

Recommendations for Conducting Cost Effectiveness: Elements of the Reference Case

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What will you learn?

- Why do we need to standardize cost-effectiveness analyses
- What needs to be standardized
- Build on details from previous lecture

PHS Recommendations

- MR Gold, JE Siegel, LB Russell, MC Weinstein (1996) Cost-Effectiveness in Health and Medicine Oxford University Press. Especially Appendix A (pp 304:311)

PHS Recommendations, JAMA

Summary

- Russell LB, et al. The Role of Cost-effectiveness Analysis in Health and Medicine. JAMA. 1996;276:1172-1177.
- Weinstein MC, et Al. Recommendations of the Panel on Cost-Effectiveness in Health and Medicine. JAMA. 1996;276:1253-1258.
- Siegel JE, et al. Recommendations for Reporting Cost-effectiveness Analysis. JAMA. 1996;276:1339-1341.

Poll

- Have you ever conducted a cost-effectiveness analysis?
- Answers
- No
- One study
- More than one study

Why Do We Need Cost-Effectiveness Analysis?

- Health care interventions affect many different outcomes, in different ways
- Need a common metric to allow comparisons across diverse diseases, conditions, and patient populations (e.g., compare the value of interventions for PTSD and coronary artery disease)

What is Cost-Effectiveness Analysis?

- Tool for making decisions, a common metric to compare diverse interventions
- An intervention or treatment is compared to an alternative; “usual care” is the standard comparator.
- Essentially asking, is the treatment being evaluated “better” than the current standard of care?

What is Cost-Effectiveness Analysis?

- Need to find both the costs of the intervention (and the comparator) and assign values to outcomes
- Outcomes must be measured on a single scale; the standard is Quality Adjusted Life Years (QALYs)

Cost-Effectiveness Analysis vs. Cost-Utility Analysis

- CUA is a specific form of CEA, where the outcome is measured in utilities (usually QALYs)
- More generalizable
- I will use terms interchangeably, but focus of this talk is only CUA

Dominance Principles

- Only available tool if outcomes are not measured in QALYs
- An intervention is favored if it is more effective and costs less
- Extended dominance can be used when 3 or more treatments are being compared

Application of Dominance

+ Change in Cost -	Standard care preferred (dominated)	?
	?	Intervention Preferred (cost saving)
	-	+ Change in Effectiveness

Examples of Strong Dominance or Cost Saving (better outcomes and lower costs)

- These are RARE!!!
- Many infant/childhood vaccinations/immunizations (Polio, MMR, etc.)
- Age appropriate cervical cancer screening
- Mandatory motorcycle helmet laws

Example of Strong Dominance (better outcomes and lower costs)

