Cost-effectiveness analysis to inform reimbursement, allocation, and prioritisation decisions in health

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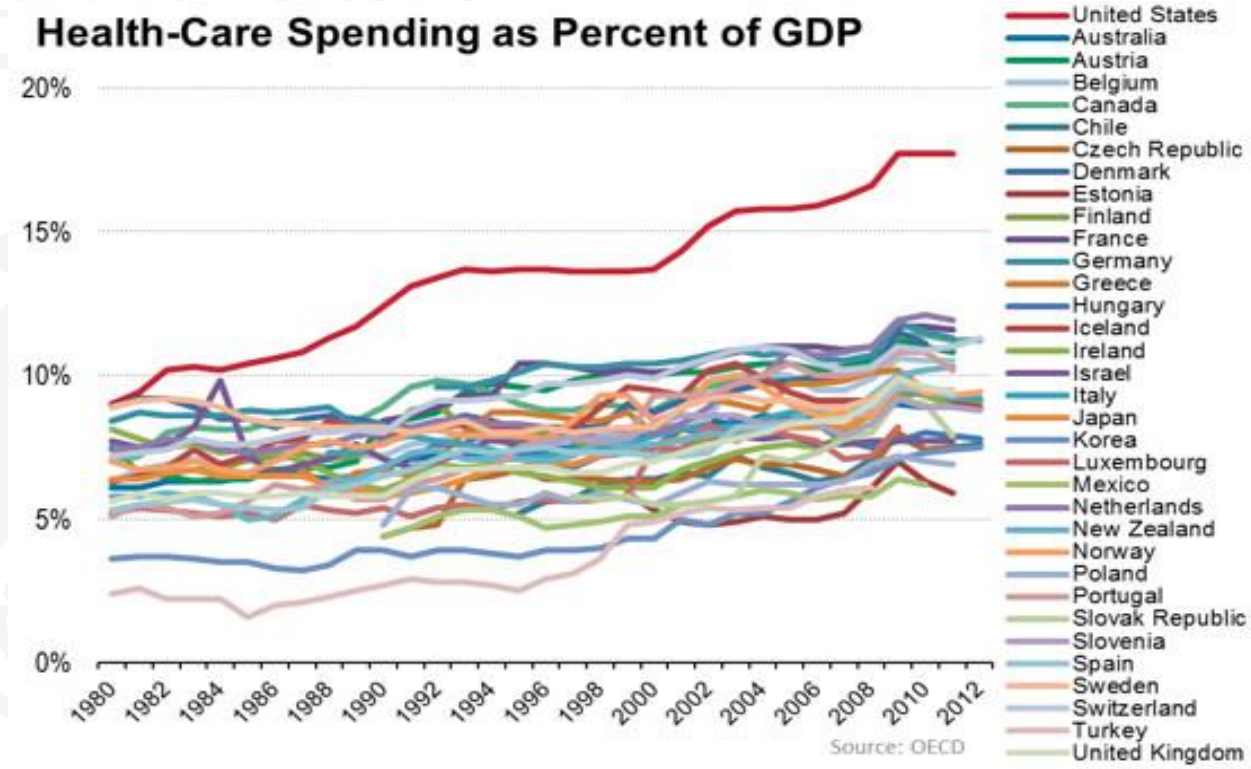
Overview of session

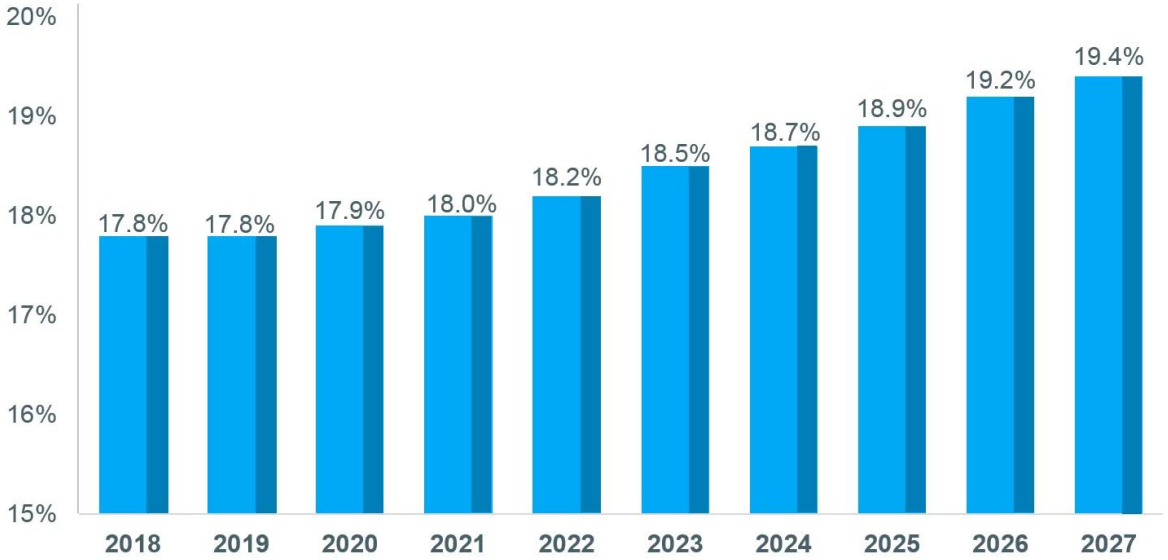
- Rationale for cost-effectiveness analysis (economic evaluation)
- How economic evaluation can support decision making
- Principles of economic evaluation
- Types of economic evaluation
- Examples in GH settings
- Challenges in applying economic evaluation methodology

