



# Recommendations for Conducting Cost Effectiveness: Elements of the Reference Case

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**January 6, 2010**

# 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.

# 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

# 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.

# 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

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

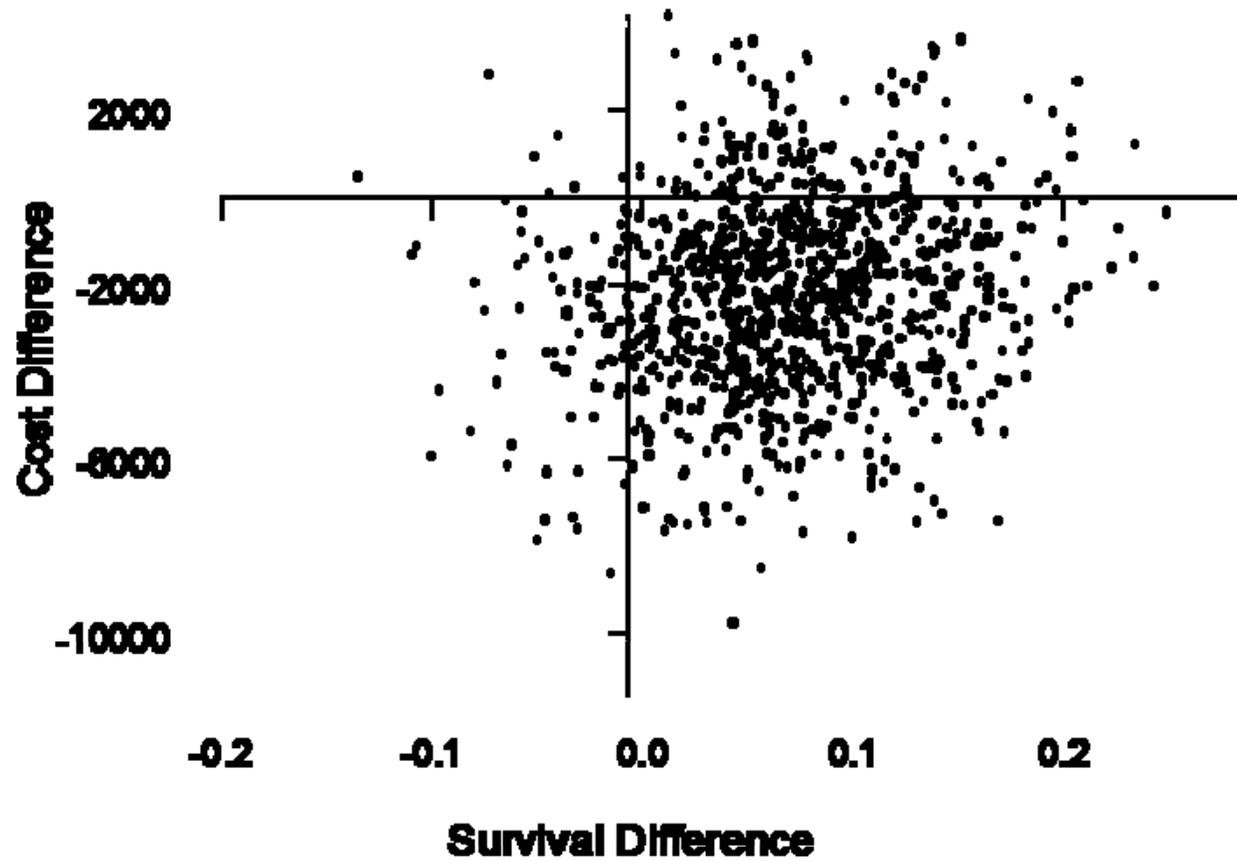
Repeat 1,000 times

Find percentage of replicates that are not “cost-effective”

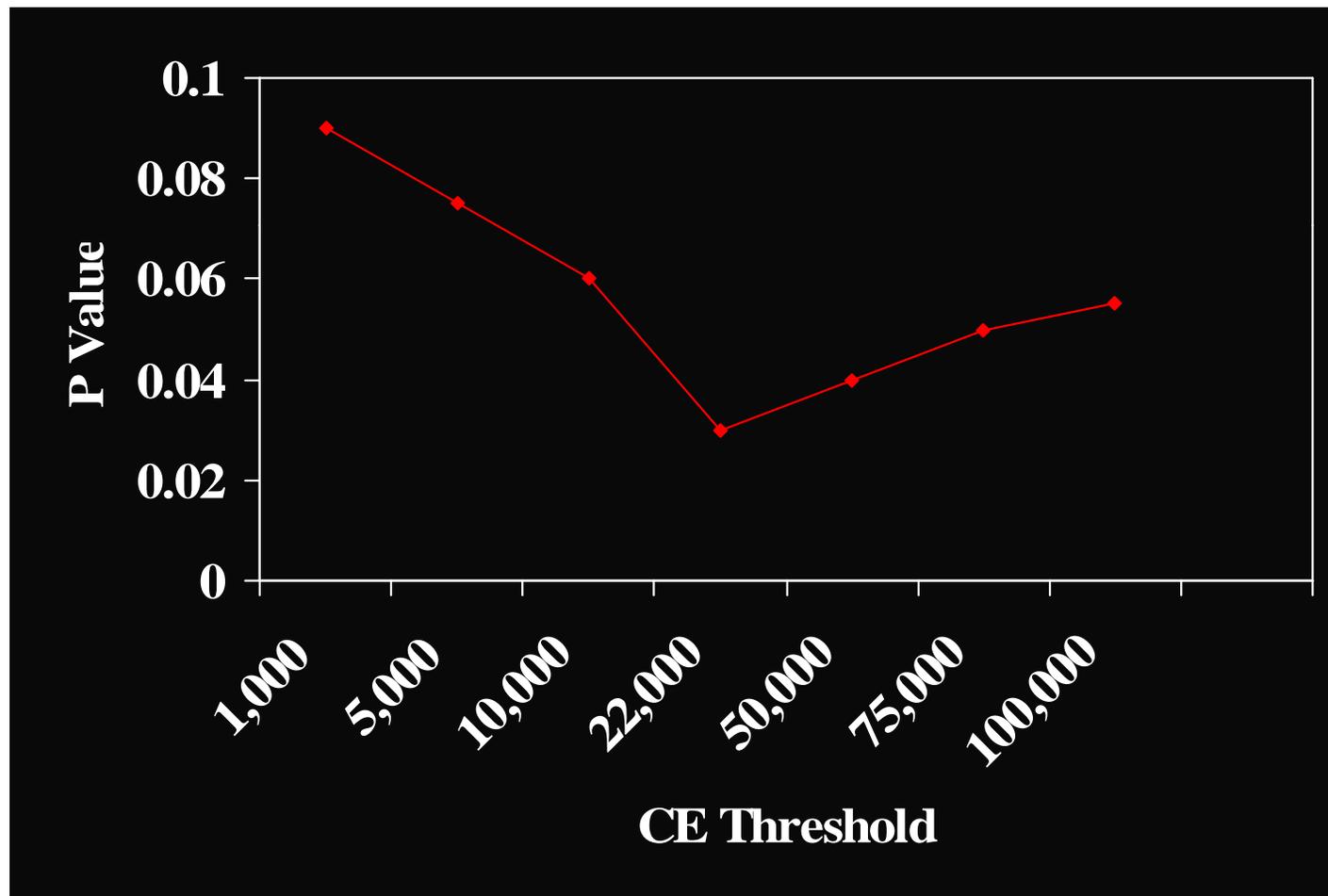
- this is the p-value

- 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

Details of recommendations in paper distributed in advance; 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.