Cost Effectiveness Analysis

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Advanced Topics in Epidemiology

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Outcomes Research

- Evaluates outcomes of medical therapies (potentially including costs) and their impacts on people, organizations, and society
- Therapies can include drugs, devices, procedures, or broader programmatic or system interventions
- Outcomes can include mortality, morbidity, functional status, mental well-being, other aspects of health-related quality of life, cost, etc.

Cost-Effectiveness Analysis

Cost-Effectiveness Analysis

- Outcomes research specifically focused on economic value of therapies / delivery systems / behavioral interventions
- Multidisciplinary methods
  - Economics
  - Epidemiology
  - Medicine
  - Pharmacy
  - Decision sciences
  - Operations research
  - Statistics / biostatistics
  - Other social sciences

Economic Messages

- Therapy is good/bad value
- Budget impact
- Burden of illness
  - Often flag waving: “This disease is important…”
- Specific messages addressed depend in part on:
  - Disease and therapy under evaluation
  - Other therapies available to treat condition
  - Interest of regulatory bodies, providers, payers, and patients

What Data / When?

What Data / When?

- Phases I and II
  - Incidence and prevalence-based burden of illness
    - Incidence-based - lifetime costs of the disease for a cohort with incident disease
    - Prevalence-based - costs of disease during a given time period for prevalent cases
  - Natural history modeling
  - Preplanning for phase III economic studies

Phase III

- Cost / Efficacy studies in clinical trials
  - Provides economic data for registration, pricing, and early use
- Decision modeling of impacts of intervention
- Budget impact studies

Phase IV

- Cost / Effectiveness studies in usual care
  - Comparisons made in more realistic settings with more realistic protocols against comparators of interest to individual decision makers
  - Allow decision makers to assess whether economic results from phase III trials are generalizable to usual care
- Decision modeling of impacts of intervention
- Post marketing surveillance studies
  - Observational data to evaluate costs, effectiveness, and adverse experiences related to the drug

Cost-Effectiveness Study Designs

Cost-Effectiveness Study Designs

- Clinical trials
  - Economic evaluation in clinical trials widespread
  - Little to no selection bias, but potential issues of generalizability
- Observational studies
  - Often more generalizable, but problems with selection bias
- Decision models
  - Often used to address pressing questions for which direct data are not available
  - Shares strengths and weaknesses of source data
  - Added uncertainties related to combining data from multiple sources and projection beyond the data

Decision Analysis Approaches

- Most frequently used healthcare decision analytic approaches
  - Decision trees
  - Markov models
- Can be used:
  - To analyze data from trial
  - To perform analyses that incorporate data from trial(s) plus observational data
  - (Most frequently) To perform analysis when trial data are unavailable

Decision Trees

- "Models" that use a tree-like structure to organize thoughts and data about problems (e.g., treatment decisions) and their consequences
- Characterized by decisions, chances, and outcomes
- Results based on probabilities and "rewards" for outcomes
- Time usually not directly modeled in decision trees

Markov Models

- Repetitive decision trees used for modeling conditions that have events that may/do occur repeatedly over time
  - e.g., Cycling among heart failure classes or screening for colorectal cancer
- Use of Markov models simplifies presentation of tree structure
- Markov models explicitly account for timing of events

Cost-Effectiveness Methods Overview

Economic Evaluation Methods Overview

- Types of analyses
- Steps in economic evaluation
- Types of outcomes
- Perspective

Types of Analyses

Types of Analysis

- Cost identification
- Cost-effectiveness / cost-utility
- Cost-benefit
- Generally distinguished by:
  - Outcomes included: e.g., costs alone vs costs and effects
  - How outcomes are quantified: e.g., as money alone or as health and money

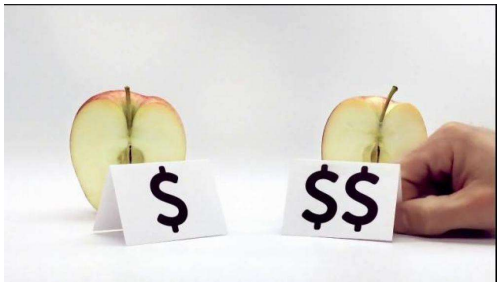
Cost Identification / Cost Minimization / Cost-Cost Analysis

Cost-Identification, etc.

- Estimates difference in costs between therapies, but not difference in other outcomes
- Commonly conducted when no difference observed in effectiveness
  - "As no statistical significant difference among the mean QALYs gained with the different [hormonal therapies] was detected (p = 0.12), CUA was replaced by a cost minimization analysis."