Unlimited resources?



Where will the spending stop?

Figure 1. Projected US Healthcare Expenditures as a Percentage of GDP, 2018–2027



Sources: Centers for Medicare & Medicaid Services. National Health Expenditure Projections 2018-2026, Forecast Summary and Selected Tables.

The underlying problem

- There is an infinite demand for healthcare.
  - Particularly in full insurance systems – moral hazard
- There is a finite amount of resources with which to provide healthcare.
- This is the ‘classic’ rationale for economics – the science of scarcity.

Key issue in applying economic evaluation: the ‘Objective Function’

- What are healthcare systems trying to achieve?
  - Protection from high medical costs?
  - Increasing life expectancy?
  - Improving Health Related Quality of Life (HRQoL)?
  - Wider Societal benefit, including productivity
  - Equity or Fairness?
  - Dignified treatment?

How can economics help

- Health economics is primarily concerned with the allocation of scarce resources to improve health.
  - Also equity concerns
- Often used in HTA processes - summarising medical & economic information related to the use of a medical technology.
  - Designing benefits packages
  - Prioritising investments or further research
  - Where transparent, robust and unbiased methodology is needed

Quantifying opportunity costs

- What happens when a new technology comes along?
  - If funded from an exhausted fixed budget
- Spending money on a new technology means withdrawing money from some other use
- The new health outcome generated from the new technology is to some extent offset by the lost health outcome from the displaced spending
  - This is the **Opportunity Cost**
- EE tries to ensure that the gain from the transfer of spending to the new technology outweighs the loss


Hours
Leisure
Opportunity Cost = Next best alternative foregon
12
10
8
If you study an extra two hours.
The opportunity cost is the two hours
of leisure that you now cannot have
2
4
www.economicshelp.org
Hours Study

Example of opportunity cost

- Public health resources are used for a scheme to improve walking among the over-60s.
- Resources are no longer available to fund a football group for children
- Economic evaluation of the over-60s walking group needs to consider:
  - Resources used to encourage the participants to walk more (costs)
  - Effects on participants' physical and mental health (benefits)
  - Resources no longer available for the football group.
  - Value of the benefits from alternative projects are the opportunity costs.

Requirements for EE

- Budget Impact isn’t always considered
  - Key issue in many settings…can we add the new intervention to the benefits package?
- Is there a common measure of ‘effect’?
- Can we estimate the opportunity cost of health - a reference cost?

What is the intention of economic evaluation?

- Economic evaluation measures the ***incremental*** (not average) costs and benefits of specific treatments relative to the provision of alternative treatments, by comparing expected ***counterfactual outcomes.***
- Various ways in which costs and benefits can be captured.

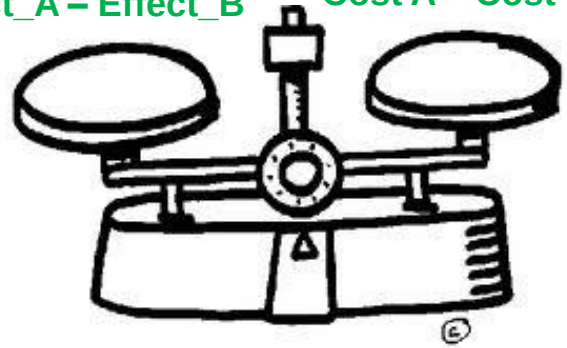
Incremental not average

- Difference in Averages = Not correct
  (health outcome A / cost A) vs. (health outcome B / cost B)

