALY per £20,000 (Budget impact 1)

QALYs gained

Cost

Price = P* £40,000

£20,000 per QALY

2 QALYs gained

2 QALYs opportunity cost
Cost-effectiveness and affordability

Health opportunity cost: 1 QALY per £20,000 (Budget impact 1)

Price = $40,000 per QALY

QALYs gained

Cost

Net Health Benefit -1 QALY

1 QALY per £13,333 (Budget impact 2)

QALYs gained

Cost

Health opportunity cost: 1 QALY per £20,000 (Budget impact 1)

Price = $40,000 per QALY

2 QALYs gained
Estimating opportunity cost by budget impact

Resolving the “Cost-Effective but Unaffordable” Paradox: Estimating the Health Opportunity Costs of Nonmarginal Budget Impacts

Lomas et al. Value in Health, 2018, 21:266-275

Evidential uncertainty – example of oncology

- Mortality risk in delayed event
- Plateau in OS
- Long-term OS treatment effect
- Long-term OS
- Separation in OS curves
- PFS as a surrogate for OS

Quality-adjusted survival
Example in oncology

Decision uncertainty and its implications

- **Cost-effective**
  - Adopt: True positive: gains in health
  - Reject: False negative: loss in health

- **Not cost-effective**
  - Adopt: False positive: loss in health
  - Reject: True negative: no change

Othus et al. Value in Health 2017;20:705-9
Dealing with uncertainty

**Analysis**
- Magnitude and cost of uncertainty
- Key uncertainties
- Feasibility & timing of research
- Irrecoverable costs
- Impact of price changes

**Policy responses**
- Fund
- Reject
- Price reduction
- Risk-sharing
- Fund only in research
- Fund with research

Should we value putative cures differently?

**Product A**
- 2 life-years gained
- Good QoL
- Die of disease

**Product B**
- 2 life-years gained
- Good QoL
- Die of other causes
Conclusions

- Evaluation challenges not unique to ‘cures’
- The toolkit exists, needs to be used
- Always need for further development
  - Survival modelling
  - Policy responses to uncertainty
  - Policy responses to high upfront costs

Thank you!
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