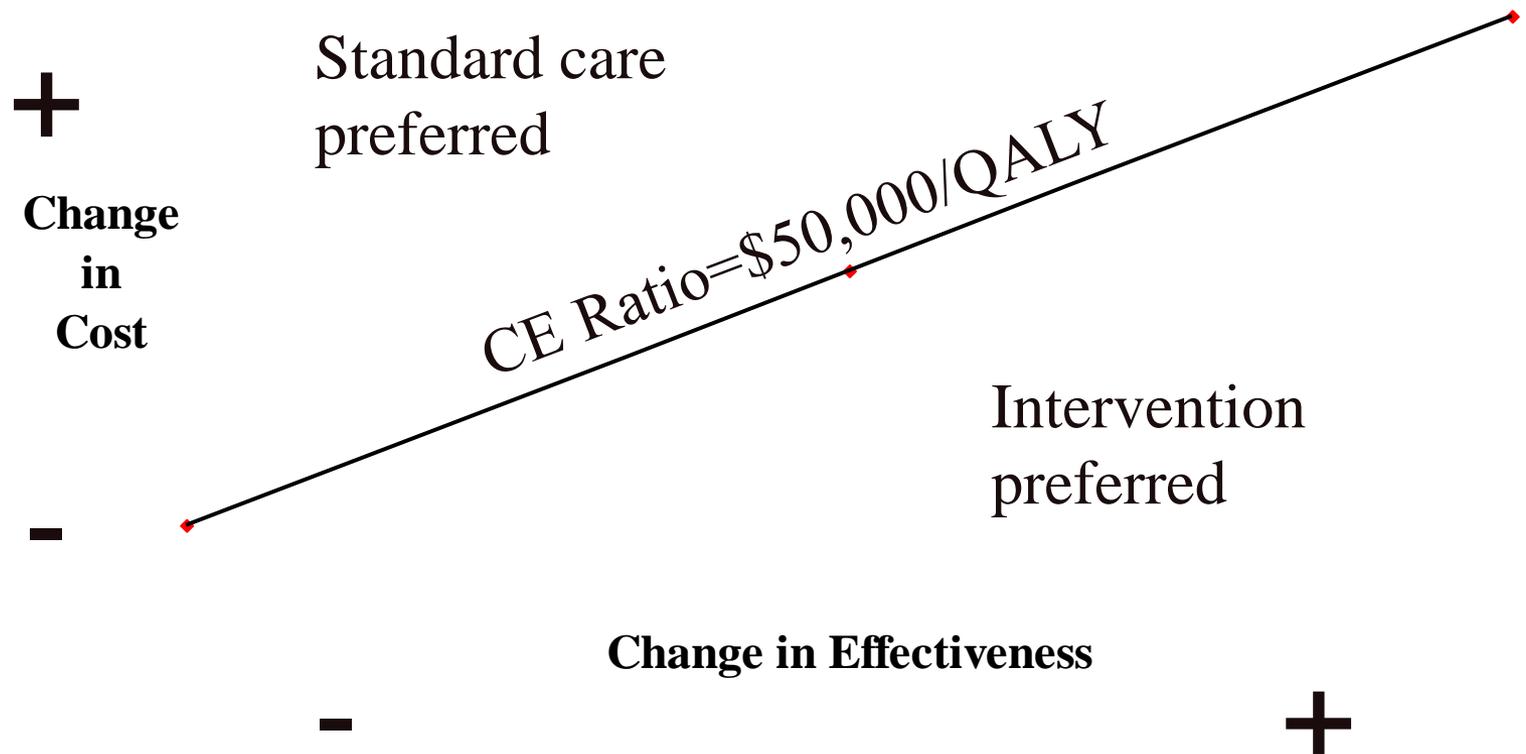
- Neonatal surfactant replacement therapy, 50% reduction on RDS mortality
- Reduced mortality increases costs
- But, surfactant reduced treatment intensity and LOS of those who would have survived anyway
- Net result, lower mortality and lower costs
- May see more of these as we develop treatments that address the underlying biologic problem

Incremental Cost-Effectiveness Ratio

- Calculated when one intervention is more effective and more costly

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

Application of Critical Cost-Effectiveness Ratio



Where does the \$50,000 per QALY Threshold Come From?

- This is both old and arbitrary.
- When Medicare extended to cover ESRD, estimated gain was \$50,000 per QALY
- Revealed public preference
- Never updated for inflation

What is the “Reference Case”

- A standard set of methods and assumptions to serves as a point of comparison across studies

Why Do We Need a Reference Case?

- There are many different assumptions, methods, and perspectives that can affect the outcomes of a cost-effectiveness analysis.
- Without standardization, it would not always be possible to compare the results across studies.
- Standardization greatly increases the policy value of C-E analysis.

PHS Recommendations: Summary

- Adopt perspective of society
- Measure all costs
 - direct cost of intervention
 - all health care expenditures
 - patient incurred cost
- Express outcomes as Quality-Adjusted Life Years (QALY)

PHS Recommendations:

Summary (continued)

- All health effects in the denominator of the C/E ratio
- The numerator of the C/E ratio captures all changes in resource consumption associated with the intervention
- Discount costs and outcomes at 3% annual rate

PHS Recommendations: Summary (continued)

- Model when effects of intervention not fully realized during the study period
- Conduct sensitivity analysis
- Test statistical significance of cost-effectiveness findings
- Standards for reporting of C/E analyses.