Lazarro et al. Archivio Italiano di Urologia, Andrologia. 2007:79:104-7

Appropriate Only When Therapies are Identical



Dish Network TV Spot, "Apples", 2015

Cost Identification ???

2016 Kia Rio, MSRP $14,165

Mercedes
2016 SLK, MSRP $47,925

Is failure to detect a difference same as a demonstration of equivalence?

Problems With Cost Identification

- Old version
  - If two therapies' effects are identical, adopt cheaper of two
    - Effect maximization corollary: If two therapies' costs identical, adopt more effective of two
- New version
  - Generally can't conclude two therapies are identical
    - At most we fail to reject null hypothesis
  - Cost-identification unlikely to be appropriate

Cost-Effectiveness Analysis

Cost-Effectiveness Analysis

- Estimates differences in costs and differences in outcomes between interventions
- Costs and outcomes measured in different units
- Costs usually measured in money terms; outcomes in some other units
- Incremental cost-effectiveness ratio

$$\frac{\text{Costs}_1 - \text{Costs}_0}{\text{Effects}_1 - \text{Effects}_0}$$

Cost-Effectiveness A Relative Measure

- Cost-effectiveness is a *relative* measure; no program is "cost-effective" in abstract
  - Results meaningful in comparison with:
    - A predetermined standard
      - e.g., $50,000 per quality-adjusted year of life saved
    - Other accepted and rejected interventions (e.g., a league table)

Cost-Utility Analysis

- Costs and Outcomes measured in different units AND outcomes expressed in units of utility (e.g., QALYs)
- Referred to either as a fourth type of analysis or as a subset of cost-effectiveness analysis

What Is Maximum Acceptable WTP?

- US Gov't
  - EPA: 9.1 M / life (~222K / undiscounted YOLS)
  - FDA: 7.9 M / life (~176K / undiscounted YOLS)
  - DOT: 6 M / life (~133K / undiscounted YOLS)
- Australia: $AU 42K - 76K /YOLS
- Italy: €60,000/QALY
- Netherlands: €80 000/QALY
- Sweden: SEK 500,000 (€54,000) / QALY
- UK: £20 - 30K / QALY
- WHO report: 3 times GDP per DALY

Cost-Benefit Analysis

Cost-Benefit Analysis

- Estimates differences in costs and differences in benefits in same (usually monetary) units
- As with cost-effectiveness, requires a set of alternatives
- Net benefit is preferred cost-benefit result
  - $(Benefit_1 - Benefit_0) - (Cost_0 - Cost_0)$

Review

Example 1

- Investigators compared 2 treatments, “LessCost” and “MoreCure”
- Found that “LessCost” was less expensive and recommended its adoption by physicians
  - 1000 vs 1200
- What type of economic analysis are investigators carrying out?
- Do you agree with their conclusion?

Example 2

- Investigators compared 2 treatments, “LessCost” and “MoreCure.” Observed the following:

	MoreCure	LessCost	Difference
Cost	1200	1000	200
Benefit	3000	1500	1500

- Authors concluded that MoreCure is net beneficial.
- What type of economic analysis are investigators carrying out?
- Do you agree with their conclusion?

Example 3

- Investigators compared 2 treatments, “LessCost” and “MoreCure.” Observed that MoreCure cost 200 more than LessCost and provided 0.03 additional QALYs
- Authors recommended that MoreCure was good value for cost
- What type of economic analysis are investigators carrying out?
- Do you agree with their conclusion?

Steps in Economic Evaluation

Steps in Economic Evaluation

Step 1: Quantify costs of care

Step 2: Quantify outcomes

Step 3: Assess whether and by how much average costs and outcomes differ among treatment groups

Step 4: Compare magnitude of difference in costs and outcomes and evaluate "value for costs"

- e.g. by reporting a cost-effectiveness ratio, net monetary benefit, or probability that ratio is acceptable
- Potential hypothesis: Cost per quality-adjusted life year saved significantly less than $75,000

Step 5: Perform sensitivity analysis

Types Costs and Effects

Types of Costs

- Direct: medical or nonmedical
- Time costs: Lost due to illness or to treatment
- Intangible costs
- Types of costs included in an analysis depend on:
  - What is affected by illness and its treatment
  - What is of interest to decision makers
    - e.g., a number of countries' decision makers have indicated they are not interested in time costs

What Effectiveness Measure?

