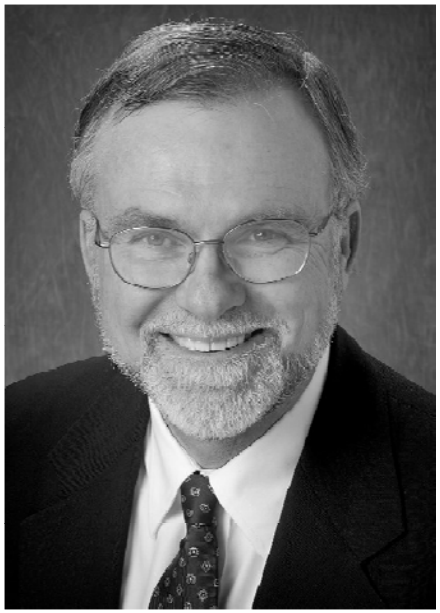
How Can Cost Effectiveness Analysis Be Made More Relevant to U.S. Health Care?



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Talk Overview

- Review of Cost Effectiveness Analysis (CEA)
- The role of CEA in the U.S. and other countries
- The barriers to implementing CEA
- Overcoming the barriers to CEA
- CEA & comparative effectiveness

Cost-effectiveness analysis (CEA)

- Compare treatments, one of which is standard care
- Measure all costs (from societal perspective)
- Identify all$_{ou}$ tcomes
  - Express outcomes in Quality Adjusted Life Years
- Adopt long-term (life-time) horizon
- Discount cost and outcomes to reflect lower value associated with delay

Review CEA (cont.)

- Test for dominance
- The more effective, less costly treatment *dominates*
  - or if they are equal cost, the more effective
  - or if they are equally effective, the less costly
- In the absence of dominance, find the Incremental Cost-Effectiveness Ratio (ICER)

Incremental Cost-Effectiveness Ratio (ICER)

$$\frac{Cost_{EXP} - Cost_{CONTROL}}{QALY_{EXP} - QALY_{CONTROL}}$$

- Decision maker compares ICER to "critical threshold" of what is considered cost-effective ($ per QALY)

Where can CEA be applied?

- How does research influence health care?
  - Individual decisions of physician and patient
  - System decisions
    - Coverage decision
    - Practice guidelines

Use of cost-effectiveness in other countries

- Canada
  - Canadian Agency for Drugs and Technologies in Health
  - Established 1989 to evaluate health technologies
  - Provincial organizations also study cost-effectiveness
- United Kingdom
  - National Institute of Clinical Effectiveness
  - Established 1999 to provide advice to National Health Service

Use of CEA in other countries (cont.)

- Sweden, Australia, Netherlands
  - Requires manufacturer to submit evidence of cost-effectiveness to add new drugs to health system formulary
- Germany
  - New institute “Institute for Quality and Efficiency in the Health Care Sector” (IQWiG)
- France
  - Unique periodic reviews of previously approved pharmaceuticals

Use of CEA in other countries (cont.)

- Health plans of most developed countries consider cost-effectiveness
- Used for coverage decisions
  - Especially for new drugs and technologies
  - Cost-effectiveness findings not always followed
  - Few cases of outright rejection based on cost
- No formal evaluations of use of technology assessment, however

Use of cost-effectiveness in U. S.

- Medicare proposed use of cost effectiveness criteria in 1989
  - Proposed regulation was withdrawn after decade of contentious debate
- Medicare Coverage Advisory Commission (MCAC) has no mechanism to consider cost or value in its decision

Use of cost-effectiveness in U. S.

- Oregon Medicaid
  - Attempted to restrict expensive treatments of low benefit
  - Negative political consequence
  - May not have been a real test of acceptance of CEA

Surveys of coverage decision makers

- Survey of 228 managed care plans (Garber et al, 2004)
  - 90% consider cost
  - 40% consider formal CEA

Question for discussion: What are the potential objections to using CEA?

Research on barriers to use of CEA

- At least 16 different surveys of decision makers' attitudes to health economic studies
- Identified decisions makers concerns

Decision maker concerns about CEA

- Lack of understanding of CEA
- Lack of trust in CEA methods
  - Lack of confidence in QALYs
  - Lack of confidence in extrapolation (modeling)

Decision maker concerns about CEA (cont.)

