Study Designs

Lecture 3

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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?

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 different in costs?
    - Is the study designed to appropriately measure a different 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

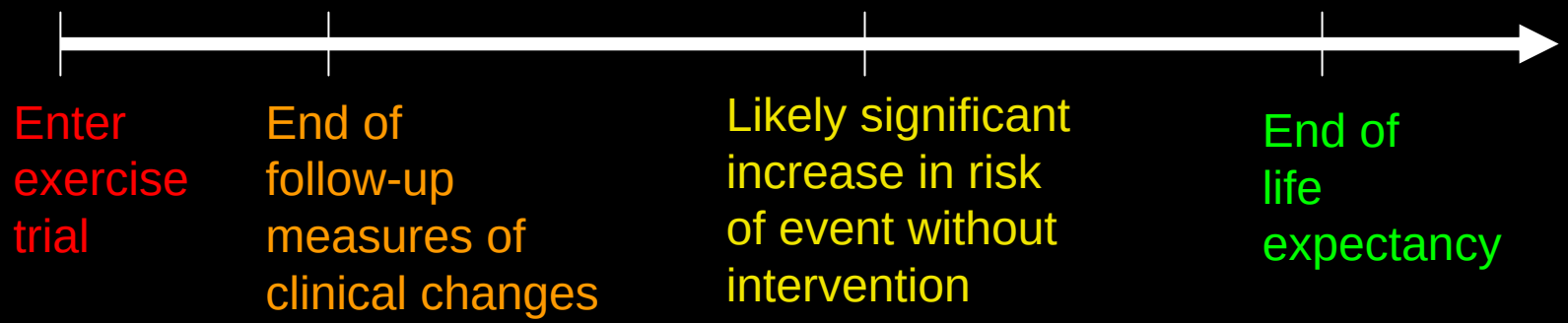
- Efficacy of an intervention demonstrated previously
- Design a trial specifically to collect cost-effectiveness data
- May be able to use more valid, reliable, unbiased, and precise measures because resources are dedicated specifically to cost-effectiveness assessment
- Aim for higher external validity

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

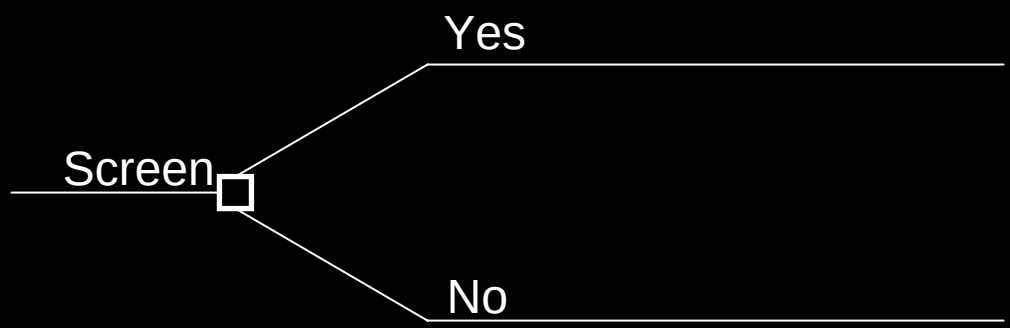


Depiction of a Model

- 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)

