How CE Analyses Are Used

Also (briefly),
Other Resources for Cost-Effectiveness Analyses,
Tornado Diagrams,
and
The Rest of the Course

How CE Analyses Are Used

• At the bedside/In the office
• Health policy
  – Public Health
  – Clinical care

CEA in Public Health

• Tobacco control
• Preventing motor vehicle occupant injury
• Cancer screening
• Childhood immunizations
• Newborn screening
• Blood product safety
Cost-Effectiveness Analysis to Inform Health Policy outside the United States

Canada

The federal Patented Medicine Prices Review Board, an independent, quasi-judicial body, regulates the introductory prices of new patented medications in Canada. The Review Board’s mandate is to ensure that patented drug prices are not “excessive,” on the basis of their “degree of innovation” and through a comparison with the prices of existing medicines in Canada and in seven other countries including the United States and the United Kingdom. . . .
Germany

...the Federal Joint Committee has wide-ranging regulatory power to determine the services to be covered by sickness funds and to set quality measures for providers...To the extent possible, their coverage decisions are based on evidence from health technology assessments and comparative-effectiveness reviews. The Federal Joint Committee is supported by the Institute for Quality and Efficiency (IQWiG), a foundation legally charged with evaluating the cost-effectiveness of drugs with added therapeutic benefits.

Australia

The Australian Government is a near-monopolist purchaser of patent medicines which, combined with tight prescribing requirements, allows it to control pharmaceutical pricing. New pharmaceuticals have to meet cost-effectiveness criteria and are subject to nationally negotiated pricing before inclusion in the formulary of publicly subsidized medicines.

Australia

• 1992
• Pharmaceutical Benefits Advisory Committee
• Pharmaceuticals, medical services, medical procedures, diagnostics, vaccines, blood products, screening programs
United Kingdom

• National Institute for Clinical Excellence (NICE)

• Started in 1999

• Problem was “Postcode Lottery”
NICE

• Only 3 of the first 22 technologies were not recommended
  – Prophylactic removal of wisdom teeth
  – Laparoscopic surgery for colorectal cancer
  – Autologous cartilage transplantation for knee joints

Some of the more controversial NICE decisions have concerned drugs for the treatment of Alzheimer's disease (donepezil, galantamine, rivastigmine and memantine) and for renal cell carcinoma (bevacizumab, sorafenib, sunitinib and temsirolimus). All are drugs with a high cost per treatment, and NICE has either rejected or restricted their use in the NHS on the grounds that they are not cost-effective.

NASTY – Not Available, So Treat Yourself
NICE and Multiple Sclerosis

- Beta interferon and glatiramer acetate
- Rejected
- Controversy over how to judge long-term results with only short-term trials
- Conditional acceptance
  - If the drugs don’t deliver a long-term ICER less than $66,000 per QALY, the pharmaceutical companies will return the monies they received from the government.
NHS reform: health and social care bill passes its final hurdle

Final vote for the bill ended more than a year of debate and several last-minute attempts to overturn or delay the legislation

Juliette Jowit, political correspondent
Beginning in April 2013

- NICE became a public body independent of the government
- New name: National Institute for Health and Care Excellence (still NICE)
- New responsibilities include
  - Guidance for social services
  - Value-based pricing for drugs (beginning in 2014)

Value-Based Pricing for Drugs

- Prices reflect factors that are not fully recognized by QALYs, for example, drugs for diseases with
  - A greater “burden of illness”
  - Unmet need
  - Particularly severe consequences
- And drugs with
  - Greater therapeutic innovation
  - Wider societal benefits

NICE times: a valedictory dispatch

Sir Michael Rawlins
"If the United States is to meet the needs of all its citizens, especially in the face of an increasingly elderly population, it will someday have to take both clinical effectiveness and cost-effectiveness into account in determining the contents of its package of universal health care. Our experience in the United Kingdom shows that, though sometimes uncomfortable, it is possible."

What about the United States?

