Cost-Effectiveness Analysis in Health Services Research: An Overview

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Health Care Choices:
The Clinical vs. Economic Paradigm
Introduction

- Provide a medical service to a patient if:
  - it has any benefit (clinical paradigm)
  - its incremental benefit outweighs its incremental cost (economic paradigm)
- Example: If a candy bar costs $1 and it’s only worth 50¢ to you, you wouldn’t buy it
- Same thing with health; if a service costs $500 and society only values the health improvements at $150, shouldn’t pay for it
“Opportunity Costs”

- Opportunity cost: value of what you give up
- With limited resources, providing service to one patient reduces resources for others
  - Resources limited, since society is unwilling to spend all of its money on health care
  - Health care will always be rationed somehow, so better to consciously decide tradeoffs
  - CEA especially important when society is practicing “flat of the curve” medicine
Diminishing Returns and “Flat of the Curve” Medicine
“Optimal” Amount of Health Care Under Clinical vs. Economic Paradigm

Health Benefits, Costs in $

Maximum health status at MB=MC

Maximum social welfare

Health Care Inputs

Costs

Health Benefits
Efficacy ≠ Effectiveness

- **Efficacy** is how well treatment works in ideal circumstances.
- **Effectiveness** is how well treatment works in real life.
- Treatments may work less well in practice than clinical settings, due to patient non-compliance and comorbidity.
- C/E analysis always based on effectiveness.
Cost-Minimization, Effectiveness and Cost-Effectiveness

- Cost-Effective $\neq$ Cost-Minimizing
  - An intervention can be cost-effective even if it increases costs

- Effective $\neq$ Cost-Effective
  - An intervention can provide a health benefit without giving the best health "value" for the $
Example

- You have $150K to treat fatal disease X
- Treatment 1
  - $150 per patient with disease X
  - 10% chance of saving person’s life
- Treatment 2
  - $50 per patient with disease X
  - 5% chance of saving the person’s life
- Important: Not enough $ to treat everybody
- Q: How should you spend the money?
Answer: Spend it all on T2

- T1 has greater benefit for individual
- But T2 has greater benefit for population
- T1 is twice as effective (2x chance of saving life) but cost 3x as much
- Spending $150K on T1 saves 100 lives ($1,500/life saved)
- Spending $150K on T2 saves 150 lives ($1,000/life saved)
Effectiveness ≠ Cost-Effectiveness

Example: Kamlet et al., 1995

- 3-year RCT of 128 patients with recurrent depression
- Interpersonal therapy improved health, but was not cost-effective compared with imipramine therapy
- Imipramine improved expected lifetime health while lowering costs
Cost-Effectiveness By Any Other Name

- **Cost-effectiveness**: Compares incremental cost to incremental effectiveness, e.g. \$/life years saved from intervention vs. usual care.

- **Cost-utility**: Special case, effectiveness measured as quality-adjusted life years (QALYs).

- **Cost-benefit**: Measures all costs and benefits in dollar terms.

- **Cost-offset**: Decline in non-intervention costs resulting from intervention.
Use of Cost-Effectiveness Analysis for Health Care Policy
Decision Rules Based on Cost-Effectiveness (Frank, 1993)

- Pay for a treatment if and only if it is found to be cost-effective
  - Primary concern: Money is wasted on a therapy that is not cost-effective
- Withhold payment for a treatment if and only if it is shown not to be cost-effective
  - Primary concern: A cost-effective therapy is excluded from coverage
Use of C/E Analysis for Policy

- C/E ratios only useful for policy if they are calculated in a consistent manner
- Fundamental problem in comparing C/E ratios to allocate resources has been the use of very different methods and definitions
  - e.g., misleading to compare cost per quality-adjusted life year for T1 vs. T2 when different costs are included and QALYs measured differently
Panel on Cost-Effectiveness in Health and Medicine

- Before, researchers could get almost any result they wanted to by making judicious choices about how to calculate C/E
- Purpose of Panel was to develop “reference case” guidelines for conducting CEA
- Although it seems straightforward to calculate a C/E ratio, involves a number of decisions to which results may be sensitive
C/E Ratio = Difference in Costs/
Difference in Effectiveness

- Costs and effectiveness of what? Compared to what?
- With which population and over what time period is the C/E of the treatment measured?
- Whose costs and benefits?
- What type of costs should we measure?
- How do we measure effectiveness?
- How are the intervention effects determined?
Costs and Effectiveness of What?
Importance of Defining the Intervention

- Without knowing what the intervention was, cannot hope to replicate if findings were positive, or understand what to change if findings were negative.
- Important to collect data on what the intervention actually was, not just rely on what it was supposed to be.
- This also allows intervention costs to be measured more easily.
Compared to What?
Importance of Choosing the Comparison Condition

- Q: What is the relevant alternative?
- A: The “next-best” option.
- This may be usual care or it may be another treatment that has been widely accepted as cost-effective and is covered by health plans
- It is misleading to compare the C/E of the intervention to a less desirable alternative
Example from the Literature

Lave et al. compared cost-effectiveness of
- Interpersonal therapy (IPT)
- Nortriptyline (NT)
- Usual care (UC)

Effectiveness measure: depression-free days

IPT was cost-effective relative to UC, but this is not the relevant comparison

IPT was NOT cost-effective relative to NT

NT, not UC, is the best treatment alternative
With Which Population?
Importance of Study Population

- Studies often impose numerous inclusion and exclusion criteria and are typically conducted in a limited number of sites.
- The more selected the sample, the lower the external validity (generalizability) of the findings.
- Both effectiveness and costs (and hence C/E) could look very different for different study populations, so doubly important for C/E analysis.
Importance of Study Population (cont’d)

- Relative prices may vary across sites, so an intervention designed to reduce IP care by increasing OP care will look more cost-effective in sites where cost of IP care relative to OP care is higher.