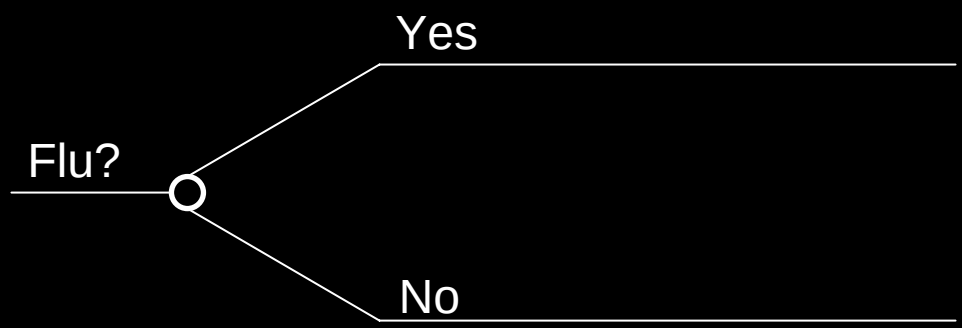
Choice Node

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



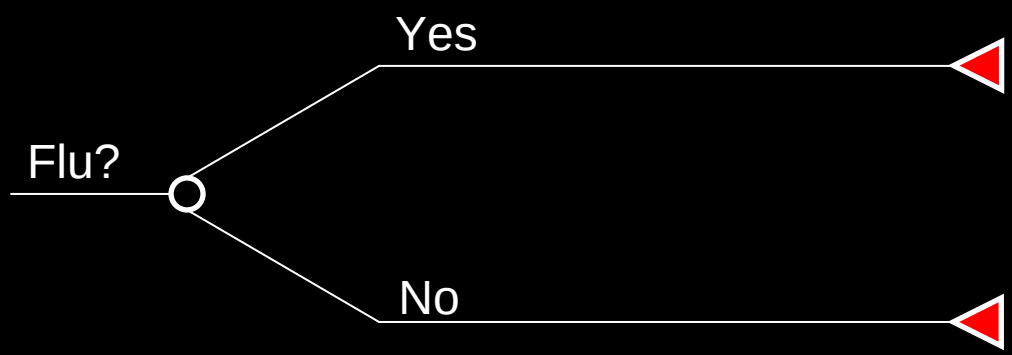
Chance Node

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



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



“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

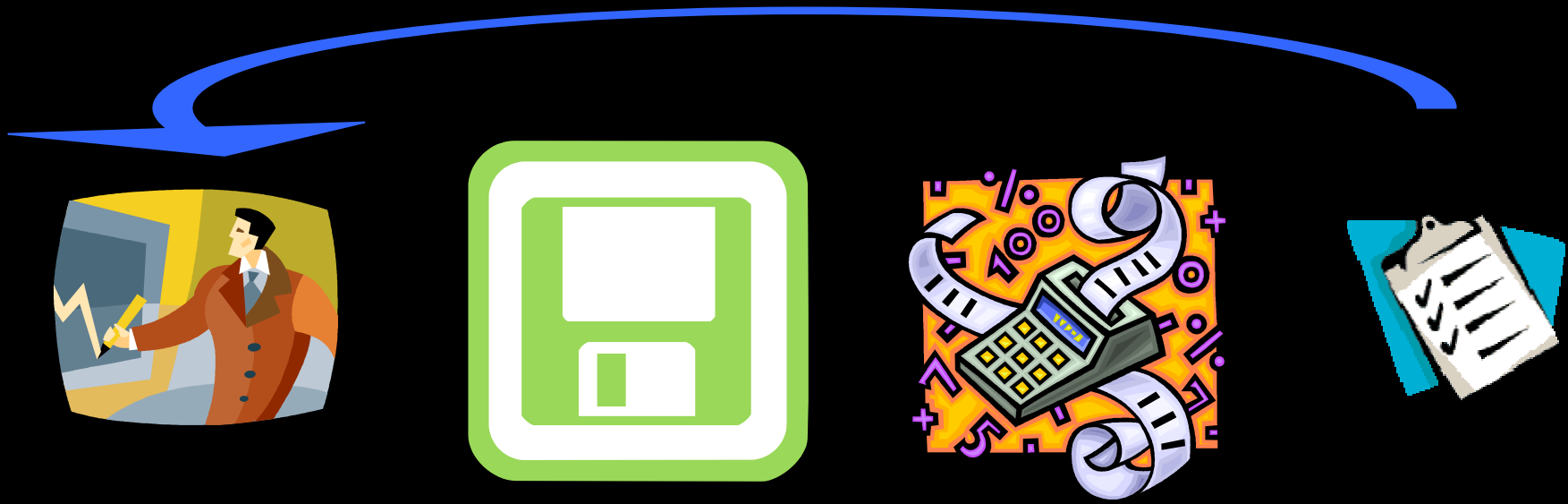
- Difference in mean costs
- Difference in mean effects
- Cost-effectiveness ratio
  - (C1-C2)/(E1-E2)
- Incremental Net Benefit
  - (B2-C2) – (B1-C1)