Oregon

- 1980s
- Expand Medicaid to cover more people
- Identify conditions paired with treatments
- Rank condition-treatment pairs
  - Use CE analyses
  - Telephone survey of utilities using rank-and-scale method
- Pay only for condition-treatment pairs above the budget line
Oregon

• 1990 list
  – Tooth capping ranked higher than surgery for ectopic pregnancy
  – Splints for TM joints ranked higher than appendectomies

• 1992 list
  – Expert judgment, not CE analyses
  – Rejected by HCFA

Oregon’s 1992 List

• Violated Americans with Disabilities Act
  – Quality of life measures were based only on the preferences of healthy individuals
  – Treatments that restored people to their usual disabled state were undervalued relative to treatments that restored people to their usual normal state

What Happened in Oregon?

“The most fundamental lesson . . . was that the use of CE analysis was unlikely to produce a socially or politically acceptable definition of necessary care” in the United States.

—Peter J. Neumann, ScD
Medicare

- 1965 authorizing legislation prohibits payment for "... items and services that are not reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member."
- Reasonable and necessary means safe, effective, generally accepted ("customary")

Medicare

- 1989 proposed regulations
  "We believe the requirement . . . that a covered service be 'reasonable' encompasses the authority to consider cost as a factor in making Medicare coverage decisions."
- Opposition ("rationing")
- 1998 Medicare Coverage Advisory Committee (MCAC)
  - Cost considered only when effects are equivalent

Medicare Exceptions

- Pneumococcal and influenza vaccines
- Screening mammography
- CE analyses can affect payment decisions, as opposed to coverage decisions, e.g., "me too" drugs.
- Implantable cardiac defibrillators
  "We don’t use cost to decide the evidence issue, but we do use cost to decide if the issue is important enough to address."
Factors Predicting Medicare National Coverage: An Empirical Analysis
James D. Chambers, MPharm, MSc,* Stephen Morris, BSc, MSc, PhD, Peter J. Neumann, ScD, and Martin J. Buxton, BA*

Results: The following variables were independently associated with positive Medicare coverage: good or fair quality supporting evidence (adjusted odds ratio, OR=6.04, P<0.01); presence of an alternative intervention (OR=0.130, P<0.01); no associated estimate of cost-effectiveness (OR=0.190, P<0.05).

Conclusions: Findings suggest that good or fair quality supporting evidence is a strong predictor of positive coverage. Availability of alternative interventions, more recent decisions, and lack of an associated estimate of cost-effectiveness are associated with a decreased likelihood of positive coverage. The findings highlight Medicare’s move to evidence-based coverage decisions, and suggest that coverage decisions are influenced by the availability of cost-effectiveness evidence.

Does Medicare Have an Implicit Cost-Effectiveness Threshold?
James D. Chambers, MPharm, MSc; Peter J. Neumann, ScD; Martin J. Buxton, BA

Background: Despite the huge cost of the program, the Centers for Medicare and Medicaid Services (CMS) have been criticized for lack of consistency in its national coverage determinations (NCDs). Although to assess whether an implicit cost-effectiveness threshold exists and to determine if necessary evidence has been considered in previous NCDs. Methods: A systematic search was conducted to identify estimates of cost-effectiveness from a sample of 374 NCDs from 1993-2007. Results: Of the 374 decisions, 123 were considered as having a corresponding cost-effectiveness estimate, 128 were not associated with a positive coverage decision and 125 with a non-coverage decision. Of the remaining decisions, 120 were associated with an economic evaluation that met current CMS criteria in its evidence (if over $50,000 greater than $100,000 for QALY gain and was less than $100,000 per QALY gain and was less than $100,000). Results: Of the 125 decisions, 120 were associated with a positive coverage decision and 15 with a non-coverage decision. Of the positive decisions, all were associated with an economic evaluation that met current CMS criteria in its evidence (if over $50,000 greater than $100,000 for QALY gain and was less than $100,000). Results: Of the 125 decisions, 120 were associated with a positive coverage decision and 15 with a non-coverage decision. Of the positive decisions, all were associated with an economic evaluation that met current CMS criteria in its evidence (if over $50,000 greater than $100,000 for QALY gain and was less than $100,000). Results: Of the 125 decisions, 120 were associated with a positive coverage decision and 15 with a non-coverage decision. Of the positive decisions, all were associated with an economic evaluation that met current CMS criteria in its evidence (if over $50,000 greater than $100,000 for QALY gain and was less than $100,000). Results: Of the 125 decisions, 120 were associated with a positive coverage decision and 15 with a non-coverage decision. Of the positive decisions, all were associated with an economic evaluation that met current CMS criteria in its evidence (if over $50,000 greater than $100,000 for QALY gain and was less than $100,000). Results: Of the 125 decisions, 120 were associated with a positive coverage decision and 15 with a non-coverage decision. Of the positive decisions, all were associated with an economic evaluation that met current CMS criteria in its evidence (if over $50,000 greater than $100,000 for QALY gain and was less than $100,000).

