Overview

- What is economic evaluation in health care?
- Conceptual basis for economic evaluation
- Framework for economic evaluation
- Measuring and valuing resource use (costs)
- Measuring and valuing outcomes
- Interpretation and application

Rationale for economic evaluation 1

- Resources are scarce
- New demands on the health care budget
  - Population growth
  - Ageing population
  - New technologies
  - New uses for technologies
  - Rising expectations
- Choices need to be made:
  - What treatments should be produced?
  - How many treatments should be produced?
  - Who should get what treatments?
- Many safe, effective health care interventions that could be funded
- Need a decision making rule to maximise benefit from available resources

Rationale for economic evaluation 2

- Basis for making collective decisions about the mix of consumption of goods and services
- A tool for addressing resource allocation questions
- Examples:
  - Should a new expensive drug be listed on the formulary?
  - Should we check the blood pressure of each adult annually?
  - Should a Pap smear be performed every 2 or 3 years, and it what age group?
  - Should nursing staff be removed from well baby clinics to carry out home visits on lapsed hypertensives?
  - Should we a national prostate cancer screening program?
  - Should we build a new public hospital?

Economics approach to decision making

- Maximise something (objective) subject to a constraint (budget/resource)
  - What do I want to achieve?
    - What is the objective?
  - What resources are available?
    - What is the budget constraint?
  - How do I achieve the most from available resources?
    - How do I allocate resources between alternatives?

Opportunity cost

- Scarce resources
- Many alternative uses of resources
- Identify the opportunity cost of each alternative
  - Opportunity cost of doing A is not doing B
  - Benefits of doing B are forgone
Applying opportunity cost

- Fixed budget:
  - Additional resources for one use have to come from somewhere
  - Shifting resources between alternatives

- What are the additional costs and additional benefits?
  - Is benefit > cost?
  - Cost is what I have to give up to get the benefits: Opportunity cost

  - Choose the alternative with the highest benefit
  - Choose the alternative with the lowest opportunity cost

Key concepts

- Efficiency
  - Technical efficiency
    - Maximise a given output from available resources
    - Technical efficiency occurs when the maximum amount of an output is produced for a given set of inputs or when the minimum amount of inputs are required to produce a given output level.
  - Allocative efficiency
    - Choose the best mix of output
    - Allocative efficiency occurs when firms produce those goods and services most valued by society. This means scarce resources are allocated to the production of the goods and services so that consumer wants and needs are met in the best way possible.

- Equity
  - How should these outputs be distributed among members of society?

Resource allocation in general

- Allocation decisions for most goods made in markets
- Rely on market mechanisms to set priorities and allocate resources
- Consumers determine what they value and decide what they wish to purchase, given prices and other alternatives
  - Maximise wellbeing (utility)
  - Producers (suppliers) decide what to produce given prices and costs of inputs
    - Maximise profit
  - When markets work, prices convey information to producers and consumers
    - In competitive markets, the market mechanism will maximise benefits within current resources (set priorities and allocate resources)
- Individual decisions lead to efficient outcomes
  - Marginal social benefit = marginal social cost

A note about markets

- Under the assumptions of perfect markets, individuals maximise welfare FOR ANY GIVEN INITIAL DISTRIBUTION OF WEALTH AND INCOMES
- Not at this stage making a judgement about the initial distribution

Resource allocation decisions

- Evaluate benefits and costs of alternative uses of resources
- Cost = Opportunity cost = benefit forgone
- Always making choices "at the margin"
  - What is the additional benefit of producing/consuming the next unit?
  - What is the opportunity cost?
- Is MB > MC?

Can markets make these decisions in health?

- Numerous sources of market failure
  - Uncertainty
  - Asymmetry of information
  - Externalities
- When markets fail we need other ways to make collective choices
- Strong case for government intervention in funding and providing health care
- Role for economic evaluation
Economic Evaluation

- The systematic comparative analysis of alternative courses of action in terms of their costs (resource use) and their consequences
- Who should do what to whom with what health care resources and with what relation to other health services?
- (Drummond et al, 1997)
- Choosing the alternative that maximises social welfare

Conceptual basis

- Individuals assumed to maximise utility
  - Consider the costs and benefits of alternative mix of goods and services
  - If no change can increase utility then individual welfare maximised
- But decisions for society involve more than one individual
- Issue of interpersonal comparability
- Use principles of welfare economics

How do we judge a welfare improvement?