- Not relevant to decision maker's setting or perspective
  - Decision maker has short-term horizon
  - Wants payer perspective, not societal perspective
- Lack of information on budgetary impact
- Concern about sponsorship bias
- See: (Drummond, 2003)

Other concerns about CEA

- American attitudes
  - Distrust of government and corporations
  - Unwilling to concede that resources are really limited

What can researchers do to improve acceptance of CEA?

ISPOR recommendations to improve acceptance of CEA

- Describe relevant population and its size
- Budget impact, including which budgets will be affected
- Provide disaggregated cost and outcomes
- Provide cost and outcome by sub-groups
- Provide key assumption, data sources, sensitivity analysis– which parameters have biggest impact?

Other ways to improve acceptance

- Make sure CEA is relevant to decision maker
  - Support coverage decisions about expensive interventions
  - In other countries CEA analyses are *commissioned by* decision makers
  - Decision makers are anxious for results

Other ways to improve acceptance (cont.)

- Provide findings that are timely
  - Easier to prevent adoption than to withdraw widely-used technology
  - Conduct preliminary studies
    - These represent pre-positioning of resources

U.S. coverage decisions

- Coverage based on effectiveness
  - Size of effect
  - Strength of evidence

Implicit use of CEA in U.S.

- Examples of behind the scenes role:
  - Decision makers require large effect if the treatment is expensive
  - Used by U.S. Preventive Services Task force recommendations for screening
  - American Managed Care Pharmacy "formulary guidelines"
  - See (Neumann, 2004)

CEA and comparative effectiveness

- Comparative effectiveness research
  - Alternative to CEA (which is seen as too controversial)
  - Study alternative treatments to find the most effective
  - The more effective treatment should be used
  - Placebo often not the appropriate comparator

Limits of comparative effectiveness

- What if most effective treatment has more side effects or higher risk?
- How to estimate long-term benefit of short-term effectiveness, e.g., what is the value of successful identification of a disease?

Use of CEA methods in comparative effectiveness

- Balance benefits with risks
  - Convert to QALYs to find net benefit and which treatment is "most effective"
- Extrapolating beyond short-term effectiveness
  - Use of Decision Models can estimate long-term benefits
- See: (Russell, 2001)

Other criticisms of comparative effectiveness

"A menu without prices."

- Garber

Priorities for comparative effectiveness

- Institute of Medicine (IOM) set priorities for comparative effectiveness research funded by economic stimulus bill
  - "Cost-effectiveness analysis is a useful tool of comparative effectiveness research"
- Cost was mentioned explicitly in 13 of 100 priorities

Exceptions to CEA

- Even when treatment is not cost-effective, physicians and patients give priority to certain groups:
  - Life threatening conditions
  - Children
  - Disabled

Exceptions to CEA

- VHA can add to this list
  - Treatment for a service-connected injury or illness

Public involvement in application of CEA

- NICE citizen council
- Experiment with individuals recruited from New York state juror pool
  - Provision of cost-effectiveness information influenced coverage decisions
- See: (Gold, 2007)

Unique role for VA

- Global budget
- Potential collaboration between decision makers and researchers
- Identified constituency of health system users who can be (must be) involved

What have we learned?

Review: How to choose a topic for CEA

- Involve decision maker at the outset
- Consider if CEA finding will be relevant to policy
  - Is treatment likely to be expensive?
  - Is treatment targeted for one of the exceptional groups?

Review: How to prepare a CEA

- Transparency in reporting
- Provide disaggregated cost and outcomes
- Describe sub-groups
- Budget Impact Analysis may be an essential adjunct to CEA
  - Describe size of population affected
  - Consider short-term horizon, payer perspective

Some further reading

- Drummond, M., et al., *Use of Pharmacoeconomics Information-Report of the ISPOR Task Force on Use of Pharmacoeconomic/Health Economic Information in Health-Care Decision Making*. Value Health, 2003. 6(4): p. 407-416.
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- Neumann, P.J., *Why don't Americans use cost-effectiveness analysis?* Am J Manag Care, 2004. 10(5): p. 308-12.
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