- Can calculate a ratio for any outcome
  - Cost per toe nail fungus day averted
- For cost-effectiveness ratios to be an informative, must know willingness to pay for outcome
  - In many jurisdictions, quality-adjusted life year (QALY) is recommended outcome of cost-effectiveness analysis

QALYS

- Economic outcome that combines preferences for both length of survival and quality into a single measure
- Help us decide how much to pay for therapies that:
  - Save fully functional lives/life years

    VS
  - Save less than fully functional lives/life years
    - e.g., heart failure drug that extends survival, but extra time spent in NYHA class III

    VS
  - Don't save lives/life years but improve function
    - e.g., heart failure patients spend most of their remaining years in class I instead of class III

QALY Scores

- QALY or preference scores generally range between 0 (death) and 1 (perfect health)
  - E.g., health state with a preference score of 0.8 indicates that year in that state is worth 0.8 of year with perfect health
  - There can be states worse than death with preference scores less than 0

Prescored Health State Classification Instruments

- Dominant approach for QALY measurement uses prescored health state classification instruments (indirect utility assessment)
- Participants' report their functional status across a variety of domains
- Preference scores derived from scoring rules that usually have been developed from samples from general public

Compare magnitude of difference in costs and outcomes and evaluate "value for costs"

Screening for Colorectal Cancer

- Suppose we can use one of 5 screening strategies for screening for cases of colorectal cancer

Screen	Cost	YOLS
S1 Sig Q10	1290	17.378
S2 U+Sig, Q10	1810	17.402
S3 C Q(10)	2030	17.396
S4 Sig Q5	1535	17.387
S5 U+Sig, Q5	2035	17.407

Frazier AL, et al. JAMA. 2000;284:1954-61.

- What calculations might help make choice between the screening strategies?

Mistake #1

- Divide therapy's cost by its outcome; compare resulting ratios

Screen	Cost		YOL		C/YOL
S1 Sig Q10	1290	÷	17.378	=	74.23
S2 U+Sig, Q10	1810	÷	17.402	=	104.01
S3 C Q(10)	2030	÷	17.396	=	116.69
S4 Sig Q5	1535	÷	17.387	=	88.28
S5 U+Sig, Q5	2035	÷	17.407	=	116.91

- Sometimes mistakenly referred to as average cost-effectiveness ratios

Dividing a Therapy's Costs by Its Effects is "Generally Uninformative"

	Cost	Effect	Ratio
Example 1			
Rx1	2,800	0.28	10,000
Rx2	5,800	0.29	20,000
Example 2			
Rx1	2,800	0.28	10,000
Rx2	11,200	0.56	20,000

Dividing a Therapy's Costs by Its Effects is "Generally Uninformative"

	Cost	Effect	Ratio
Example 1			
Rx1	2,800	0.28	10,000
Rx2	5,800	0.29	20,000
	(5,800-2,800) / (0.29-0.28) = 300,000		
Example 2			
Rx1	2,800	0.28	10,000
Rx2	11,200	0.56	20,000
	(11,200-2,800) / (0.56-0.28) = 30,000		

Mistake #2

- Calculate ratios for all therapies versus S1, Sig Q10

Screen	Cost	ΔCost	YOL	ΔYOLS	Ratio *
S1 Sig Q10	1290	0	17.378	0	--
S2 U+Sig, Q10	1810	520	17.402	0.024	21667
S3 C Q(10)	2030	740	17.396	0.018	41111
S4 Sig Q5	1535	245	17.387	0.009	27226
S5 U+Sig, Q5	2035	745	17.407	0.029	25690

* $(C_i - C_1) / (E_i - E_1)$

Average Cost-Effectiveness Ratio

- Ratios in prior table correctly referred to as average cost-effectiveness ratios
- Definition: Comparison of costs and effects of each intervention with a single option, often "do nothing" or usual care option
  - Sometimes study sponsor's therapy

Average Cost-Effectiveness Ratios

- Goal of algorithm: choose strategy that provides largest health outcome that we are still willing to pay for

Screen	Cost	ΔCost	YOL	ΔYOLS	Ratio *
S1 Sig Q10	1290	0	17.378	0	--
S2 U+Sig, Q10	1810	520	17.402	0.024	21667
S3 C Q(10)	2030	740	17.396	0.018	41111
S4 Sig Q5	1535	245	17.387	0.009	27226
S5 U+Sig, Q5	2035	745	17.407	0.029	25690

- Why don't average ratios allow identification of this strategy?

What's Wrong with the Average Cost-Effectiveness Ratio?