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

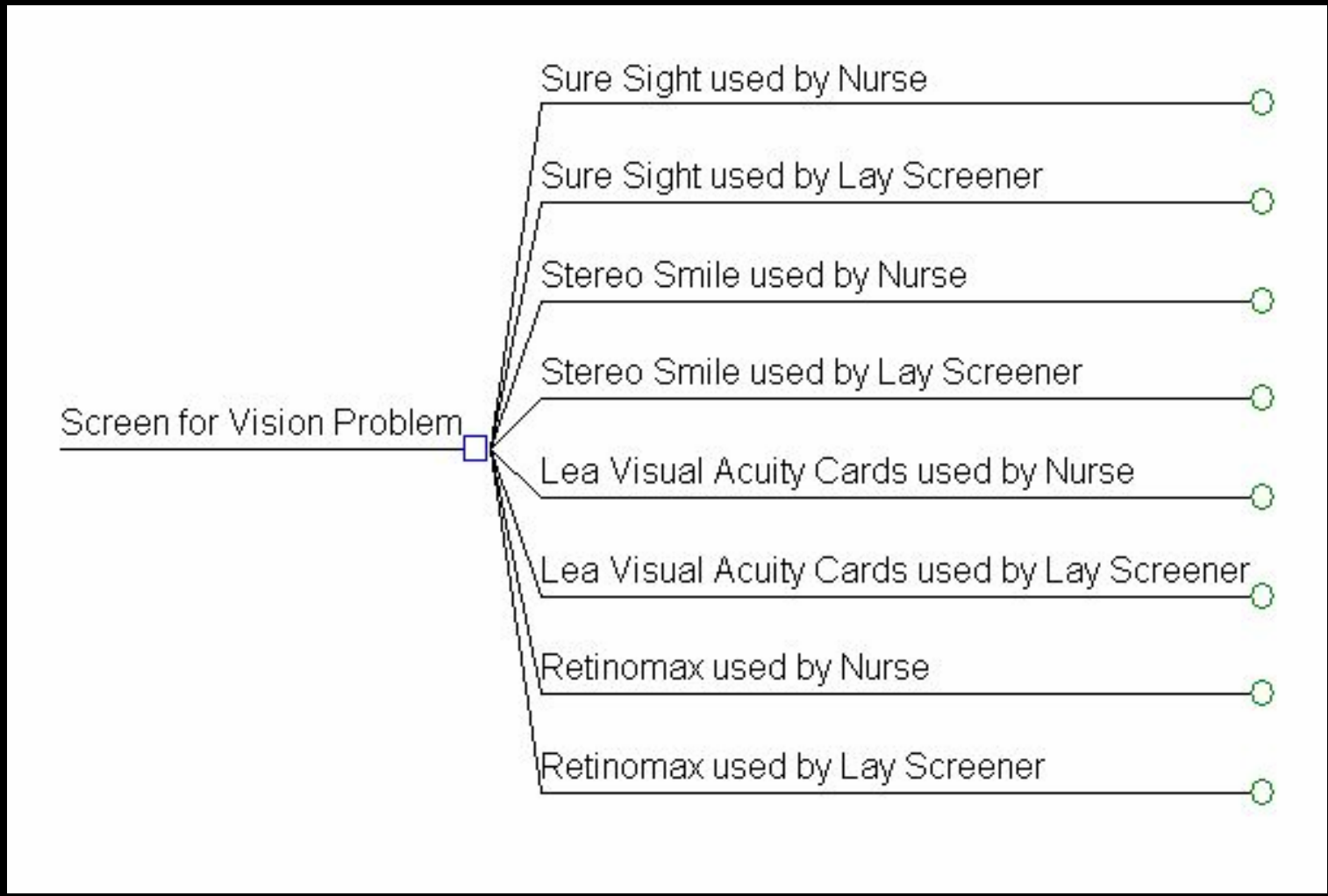
Increasing Breastfeeding

- 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