- If comparison is “usual care,” may differ dramatically across sites; intervention will look more effective in sites where access and quality of care are poor.
Example from the Literature

- Effectiveness of intervention will also depend on sample
- Kamlet et al. (1995) studied the cost-effectiveness of imipramine relative to IPT
  - Conclusion: Imipramine was cost-effective relative to IPT
  - However: The study was based on patients initially stabilized with imipramine
  - Results may not generalize, due to selection
  - IPT might look better among patients who never used imipramine
Over What Time Period?
Importance of Study Period

- If the study is based on data from more than one year, inflation adjustments / discounting may be necessary.
- Intervention costs are often incurred up-front, while benefits tend to accrue slowly over time => use of short followup periods may underestimate the cost-effectiveness of interventions.
Example from the Literature

- The Lave et al. study found that relative to nortriptyline, interpersonal therapy was not cost-effective over one year.

- However, IPT might have looked more cost-effective if a longer follow-up period had been used; drugs may work faster, but perhaps therapy has a longer-lasting effect.
Whose Costs and Benefits?

Importance of Study Perspective

- Insurers care most about covered health care costs, including potential cost-shifting.
- Employers care most about job productivity losses, employee turnover, insurance premiums.
- Government cares most about transfer program costs, foregone income tax, criminal justice costs, crime and accidents.
Study Perspective (cont’d)

- Families care about out-of-pocket health care costs, informal caregiving burden, patient time and suffering, lost earnings

- “Social planner” cares about all costs and benefits (does not matter who incurs them)
  - Pure transfers between individuals (e.g., welfare payments) are not counted as costs, since one person’s loss is another one’s gain
  - In other words, all individuals are given equal weight in calculation of costs and benefits
“Gold standard” analyses are usually conducted from the perspectives of the social planner and the relevant decision maker, i.e., the funder of the intervention or treatment (usually the insurer).

In the absence of insurance, the health care decisions of individuals can be thought of as CEA from the patient perspective.

These decisions may be rational given the patient’s information, but this information is often poor, leading to bad decisions.
A study of economic burden of depression in 1990 (Greenberg et al., 1993) looked at:

- medical, psychiatric, pharmacologic costs
- mortality costs from suicide
- workplace productivity costs

The “social planner” would care about all of these costs.

- Insurer cares most about health care costs
- Employer cares most about productivity
Measuring Costs
Definitions of Costs

1. An amount paid or required in payment for a purchase; a price

2. The expenditure of something, such as time or labor, necessary for the attainment of a goal.

- American Heritage Dictionary via Yates

CEA uses economic costs, which is based on the real value of resources to society (i.e., what you gave up to produce the health care).
Which Costs Should Be Measured?

- Medical and behavioral health care
- Prescription drugs
- Informal caregiver time
  - “Opportunity costs”: person could have been working or doing other activities instead
- Social services or transfer programs, e.g., welfare (depending on perspective taken)
Costs to be Measured (cont’d)

- Crime and accidents (e.g. robbery or drunk driving fatalities)
  - Victim losses (some or all, depending on perspective and crime) and externalities
  - Law enforcement and criminal justice systems
- Patient time spent getting care
- Earnings losses (if not implicitly included in effectiveness measure or in patient time)
- Consumption (if life expectancy changes)
Intervention vs. Non-Intervention Costs

- Easier to measure intervention costs, but important to measure both

- Example: Intervention may increase physician visits through referral, or decrease inpatient stays through better outpatient care

- Unless non-intervention costs measured,
  - If intervention increases utilization of other services, will look more cost-effective than it is
  - If intervention decreases utilization of other services, will look less cost-effective than it is
Construction of Cost Measures

- Costs are often constructed as weighted sum of utilization, especially when using survey data.
- Unit cost of each type of service is the weight for that service.
- Example: Patient has 2 office-based physician visits @ $100 each and 1 hospital outpatient visit @ $150 each.
  - Total cost = \(2 \times $100 + 1 \times $150 = $350\)
How Is Service Use Measured?

- Determine generic types of services important for the population
- Only necessary to distinguish between two services if unit costs differ sufficiently
- Identify the most important services for measuring C/E of intervention vs. comparison condition, i.e., “care as usual”
  - Expensive
  - Intervention likely to affect use
Example: Importance of Utilization Data

- **Intervention patient**
  - 5 psychiatrist visits @ $100/visit
  - $500 of medications
  - Total = $1,000

- **Comparison group patient**
  - 1 psychiatric hospital stay (3 days) @ $750/day
  - Total = $2,250

- **Note:** If hospitalization not reported, comparison yields wrong answer
Prices

- The biggest challenge in cost analyses is obtaining prices to weight utilization.
- Administrative (claims) data may have information on charges and/or reimbursement. Neither is the same as costs, but they are often used as proxies.
- Reimbursement probably closer to costs, although cost-to-charge ratios can sometimes be used to deflate charges, especially for hospital services.
Assigning Prices to Self-Reported Service Utilization

- With self-reported utilization, prices must come from external sources
  - Published literature
  - Medicare, Medicaid or private insurance fee schedules
  - Administrative databases for random patient samples
- Relative, not absolute, prices are the most important
Common Problem to Watch Out For

- Intervention costs tend to be measured as true costs (because the information comes from the grant budget)
- In contrast, non-intervention costs ("cost offset") are often measured as charges or list prices, which overstate costs
- In this case, cost-effectiveness of the intervention may be overstated