

# THERE'S SOMETHING MISSING FROM THIS GRANT....MAKING SENSE OF THE DOLLARS.

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# What's it all about?

# Making Decisions.

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- Many safe, effective health care interventions that could be funded
- But... resources are scarce
  - ▣ Demands on the health care budget from technological advances, population change and expectations
- Health care budget is limited relative to available treatments
  - ▣ Choices need to be made:
  - ▣ Which interventions, how much, how often, for whom?
- Need a decision making rule to maximise benefit from available resources

# A Role for Economic Evaluation

- Economic evaluation is a way of informing decision making.
- It compares costs and outcomes of two (or more) approaches to the same problem.
  - *eg. What is the cost per new case detected for liquid based cytology compared with Pap testing for cervical cancer screening?*



# Economic Evaluation

- ▣ Aims to answer the question: “will this program/intervention increase the total health of society?”
- ▣ While the intervention may be more effective than the alternative, it will displace something else (finite budget)
  - Trials provide information on both elements: whether the intervention(s) works, and what it displaces.
  - Economic evaluation asks whether the additional outcomes justify what has been displaced.

# Key Steps

- Define alternatives A and B (always comparative)
- Define perspective and time frame
- Choose appropriate evaluation tool
- Identify, measure and value costs and outcomes
- Combine costs and effectiveness

$$ICER = \frac{Cost_{New} - Cost_{Comparator}}{Effectiveness_{New} - Effectiveness_{Comparator}}$$

- Assess robustness of results (sensitivity analysis)
- Interpret

# Types of Economic Evaluation

	Costs	Outcomes
Cost-minimisation: CMA	Which is the cheapest?	No difference in type or efficacy.
Cost-effectiveness: CEA	Compare costs between alternatives.	Differ in efficacy. Measured in natural units eg. patients with a response.
Cost-utility: CUA	Compare costs between alternatives.	Differ in efficacy. Measured in common metric: QALYs.
Cost-benefit: CBA	Compare costs between alternatives.	Differ in efficacy. All outcomes are measured in dollar values.

# Costs and consequences

- Sometimes, it is not possible or we don't wish to compare interventions directly
- Cost consequence analysis allow us to present the relevant information in a meaningful manner:
  - ▣ estimate the costs and consequences of an intervention and “lay them out” but make no assessment of relative value.



# Thinking About Costs

- All the resources used by patients undergoing care (in both treatment/study groups):
  - ▣ Drug costs including administration (eg. infusion)
  - ▣ Monitoring and diagnostics
  - ▣ Treatment and adverse events
  - ▣ Radiotherapy
  - ▣ Other medical services
  - ▣ Events associated with the condition (eg. cognitive decline).
  - ▣ Within study and downstream.

# Calculating Costs

- There is no magic to estimating the costs of adding a new treatment/service or expanding an existing one.
- Three steps:
  - ▣ Identify the resources used:
    - All inputs into a service
    - Before, during and after
    - Side effects and unintended consequences
  - ▣ Measure the number or amount of resources used
  - ▣ Assign unit costs (prices) to each type of resource

# Issues to Consider

- Prices are not the same as cost (and certainly not opportunity cost).
- Costs can differ by age, sex, disease severity, co-morbidities, case mix etc.
- Which costs - related to perspective (*covered later*).
- Sources for costing information (*next slides*).
- Is the resource use driven by the trial?

# Sources of Data

- Clinical trials:
  - ▣ Direct measure of resource use
- Secondary databases (Medicare data, hospital datasets etc.):
  - ▣ Provide measure and value of resource use
- Other primary data-collection:
  - ▣ Patients (recall can be an issue)