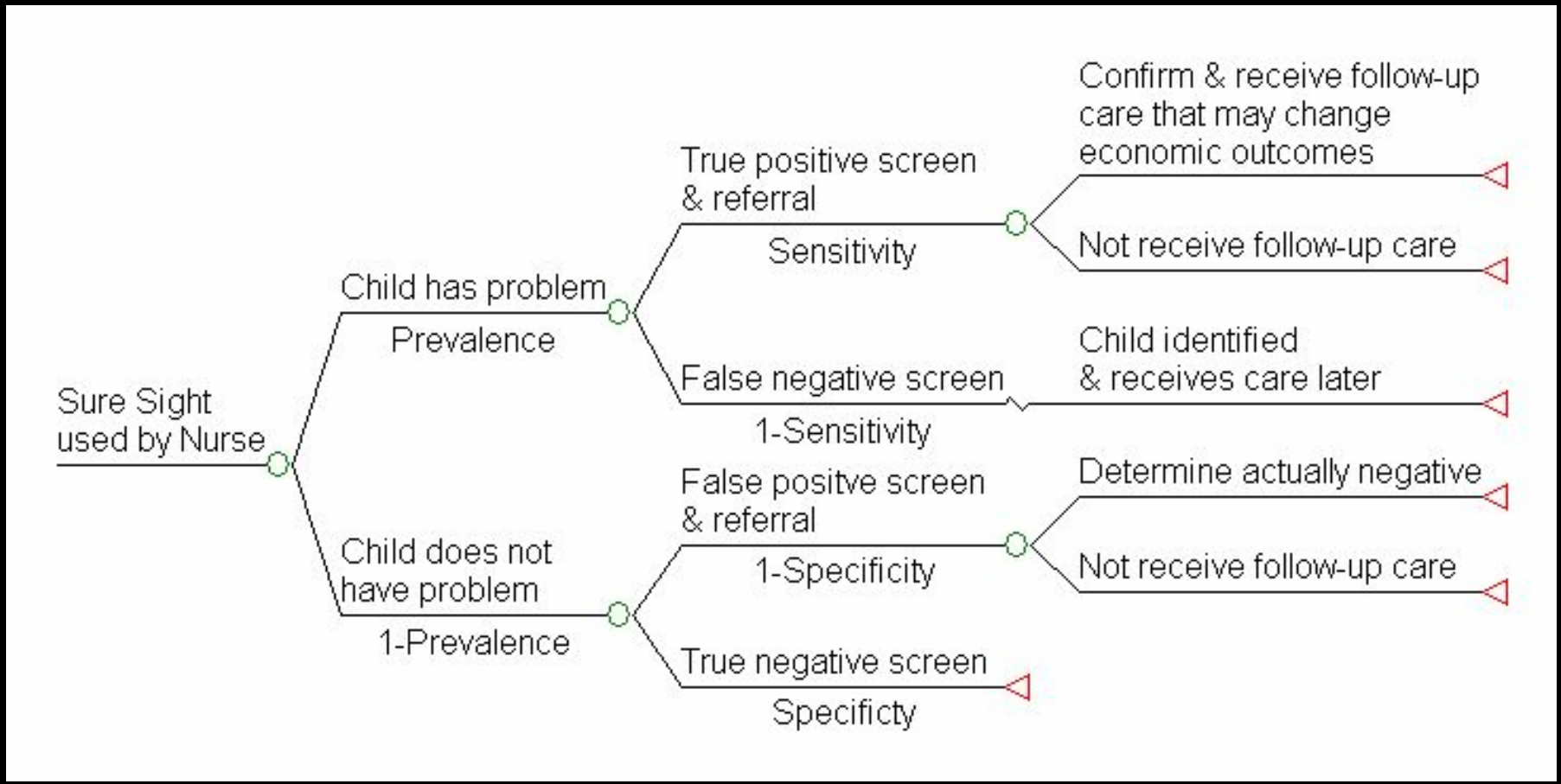
Vision Screening in Preschoolers

- 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



Vision Screening Model - II



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