Other Organizations that Use Cost Analyses

- Some state Medicaid programs
- Blue Cross Technology Evaluation Center
- Pharmaceutical benefits managers (PBMs)
- Department of Veterans Affairs
- Department of Defense
- Health plan and hospital drug formularies
- Centers for Disease Control (CDC)
- Agency for healthcare Research and Quality (AHRQ)
Comparative Effectiveness Research

- 2003 Medicare Prescription Drug, Improvement, and Modernization Act – AHRQ Effective Health Care Program
- 2008 IOM recommended national program of comparative effectiveness research
- 2009 American Recovery and Reinvestment Act (ARRA)

American Recovery and Reinvestment Act (ARRA) of 2009 (P.L. 111-5)

- Comparative-effectiveness research (CER) covers "research that compares the clinical outcomes, effectiveness, and appropriateness of items, services, and procedures that are used to prevent, diagnose, or treat diseases, disorders, and other health conditions."

JAMA. 303(10):951-8, 2010 Mar 10

- Analyzed 328 medication studies recently published in 6 top medical journals
- Just 32% were aimed at determining which available treatment was best
- The rest compared a medication with a placebo
- 87% of the comparative effectiveness studies were funded entirely or in part by nonprofit foundations or government institutions
Patient Protection and Affordable Care Act, March 2010

• The bill establishes an independent, not-for-profit corporation, the Patient-Centered Outcomes Research Institute (PCORI)

• “to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions . . . with respect to the relative health outcomes, clinical effectiveness, and appropriateness of medical treatments, services, . . . .”

Patient Protection and Affordable Care Act, March 2010

• Patient-Centered Outcomes Research Trust Fund ($500 m/yr)

• Research priorities based on the prevalence and burden of diseases and patient care

• Primary research and systematic reviews of existing studies

• Contracts with NIH, AHRQ, and non-government researchers
PCORI and AHRQ

Non-overlapping Areas

- PCORI is now the only one of our two agencies authorized to fund CER, including CER infrastructure; AHRQ's funding in this area has been eliminated.
- AHRQ has numerous mandates that PCORI does not share:
  - Quality performance measurement and quality improvement;
  - Patient Safety
  - Health IT
  - Data Collection/Surveillance (e.g., HCUP, MEPS)
  - Knowledge Management
  - Workforce Training in CER/PCOR
  - Technology assessment

Legislating against Use of Cost-Effectiveness Information

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 advanced 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
The Incidental Economist
Who says PCORI can’t do cost-effectiveness?
Posted: 14 Oct 2013 03:00 AM PDT
The following is a guest post by Nicholas Bagley, University of Michigan Assistant Professor of Law.

• The first thing to notice is that this isn’t a flat prohibition on folding cost into PCORI research. It’s drafted much more narrowly. The statute just forbids PCORI from using a dollar-per-QALY metric “as a threshold” for establishing cost-effectiveness or for making recommendations. What does that mean? Well, it means that PCORI can’t say that a treatment costs “too much” just because its costs exceed, say, $50,000 for every QALY saved. That $50,000-per-QALY line would be a threshold.

• But does the statute prohibit PCORI from considering costs altogether? Nope. So far as the ACA is concerned, it’s perfectly OK for the institute to use dollar-per-QALY metrics. It just can’t use those metrics as thresholds. In practice, that leaves a lot of room for PCORI to think about costs. The institute could, for example, compile cost information about the treatments that it studies. No thresholds there. Alternatively, it could rank the cost-effectiveness of alternative treatments.

• None of this is to say that PCORI must consider costs. Indeed, in the current political environment, it would be foolish—maybe suicidal—for the institute to dwell on cost concerns. The thing about statutes, though, is that they tend to persist even as the political environment changes. Today, PCORI isn’t in the business of considering the costs of different asthma medications. But tomorrow? Stay tuned.

