Economic appraisal in health care: study design and patient registries

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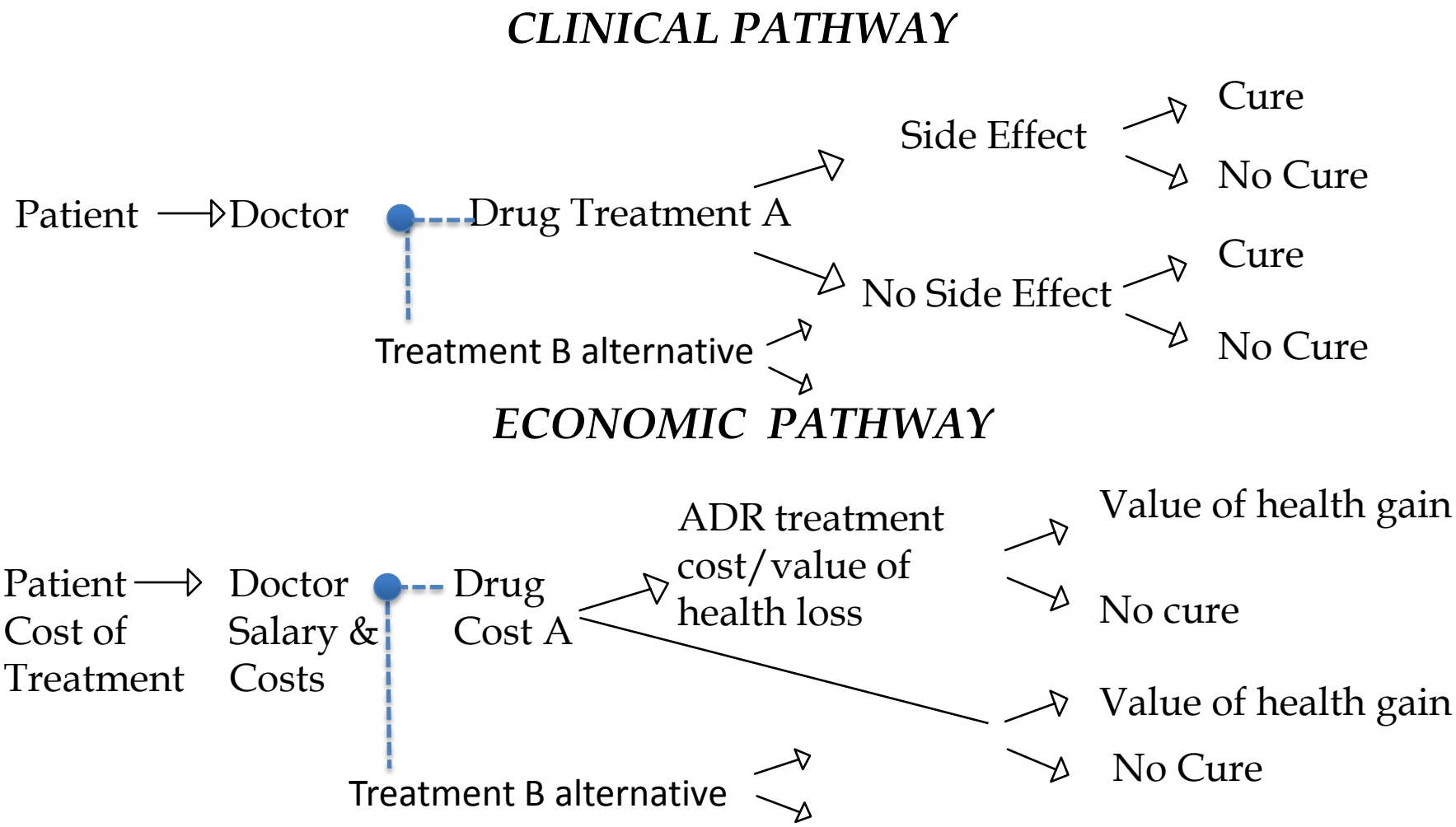
What is economic appraisal in health

- Economic appraisal is concerned with the analysis of alternative courses of action.
- The aim is to choose that course of action which maximises social welfare (subsidise either a new drug, procedure or test or stay with the status quo)
  - recognising the scarcity of resources means we do have to make choices and that will often mean giving up the welfare of some people to improve the welfare of others