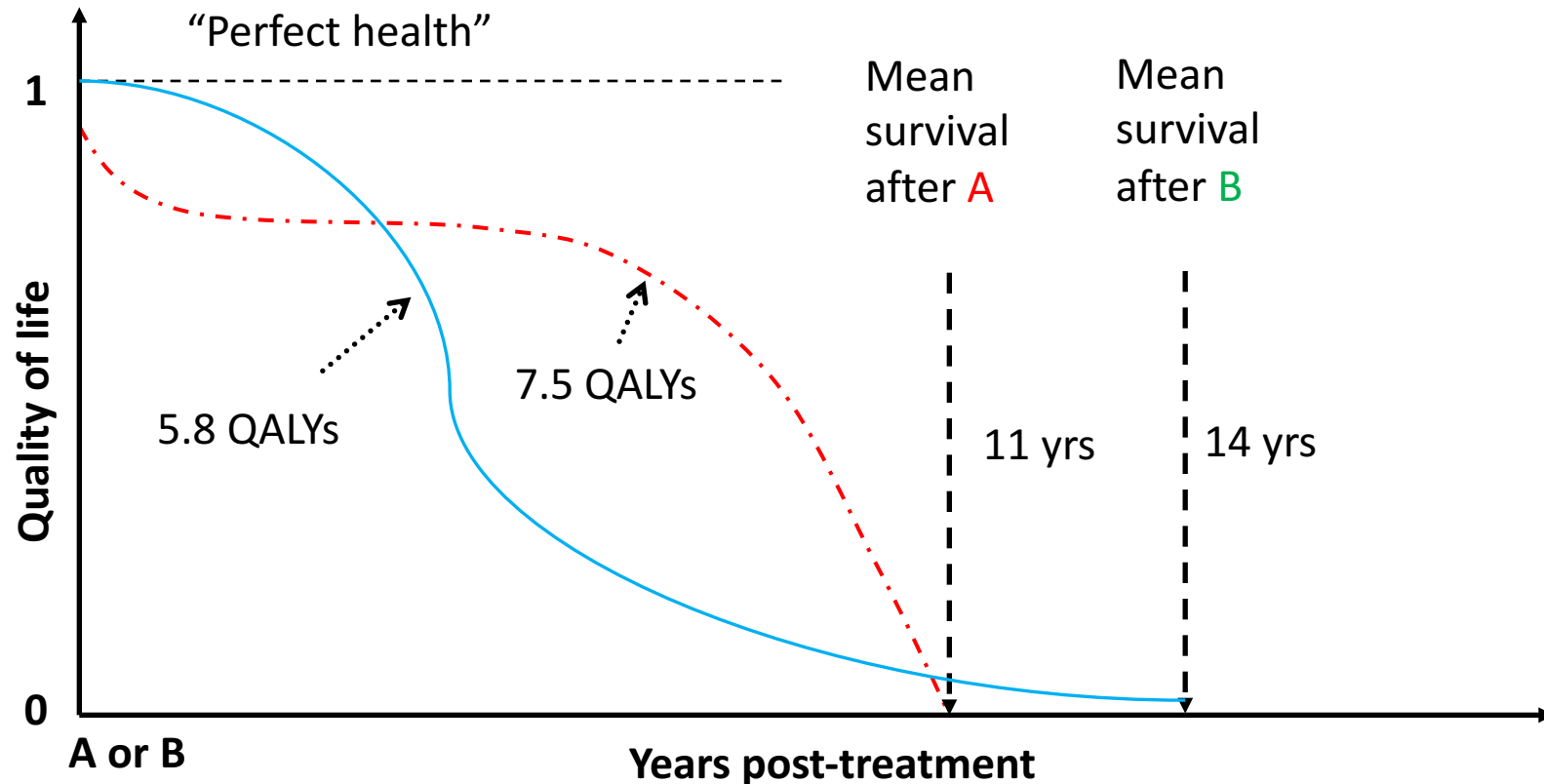
# Outcomes – the Other Side of the Coin

- Same three steps as costs: identify, measure and value
- Identify
  - ▣ What is the outcome in which we are interested; is it intermediary or final (more on this in minute)?
  - ▣ How much of that outcome is produced?
  - ▣ What is the value we place on that outcome (defined by our evaluation approach)?

# Outcomes – the Other Side of the Coin

- What are the relevant clinical outcomes or patient relevant outcomes?
  - ▣ Trial based surrogate outcomes (eg. tumour response) difficult.
  - ▣ More relevant outcome is the impact on final outcomes – survival and quality adjusted survival.
- Applying quality of life weights (utility values) to survival allows QALYs to be estimated (CUA) – CBA would use \$.