- Issue of comparability of individual welfare
  - How does my gain compare with your loss?
  - Therefore how can we judge a welfare improvement?
- Pareto criterion
  - A change in the distribution/mix of goods and services achieves a Pareto improvement if at least one individual is made better off and no-one is made worse off
  - A Pareto optimum occurs when there is no shift from the current distribution that can achieve a Pareto improvement
  - Note that in perfect markets voluntary trade leads to a Pareto optimum

So is the Pareto criterion practical?

- Most programs/interventions make some people better off and some worse off
  - Eg all tax financed interventions/programs
- Back to interpersonal comparisons!
- But if the benefits are large enough that the winners could compensate the losers we have a Potential Pareto Improvement even if the compensation doesn’t occur
- Potential Pareto Improvement can be used as a basis for decision making
- Rationale behind economic evaluation (particularly cost-benefit analysis)
- Note the PPIC has a pitfall (see Skitovsky Paradox)

From individual welfare to social welfare: 1

- Pareto improvement makes no judgement about interpersonal comparability
  - Social welfare depends on individual welfare, but no value judgement about how individual welfare contributes
- Societies may make judgements about how to aggregate from individual welfare to social welfare
  - Eg simple aggregation compared with weighted aggregation

From individual welfare to social welfare: 2

- How do we aggregate individual welfare
- Utility functions not directly comparable
- But revealed preference gives us a mechanism
  - Willingness to pay
  - Gives monetary measures of welfare gains
  - Allows aggregation of individuals using the common metric of money
  - Weighting of individual welfare gains still feasible
Basis of economic evaluation

- The aim is to answer the question: “will this program/intervention increase the welfare of society?”
- Assumes that we agree that increasing welfare of society is the objective (i.e., maximizing a function that is some aggregation of individual utility functions)
- Some economists have argued for different maximands in health sector – eg maximize health
  – Extra-welfarist approach

Summary

- Economic evaluation as a tool in the analyst’s kit is necessary because we cannot rely on markets to provide efficient outcomes in many cases (especially in health care)
- Still using the principles of maximizing welfare to make decisions
- Requires some way of aggregating across individuals
- Requires some value judgment about the current distribution of wealth/income
- Potential Pareto Improvement Criterion gives a theoretical basis for economic evaluation
- Other criteria are possible

Economic evaluation - defined

“the comparative analysis of alternative courses of action in terms of both their costs and consequences”

Key points in definition

- Always comparing alternatives (even if alternative is “do nothing”)
- Comparison of alternatives means deciding at the margin (marginal or incremental analysis)
- Identifying, measuring and valuing costs (resource use) and consequences
- Underlying criteria is efficiency
- Assessing efficiency implies know effectiveness
- Social decision making context
- Explicit valuation of consequences: making trade-offs clear

Economic evaluation: principles to practice

- Define the appropriate margin for the decision
  – what are the alternatives?
- Identify opportunity costs of alternatives
  – what resources are used/freed up by the intervention?
  – what resources would be used without the intervention?
- Identify and value consequences of alternatives
  – what happens to whose health or wellbeing with the intervention?
  – What would happen to whose health/wellbeing without the intervention?
Types of Economic Evaluation

- Economic evaluations in the healthcare literature are usually one of these four:
  - Cost-minimisation analysis (CMA)
  - Cost-effectiveness analysis (CEA)
  - Cost-benefit analysis (CBA)
  - Cost-utility analysis (CUA)

- They all asking the same question, but differ in their choice of outcome measure.