- Comparison of **Incremental** (marginal) differences = Correct

ΔE ΔC

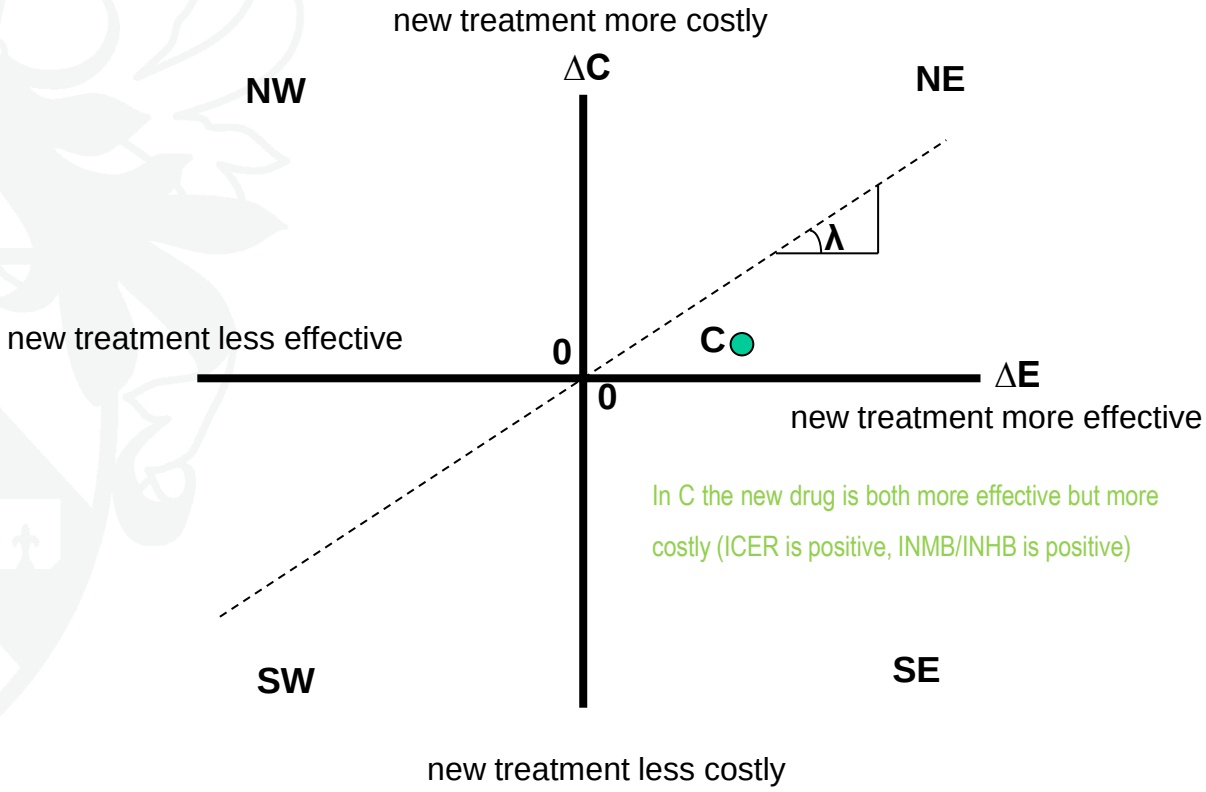
Effect_A – Effect_B **Cost A – Cost B**



The Incremental Cost-Effectiveness Ratio

- The ICER …. $\Delta C/\Delta E$ is effectively a cost per unit of effect
- Where Treatment A is more effective but more costly:
  - e.g it is £1,004/0.096 = £10,510 per QALY/DALY
- If incremental effectiveness is positive then:
  - high values bad
  - low values good

Cost-Effectiveness Plane



Willingness to pay for a QALY/DALY averted?

- Also known as threshold value and you may have heard of some values
  - England - £20k to £100k cost per QALY (£20k - £30k standard)
  - Australia - AU$ 69,900 per QALY
  - Netherlands – €80,000 per QALY
  - Sweden – €90,000 per QALY
  - US - $50k per QALY
  - WHO – 3% GDP per capita

