Study Design Workshop

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Objectives of Session

- Describe different study designs and when to use them
- Compare different study designs in terms of timing/duration, sample size, cost, validity/bias, causation
- Identify resources (iTHRIVE) to determine study feasibility

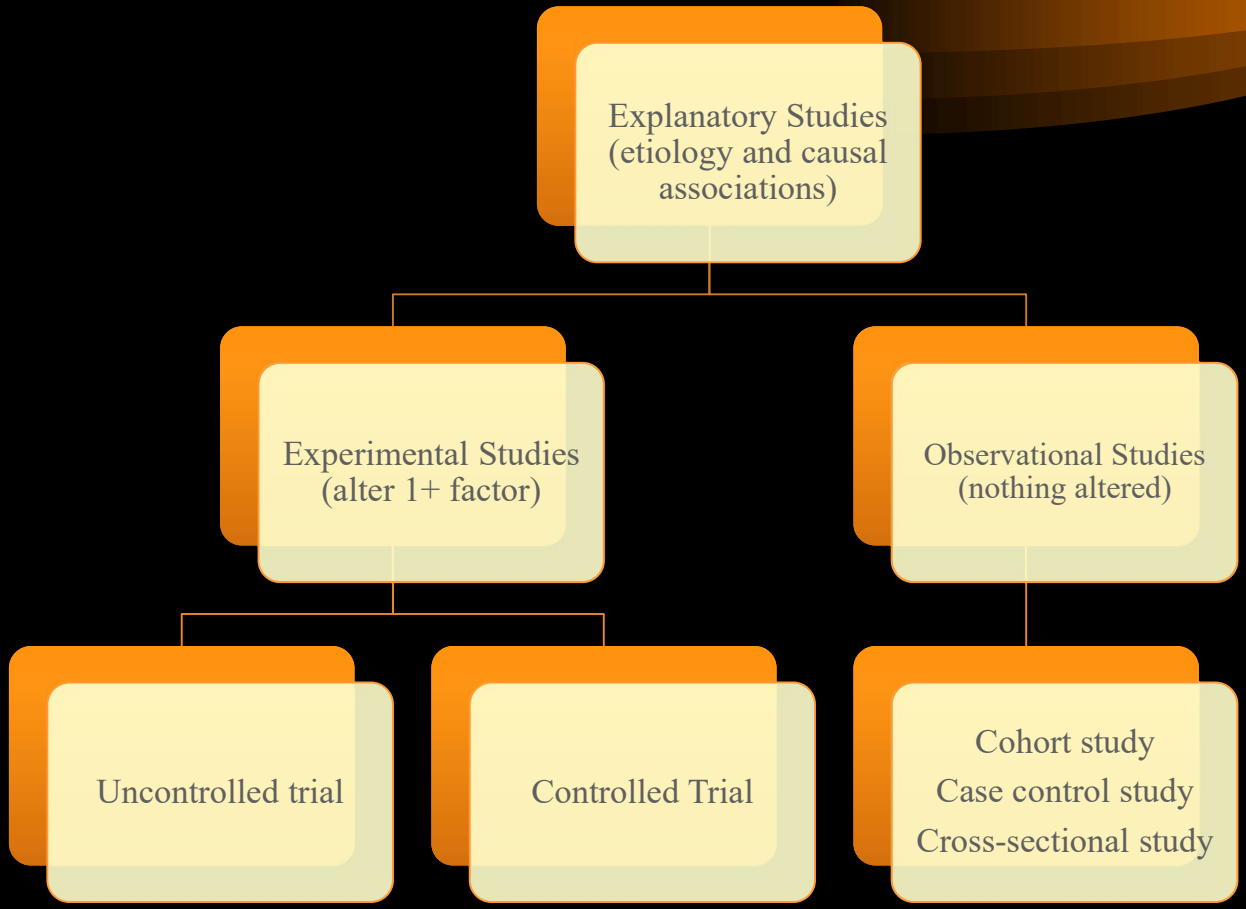
The Design

- There are several different kinds of study designs
- Choice of design will depend on the research question and nature of the problem to be studied, timeframe, budget, expertise, and other factors (e.g., availability of data)
- Often begin with a descriptive study design

Descriptive Studies

- Describe distribution of diseases, health related characteristics in a population
- Case report or series
- Common diagnoses seen in family practice
- Community survey of needs of the elderly

