Slide 1

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

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Slide 2

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

Slide 3

Unlimited resources

A graph presenting healthcare spending as percent GDP for different countries

Slide 4

Where will the spending stop?

Figure 1 presenting projected US health expenditure as percentage of GDP, 2018 – 2027

Slide 5

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.

Slide 6

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?

Slide 7

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

Slide 8

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

Slide 9

Graph

Opportunity costs = next best alternative foregone

Slide 10

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.

Slide 11

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?

Slide 12

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.

Slide 13

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

Slide 14

The Incremental Cost-Effectiveness Ratio

The ICER …. ∆C/∆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

Slide 15

Cost-Effectiveness Plane

Slide 16

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

Slide 17

Figure 1. Cost per DALY averted estimates for low-income country

Slide 18

FoCost 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)rms of Economic Evaluation

Slide 19

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

Slide 20

Measuring benefits

Picture explaining DALYs

Slide 21

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

Slide 22

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)

Slide 23

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.

Slide 24

Graphs

Source: Vassall, et al, 2017

Slide 25

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*

Slide 26

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

Slide 27

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!

Slide 28

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.

Slide 29

Graph

Source: Reddy, et al, 2021

Slide 30

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

Slide 31

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

Slide 32

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

Slide 33

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

Slide 34

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

Slide 35

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

Slide 36

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 ε = 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.

Slide 37

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

Slide 38

Questions and answers