Cost-minimisation analysis

- Assumes effects are the same, but the costs differ
  - which treatment costs the least?
  - E.g. Surgical repair of varicose veins (VV)
    - Option 1: inpatient treatment
    - Option 2: day patient treatment
  - Both have identical outcomes e.g. VVs repaired
  - However, treatment costs are different
  - Compare costs to identify least costly alternative

- Appropriate when consequences identical in every respect that matters (which needs to be justified)

- Typically applied in drug reimbursement decisions for “me too” drugs (or where there is a claim of equivalent therapeutic effect)

- Problems
  - Cannot compare programs with different outcomes
  - Does not answer the question of whether we should invest in the least costly alternative

Cost-effectiveness analysis

- The outcome in CEA is a common unit of effect
  - E.g. death averted, one point reduction in cholesterol
  - Measured in “natural units”

- Unlike CMA the effectiveness of each alternative is different

- How do we maximise a given health output from available resources?
  - Can be performed on any alternative with common outcomes
  - Identify cost per unit of effect
  - Compare costs and consequences to find the most cost-effective alternative

- Problems
  - Unit of effect can be narrow (stroke prevented) or broad (life years gained)
  - Cannot compare program with different outcomes
  - Outcome is unidimensional – so cannot capture morbidity and mortality
  - Does not answer the question of whether we should invest in the most cost-effective alternative

Cost-utility analysis

- Outcomes measures by an index of “utility”;
  - e.g. Quality Adjusted Life Year (QALY)
- Cost per Quality Adjusted Life Year

- Advantages
  - These allow for patient preferences
  - Can compare across wide variety of health care programmes
  - Measure of effect combines quality of life and survival
  - Can be used to identify whether we should invest in the most cost-effective option.

- Problems
  - Concerns regarding measuring QALYs
  - Equity issues

Cost-benefit analysis

- Cannot always find a common effect
  - hypertension screening programme (premature death)
  - influenza vaccination programme (disability days)

- Require a common denominator in order to compare programmes
  - CBA solution is to assign a monetary value to benefit
  - Advantage of giving a net benefit (loss) of one programme over another

- Assigning monetary value
  - Productivity
  - Willingness-to-pay
  - Can answer questions of whether an intervention increases social welfare

- Problems
  - Often difficult to assign a money value to a benefit
  - Rarely used in health care

In health typically use CEA/CUA

- Welfare economics underpinnings (similar to CBA)

- Aim to reflect society’s preferences for different treatments and outcomes

- Allows (requires) comparison across health care interventions

- Provides a common metric for comparing cost per unit of health gain

- Avoids pesky ethical concerns about market allocations or CBA (maybe)
**Steps in economic evaluation**
- Define alternatives A and B
- Define perspective and time frame
- Choose appropriate evaluation tool
- Identify, measure and value costs
- Identify measure and value consequences
- Combine costs and consequences
  \[ \text{ICE Ratio} = \frac{(\text{Net costs of A} - \text{Net costs of B})}{(\text{Consequences of A} - \text{Consequences of B})} \]
- Assess robustness of results (sensitivity analysis)
- Interpret

**Defining the intervention**
- Components of the program
- Frequency of the intervention
- Specific technologies used
- Method of delivery
- “Bundling” of services
- “Starting point” for intervention
- Target population

**Defining the intervention target population**
- Defining target group important for resource use and consequences
  - Age groups
  - Comorbidities
  - Risk Factors
  - Urban/rural
  - Socioeconomic factors

**Choice of comparator**
- Identifying incremental costs and incremental effects
- Comparator will depend on the policy context
- May need a range of comparators
- “Do nothing” / No treatment
- Status quo (may involve a range of programs): choose one of these or what happens now?
- Current best practice
- 2+ alternatives against same base case
- What do we know about current practice?
- Comparing programs of varying intensity/duration

**Basic conceptual model**
- Decision tree framework
  - Epidemiological information used to identify
    - disease states
    - outcomes of treatment
    - probability of different outcomes (proportion of a population experiencing the outcome)
    - survival (durations) associated with different outcomes
    - quality of life associated with different outcomes
    - resource use associated with each alternative
- Model expected health gain and resource consequences of each alternative
- Use this to estimate the incremental cost effectiveness ratio

**Valuing health outcomes is complex**
- Health is uncertain and therefore the relevant health outcome of an intervention is “always a probability distribution over a large number of possible lifetime paths …[consisting] of a sequence of health states through which the individual would pass up to the point of death”
  
  (Torrance, 1995)
Very simplified decision tree for Tamoxifen therapy for breast cancer chemoprevention

