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 outcomes
  - 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
  - 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

- Patient Protection &Affordable Care Act 2010
- Created Patient-Centered Outcomes Research Institute (PCORI)
  - assess outcomes, effectiveness, and appropriateness
- Prohibited use of dollars per QALY thresholds
  - For PCORI recommendations
  - For HHS coverage decisions

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
  - Oregon continues to prioritize Medicaid services (Saha, 2010)

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

Examples of research partners

Operations partner	**Potential Topics**
Pharmacy Benefits Management	New pharmaceuticals
National Center for Health Promotion & Disease Prevention	Screening and prevention
Office of Public Health	Screening and treatments for HIV, Hepatitis C, tobacco
Office of Specialty Care Services	New interventions effecting that service
Chief Business Office	Make or buy choice

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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