Screen	Cost	ΔCost	YOL	ΔYOLS	Ratio *
S1 Sig Q10	1290	0	17.378	0	--
S2 U+Sig, Q10	1810	520	17.402	.024	21,667
S5 U+Sig, Q5	2035	745	17.407	0.029	25690

- 25,690 ACER for S5, U+Sig, Q5s takes credit for the $1810 we are already spending on S2 and the 17.402 YOL we live with S2
- Compared to S2, we are spending $225 more for S5 and gaining only 0.005 YOL ($225 / .005 = $45,000)

Incremental Cost-Effectiveness Ratios

- Compares costs and effects among alternative options
- When there are only 2 options being evaluated, average and incremental cost-effectiveness ratios are identical

3 Potential Problems for ICER Calculation

1. Treatments must be correctly ordered
2. Never want to spend more and obtain less outcome
3. Don't want to buy less outcome for a higher cost per unit of outcome

Incremental Cost-Effectiveness Ratios

- Basic idea: calculate ratios for succeeding pairs of therapies, e.g., 2 vs 1, 3 vs 2…

Screen	Cost	ΔCost	YOL	ΔYOLS	Ratio *
S1 Sig Q10	1290	--	17.378	--	--
S2 U+Sig, Q10	1810	520	17.402	.024	21667
S3 C Q(10)	2030	220	17.396	-.006	-36667
S4 Sig Q5	1535	-495	17.387	-.009	55000
S5 U+Sig, Q5	2035	500	17.407	.020	25000

* $(C_i - C_{i-1}) / (E_i - E_{i-1})$

- What's wrong with these numbers?

Problem/Complication 1

- Treatments must be correctly ordered

Efficient Algorithm: Step 1

- Rank order therapies in ascending order of either outcomes or cost

Treatment	Cost	YOLS
S1 Sig Q10	1290	17.378
S4 Sig Q5	1535	17.387
S3 C Q(10)	2030	17.396
S2 U+Sig, Q10	1810	17.402
S5 U+Sig, Q5	2035	17.407

- 5 strategies not in ascending order of either cost or effect
- Revised so correctly ordered by effect
- Final recommendation unaffected by ranking variable

Problem/Complication 2

- Never want to spend more (increased cost) and obtain less outcome (reduced effects) than at least one other alternative
  - Referred to as "strong" dominance

Efficient Algorithm: Step 2

- Eliminate therapies that are strongly dominated

Treatment	Cost	YOLS
S1 Sig Q10	1290	17.378
S4 Sig Q5	1535	17.387
S3 C Q(10)	**2030**	**17.396**
S2 U+Sig, Q10	**1810**	**17.402**
S5 U+Sig, Q5	2035	17.407

- S2 strongly dominates S3
- Eliminate S3 from consideration for adoption

Efficient Algorithm: Step 3

- Compute incremental cost-effectiveness ratios for each adjacent pair of outcomes
  - i.e., between options S1 and S4; options S4 and S2; and options S2 and S5

Treatment	Cost	Δ	YOLS	Δ	ICER
S1 Sig Q10	1290	--	17.378	--	--
S4 Sig Q5	1535	245	17.387	.009	27,222
~~S3 C, Q10~~	~~2030~~	~~495~~	~~17.396~~	~~.009~~	~~SDOM~~
S2 U+Sig, Q10	1810	275	17.402	.015	18,333
S5 U+Sig, Q5	2035	225	17.407	.005	45,000

Efficient Algorithm: Step 3 (2)

- If resulting incremental ratios ranked from lowest to highest, skip to Step 6
- If not, need to address problem/complication 3

Treatment	Cost	Δ	YOLS	Δ	ICER
S1 Sig Q10	1290	--	17.378	--	--
S4 Sig Q5	1535	245	17.387	.009	**27,222**
~~S3 C, Q10~~	~~2030~~	~~495~~	~~17.396~~	~~.009~~	~~SDOM~~
S2 U+Sig, Q10	1810	275	17.402	.015	18,333
S5 U+Sig, Q5	2035	225	17.407	.005	45,000

Problem/complication 3

- Rather buy more outcome for a lower cost per unit than less outcome for a higher cost per unit
  - Referred to as “extended” or “weak” dominance
- May need to repeat evaluation of weakly dominated therapies

Efficient Algorithm: Step 4

- Eliminate weakly dominated therapies

Treatment	Cost	Δ	YOLS	Δ	ICER
S1 Sig Q10	1290	--	17.378	--	--
S4 Sig Q5	**1535**	**245**	**17.387**	**.009**	**27,222**
~~S3 C, Q10~~	~~2030~~	~~--~~	~~17.396~~	~~--~~	~~SDOM~~
S2 U+Sig, Q10	**1810**	**275**	**17.402**	**.015**	**18,333**
S5 U+Sig, Q5	2035	225	17.407	.005	45,000

