How CE Analyses Are Used

Also (briefly),
Other Resources for Cost-Effectiveness Analyses,
Tornado Diagrams,
and Changes in Course for Next Year

How CE Analyses Are Used

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

CEA in Public Health

• Tobacco control
• Preventing injury to motor vehicle occupants
• Screening
• Immunizations
• 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.

United Kingdom

• National Institute for Clinical Excellence (NICE)

• Started in 1999

• Problem was “Postcode Lottery”
NICE

- Guidance based on cost-effectiveness analyses, modified by “other social values”
- NICE recommends against proposed drug coverage 10-15% of time
  - 30% of these recommendations are appealed
  - 10% of these appeals are successful

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 on the grounds that they are not cost-effective.

NASTY -- Not Available, So Treat Yourself

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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

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NICE and Blockers of Proprotein Convertase Subtilisin/Kexin type 9 (PCSK9), Which Lower Cholesterol

- In November 2015
  - NICE rejected Repatha (Amgen) for reasons of cost and effectiveness
- In February 2016
  - NICE approved Repatha for limited use in specific types of patients contingent on Amgen offering a discount
  - Rejected Praluent (Sanofi/Regeneron)
In general, NICE considers treatments cost-effective if their incremental cost-effectiveness ratio is £30,000 per QALY or less. On occasion, NICE accepts values between £30,000 and £50,000 per QALY. On rare occasions, it accepts values beyond £50,000. This ap-...
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: Moving Onward
Michael D. Rawlins, M.D.

“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

• 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
  – 688 procedures were ranked, and only the first 568 were covered.

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

Oregon’s 1992 List

• Challenged by the federal government
• 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
But the plan hit a snag in 2008 when a woman with recurrent lung cancer was denied a drug that cost $4,000 a month because the proven benefits were not enough to warrant the costs. The national backlash to this illuminated our collective difficulty in discussing the fact that some treatments might not be worth the money. The Oregon health plan made things worse in this case, however, by offering to cover drugs for the woman’s physician-assisted suicide, if she wanted it. Even supporters of the plan found the optics of this decision difficult to accept.

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.”

If You’re Going to Use CE Analyses, What Is the Maximum Acceptable Ratio?

• US Government
  – EPA: $9.1 M/life (~ $222,000/undiscounted YOLS)
  – FDA: $7.9 M/life (~ $176,000/undiscounted YOLS)
  – DOT: $6.0 M/life (~ $133,000/undiscounted YOLS)
• Australia: ($AU 42,000 to 76,000)/YOLS
• Italy: € 60,000/QALY
• Netherlands: € 80,000/QALY
• Sweden: € 54,000/QALY
• UK: (£ 20,000 to 30,000)/QALY
• WHO for developing countries: 3 times GDP per DALY
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
- Will contract 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. PCORI’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 CERP/COR
  - Technology assessment

Legislating against Use of Cost-Effectiveness Information

The Patient-Centered Outcomes Research Institute . . . shall not develop or employ a dollar 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 a threshold to determine coverage, reimbursement, or incentive payments 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. Again, no thresholds.

Joe V. Selby, MD, MPH, Executive Director

16
Why the American Aversion to CEA?

- People don’t understand CEAs
  - “Cost-effectiveness analysis” = “death panels”
- 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

Value Pricing in the US

[The outcomes used by the Institute for Clinical and Economic Review (ICER) include] comparison of clinical effectiveness, economic (cost-effectiveness), ethical, and societal factors.

The ICER process is not purely formulaic. It includes public input and committee votes on value and strength of evidence. The additional factors the committee considers vary, by treatment and context. They might include, for example, whether treatment outcomes reduce disparities across patient groups, whether treatments facilitate greater productivity through, for instance, more rapid return to work, or whether there are any treatment alternatives, among others. The inclusion of additional factors in the process is important, as a pure cost-effectiveness evaluation can leave important considerations out of the process.

Two aspects of ICER process warrant emphasis because they are approached by other organizations differently, or not at all. First, the effect of additional factors is approached qualitatively. They are not included directly into a formula, nor quantitatively combined with other notions of value. Second, an "ICER price" is not only a cost-effective one, but one that would bring the total cost of its use in line with total economic growth (GDP+1%).

Austin B. Frakt, PhD

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/](http://www.smdm.org/)

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

Tornado Diagrams
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.

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
Suggestions about Course Changes for Next Year?

• Location
• Time
• Notes and handouts
• Reading
• Homework
• TreeAge software
• Critical appraisals
• Quizzes
• Other