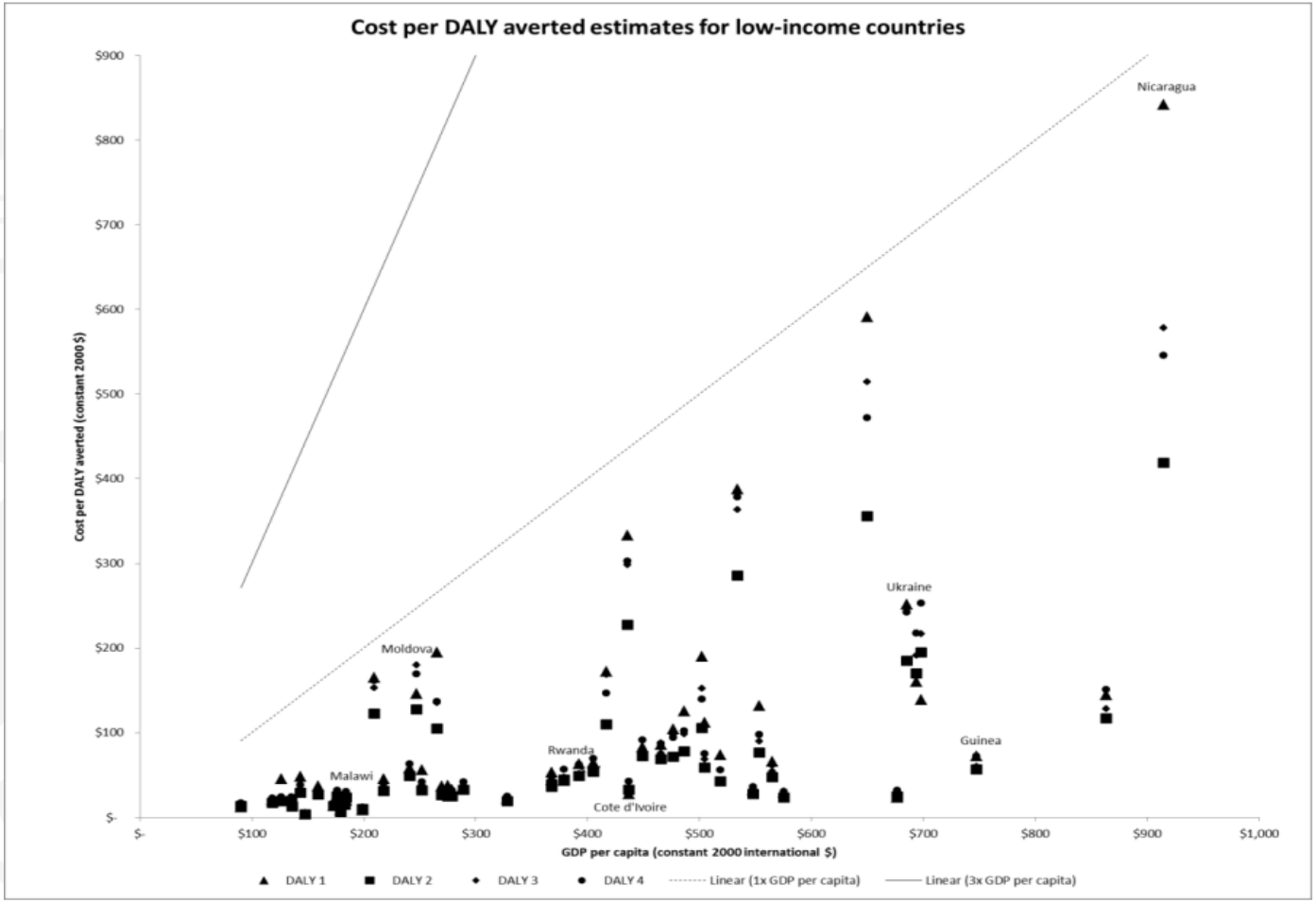


Figure 1. Cost per DALY averted estimates for low-income countries from Chapter 2[21]

