# Introduction to Economic and Cost-Effectiveness Analyses

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# Learning Objectives (1)

- Learners will be able to:
  - Indicate why cost considerations are relevant to health and health research
  - Describe the major types of economic analyses used to evaluate health interventions
  - Identify key ingredients of an economic analysis in the health setting
  - Define possible ways to express the benefits of health interventions, for economic purposes

## Learning Objectives (2)

- Learners will be able to:
  - Identify elements of health care cost estimates, and some potential challenges
  - Identify features of a credible and informative analysis
  - Understand the audience for, and potential impact of, economic analyses for the assessment of health interventions

## Decisions, decisions...

- Why have "we" chosen to allow:
  - Gaps in basic TB diagnosis and care?
  - Gaps in health care insurance and coverage?
  - Emergence of a parallel private system of TB care in many settings?

## Decisions, decisions...

- As societies, we (through elected officials, other policymakers as well as individually) decide how much to spend on health care, versus
  - Education
  - Road work and repair
  - Job creation
  - Housing
  - Food
  - Etc etc

## Decisions, decisions...

- Within the health sphere, we must distribute resources between
  - Different institutions and care settings
  - Different health conditions and goals
  - Different approaches (primary prevention, acute treatment, rehabilitation, secondary prevention, chronic disease management)
  - New vs. established technologies and treatments

## Why so many decisions???

- The fundamental issue is:
  - The demand and potential uses for finite resources exceed their availability
  - So we are obliged to choose between competing uses of these resources
- We face this as individuals, and as societies
- Note that resources are often expressed in, but are not limited to, \$
  - Trained personnel, space, time, etc.

#### Health economics

- "Economics is the social science that analyzes the production, distribution, and consumption of goods and services."
- "Health economics is a branch of economics concerned with scarcity in the allocation of health and health care."

Both from Wikipedia: http://en.wikipedia.org/wiki/Economics

## Economic analyses in health care

- Designed to help clinicians, policymakers, payers make choices
- As such, any economic analysis always involves a choice between ≥ 2 options
  - There must always be a comparison option
  - It may be explicit e.g. treatment B compared to treatment A
  - Or the comparator may be implicit e.g. current practice or "do nothing"

## All health economic analyses:

- Share two major ingredients
  - Cost (what do we pay or save if we choose one course of action vs. another)
  - Health outcomes (what do we get accordingly?)
- May be classified and understood according to how they address these two dimensions

## 1. Cost minimization analysis

- "Pure" cost comparison
- The cheapest intervention is preferred
- Health outcomes are not explicitly assessed
  - They are implicitly assumed EQUIVALENT
  - If health outcomes are not equivalent, then the analysis should not be restricted to costs, and simple cost minimization is inappropriate

## Cost minimization analysis

 Example: sputum induction in an Arctic setting, vs. transfer of patients South for diagnosis

## Cost minimization analysis

- Note that even if the focus is on one intervention (e.g. a new technology), there is still an implicit comparison with current practice
- In this example we would want to know if the cost of the necessary equipment, supplies and personnel is outweighed by savings on transport of patients out of the community
- We also need to know how long the equipment will last

## 2. Cost-effectiveness analysis

- Here, both cost and health outcomes are explicitly considered
  - Costs usually expressed as \$
  - Outcomes expressed in relevant health units
    - Cases of TB diagnosed, years of life gained, cases cured, etc.
- Again, at least two alternatives are compared

## Cost-effectiveness analysis

- Imagine we are comparing interventions
   A and B
  - A is standard treatment, and B is a novel treatment for the same condition
- If B is cheaper than A AND more effective, then which should we choose?
- If B is more expensive than A AND less effective, then which should we choose?

## Cost-effectiveness analysis

- Suppose B is more expensive than A, but also more effective—or that B is cheaper than A, but also less effective
- Then which should we choose?

#### The incremental cost-effectiveness ratio

- Basically "it depends"
- We need to know the additional cost of the more expensive treatment, per additional gain in health, i.e.

#### $\Delta$ cost $\div \Delta$ health effect

 This is known as the "incremental costeffectiveness ratio," or ICER

## The ICER: an example

- Drug combination A is associated with a 90% cure rate. It costs the NTP \$100 per treatment course.
- Drug combination B is associated with a 95% cure rate, and is shorter. It costs the NTP \$1000 per treatment course.