- S4 is weakly dominated by S2
  - S2 more effective than S4: 17.402 vs 17.387
  - Ratio for S2 vs S3 (18,333) less than ratio for S4 vs S1 (27222)

Efficient Algorithm: Step 5

- Eliminate S4 and **RECALCULATE** ratio for S2 vs S1

Treatment	Cost	Δ	YOLS	Δ	ICER
S1 Sig Q10	1290	--	17.378	--	--
~~S4 Sig Q5~~	~~1535~~	~~--~~	~~17.387~~	~~--~~	~~WDOM~~
~~S3 C, Q10~~	~~2030~~	~~--~~	~~17.396~~	~~--~~	~~SDOM~~
S2 U+Sig, Q10	1810	520	17.402	.024	21,667
S5 U+Sig, Q5	2035	225	17.407	.005	45,000

- Resulting ratio will always be less than ratio of weakly dominated therapy and greater than weakly dominating therapy's original incremental ratio
  - E.g., 18,333 < 21,667 < 27,222

Efficient Algorithm: Step 6

- Identify acceptable therapy

Maximum WTP	Therapy
<21,667	S1
21,667 to 45,000	S2
45,000+	S5

Full Cost-Effectiveness Table

Treatment	Cost	ΔC	YOLS	Δ Y	ICER
S1 Sig Q10	1290	--	17.378	--	--
~~S4 Sig Q5~~	~~1535~~	~~--~~	~~17.387~~	~~--~~	~~WD~~
~~S3 C Q(10)~~	~~2030~~	~~--~~	~~17.396~~	~~--~~	~~SD~~
S2 U+Sig, Q10	1810	520	17.402	0.024	21,667
S5 U+Sig, Q5	2035	225	17.407	0.005	45,000

SD = strong dominance; WD = weak dominance

Reduced Cost-Effectiveness Table

Treatment	Cost	ΔC	YOLS	Δ Y	ICER
S1 Sig Q10	1290	--	17.378	--	--
S2 U+Sig, Q10	1810	520	17.402	0.024	21,667
S5 U+Sig, Q5	2035	225	17.407	0.005	45,000

Cost-Effectiveness Exercise

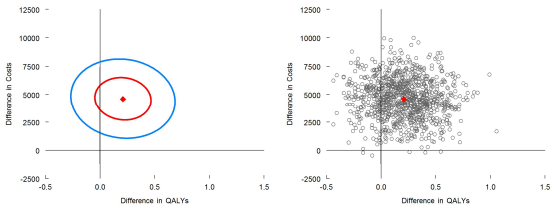
Sampling Uncertainty

Confidence About Value for Cost

- Common goal of economic analysis: identify when we can be confident that a therapy is good value compared to another
- Threat to confidence: economic result observed in experiment may not reflect result in the population
  - Single sample drawn from population
- Referred to as sampling (or stochastic) uncertainty
- Methods for estimating sampling uncertainty for economic outcomes have much in common with methods used for clinical findings

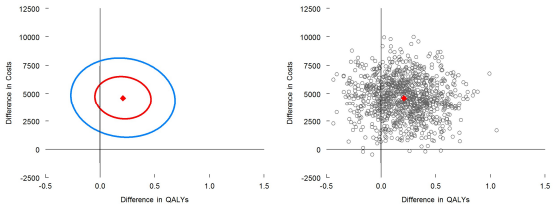
Cost-Effectiveness Plane

Joint Distribution of Cost and Effect



- Bivariate normal curves (Δc, SEc, Δq, SEq, ρ) (left)
- Bootstrap of patient level data (right)
- Second order Monte Carlo (decision analysis with variables represented as distributions) (right)

Joint Distribution of Cost and Effect (2)

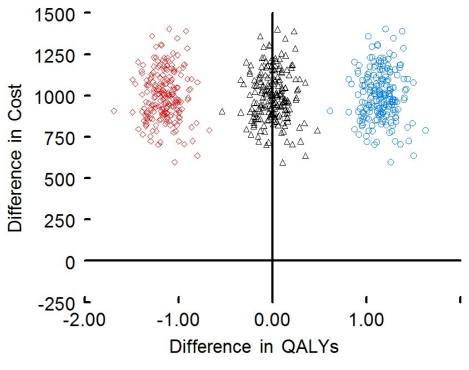


- Mean cost difference, $4600, SE, 1803
- Mean QALY difference, 0.2090, SE, 0.2430
- Correlation of difference, -0.045
- ICER Point estimate = 22,010 (4600 / 0.2090)