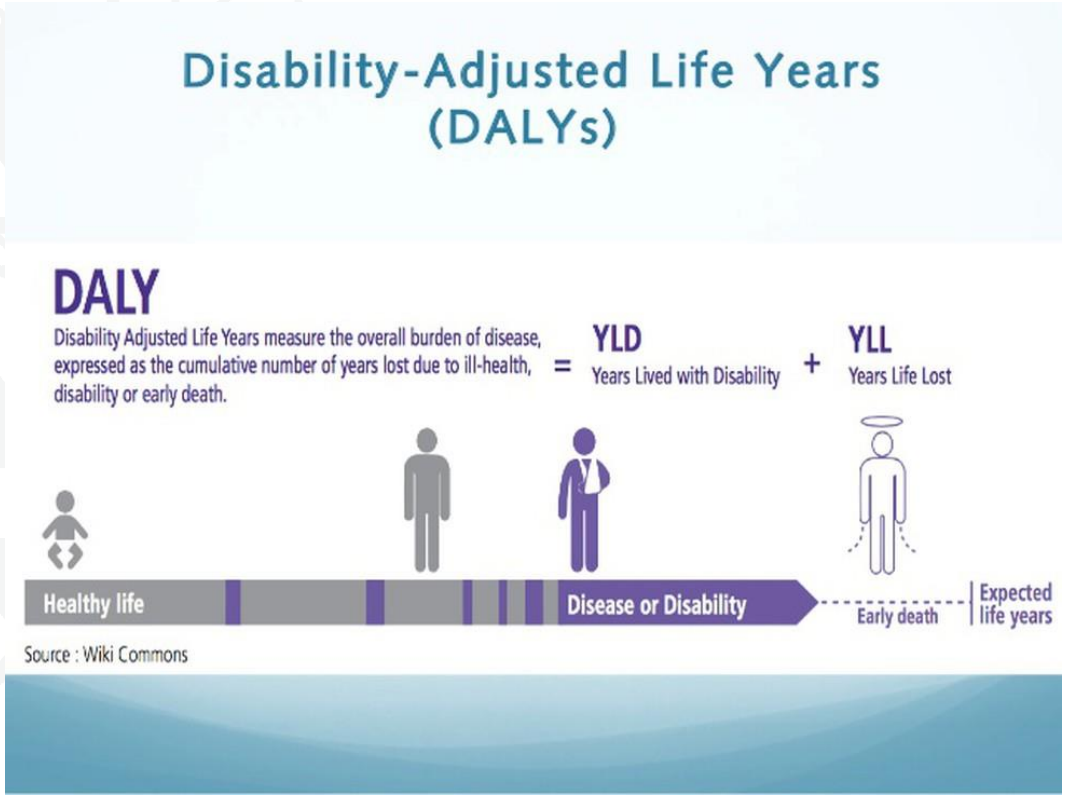
Forms of Economic Evaluation

- Cost Analysis
  - Assumes benefits the same (or alternatively *ignores* benefits)
- Cost-Benefit Analysis
  - Benefits are naturally measured in monetary metric
- Cost-Effectiveness Analysis
  - One outcome not converted to monetary outcome
  - disease specific therapeutic benefit
- Cost-Utility Analysis
  - Index of outcomes converted to single non-monetary metric e.g. Quality-Adjusted Life Years (QALYs)

Types of costs included

- Which costs – driven by perspective
- Health sector costs: Medicines, procedures, tests, hospital costs, staff costs, transport
- Other sector costs: educational aids, living aids, welfare payments
- Patient costs:
  - Out-of-pocket expenditures, direct and indirect, e.g. transport
  - Productivity/economic losses
- Carers expenses: productivity losses, travel
- Costs collected from patients or routine records

Measuring benefits



How do we compile these costs and QALYs/DALYs?

- Trial based or model based economic evaluation
- Trial based:
  - Prospective or retrospective
  - Use of non-randomised data also possible
  - Single study
- Model based
  - Utilises evidence from multiple sources