Societal Perspective

- Adopt perspective of society
- Payer perspective may yield very different results; benefits or costs may occur to others, including:
 - Patient
 - Other payers
 - Other individuals (e.g., family members)
 - Employers

Budget Impact Analysis

- For VA studies, may also consider doing a Budget Impact Analysis, in addition to a CEA
- Provides VA managers with information about the cost of implementation, time line of the costs and benefits; important for budget planning.
- May help speed adoption/implementation
- Will be covered in a later lecture

Denominator vs. Numerator

- All health effects in the denominator, expressed in QALYs
- The numerator of the C/E ratio captures all changes in resource consumption associated with the intervention
- There are gray areas, that could be placed in either
- Avoid double counting.

Poll: Do these belong in the numerator of the ICER? Yes/no answers

- Health care costs associated with the intervention
- Length of stay
- Costs of patient time
- The value of lost productivity

Components Belonging in the Numerator of the C/E Ratio

- Costs of health care services
- Costs of patient time ***
- Costs of care-giving (paid and unpaid)
- Other costs (e.g. travel time)
- Costs measured in constant dollars
- Use wage rates to value time costs

Components Belonging in the Numerator of the C/E Ratio (cont)

- Non-health care costs
 - E.g., education, criminal justice, environment
- Costs imposed on others
 - E.g., employers, rest of society
- Do **NOT** include lost productivity; would result in double counting

Components Belonging in the Numerator of the C/E Ratio (cont)

- Health care costs that result from living longer
 - Include costs for intervention-related diseases within original expected life span, and for added years of life
 - Include costs of treating adverse events
 - Exclude unrelated health care costs and non-health care costs within original expected life span
 - Exclude non-health care costs for added years of life
 - No recommendation for unrelated health care costs for added years of life

Components Belonging in the Denominator of the C/E Ratio

- Measure health effectiveness in QALYs
- QALYS should be preference based
- Weights based on community preferences
- Use a generic health-state classification, as opposed to disease-specific
- Use age- and sex-specific HRQL to value gains and losses

Modeling May Be Necessary

- Most clinical trials don't cover full time horizon of the potential effects, both costs & QALYs
- It is allowable to use modeling and/or data from other sources to complete the analysis
- Use of expert judgment should be avoided, if possible

Discounting

- Real discount rate of 3%
- All costs should be adjusted for inflation
- Both costs and health outcomes should be discounted
- Conduct sensitivity analysis of the discount rate.