# Life-years or QALYs



# QALYs as an Outcome

- “Quality Adjustment” (utility value) reflects value of health states relative to full health
  - ▣ QALY weight = 1 for “full health”
  - ▣ QALY weight = 0 for death
  - ▣ “Worse than death” health states permissible (QALY weight < 0)
- Population based preference weights
  - ▣ Preference based *index*
  - ▣ Measured on a cardinal scale, anchored at 0 and 1
- The metric for measuring strength of preference is survival
  - ▣ QALYs measure strength of preference for survival and quality of life and trade-offs between the two



# Where do we get the QALY weights?

- Identification/Description/Measurement of health state
  - ▣ Measured in patients
  - ▣ With accurate methods that are sensitive to relevant effects
  - ▣ Use multi-attribute utility instruments eg. EQ-5D-5L.
- Valuation of health states among a community sample
  - ▣ Scenarios based on QOL typically used
  - ▣ Use population values to reflect preferences of the “tax-payer”
- Patient rated and community valued.

# Comparing Costs and Outcomes

$$ICER = \frac{Cost_{New} - Cost_{Comparator}}{Effectiveness_{New} - Effectiveness_{Comparator}}$$

- Does this represent value for money?
  - ▣ No explicit decision rule for Australia, depends on how much society is willing to pay for the outcome of interest.
- Have we captured all the relevant cost and outcomes?
- Are they measured and valued appropriately?
- What is missing?
- Do the results stand up to sensitivity analyses?

# Why do I care?

# Do all Studies Need Economic Evaluations?

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- Not all studies need or are amenable to an economic evaluation:
  - ▣ It might be a single arm study, or not aimed at changing practice.
  - ▣ It is early in the development phase, so it is not clear how it will be used in “usual” care.
- An economic evaluation might be needed if:
  - ▣ Aim to change practice
  - ▣ Resource allocation decisions need to be made
  - ▣ When two or more alternatives may be appropriate
  - ▣ When there is evidence of differences in effect and/or resource use

# Do I Need an Economic Evaluation?

- Is there a clinical or policy imperative to evaluating costs and outcomes of the alternatives being studied?
- If yes, what is the nature of the economic issue?
  - ▣ What are the anticipated differences between the alternatives?
    - In effects?
    - In costs?
    - In both?

# Approaches to Economic Evaluation

- *‘Trial based’ studies* – data on resource use and quality of life are collected alongside the clinical trial
  - ▣ Trial design (e.g. comparators, endpoints, length of trial, practice settings)
  - ▣ Analysis of multinational trials to generate locally specific estimates of cost-effectiveness
- *‘Modelling’ studies* – data from several sources are synthesised in a decision-analytic model
  - ▣ Model structure, that can then be populated with relevant data
  - ▣ Attention to issues surrounding the transferability of economic data
  - ▣ Attention to applicability of data from other sources to the trial data

# Economic evaluations in clinical trials

- The economic question is rarely the primary aim of a clinical trial
- Why the economic question may be important
  - ▣ Funding new technology
  - ▣ Changing behavior/uptake
  - ▣ Efficient allocation of resources
- External validity is very important for the purposes of the EE

# Threats to external validity

- Most clinical trials have high internal, but may have low external validity.
- The threats to external validity come from:
  - ▣ restrictive inclusion and exclusion criteria (patient population, disease severity, co-morbidities)
  - ▣ protocol-driven resource use (could bias costs in each treatment arm)
  - ▣ unrepresentative recruiting centres (e.g. academic hospitals),
  - ▣ inclusion of study sites from countries with varying access and availability of healthcare services
  - ▣ artificially enhanced compliance.



# Why do Economic Evaluation Within Trials?

- Capture resource use data
- Can measure patient relevant outcomes in patients undergoing the treatments
- Timely translation of trial outcomes to health care decision making, including funding decisions
- Potentially, reduce uncertainty

# Why use models?

- To synthesize head-to-head comparisons where relevant trials do not exist
- To apply the trial analysis to the relevant clinical context
  - ▣ Local treatment patterns, or patient characteristics
- To inform decisions in the absence of definitive data
  - ▣ Extrapolate beyond period of trial
  - ▣ Link intermediate outcomes to final, or apply 'values' (utility)

# Resulting Grant Input...a combination

We will undertake an economic evaluation of .....compared with .....

The evaluation will be undertaken from the health system perspective/societal perspective.

The economic evaluation will comprise a cost-effectiveness analysis and a cost-utility analysis. For the cost-effectiveness analysis we will use the primary outcome and estimate the cost per YY over the duration of the trial and follow-up period.

We will also use the individual quality of life information from the trial to undertake a cost-utility analysis over the duration of the trial and follow-up period. We will then develop an economic model to estimate the costs and outcomes over an XX year time frame.