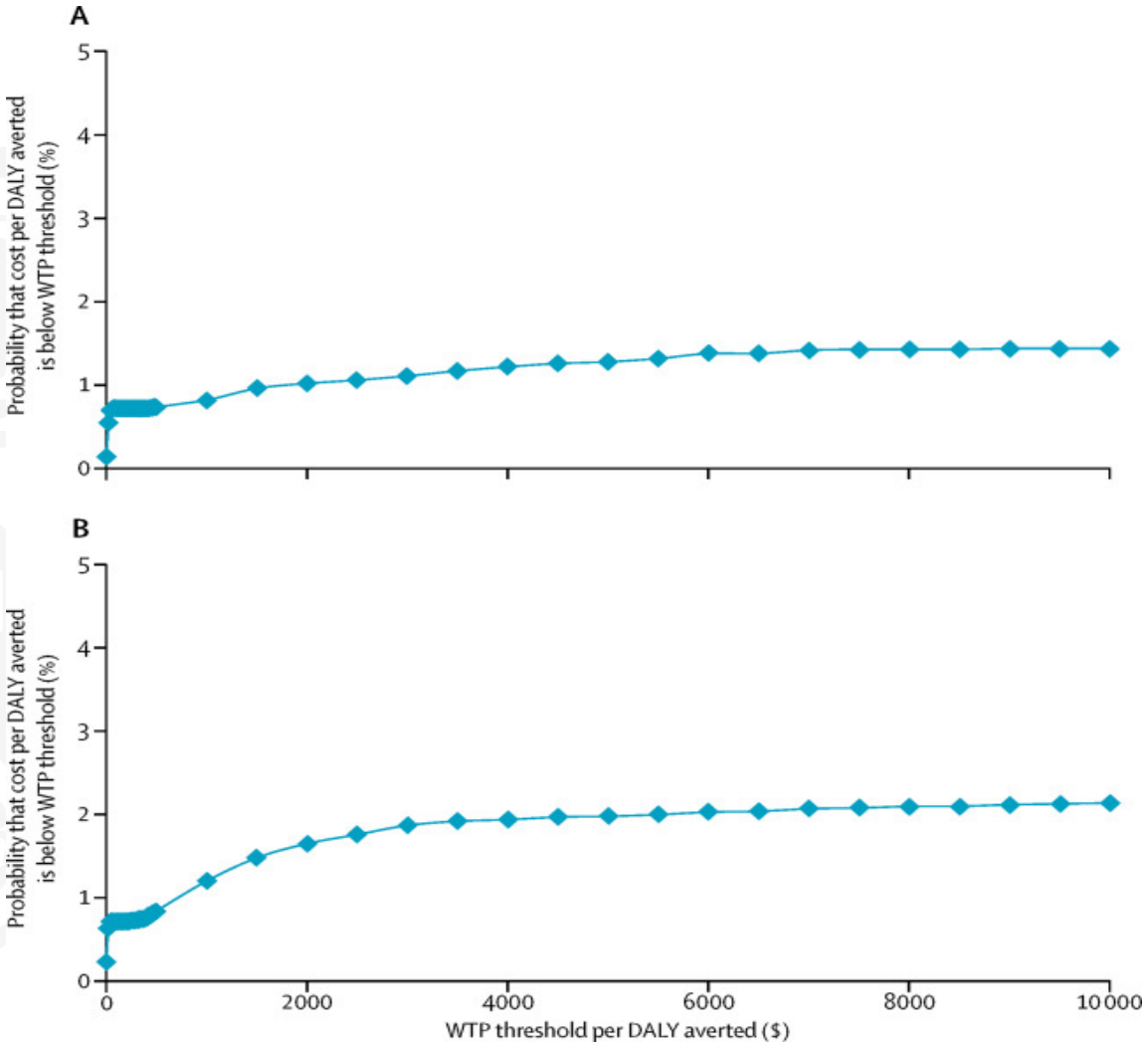
Characterising uncertainty

- Trial-based analysis
  - Deterministic sensitivity analysis – OWSA, MWSA
  - Scenarios
  - Threshold analysis
  - Parametric bootstrapping
- Model based analysis
  - Deterministic sensitivity analysis – OWSA, MWSA
  - Scenarios
  - Threshold analysis
  - Probabilistic sensitivity analysis (recommended)

Example: Trial/study-based cost-effectiveness

- Vassall, et al. Cost-effectiveness of Xpert in the real world during national roll-out in South AfricaRCT of PP intervention or waiting list group

- Pragmatic cluster-randomised trial, 20 clusters in four provinces in South Africa
  - Xpert versus sputum smear microscopy
  - Followed up for 6 months
  - Data collection within trial
  - Used a societal perspective
  - Cost per person investigated for tuberculosis and the cost per disability-adjusted life-year (DALY) averted.



Source: Vassall, et al, 2017

Any issues with within trial analysis?

- Did the trial completely address the decision problem?
  - Has the right population
  - Over the full time period
  - With all the correct comparators
  - Utilises all available data
  - *And is randomised treatment allocation*

Why use cost-effectiveness modelling?

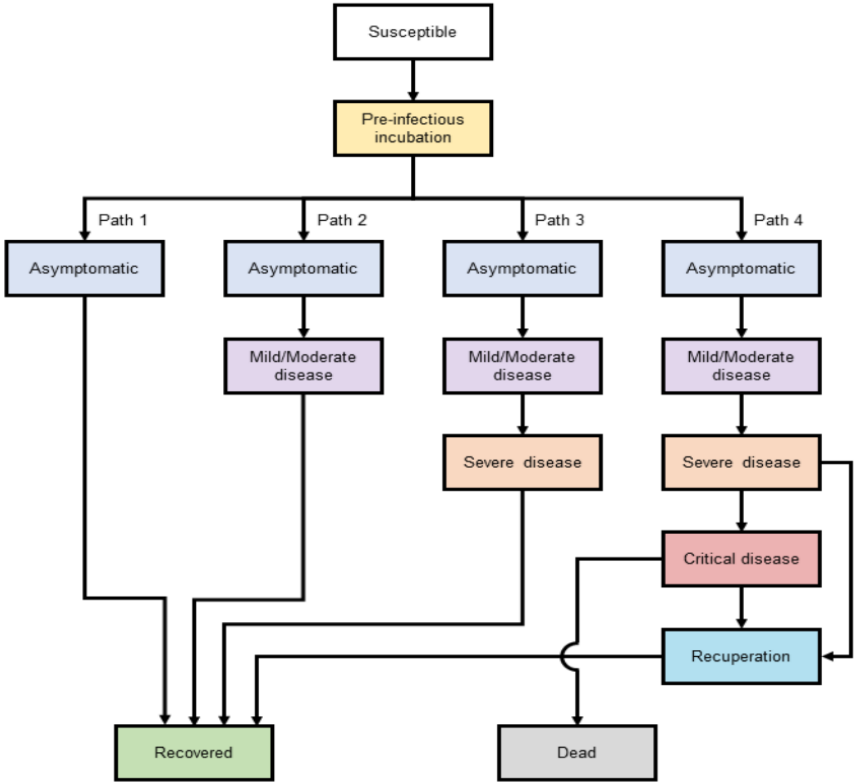
- Trials alone may not be sufficient for decision making
  - Selective inclusion of comparators
  - May need to synthesise evidence from different sources
    - In particular costs and utilities (outcomes)
  - Insufficient time horizon
    - Extrapolate intermediate (observed) outcomes to long term QALYs