Information from the Plane

- Cost-effectiveness plane provides information about point estimates, confidence intervals and p-values for:
  - Difference in effect
  - Difference in cost
  - Cost-effectiveness analysis

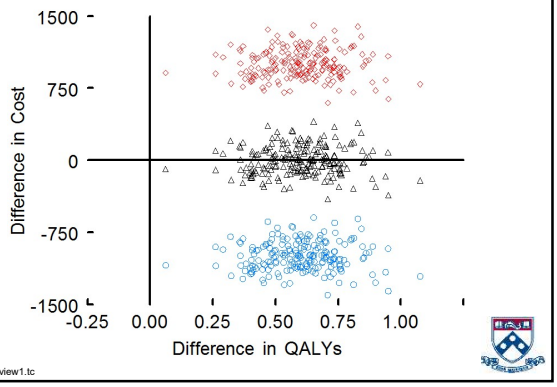
In Which Experiment(s) is ΔQ Significant?



Red and blue
(because all of their densities fall on
one side of 0 on Y-axis)

Black triangles not significantly different
(because too large a density falls on each
side of 0 on X-axis)

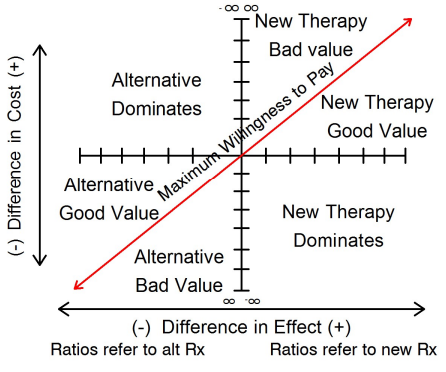
In Which Experiment(s) is ΔC Significant?



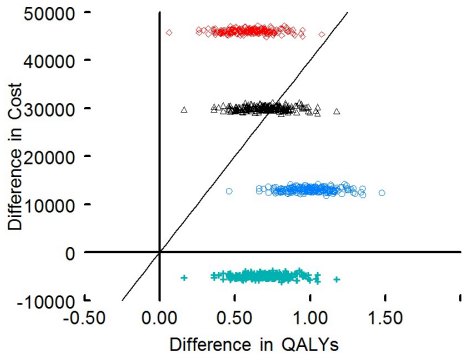
Red and blue
(because all of their densities fall on
one side of 0 on X-axis)

Black triangles not significantly different
(because too large a density falls on each
side of 0 on Y axis)

Value and the Cost-Effectiveness Plane



In Which Experiments Can We Be Confident of Value?

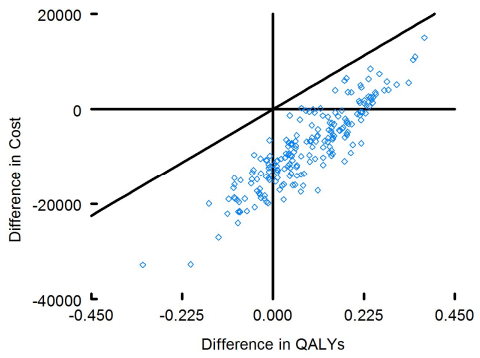


Red, blue, and cyan
(because all of their densities fall on one side of WTP)

Black triangles not confident because large fractions of density fall on both sides of WTP

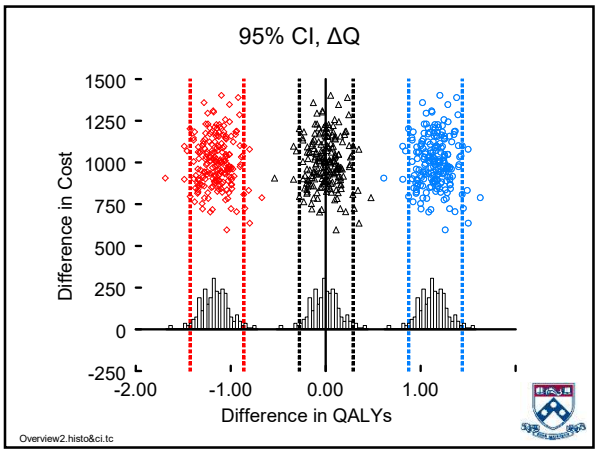
For red, blue and cyan,
what confidence statements
can we make?

What Can We Conclude About ΔC, ΔQ, Value?