Need to model costs and outcomes
- Starting point is a (usually trial based) estimate of the effect of the intervention compared to comparator
- No trials give the information needed for economic evaluation
- Apply this to the relevant population over the relevant time frame with appropriate assumptions about:
  - Population characteristics; Baseline risks; Disease process; treatment algorithms
  - Extrapolation to an economically relevant time frame
  - Extrapolation from trial outcomes to economically relevant outcomes
- Model structure should capture and reflect these assumptions

Modelling approaches
- Decision analysis (basic decision tree structure)
- Markov models
- Monte Carlo simulation
- Regression models

Attach costs to relevant branches and ‘payoffs’ to final outcomes

‘Roll back’ the tree (i.e. estimate the EV of the alternatives)

Markov models
- Most models for computing long-term outcomes of treatment are Markov models
  - e.g. progression of chronic disease (HIV-AIDS, cancer, heart disease, cystic fibrosis)
- Also, good for modelling repeat events such as:
  - headaches
  - hospitalisation episodes
**Markov models - basic idea**

- Individuals at any time are in one of a finite set of states
e.g. well, ill, or dead.
- Changes over time from state to state occur according to a set of ‘transition probabilities’

**A Markov model of Cystic Fibrosis**

- WELL → CLD → AILD → DEAD

**Markov model of Cystic Fibrosis - results**

- CLD = Chronic Lung Disease, AILD = Advanced Irreversible Lung Disease

**Progression of Chronic Hep. C after combination therapy**

- Clear of HCV, Chronic HCV infection, Cirrhosis, Dead

**Moving from clinical data to economic model, need to address:**

- **Applicability issues**
  - ways in which the participants and circumstances of use in the trial differ from the proposed population for treatment (including the baseline risk of participants and circumstances of use)
- **Extrapolation issues**
  - is there a need to extrapolate the outcomes reported in the clinical evaluation beyond the trial or study horizon
- **Transformation issues**
  - is a need to transform the nature of the outcome measured in the clinical evaluation

**Variables for the economic evaluation**

- **Examples include**
  - health care resource items (quantities and unit costs)
  - outcomes & probabilities within each branch of a decision analysis (including transition probabilities or rates in a state transition decision analysis)
  - the discount rate
- **Stochastic and deterministic variables**
- **Appropriate treatment of stochastic variables**
Time Horizon

- Most health care interventions have future costs and consequences
- Impacts in future less certain but still relevant
- Relates to perspective/decision context and to choice of outcome measure
- Trying to capture all relevant future costs and consequences
- Discounting of future costs and consequences

Discounting

- Adjusting for society's rate of time preference
- More preferred now rather than later
- Time preference NOT inflation
- How do we discount?
- Choice of discount rate

Discounting

Discount Factor: \( 1/(1+r)^t \)
(PV of $1 in t years time, given a rate of time preference of r)
See Drummond et al pp 92-93

<table>
<thead>
<tr>
<th>Year</th>
<th>Costs</th>
<th>Discount Factor (5%)</th>
<th>Discounted Costs</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>$10,000</td>
<td>1</td>
<td>$10,000</td>
</tr>
<tr>
<td>1</td>
<td>$10,000</td>
<td>0.9524</td>
<td>$9,524</td>
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<tr>
<td>2</td>
<td>$10,000</td>
<td>0.9070</td>
<td>$9,070</td>
</tr>
<tr>
<td>3</td>
<td>$10,000</td>
<td>0.8638</td>
<td>$8,638</td>
</tr>
<tr>
<td>10</td>
<td>$10,000</td>
<td>0.5584</td>
<td>$5,584</td>
</tr>
</tbody>
</table>

Opportunity Cost (aka Economic Cost)

- Opportunity cost is the cost of something in terms of an opportunity forgone (and the benefits that could be received from that opportunity).
- In costing, we do not want to ignore costs because of who they accrue to, unless there is a good reason (issue of cost-shifting).
- Therefore, a perfect economic evaluation would take a societal perspective, which includes all costs of the intervention irrespective of who pays.