How do we build cost-effectiveness models?

- Process of bringing all the different components in a decision problem into a formal structured framework
- Model should represent the disease process and capture any differences in costs and outcomes between competing interventions
- Range from very simple structures to incredibly complex models
- It allows decision makers and industry to understand the value of the product and which elements of the problem are most important to resolve --- EQUI-Injury model!

Example: model-based cost-effectiveness

- Reddy, et al. Clinical outcomes and cost-effectiveness of COVID-19 vaccination in South Africa
- Microsimulation model to evaluate cost-effectiveness of a COVID-19 vaccination program.
  - Microsimulation approach needed for infectious diseases
  - Simulated COVID-19 outcomes over 360 days (infections, deaths, years-of-life lost), health care costs
  - Modelled various strategies (% coverage, pace, acceptance)
- Providing vaccines to at least 40% of the population and prioritizing vaccine rollout prevented >9 million infections, >73,000 deaths, fewer hospitalisations.



Supplementary Figure 1. Health states and disease paths in the Clinical and Economic Analysis of COVID-19 Interventions (CEACOV) model. Mild/moderate COVID-19 disease is that which does not prompt hospitalization. Severe disease prompts hospitalization if hospital (non-ICU) beds are available. Critical disease prompts ICU admission if ICU beds are available.

Source: Reddy, et al, 2021

Key challenges in applying economic evaluation methodology

- Data – challenging for more complex evaluations, including treatment pathways, complex interventions.
- Complex models are data hungry
  - Do not rely on statistical inference but sparse data produces v uncertain estimates of cost-effectiveness
- Generic measures of health may not capture all health benefits
  - Need to compare across diseases/populations
  - Additional complexity of non-health outcomes

Reflecting efficiency-equity trade-offs

- Implicit equity judgement that additional outcomes worth the same no matter to whom they accrue
  - Limited informal evidence for distribution of intervention benefits
  - No information on distribution of opportunity cost

Equity and Inequality

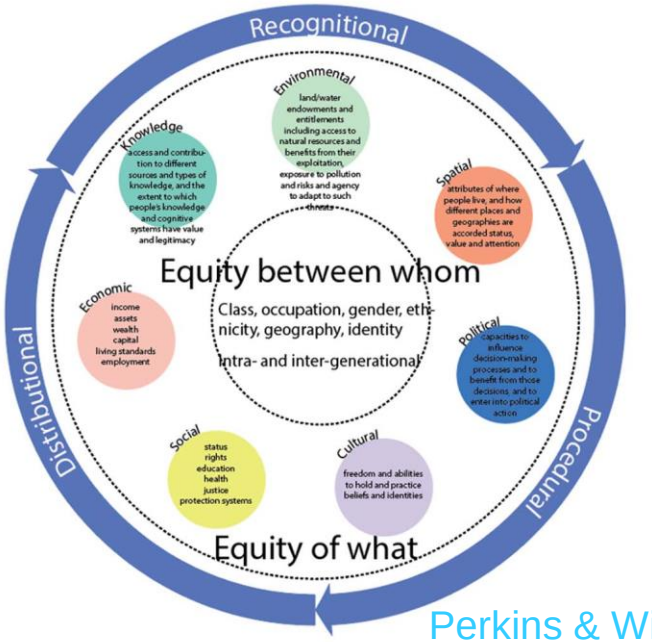
- People are averse to inequality in health
  - Surveys indicate willingness to sacrifice health for a more equal distribution
- “Equity” and “fairness” are common decision criteria for resource allocation in healthcare decision making
- Defining what is fair is contentious but unavoidable
  - Inaction or focus on average health gain implements no inequality aversion

Need for equity considerations in resource allocation decisions

- Uptake, adherence, efficacy may be better in socially advantaged
- Existing resources often targeted at most deprived, opportunity costs borne most by disadvantaged groups
- Interventions can increase health inequality (intervention generated inequality)
- Policy that would result in most equal distribution may not provide greatest increase in overall health
- Interventions may be more costly to deliver to socially disadvantaged
- Need to acknowledge potential conflicts
  - Trade offs whereby we forgo population health to reduce health inequality or exacerbate health inequality through interventions that improve overall health