Clinical and Economic Pathways



Classic Cost Effectiveness Methodology

From Weinstein & Stason

$$C/E = \frac{\Delta C_{RX} + \Delta C_{SE} - \Delta C_{morb}}{\Delta E}$$

Decisions made easy

- Compare the ICER of any intervention to a threshold value social willingness to pay for the outcome (life year, life years adjusted for quality of life, months in remission, heroin free days)
- If less than the threshold decide to fund if greater than decide not to fund. The total spent will then be the implicit budget constraint
- So if service A has an ICER of $50,000 and your threshold is $100,000 fund it.
  - Note you need to look at all of the possible interventions at once or your budget might blow out especially if the assumption of divisibility does not hold

Economic evaluation study designs

1. Experimental
   - Economic evaluation as part of a clinical trial
   a) Many examples and a textbook
2. Observational
   - data on use, survival, quality of life and health costs collected in a longitudinal study/ before and after design
3. Modelled evaluation
   - Clinical results used combined with economic data from a variety of sources perhaps extrapolated beyond the time frame or population of the clinical study

Modelled evaluation and registry data

a) Example: in a decision analytic models of evaluation of hereditary predisposition colorectal cancer Ramsey et al (2001) estimated the survival advantage for subtotal colectomy compared with standard therapy, in a matched case–control study using data from a registry.
b) Genotype-assisted antiretroviral resistance testing (MSAC report 1067) where the treatment regimens over time for HART were taken from the experience of the patients followed in the Australian HIV Observational Database.
c) Quality of life data could be collected prospectively in registry (Brennan et al 2007)
d) Resource use data (e.g. hospitalisation) could be collected retrospectively in a registry

Determination of Costs

What Costs to Include in Study?

- Depend whether the aim is a social evaluation or a managerial one – whose objective and whose budget
- Perspective of study (e.g. societal, health sector; hospital)

Examples of what has been included as a cost

- Societal costs = health + non health
- Health
  - Medical- most of these trials included visits to GPs, specialists, drugs, tests, emergency, hospitalisations
  - Non medical – in wound care “waste disposal” a significant cost of traditional dressings (comparator)
- Non health
  - in IDEAL labour force attachment
  - in bupernorphine crime and employment also less travel for buprenorphine than methadone

Direct Health Care Costs (or Savings)

- surgery, drugs, ward costs (re-treatment in shoulder trial)
- costs (savings) of tests and treatments induced as a result of treatment or information provided
  - Saving in waste disposal for wound dressings
- costs of treating side effects - dialysis
- savings from avoided morbidity costs of treating conditions during any added years of life – ESRF comorbidity

Non health system costs

Direct Personal costs

- travel costs – buprenorphine vs methadone
- home care services – home dialysis
- equipment for use at home - home dialysis

Indirect Personal Costs

- Current or future productivity gains or losses – buprenorphine studies
- opportunity cost of time spent by patients (e.g. travel, waiting time, treatment time) buprenorphine in NDARC study
- Costs to others
  - Crime in heroin addiction studies

Collecting resource use and unit cost data

- Collect resource use in natural units
- Administrative data sources for resource use and unit costs – not always a good match (dialysis in IDEAL trial)
- Medicare Australia, hospital records by individual patient (IDEAL trial)
- Diaries or survey for resource use over time –(crime in buprenorphine trial; our of study centre doctor visits in IDEAL trial)
- Value units at opportunity cost in principle
  - In practice prices are often used eg cost of physio

Example of a question in the monthly patient survey in physio trial

VISITS TO DOCTORS: Have you been seen by any doctors in the community this month?

Yes / No

If yes, please write the number of times you were seen by a GP and the number of times you were seen by a specialist (other than the regular trial visits):

____ GP visits

Specialist visits:

No of Rheumatologist visits_____

No of Orthopaedic surgeon visits_____

No of Musculosketal doctor visits_____

No of Sports Physician visits_____

Other specialist doctor_____

Issues in the analysis of cost

- Intention to treat
- Missing data
- Non normal distribution of cost data
- Repeated measures
- Censoring
- Adjustment for any baseline differences
- Clustering of costs and outcomes by centre or patient

Measuring outcomes in an economic evaluation

- Most often restricted to the difference in the sum of health benefits across individuals in each arm of the study
- Health benefits often measured as one or more of:

