

# Cost-Effectiveness Analysis in Health Services Research: An Overview

Susan L. Ettner, Ph.D.  
Professor, UCLA Schools of  
Medicine and Public Health

# Health Care Choices: The Clinical vs. Economic Paradigm

# Introduction

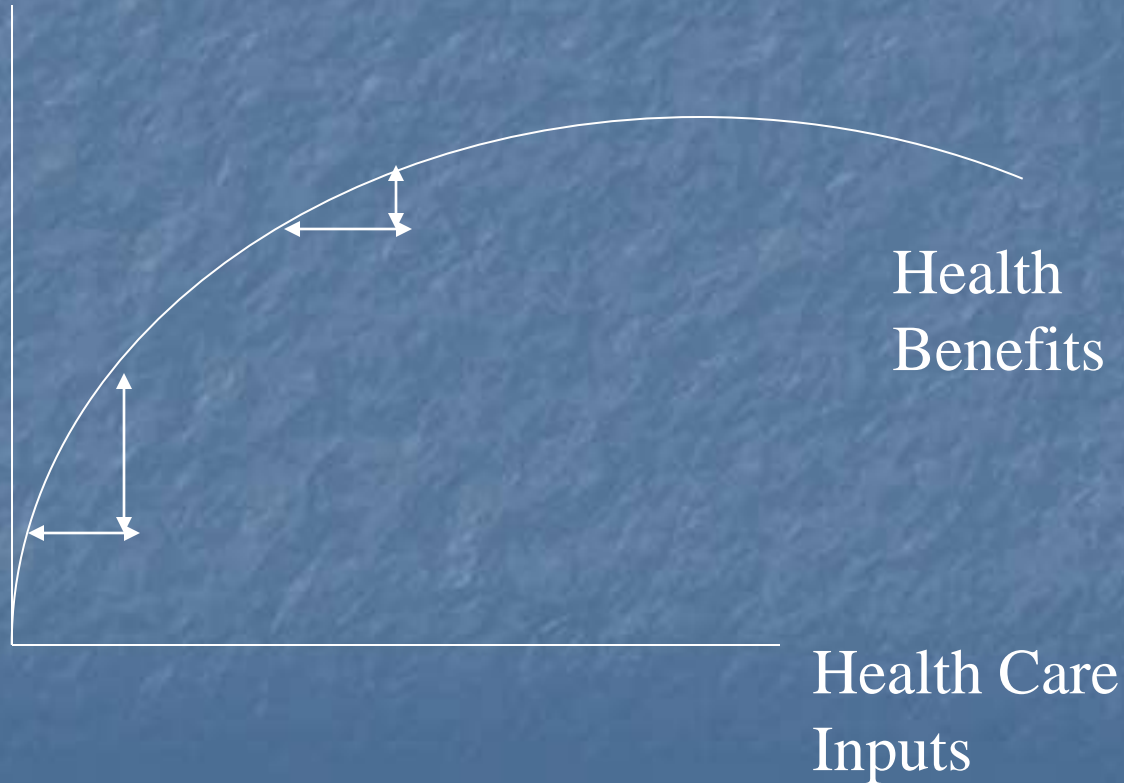
- Provide a medical service to a patient if:
  - it has any benefit (clinical paradigm)
  - its incremental benefit outweighs its incremental cost (economic paradigm)
- Example: If a candy bar costs \$1 and it's only worth 50¢ to you, you wouldn't buy it
- Same thing with health; if a service costs \$500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

