Outline

• What do we call a cost-effectiveness expert?
• Carry over of epidemiological and statistical concepts into economic evaluation
• Study designs
• Modeling
• Examples
10 Things We Might Call An Economist Specializing in Health-Related Cost-Effectiveness?

- Utility Player
- Profitable
- Health Economist
- Not a real economist
- Consultant
- Applied Economist
- Quality of Life Researcher
- Accountant
- Busy
1 Interpretation of the #1 Thing to Call an Economist Specializing in Health-related Cost-Effectiveness
Epidemiological, Statistical, and Economic Concepts

- Reliability
- Validity
  - Internal
  - External
- Bias
- Precision
Reliability

- Generally has to do with reproducibility
- Reliability of economic measures is no different an issue than reliability of other measures
  - Key is that we now have not only one construct associated with our primary outcome but at least two constructs
    - Effectiveness and costs
Internal Validity

• Generally, is a study designed to appropriately measure a difference in outcome between treatment groups?
  – In cost-effectiveness
    • Is the study designed to appropriately measure a difference in costs?
    • Is the study designed to appropriately measure a difference in outcomes?
External Validity

• Can we generalize findings from a study?
  – Can costs be generalized?
  – Can effects be generalized?
Bias

• Bias in one measure can lead to incorrect inferences of something being different when it is not or something not being different when it is

• Bias in a combination of measures can have ambiguous effects on the cost-effectiveness ratio
Effects of Bias

• Assume that one intervention is more expensive and more effective
  – Bias toward larger difference in costs and smaller difference in effectiveness
    • Higher ratio
  – Bias toward smaller different in cost and larger difference in effectiveness
    • Smaller ratio
– Bias for costs and effects in same direction
  • Ambiguous effect on ratio
Precision

- More precise measure associated with a smaller confidence interval in general study
- More precise measures in cost-effectiveness
  - Effect on estimate of confidence interval of ratio is less clear
  - Concern about covariation as well as variation
Designs

• Cost-effectiveness alongside a clinical trial run to measure effectiveness
• Cost-effectiveness trial
• Epidemiological model
Cost-Effectiveness Alongside an Effectiveness RCT

- Often referred to as piggy-backing the economic evaluation
- Take advantage of fixed costs of developing and running an RCT
- Opportunity for excellent internal validity
- May lack external validity
  - Patients in RCT are probably atypical
Special Type of RCT

- Multi-center trials may have even more threats to external validity of economic evaluation
  - Varying local costs
  - Varying local styles of practice
  - Varying populations
  - Varying expectations for quality of life
  - Possibly even international variation
Multi-Center RCT and Cost-Effectiveness

• Framing a study becomes exceptionally important in this case
  – Are we interested in the variation among sites?
  – Are we interested in a specific type of practice?
  – Are we interested in a specific price structure?
Cost-Effectiveness Trial

• Efficacy of an intervention demonstrated previously
• Design a trial specifically to collect cost-effectiveness data
• May be able to use more valid, reliable, unbiased, and precise measures because resources are dedicated specifically to cost-effectiveness assessment
• Aim for higher external validity
Epidemiological Model

- Use past results or publicly available data
- Probabilities
- Prices
- Quantities
- Prevalence
- For screening-sensitivity and specificity
Combination

- May have a trial that covers several parts of a process we would like to model but then need to extend the model further
Depiction of a Model

• Model is often depicted as a “decision tree”
• Decision trees include choices, random events, sometimes repeated random events, and “payoffs”
  – Payoffs can be negative (costs or health decreases) or positive (effects or cost savings)
Choice Node

- More than one path that is chosen by a policy maker, a physician, or a patient
- Generally depicted by a square with “branches”
Chance Node

- More than one possible event
- Generally depicted by a circle with "branches"

Flu? -> [Yes] -> 

Flu? -> [No] -> 

Flu?
End Node

- Last item represented in a sequence of events
- Does not necessarily have to be the “final event in a person’s lifetime”
- Generally represented as a triangle and describe payoffs

Flu? 

- Yes 
- No
“Advanced” Chance Node

• Build a “Markov model”
• Repeated sequence of events
• Often represented similarly to a chance node, but with an “M” inside
• Example
  – Cancer free person at risk for progressing to cancer each year
    • Can remain cancer free
    • Can have an incident case of cancer
    • May die
Data Analysis – Point Estimate

- Difference in mean costs
- Difference in mean effects
- Cost-effectiveness ratio
  \[ \frac{(C1-C2)}{(E1-E2)} \]
- Incremental Net Benefit
  \[ (B2-C2) - (B1-C1) \]
Data Analysis – Bootstrapping

• Use data collected
  – Draw a sample that is the size of the original study
    • Sample with replacement
  – Conduct analysis after random draws
  – Repeat and describe distribution of economic evaluation results
Data Analysis - Simulation

• Describe distributions to software
  – Draw parameters from distributions
  – Perform analyses
  – Repeat and describe distribution of economic evaluation results
Examples

- Surgery for dysfunctional uterine bleeding
- Intervention to increase breastfeeding duration among low income mothers
- Screening preschoolers for vision disorders
Surgery for Dysfunctional Uterine Bleeding

• Multi-center randomized clinical trial
• Originally designed as an effectiveness trial but not a cost-effectiveness trial
  – Randomization between two alternative surgeries
  – Performed at over two dozen centers
  – Measures actual resources used
• 24 month follow-up
  – Main “events” of interest were time to return to usual activities, need for follow-up treatment, and relief of symptoms
• Gather QOL and resource utilization data
• Assign costs
• Would like to model out to menopause or entire lifetime
  – Need epidemiological, QOL, and cost data from other sources
Increasing Breastfeeding

- Study designed primarily for cost-effectiveness
- Randomization at two hospitals in one city with one set of staff
- Community-based intervention
  - Had been shown to be effective in pilot work
  - Is pilot work externally valid over time?
  - Is pilot work externally valid for different personnel?
- Focus on infancy
- Gather resource utilization data
- Use publicly available data on costs
- Could model mothers’ and children’s lifetimes
Vision Screening in Preschoolers

• Build on study that conducted multiple screening activities and gold standard exams on the same set of children
  – Compare 4 screening devices and two types of personnel
• Use point estimates and standard errors of sensitivity from study
• Use distribution of times taken for screening from study
• Take specificity as fixed
• Use publicly available resource values
Vision Screening Model - I

Screen for Vision Problem

- Sure Sight used by Nurse
- Sure Sight used by Lay Screener
- Stereo Smile used by Nurse
- Stereo Smile used by Lay Screener
- Lea Visual Acuity Cards used by Nurse
- Lea Visual Acuity Cards used by Lay Screener
- Retinomax used by Nurse
- Retinomax used by Lay Screener
Vision Screening Model - II

Sure Sight used by Nurse

- Child has problem
  - Prevalence
    - True positive screen & referral
      - Sensitivity
        - Not receive follow-up care
      - 1-Sensitivity
        - Child identified & receives care later
    - False negative screen
      - 1-Specificity
        - Determine actually negative
      - False positive screen & referral
        - Not receive follow-up care
  - 1-Prevalence
    - True negative screen
      - Specificity

Vision Screening Model - III

• Is it appropriate to end at “confirm and receive follow-up care” for the true positives rather than trying to develop a full model over the course of a lifetime
  – Yes because intervention does not affect anything after treatment is initiated