## ICER example continued

- The price difference between B and A is \$900 per treatment course (\$1000 -\$100)
- But the cost to the NTP per additional TB case cured is:

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($1000 - $100)/(0.95 - 0.9)
= $18,000
```

#### Is combination B worthwhile?

- The decision to adopt B depends whether we consider \$18,000/additional TB case cured a reasonable figure
  - Would it be better to spend this on something else?
- The answer may depend on whose perspective is used
- The perspective may also influence the cost figures (more on this later)

## Is this ICER useful?

- By itself, this result cannot tell us whether we should continue with combination A, or adopt B instead
- It really depends on our values, and on other possible choices and constraints
- However, it makes the decision making process more explicit
  - We have some data to work with, rather than just a judgment or "gut feeling"

## 3. Cost-utility analysis

- A special type of cost-effectiveness analysis in which health effects are expressed in units of quality-adjusted survival
  - Quality-adjusted life years (QALYs)
  - Disability-adjusted life years (DALYs)
- These allow us to capture both morbidity and mortality

## Quality-adjusted life years

- The basic premise is that "quality" of a particular state of health is captured on a scale ranging from 0 (death) -1 (perfect health), which is then used to adjust survival
- For example, 0.5 QALY could be
  - Six months spent in perfect health
  - One year spent in poor health, valued as halfway between death and perfect health

## Quality-adjusted life years

- Several methods exist for obtaining these quality scores
- As it is not specific to any particular disease or condition, quality-adjusted survival can be used to compare the health impacts of interventions that target different illnesses
- Analogous to generic (not diseasespecific) quality of life measures

## Disability-adjusted life years

- Used by the World Health Organization
- Similar to QALYs in general terms
- More narrow range of disability adjustment weights (only 7 values other than 0 and 1)
- Derived from a panel of health care workers in 1995
- Weighted by age

## Cost-utility analysis

- Incremental cost-effectiveness ratios expressed as \$ per QALY/DALY gained
- Some suggest that thresholds of \$50,000 or \$100,000 per QALY gained may be applied to determine whether interventions represent a reasonable investment
- Based on estimates for standard treatments e.g. hemodialysis

## 4. Cost-benefit analysis

- Health outcomes valued in dollar terms (e.g. a dollar value is attached to each year of life gained) instead of health units
- Several techniques used to derive these dollar values, e.g. "willingness to pay" surveys, "revealed preferences" based on additional pay for hazardous work

## Cost-benefit analysis

- Then net costs or savings for any intervention can be estimated, and compared to other interventions
- Very controversial, because it involves direct attribution of dollar value to health and survival
- Performed much less often than costeffectiveness and cost-utility analyses
- May be most relevant for comparing alternatives inside and outside the health sphere

## Perspective

- The perspective from which the analysis is conducted is crucial; it must be stated
- It dictates which costs are included
  - Health care system perspective includes all costs borne by the health system, but none borne by patients e.g. travel, time off work
  - Societal perspective also includes costs borne by patients and families (e.g. lost work time, out-of-pocket expenses, etc.)
    - This is considered the preferred approach

#### Time frame

- As with perspective, the time frame of the analysis must also be specified
- Many interventions bring immediate costs, but longer-term savings
- Similarly, some interventions bring substantial health benefits, but only further in the future

#### Time frame

- The time frame of the analysis should be appropriate to the intervention(s) being evaluated, and must be specified
  - You would not accept a one-year time frame for an analysis of a vaccination program, or an LTBI screening and treatment program
  - The time frame must be the same for both costs and outcomes in a given analysis

### Year for costs

- The year for costs e.g. 2014 Canadian dollars must be specified
- This is to account for inflation e.g. if you know how much a nurse was paid in 2006 and the rest of your cost data are from 2014, you would express all costs in 2014 dollars and adjust the cost of nurses' time to 2014 using inflation rates between 2006-2014

## Discounting

- Refers to the notion that money spent or gained in the future is valued less by society than money spent or gained today
  - This is above and beyond inflation
  - It is the reason that we expect to earn interest or income on investments
- The same applies to health outcomes, e.g. a year of life gained today is valued more than a year of life gained 20 years in the future

## Discounting

- The discount rate (usually abbreviated as r) is used to convert costs and health events in the future to present-day values
- \$1 spent or saved in year  $X = \frac{1}{(1+r)^x}$
- A discount rate of 3% (r = 0.03) is recommended [though this can vary]
- So \$1000 saved 10 years from now =  $$1000/(1.03)^{10} = $744 \text{ today}$

## Estimating costs

- Expendable materials and supplies
- Personnel time
  - Direct observation ("time and motion" studies) vs. estimated share of total hours worked
- Shared costs (overhead) e.g. prorated share of hospital administration, medical records, housekeeping, heating, etc.
- Capital depreciation
  - Most relevant for expensive equipment that is used repeatedly but has a limited life span
  - Like cars...