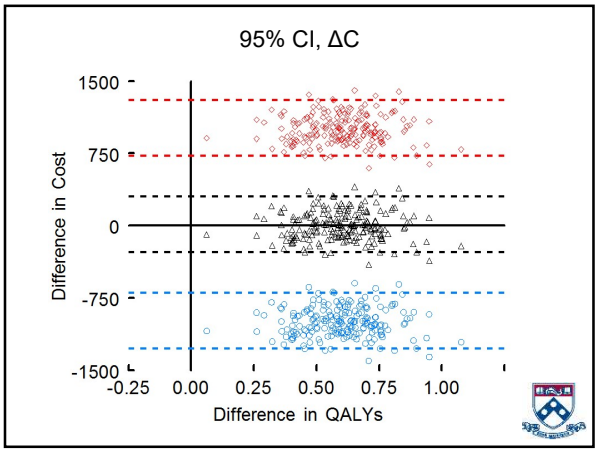
Confidence Intervals

- Graphs above provide examples of 0 (for differences in means, including NMB) or willingness to pay (W) (for CI for CER) falling either well inside or fully outside distribution of results
- Don't typically require that results be fully outside distribution to conclude they differ from 0 or W
  - Parametrically never happens
- Usual strategy: Identify a tolerance – e.g., 2.5% for 95% confidence – for the maximum fraction of results that can fall on one side of 0, 1, or W
- Conclude with 95% confidence that result excludes 0 or W if 0 or W fall outside 95% CI



Can be 95% confident of a difference for red and blue (because 0 on X-axis does not fall within the 95% CI)

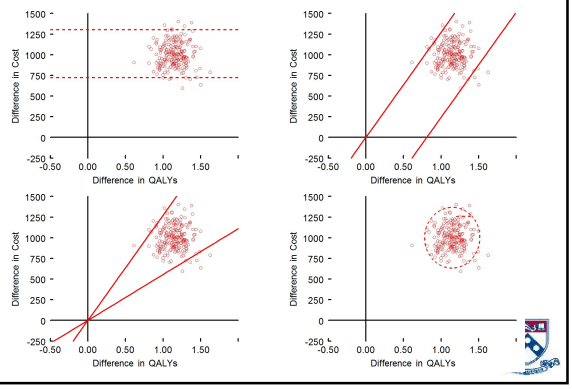
Can't be 95% confident of difference for black triangles (because 0 on X-axis falls within 95% CI)



Can be 95% confident of a difference for red and blue (because 0 on Y-axis does not fall within the 95% CI)

Can't be 95% confident of difference for black triangles (because 0 on Y-axis falls within 95% CI)

95% CI for CER?



95% CI

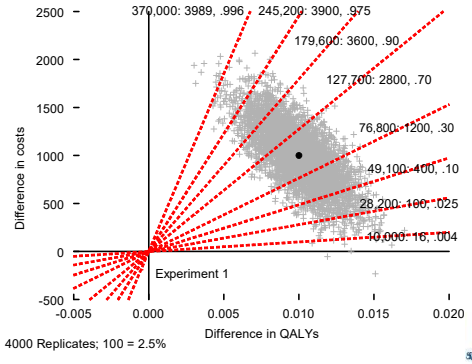
- Upper left: CI for ΔC
- Upper right: CI for NMB
- Lower right: 95% confidence ellipse around the point on the C/E plane defined by ΔC and Δq (CE for point, not CI for ICER)
- Lower left: 95% CI for the ICER

Confidence Intervals for ICER

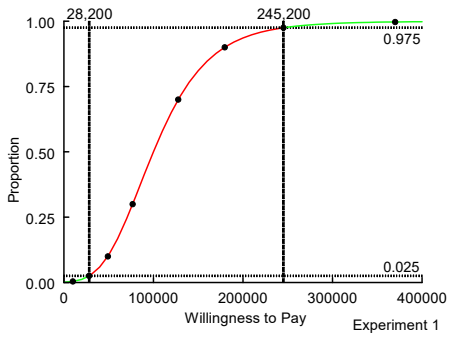
- Commonly thought to be an “order” statistic
  - Order ratios from smallest to largest
  - Identify 2.5th percentile (e.g., 26th of 1000) and 97.5th percentile (e.g., 975th of 1000)
- Technically NOT an order statistic
  - But situations exist when ordering “works”
- CI for ICER defined by lines through origin that exclude α/2 of joint distribution of difference in cost and effect

Acceptability Curve

Constructing Acceptability Curve



Acceptability Curve



"Common" Conclusions from Acceptability Curves

W	What is often said
28,200	"97.5% chance Rx A not good value"
76,800	"70% chance Rx A not good value"
100,000	"50% chance either therapy good value"
127,700	"70% chance Rx A good value"
245,200	"97.5% chance Rx A good value"