Perspective

- Societal – all costs and benefits, irrespective of to whom they are accrued, are included
- Government
- Third party payer
- Health system
- Institution
- Implications for how costs and consequences defined and measured
- May need to present results from different perspective

Issues in the measurement of costs
**An Example of Perspective – Drug A vs. No Treatment**

<table>
<thead>
<tr>
<th>Cost</th>
<th>Societal Perspective</th>
<th>Health Sector Perspective</th>
<th>Accountancy Perspective</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug A</td>
<td>Yes</td>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td>Doctor Time</td>
<td>Yes</td>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td>Future Costs to the Hospital</td>
<td>Yes</td>
<td>Yes</td>
<td>Probably Not</td>
</tr>
<tr>
<td>Patient Transport, Prescription charges etc</td>
<td>Yes</td>
<td>No</td>
<td>No</td>
</tr>
</tbody>
</table>

**Societal vs Health Sector Perspective**

- **Societal**
  - Aims to include all costs
  - Prevents cost-shifting from affecting analysis

- **Health Sector**
  - Excludes certain costs, often those which are difficult to calculate or open to much uncertainty.
  - Simpler analysis
  - Is our aim to maximise health gain from the activities of the system, or health per se?

**Identify, measure, value**

- Aim of costing is to identify, measure and value the resource use associated with each alternative over an appropriate time frame

**Exercise:** Identify the resource use associated with:
- A hospital admission for the flu
- A ‘shared care’ GP visit for Hep C therapy
- A woman having a Pap smear taken

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**Counting the resources used: What to include?**

- Not everything!
- First – list “ingredients”/activities (tree?)
- Which form a large proportion of all resources used? (e.g. staff time)
- Which are relatively expensive items? (staff, drugs, programme start-up costs)
- Conundrum!! … preliminary investigations

**Counting the resources used: What to exclude?**

- Small costs/outside time-frame
- Costs common to all alternatives
- Costs which are too expensive to accurately estimate!

**Tricky issues:**
- “Transfer payments” and patient charges
- Double-counting

**Counting the resources used: Two broad approaches**

- **Ingredients method** (micro-costing, “bottom-up”)
  - e.g.
  - No. minutes with GP
  - No. tests ordered
  - Type and no. of drugs prescribed
  - No. of visits to GP

- **Accounting method** (macro-costing, “top-down”)
  - e.g.
  - No. bed-days per admission
  - No. of admissions per year (DRG)
**Costing - some common terms**

- **OPPORTUNITY COSTS**
- Marginal costs or incremental cost
- Average costs
- Fixed vs. variable costs
- Other accounting terms: Capital vs. revenue costs, Overheads (joint)

**Valuation of resources**

STRICTLY – Opportunity costs

Market Prices
- = opportunity cost when perfect competition
- Non-market prices
  - “imputed” costs or “shadow prices”
  - Difference between charges and costs (nb. MBS item fees)

**Costs – Issues to Consider**

- Does the observed price represent the opportunity cost?
- How do we include overhead costs?
- Is a single unit cost appropriate in situations with economies of scale?

**What other factors may make a list price not appropriate?**

- Lumpy technology – e.g. scanners. Low cost until another scanner is needed, then one-off large cost.
- Economies of scale – would an expansion of a service really have the same average cost as the previous position?*
- Imperfect markets for health technologies can artificially inflate the cost of drugs away from the opportunity cost.

* See Abrantes et al. Reference on last slide

**Sensitivity Analysis on Costs**

- This is used to identify how certain we are about the baseline cost value for an intervention.
- We have reached a conclusion about the costs of an intervention.
- This conclusion is based on assumptions (e.g. cost of drug, patient re-admission etc)
- Sensitivity analysis asks the question “What would happen to the cost if Assumption X changed?”
**Cost issues**

- **Productivity**
  - What if one technology enables a patient to return to work more quickly?
  - Methodologically difficult
  - Equity issues – (elderly or unemployed)

- **Informal care**
  - Most informal carers are family or friends of the person receiving care
  - Provided care is usually free! However, it is not free in an economic sense, as time spent caring is time that cannot be directed to other activities such as paid work, unpaid work (such as housework) or leisure
  - Similar issues to measuring productivity

**Other issues in costing**

- **Different methods for apportioning overhead costs**
- **Costing methods should be described as thoroughly as outcome measurement**
- **Be explicit about uncertainty and missing data**
- **What is the base year?**
  - Inflate past cost estimates to PV and discount future costs to their PV …