## Estimating costs

- Suppose you are asked to advise the government about a new diagnostic modality for smear-negative pulmonary TB
- How would you estimate the costs, from a societal point of view?

## Estimating costs

- Cost of equipment, and its lifespan, i.e. how many samples it can analyze
- Supplies/expendables for each sample
- Power needed
- Technician time, including training
- Patient/family travel time, time off work, child care, etc.
- If you expect cost differences related to differences in diagnostic yield for TB, you will need to account for these

## Challenges

- Charges vs. costs
- Complex nature of some costs e.g. shared costs
- Hospital "per diem" often used—sometimes broken down by ward type
  - Less precise, since it averages out patients with different problems and severity of illness
  - Much simpler to obtain than highly detailed costs for every activity
  - May be adequate for many analyses where precise detail is less important

## Challenges

- Some hospitals or groups of hospitals have cost data according to primary diagnosis and level of complexity (e.g. ICU vs. no ICU)
- Some events e.g. complications are very rare but extremely expensive, and can skew cost estimates
- Patient and family time for travel, time off work, etc. may be difficult to obtain unless specifically sought, and may also be difficult to value
  - Most people are understandably reluctant to provide details of their income
  - May be best to impute "typical" wage for a person of the age in question

### Health outcomes

- Should be expressed in terms that are relevant to patients, providers and decision makers
- Should reflect the question at hand
  - If you are comparing two interventions for TB treatment, then it would be appropriate—and simpler—to focus on cure rates after 1 year, rather than on long-term health outcomes or QALYs
  - If you are trying to decide whether to invest in vaccination or new diagnostics, then you need common units (e.g. QALYs gained) and a longer time horizon

### Data sources: Health outcomes

- Traditional hierarchy of evidence e.g. metaanalysis of multiple randomized clinical trials > single RCTs > observational studies
- Ideally these compare the strategies in which you are interested, in a similar spectrum of patients
  - If the evidence does not support an intervention, then economic analysis is irrelevant
- Quality weights may have to be obtained separately

### Data sources: Costs

- Ideally should come from same setting as health outcome data
  - e.g. costs for each patient estimated in an RCT comparing two interventions
- Frequently not possible
  - The source should at least be consistent, credible, appropriate to the question at hand, and (ideally) accessible
- Most economic analyses tabulate the volume of services used, and the cost per service
- This makes it easier for others to substitute their own local costs, and know what to expect

### Techniques for cost estimates

- 1. Direct measurement
  - Cost minimization studies, for research questions where it is reasonable to measure costs and savings using a short time frame
  - Clinical studies (RCTs) where relevant health outcomes and costs can be gathered as part of study procedures and follow-up

### Direct measurement

- Same concerns as in other clinical studies regarding variance in data, bias, generalizability etc.
  - RCTs challenging for representative costs, since patients are highly selected
  - Study procedures (e.g. intensive follow-up) may further distort costs and inflate adherence
- Uncertainty and bias handled as in other clinical studies e.g. significance testing, confidence intervals, multivariate adjustment, etc.

### Techniques for cost estimates

#### 2. Prediction

- Incorporation (often extrapolation) of health and cost data from different sources, which may extend beyond the scope and time frame of the primary research studies
- Integrated to provide expected outcomes and costs for different interventions e.g. meta-analyses, decision analysis or transmission models
- Issues of adequacy of source data
- Impact of uncertainty in model inputs usually addressed by sensitivity analysis and/or probabilistic techniques e.g. Monte Carlo simulation

## Who is the target audience?

### Funders/payers

- Very interested in costs, and in what costs are borne by whom
- May be less interested in subtle differences in health outcomes, and in prevention of events further into the future

#### Clinicians

- Focus on the patients in front of them
- Primarily concerned with discrete health outcomes
- Would certainly favour cheaper interventions with similar or better outcomes

## Who is the target audience?

#### Patients and families

- Most concerned about health outcomes, including complications
  - Focus on specific outcomes as opposed to QALYs etc.
- Concerned about costs to them e.g. lost work, travel, child care, other out-of-pocket costs
- Public health decision makers
  - Optimal resource allocation across many competing alternatives
  - "Generic" outcome measures (e.g. QALYs) more relevant
  - Additional health gain per additional cost
  - Questions of equity

# Summary (1)

- As a society and as individuals, we must choose between competing uses for limited resources
- Economic analyses of existing and novel interventions can help us make better informed choices
  - They are not the whole answer, and other considerations must be kept in mind
- We must consider both cost and health outcome components

# Summary (2)

- Cost-minimization and cost-effectiveness (± cost-utility) analyses are most frequent in health research
- Costing may not be straightforward
- Role of perspective and timing
- Importance of relevant health outcomes and evidence
- Approach health economic analyses with both curiosity and skepticism, as you would any other clinical research evidence
- THANK YOU!