- Common to adopt 1-tailed interpretation of acceptability curve
- Ignores fact that 50% – not 0% – represents no information

Sampling Uncertainty Exercise

Study Perspective

Study Perspective

- Economic studies should adopt 1 or more “perspectives”
  - Societal
  - Payer (often insurer)
  - Provider
  - Patient
- Perspective helps identify services that should be included in analysis and how services should be cost out
  - e.g., patient out-of-pocket expenses may be excluded from insurer perspective
  - Not all payments may represent costs from societal perspective

Comparison Across Multiple Time periods

Comparison of Cost and Outcome in Multiple Periods

- Because costs and outcomes in different time periods are not directly comparable, their comparison requires conversion to a common time period
- Conversion accounts for:
  - Changes in purchasing power of dollar over time

    Inflation
  - Differential valuation of cost and outcome depending on when they occur:

    Discounting / Social rate of time preference
- Inflation NOT same as time preference
  - Still discount even if inflation rate equals 0!!

Inflation

- Inflation accounts for fact that purchasing power of a dollar changes over time
  - Stream of dollars without inflation adjustment: Nominal $
  - Stream after inflation adjustment: Real $
- Common measure of inflation
  - Consumer price index
    - Defined for a market "basket" of goods and services
      - Can be problematic, given market basket has to change over time

Time Preference

- Unlike inflation -- which accounts for changes in purchasing power over time -- discounting accounts for our preferences for costs incurred and outcomes obtained in different periods
  - Tend to prefer to consume immediate benefits to those occurring in the future (Marginal rate of time preference)
  - Investment today could produce more in the future (Marginal rate of return on private investment)
    - Market interest rate

"When" to Inflation-Adjust and Discount

- Need to adjust for inflation depends on whether costs are measured in "constant" dollars (e.g.by use of data from 2013 fee schedules) or in dollars from different years (e.g., by use of billing data from different years)
- Need to discount a function of duration of follow-up per participant, not duration of study

Who is Listening?

Not the U.S. Congress

"The Patient-Centered Outcomes Research Institute . . . shall not develop or employ a dollars per quality adjusted life year (or similar measure that discounts the value of a life because of an individual's disability) as a threshold to establish what type of health care is cost effective or recommended. The Secretary shall not utilize such an adjusted life year (or such a similar measure) as a threshold to determine coverage, reimbursement, or incentive programs under title XVIII"

The Patient Protection and Affordable Care Act

Is Some Use in US

- Common Belief: "Pharmacoeconomic data not used in US"
  - NIH expert guideline panels and Environmental Protection Agency can and do use
  - Chambers et al.: Lack of an estimate of cost-effectiveness associated with a decreased likelihood of Medicare coverage
  - Aspinall et al.: Veterans Health Administration "has emphasized use of cost-effectiveness data, especially for newer, costly drugs."
  - Neuman and Bliss: 12% of FDA DDMAC warning letters between 2002 and 2011 cite health economic violations

But Not All Agencies

- Medicare and Medicaid prohibited from consideration of costs and cost-effectiveness in recommendations and policies (but use informally)
- ACIP and USPSTF prohibited
- VA, NIH expert guideline panels, EPA can and do use

Medicare's Coverage Policy

- So far, inclusion of economic considerations limited to:
  - If new technology is worse, don't cover no matter what the cost
  - If new technology is no better and costs more, don't cover
  - If new technology is possibly better but possibly not, don't cover unless it costs less
  - If new technology is definitely better, always cover

Others

- AMCP Guidance for Submission of Clinical and Economic Evaluation Data to Support Formulary Listing in U.S. Health Plans and Pharmacy Benefits Management Organizations
- Cost effectiveness analysis (never cost benefit) used in other countries (UK, Canada, Australia, etc.) to suggest/determine what will be paid for under a (nearly) free single insurance plan. The plan either pays in full or pays nothing

Who is Listening

- PE Recommendations/Guidelines (Partial list)
  - Australia
  - Austria
  - Brazil
  - Baltic countries
  - Belgium
  - Brazil
  - China
  - Denmark
  - Egypt
  - Finland
  - France
  - Hungary
  - Italy
  - Mexico
  - Netherlands
  - Norway
  - Poland
  - Russia
  - South Korea
  - Spain
  - Sweden
  - Taiwan
  - Thailand
  - U.K.

Summary

- Use of pharmacoeconomic data growing
  - Improve value of healthcare
  - Manage healthcare budgets
- Multidisciplinary science: medicine, pharmacy, economics, decision sciences
- General methods well developed, but some areas still undergoing development