Equity-relevant social variables

- Groups can be defined socially, economically, demographically, or geographically.
- Often use measures of income, social deprivation, ethnicity.
  - Collected directly on population of interest



Perkins & Will

Distributional cost-effectiveness analysis (DCEA)

- Provides distributional breakdowns of who gains most and who bears the largest burdens (opportunity costs)
  - According to equity relevant social variables
  - Can also employ equity weighting to explore trade offs between efficiency and equity
- In decision modelling, parameters reflect relevant characteristics
  - Total cost becomes a distribution of health opportunity cost

DCEA Example

- Dawkins, et al . Distributional cost-effectiveness analysis in low- and middle-income countries: illustrative example of rotavirus vaccination in Ethiopia
  - Hypothetical re-designed rotavirus vaccination programme
- ICER of US$69 per health-adjusted life year (HALY) compared with the standard programme - potentially cost-ineffective when compared with current estimates of health opportunity cost in Ethiopia.
- The more equitable programme would be considered worthwhile by a decision maker whose inequality concern is greater than $\varepsilon = 5.66$ (inequity aversion parameter)
  - Health gains are weighted at least 3.86 times more highly in the poorest compared with the richest wealth quintile group.

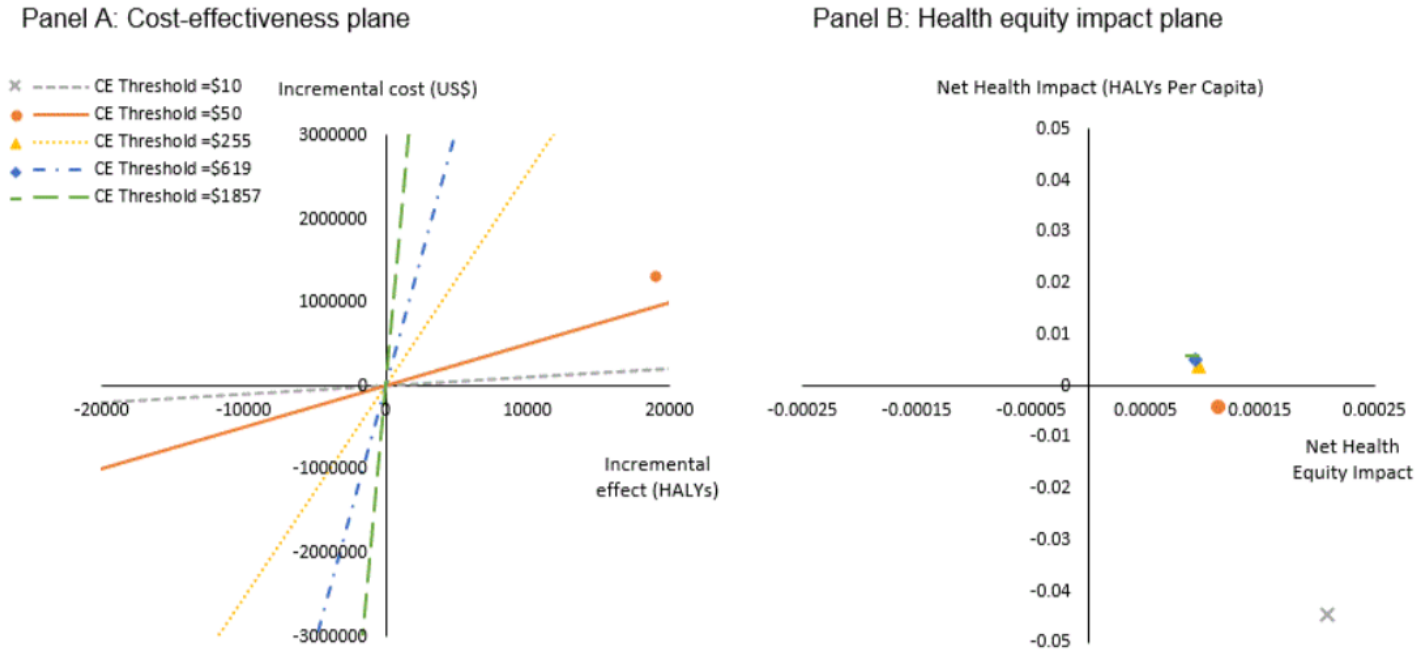


Figure 3: Incremental analysis of pro-poor vaccine compared to standard vaccine: Cost-effectiveness plane vs. Health equity impact plane

Source: Dawkins, et al, 2018

Questions & Answers

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