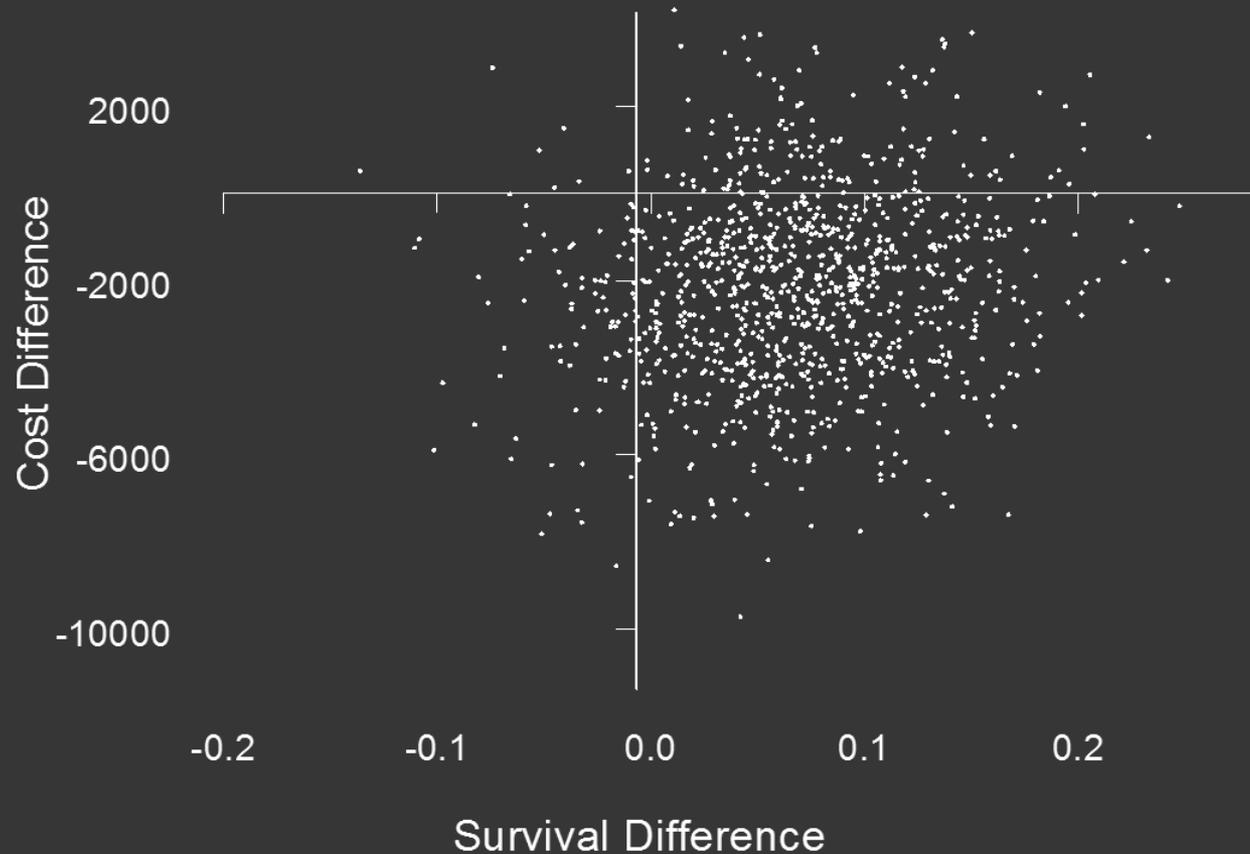
Sensitivity Analysis

- Conduct sensitivity analysis
- 1-way sensitivity analysis for key assumptions
- 1-way sensitivity analysis under-state overall uncertainty; should also conduct multivariate sensitivity analysis