1. clinical endpoint e.g. increment in death/life years; time free of illness or symptoms
2. Difference in quality adjusted life years (QALYs)
3. Value of those QALYs

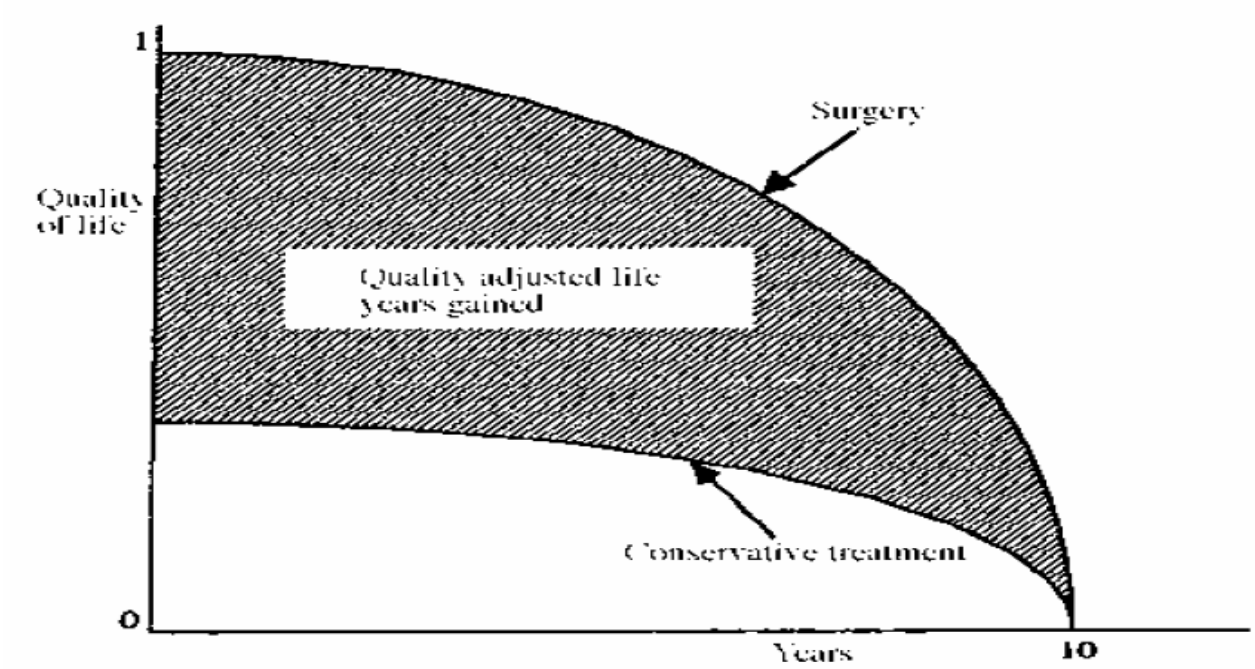
TO QUALITY ADJUSTED LIFE YEARS

Health is a combination of
life expectancy (time) and quality of life

A useful summary measure therefore is the
QUALITY ADJUSTED LIFE YEAR
or QALY

QALYs

Quality Adjusted Life Years Gained – Surgery Versus Conservative Treatment



Note that the quality of life has been standardise d to a 0-1 scale with perfect health equal to 1 and death equal to 0

Measurement of QALY Weights

- Properties of an index of health status
  - Need to have a scale that is between 0-1 that has ratio properties that reflects preferences over health states
  - The content of the scale; perfect health – good health – death - worse than death

- Methods of eliciting preference values for health states. Time trade off, rating scale
- There are some off the shelf preference based measurement instruments that have been designed to have these properties and can be administered in a study
- e.g. AqoL, EQ5D, HUI, SF6D mapping from SF36 or SF12
  - Example of use in a registry cohort: Kobelt et al 2002. 500 patients scheduled for cataract extraction were asked to complete the EQ-5D,, before surgery. Multiple regression analysis was used to estimate the QALY gain through the intervention