Joe V. Selby, MD, MPH, Executive Director

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Fieldwork was conducted using SSRS (Social Science Research Solutions; Media, Pennsylvania) via telephone (land line and cell) with a nationally representative sample of 1017 adults 18 years or older. The margin of error is ±3.9 percentage points at the 95% confidence level.

The primary outcome measures for this study are support for a government CER agency and support for each of 4 CER-driven decision vignettes: (1) Avastin/bowel cancer/United Kingdom, access to the drug limited to a subpopulation; (2) Avastin and/or Lucentis/wet age-related macular degeneration (wet AMD)/Italy, reimbursement provided only for an off-label treatment; (3) β-interferon/multiple sclerosis/United Kingdom, drug not provided because life extension judged too short; and (4) positron emission tomography (PET) scans/head and neck tumors/Germany, imaging method allowed only for a subset of cancers.

This study should offer a warning to the research community that, despite the cost-saving potential of CER, it is likely to engender widespread opposition when put into practice in the United States. . . . Growing health care spending will require smarter choices on the part of health care payers and consumers. This research suggests that the public often will not support the federal government making those decisions for them.

Why the American Aversion to CEA?

- People don’t understand CEAs
- People don’t trust CEAs
  - Pharmaceutical sponsorship
  - NEJM limitations
- CEAs are not relevant
  - Budget constraints
  - Indirect social benefits are difficult to estimate
- CEAs threaten corporate profits
Legal Issues

• State regulations
  – California requires insurance coverage “unhindered by a plan’s fiscal concerns”

• Courts
  – Often have overturned coverage decisions
  – Standard is “usual, customary, and reasonable,” not scientific evidence

Ethical issues

• “Veil of Ignorance”
  Decision makers should have no knowledge of their future health needs, so self-interest cannot affect decisions

• “Rush to Rescue”
  Small gains for many, invisible people vs. big gains for a few, highly visible people

• Heterogeneity in preferences

• Inherently immoral
Let’s Switch Direction
Journals That Are Resources for CEAs

- Methodology and CE Analyses
  - Medical Decision Making
  - Health Economics
- CE Analyses
  - Value in Health
  - Pharmacoeconomics
Resources for CEAs: Societies and Meetings

- Society for Medical Decision Making
  - http://www.smdm.org/

- International Society for Pharmacoeconomics and Outcome Research (ISPOR)
  - http://www.ispor.org/
Let's Switch Directions Again

Tornado Diagrams
Net Monetary Benefits (NMB)

\[ \text{NMB} = (W \ast \Delta Q) - \Delta C \]

where:
- \( W \) = willingness to pay
- \( \Delta Q \) = change in effectiveness
- \( \Delta C \) = change in cost
Bottom Line

- A tornado diagram uses multiple, one-way, deterministic, sensitivity analyses to identify variables with greater and lesser influence on the decision choice.
- In TreeAge, do NOT use the NMB (Net Monetary Benefits) option for a tornado diagram, even though it is the program's default option.
- Use the option for ICER (Incremental Cost-Effectiveness Ratio), instead.
Let’s Switch Directions Again

The Rest of the Course

COURSE GOAL
The overall goal of this course is for students to learn quantitative methods for understanding medical decisions.

COURSE CONTENT AND APPROXIMATE SEQUENCE
- Diagnostic tests with dichotomous results
- Diagnostic tests with continuous results
- Prediction rules
- Understanding Cost / Measuring and Analyzing cost / Discounting
- Mathematical modeling with decision trees
- Mathematical modeling with Markov techniques
- Measuring outcomes in terms of "utility"
- Conducting, analyzing, and understanding cost-effectiveness analysis
- Economic assessment and policy analysis

Rest of the Course


4/22 Sampling Uncertainty in Cost-Effectiveness Analysis - 1
4/24 Sampling Uncertainty in Cost-Effectiveness Analysis - 2
Design issues for economic assessments in trials; confidence intervals for cost-effectiveness ratios; acceptability curves; confidence intervals for net monetary benefits; sample size

4/29 Wrap-Up and Review for Final Exam

5/4 – 5/11 Final exam