

# 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
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# 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
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# 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)
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# Incremental Cost-Effectiveness Ratio (ICER)

$$\frac{\text{Cost}_{\text{EXP}} - \text{Cost}_{\text{CONTROL}}}{\text{QALY}_{\text{EXP}} - \text{QALY}_{\text{CONTROL}}}$$

- Decision maker compares ICER to “critical threshold” of what is considered cost-effective (\$ per QALY)
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# Where can CEA be applied?

- How does research influence health care?
    - Individual decisions of physician and patient
    - System decisions
      - Coverage decision
      - Practice guidelines
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# 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
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# 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
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# 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
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# 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
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# 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)
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# Surveys of coverage decision makers

- Survey of 228 managed care plans (Garber et al, 2004)
    - 90% consider cost
    - 40% consider formal CEA
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**Question for discussion:  
What are the potential  
objections to using CEA?**

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# Research on barriers to use of CEA

- At least 16 different surveys of decision makers' attitudes to health economic studies
  - Identified decision makers concerns
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# 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)
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# 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)
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# Other concerns about CEA

- American attitudes
    - Distrust of government and corporations
    - Unwilling to concede that resources are really limited
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**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?
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# 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
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# 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
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# U.S. coverage decisions

- Coverage based on effectiveness
    - Size of effect
    - Strength of evidence
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# 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 )
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# 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
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# 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?
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# 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 )
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# 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
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# 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)
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# 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
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# Examples of research partners

| <b>Operations partner</b>                                 | <b>Potential Topics</b>                                |
|---|--|
| 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                                     |

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# 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?
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# 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
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# 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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