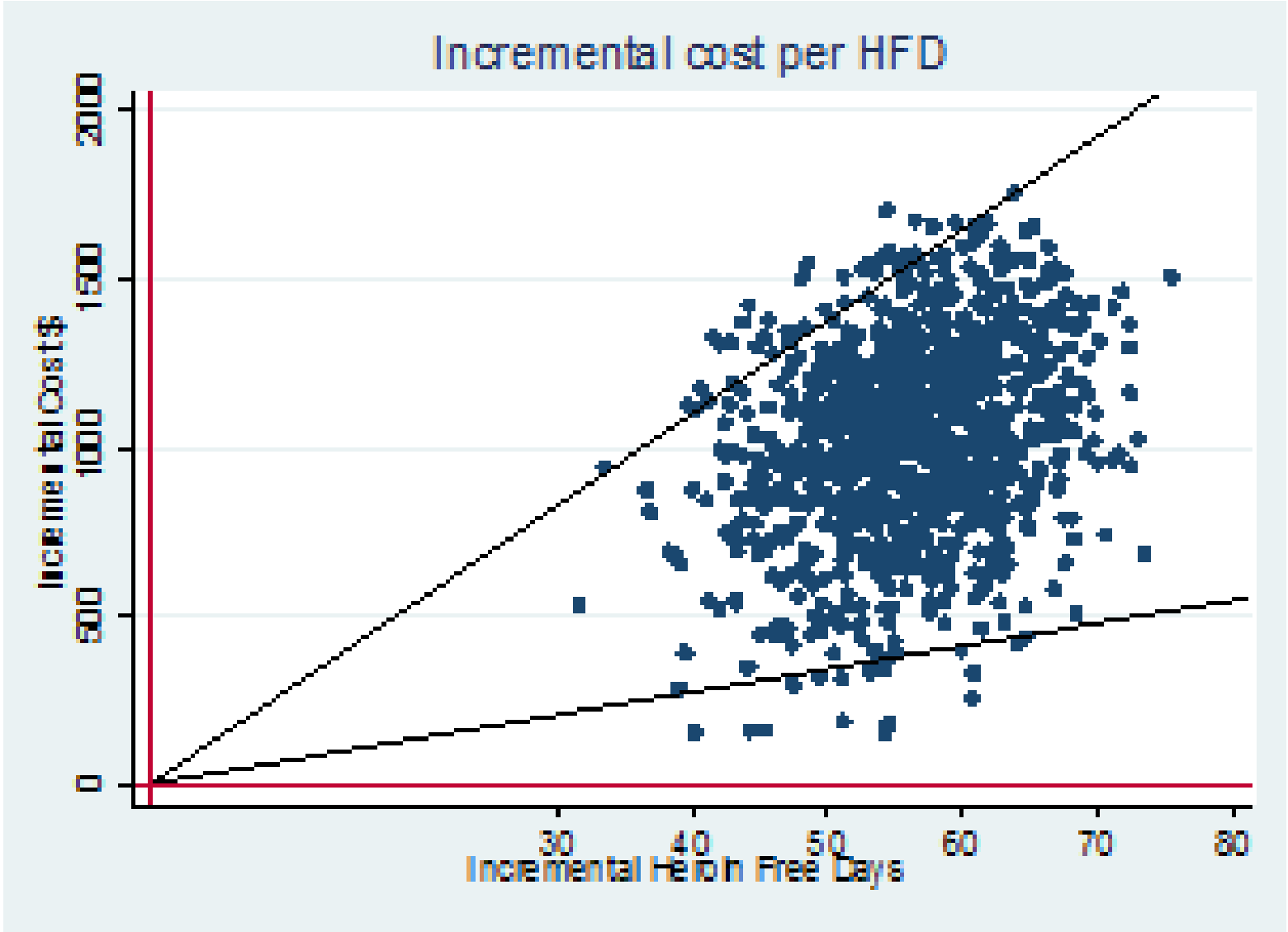
Issues with QALYs

- How to measure and interpret the quality of life weights for each time period;

- Whether it is legitimate to combine such weights over time and across individuals.

Calculation of ICER

- In two trials of a pharmacotherapy for heroin addiction we constructed a picture of distribution of the incremental cost effective ratio (ICER = additional cost/additional outcome)
- We used non parametric bootstrapping
- Criterion for cost effectiveness was that 95% of ICERs were less than an acceptable threshold
  - (and in the appropriate quadrants of the ICER space)


Incremental cost per HFD
Incremental Cost ($)
0
500
1000
1500
2000
30
40
50
60
70
80
Incremental Health Free Days