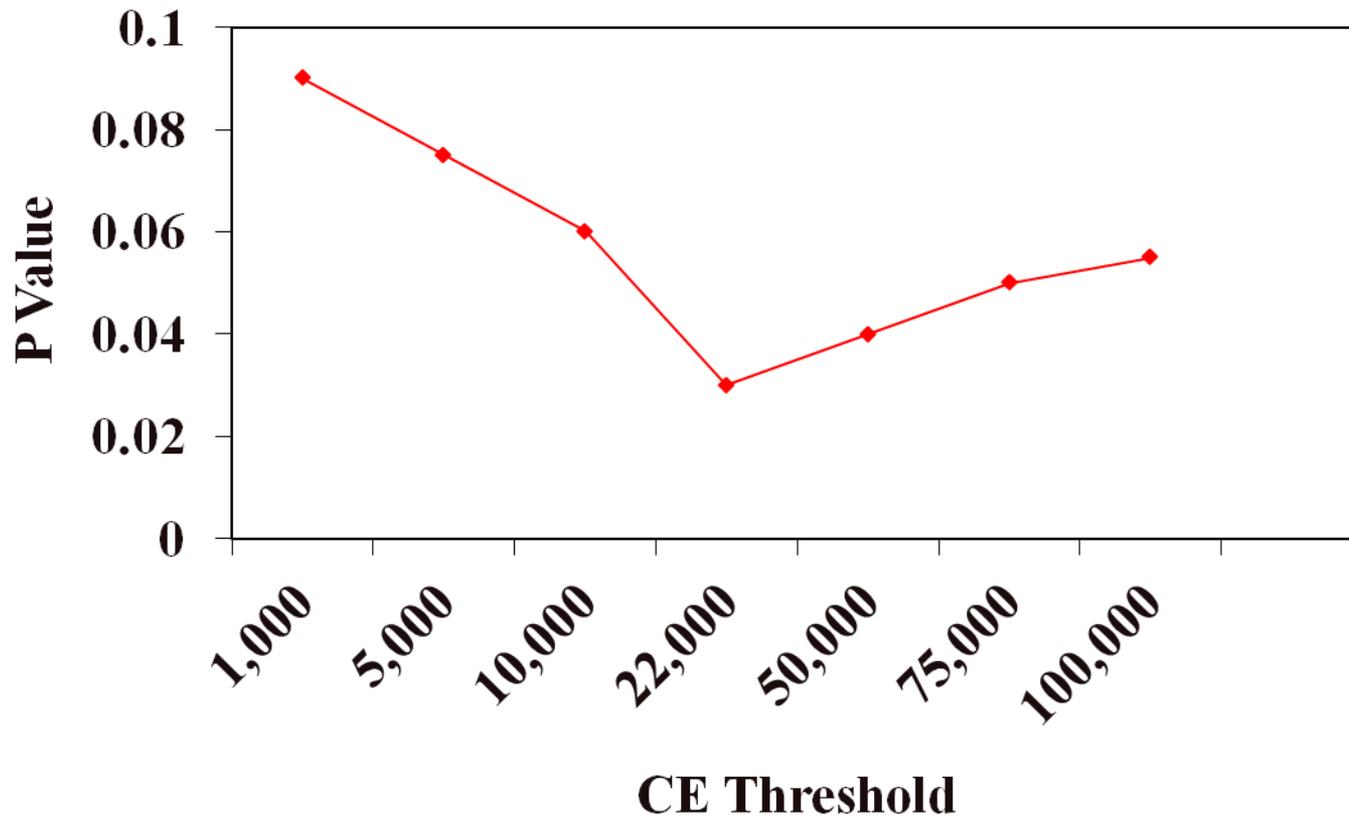
Bootstrap Determination of Cost-Effectiveness Confidence Region

- Sample n observations with replacement
- Find incremental cost-effectiveness ratio
- Repeat 1,000 times
- Find percentage of replicates that are not “cost-effective”
 - this is the p-value
 - p-value may vary by threshold

Distribution of Bootstrapped Cost-Effectiveness Ratios



Sensitivity Analysis: How Does Significance Vary by CE Threshold?



Standards for Reporting Results

- Siegel JE, et al. Recommendations for Reporting Cost-effectiveness Analysis. JAMA. 1996;276:1339-1341. Checklist
- List of information that needs to be included to allow comparison across studies
- This is very important from a policy perspective

Alternative Method

- Just to mention, alternative to reporting ICER, net benefit regression.
- Hoch JS, Briggs AH, Willan AR. Something old, something new, something borrowed, something blue: a framework for the marriage of health econometrics and cost-effectiveness analysis. *Health Economics*. 2002;11:415-430.
- HERC Cyber-Seminar, Hoch 8/23/2006

Other References

- **Methods for the Economic Evaluation of Health Care Programmes (Paperback)**
by Michael F. Drummond, Mark J. Sculpher, George W. Torrance, Bernie J. O'Brian, Greg L. Stoddart Oxford 2005
- **Hayward RA, Kent DM, Vijan S, Hofer TP. Reporting clinical trial results to inform providers, payers, and consumers. Health Affairs 2005;24(6):1571-1581.**
- **Heitjan DF. Fieller's Method and Net Health Benefits. Health Economics 2000;9:327-335.**

Other References

- **ISPOR Task Force for CEA in clinical trials, see:**
- **Ramsey, Scott, et al. Good Research Practices for Cost-Effectiveness Analysis Alongside Clinical Trials: The ISPOR RCT-CEA Task Force Report. *Value in Health* 2005;8 (5), 521-533. Also available on the ISPOR web page, http://www.ispor.org/workpaper/clinical_trial.asp**

Next HERC Cyber Course

January 27, 2016

Estimating the Cost of an Intervention

Todd Wagner, Ph.D.