**Issues in measuring and valuing outcomes in economic evaluation**

**Why do we need to value outcomes?**

- When goods are traded in a market, the market provides information about the value to individuals
  - Eg wage rate gives a value of time
- But many health outcomes not typically traded in markets
- So for economic evaluation, need to identify, measure and value outcomes
- Clinical studies typically give us some measure of outcome
- How this is applied and valued determines the type of economic evaluation undertaken

**What are the outcomes of health care interventions?**

- **Health gain**
  - Reduction in mortality
  - Reduction in morbidity
  - Improvements in quality of life
- **Other outcomes**
  - Information
  - Convenience
  - Reassurance
  - Satisfaction with process of care
- **Impacts on productivity**
  - Treated differently from other consequences
- **Improvements in individual welfare**

**Measures of health gain (effectiveness)**

- Ideally want measures that are comparable across different diseases and treatments
- "Intermediate"/"surrogate" (clinical) outcome not comparable
- Life years saved – not all years have the same quality
- Quality adjusted life years saved (QALYs)
Quality Adjusted Life Years

- “Quality Adjustment” reflects value of health states relative to full health
- Captured by applying a QALY weight to survival
  - QALY weight = 1 for “full health”
  - QALY weight = 0 for death
  - “Worse than death” health states permissible (QALY weight < 0)
- Cardinal ‘utility’ scale anchored at death and full health
- Relies on assumptions about how health states are valued (see below)
- QALYs measure strength of preference for survival and quality of life and trade-offs between the two

Example: QALY Weights

<table>
<thead>
<tr>
<th>Health State</th>
<th>QALY Weight</th>
</tr>
</thead>
<tbody>
<tr>
<td>AIDS, CD4 count range 0-50</td>
<td>0.79</td>
</tr>
<tr>
<td>Arthritis, before treatment</td>
<td>0.61</td>
</tr>
<tr>
<td>Arthritis, after treatment</td>
<td>0.65</td>
</tr>
<tr>
<td>Cancer, breast, after surgery, first recurrence</td>
<td>0.85</td>
</tr>
<tr>
<td>Cancer, breast, after surgery, third recurrence</td>
<td>0.30</td>
</tr>
<tr>
<td>Cancer, prostate, metastatic, early progressive disease</td>
<td>0.83</td>
</tr>
<tr>
<td>Proctitis/cystitis after radiation therapy for prostate cancer</td>
<td>0.90</td>
</tr>
<tr>
<td>Stroke, severe, motor deficit</td>
<td>0.03</td>
</tr>
<tr>
<td>Stroke, mild cognitive deficit</td>
<td>0.54</td>
</tr>
</tbody>
</table>

Source: Tengs, T and Wallace A. One Thousand Health-Related Quality of Life Estimates Medical Care 38(6) 583-637, 2000

Why use QALYs?

- Increasing preference for cost-utility analysis (eg NICE, PBAC)
- Comparability across different diseases and treatments
  - “Intermediate” (clinical) outcomes not comparable
  - Life years saved – not all years have the same quality; and not all treatments aim to extend life
- QALYs provide a common metric for comparing cost per unit of health gain
- Aim to reflect society’s preferences for different treatments and health outcomes

Interpreting QALYs

- Trade-off between quality of life and survival
- QALY weight of 0.5:
  - individual is indifferent between current lifespan in current health state and full health for half their remaining lifespan
- QALY gain of 0.5 (eg 0.3 to 0.8):
  - individual is willing to give up nearly 70% of remaining lifespan to achieve this improvement

Where do QALY weights (ideally) come from?

- Population based preference weights
- Accurate description of the health state
  - Valid, reliable instruments for measuring/describing quality of life
  - Measured in patients experiencing the relevant disease processes and treatments
- Valid preference based methods to provide a cardinal ranking of health states (valuation task)
  - Theoretical model of preferences
  - Preference elicitation task that links to theory
  - Data collection (sampling frame; survey design)
  - Statistical analysis

Three broad approaches to “description”

- Scenario based using information from patients
- Patients complete a multi-attribute utility instrument (MAUIs)
- Patients directly value health states
- Other approaches are more ad hoc (but commonly used)
  - Views of experts
  - Literature review
  - Mapping between quality of life instruments
Three broad approaches to “valuation”