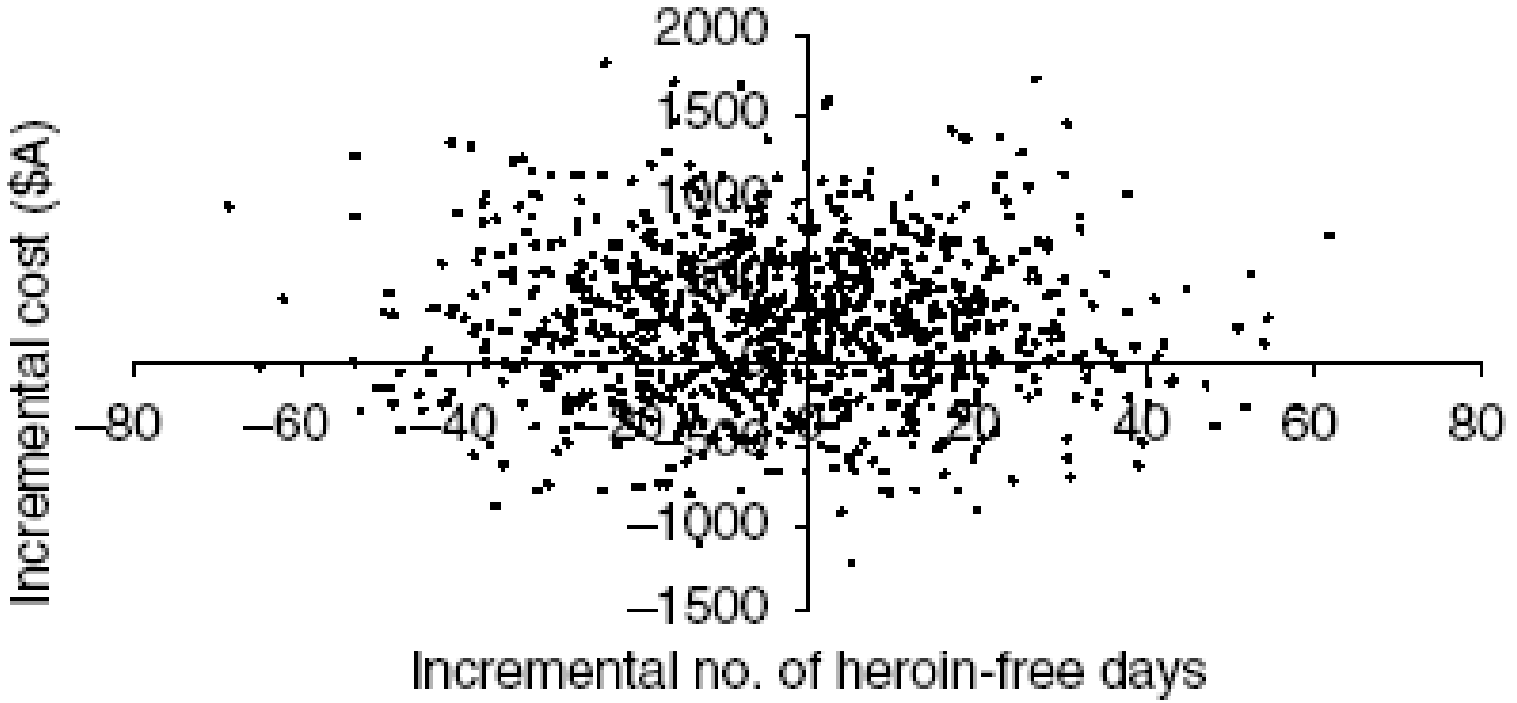


Fig. 2. Bootstrapped unadjusted mean incremental cost per extra heroin-free day for buprenorphine compared with methadone for the whole sample (excluding crime costs). **$A** = Australian dollars (1999 values).

Use of patient disease or exposure registry data in an economic analysis

- Controlled trials may be too short term for full health and economic impact to be determined with confidence e.g. cancer screening, long term costs with overdispersion
- Registries as a source of data for decision analytic modelled cost effectiveness studies
  - Explore broader outcomes, including health-related quality of life & other patient-reported outcomes
  - Assess economic costs
- Registries as a source of primary cost effectiveness data
  - Examine associations between care and outcomes in case control or cohort studies
  - Inform policy questions e.g. compliance with (funded) appropriate use
    - It seems likely that more reimbursement decisions will be conditional on establishment of a registry to confirm trial based patient outcomes (CED) and event rare outcomes

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