# “Opportunity Costs”

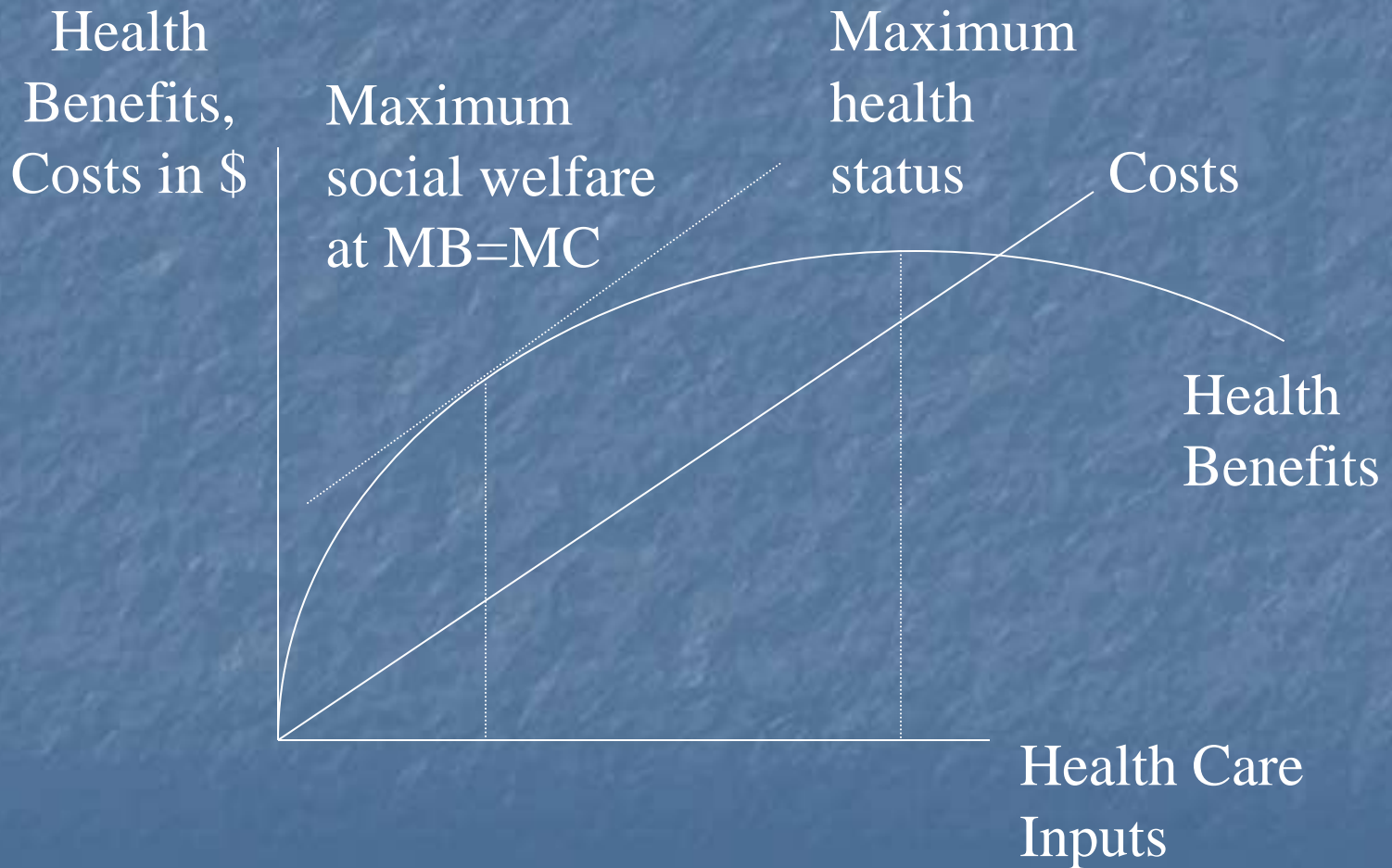
- Opportunity cost: value of what you give up
- With limited resources, providing service to one patient reduces resources for others
  - Resources limited, since society is unwilling to spend all of its money on health care
  - Health care will always be rationed somehow, so better to consciously decide tradeoffs
  - CEA especially important when society is practicing “flat of the curve” medicine

# Diminishing Returns and “Flat of the Curve” Medicine

Health  
Benefits in \$



# "Optimal" Amount of Health Care Under Clinical vs. Economic Paradigm



# Efficacy $\neq$ 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 $\neq$ 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 followup 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

# Study Perspective (cont'd)

- “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.

# Example from the Literature

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 =  $2 * \$100 + 1 * \$150 = \$350$

# 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