- Rating scales/Visual analogue scales
- Time trade-off experiments
- Standard gamble experiments
- Other approaches are more ad hoc
  - Views of experts
  - Literature review
  - Mapping between quality of life instruments
- New approaches in early stages of development

Visual analogue/rating task

- Health State 1
- Worst Health State
- Best Health State

Standard gamble valuation task

- Specify $h_1$
- Find $p$ such that utility of the gamble, $B$, is equal to $A$ - the certainty of health state $h_1$ for $T$ years
- ie
  \[ p \cdot v(FH) + 0 = v(h_1)'T \]
- Normalise: $v(FH) = 1$

Time trade-off

- Find $x$ such that utility of the health state 1 for time $T$, $h_1'$ is equal to the utility of full health for a proportion $x$ of $T$ years
- $v(h_1)'T = v(FH)xT$
- $x = v(h_1)'/v(FH)$

Things to note about valuation tasks

- All approaches require “thought experiments”
- All common approaches assume a constant health state applies for remaining survival duration
  - Assumed in the valuation task, but not in the application
  - Important to specify how long is “the rest of your life”
- All impose simplifying assumptions
  - Value of health state independent of:
    - how long it lasts,
    - when in lifetime it occurs,
    - other health states experienced over life cycle
  - Individuals assumed to be “risk neutral” with respect to survival and to have a simple “expected utility” approach to gambles
  - Health state doesn’t influence the value of consumption goods or activities and vice versa
- Population values obtained by simple averaging over individuals
- Whose values?

Implications for interpreting QALYs

- The valuations likely to be highly sensitive to:
  - how the health states are described
  - how the choices are framed
  - the nature of the trade-offs presented
  - how the choices are presented
- Some health states present particular challenges for the QALY approach and methods:
  - temporary health states
  - close to death health states
  - health states for children, for people with mental health problems, cognitive problems
- There is and can be no external validation
- Do the valuations pass a “plausibility” test?
Multi-attribute utility instruments: what are they?

- Generic descriptive QOL instrument
- Can be filled in by patients in trials or clinical settings
- Scoring "algorithm" that can be applied to generate valuations of health states
- Preference based measurement of MAU health states (SG, TTO or other)
- General population sample provides the valuations
- Theoretical and/or statistical methods to develop the scoring algorithm from these valuations
- The scoring algorithm gives the QALY weights for each health state described by the MAU
- Patient based measurement, community based valuation

Example: EQ-5D

- Mobility
  - No problems in walking about
  - Some problems
  - Confined to bed
- Self care
  - No problems
  - Some problems
  - Unable to wash or dress self
- Usual activities
  - No problems
  - Some problems
  - Unable to perform usual activities
- Pain/Discomfort
  - No pain or discomfort
  - Moderate pain or discomfort
  - Extreme pain or discomfort
- Anxiety/Depression
  - Not anxious or depressed
  - Moderately anxious or depressed
  - Extremely anxious or depressed

- 5 dimensions, each with 3 levels = 243 states
- General population TTO to develop scoring algorithm
- Different scoring algorithms for different countries, but UK one commonly used
- Examples
  - "11111" = 1.0
  - "11223" = 0.255
  - "21111" = 0.85

Acceptable MAUIs (PBAC guidelines)

- Health Utilities Index (HUI2, HUI3)
- Assessment of Quality of Life (AQOL)
- SF-6D (based on the SF-36)
- EQ-5D

- Note that different MAUIs may give different results

Preference for MAUIs in trial settings

- Can be readily implemented in trial setting
- Direct patient based measurement of health states
- Transparent translation from "description" to "valuation" of quality of life through the MAUI
- Repeated measures on individuals
- Avoids observer bias, framing bias etc
- Comparability across different settings

Interpretation of economic evaluation results

Incremental Cost-Effectiveness Ratio (ICER)

- The tool we use for the comparison between two options is the ICER
  \[ ICER = \frac{\text{Cost}_{\text{New}} - \text{Cost}_{\text{Comparator}}}{\text{Effectiveness}_{\text{New}} - \text{Effectiveness}_{\text{Comparator}}} \]

- How much are we, as a society, paying for each unit of outcome (death averted, sight regained etc)? Could we gain more of these units by putting our limited resources elsewhere?
- The fundamental question is this: "Does the difference in outcome between the approaches justify the difference in costs?"
### 4 Quadrant Diagram

- **Intervention:** more effective
- **Less costly**

- **Intervention:** less effective
- **More costly**

**Incremental Cost**

<table>
<thead>
<tr>
<th>Option</th>
<th>Cost (relative to comparator)</th>
<th>Outcome (relative to comparator)</th>
<th>Decision</th>
</tr>
</thead>
<tbody>
<tr>
<td>A</td>
<td>Positive</td>
<td>Positive</td>
<td>???</td>
</tr>
<tr>
<td>B</td>
<td>Positive</td>
<td>Negative</td>
<td>B is dominated</td>
</tr>
<tr>
<td>C</td>
<td>Negative</td>
<td>Negative</td>
<td>???</td>
</tr>
<tr>
<td>D</td>
<td>Negative</td>
<td>Positive</td>
<td>Comparator is dominated</td>
</tr>
</tbody>
</table>

### Making Decisions

- **Option Cost** (relative to comparator)
- **Outcome** (relative to comparator)

### Focusing on A

- A is better than the comparator, but costs more. One approach is to value each unit of outcome and then to ask whether we are getting enough benefit for our extra spending.

- Let’s say that each unit of outcome is worth $1000.

### Important note

- All health services face a budget constraint
- The ICER does not give us information about total costs and total benefits
- Total costs and benefits is also relevant to the decision
- Need to consider opportunity cost
- Willingness to pay may not stay the same — i.e., we may not have a constant threshold

### Example – An Eye Service

<table>
<thead>
<tr>
<th>Option</th>
<th>Cost ($)</th>
<th>Additional years of sight</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>2000</td>
<td>100</td>
</tr>
<tr>
<td>2</td>
<td>3000</td>
<td>90</td>
</tr>
<tr>
<td>3</td>
<td>4000</td>
<td>160</td>
</tr>
</tbody>
</table>

What is the first step in making a decision? Why do we do this?
Comparing Non-Dominated options

<table>
<thead>
<tr>
<th>Option</th>
<th>Cost ($)</th>
<th>Additional years of sight</th>
<th>Cost per year of sight</th>
<th>Incremental cost (relative to Option 1)</th>
<th>Incremental outcome (relative to Option 1)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>2 000</td>
<td>100</td>
<td>20</td>
<td>-</td>
<td>-</td>
</tr>
<tr>
<td>3</td>
<td>4 000</td>
<td>160</td>
<td>25</td>
<td>2 000</td>
<td>60</td>
</tr>
</tbody>
</table>

If we are unwilling to pay $20 per extra year of sight, we can rule out both options. If we are willing to pay $20 or more, we can now plot 1 and 3 on a 4-quadrant diagram, with 1 acting as the comparator.

4 Quadrant Diagram

- Incremental Cost
- Incremental benefit

Option 1

Threshold Line

Which of the two would we prefer? If we are willing to pay $2000 / 60 = $33 per year of sight, we prefer Option 3. If not, we prefer Option 1.

Issue to consider: what are the implications for the budget?

Remember opportunity cost!

Sensitivity Analysis

- Most published analyses will contain a sensitivity analysis. This is an attempt to show how confident the authors are in their conclusions.

- The values used in the analysis (e.g. the drug cost, the mortality improvement) are varied within ‘reasonable’ limits to see if the results and conclusions change.

- If the conclusions do not change, they are said to be ‘robust’.

So What? – The Transferability of Results

- Given the caveats and uncertainties we have about how costs and benefits are measured, an economic evaluation will show us the preferred option between two in a particular area in a particular group of people. Unless we are dealing with that specific group, the question is “So What?”

- Inevitably, different groups will have different costs and different outcomes – what determines whether a result is transferable?

Transferability Criteria - Example

A study shows that Intervention X is cost-effective in a German population. Is this transferable to an Australian population?

Possible questions to ask:
1. Are the costs of Intervention X likely to be similar in Australia and Germany?
2. Are the outcomes likely to be similar?
3. How convincing is the conclusion regarding the German population?
4. Is there any reason to suppose the two populations value health differently?