Explanatory Studies



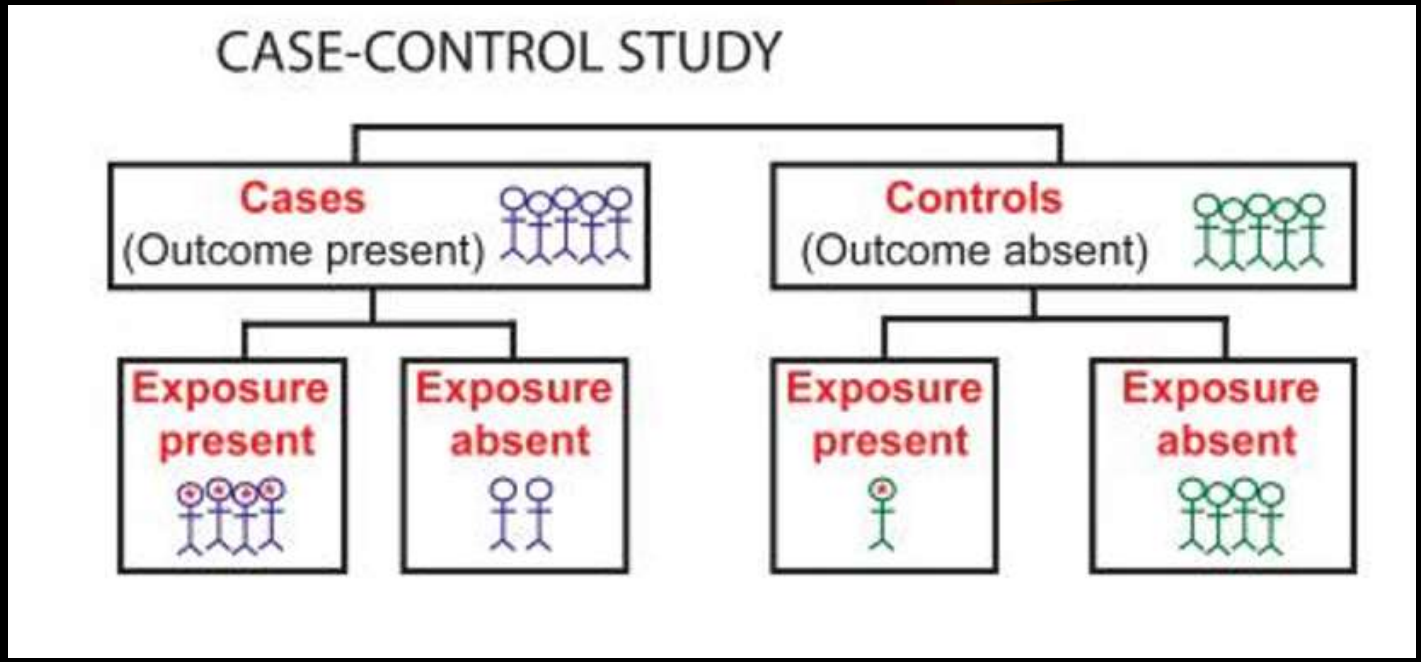
Explanatory Studies

- Experimental: evaluate the efficacy of therapeutic, educational or administrative interventions
- Investigator controls allocation
  - Clinical trial
  - Educational intervention

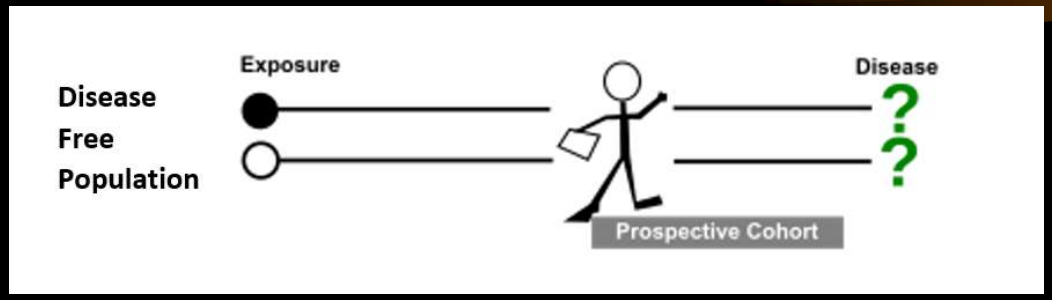
Explanatory Studies

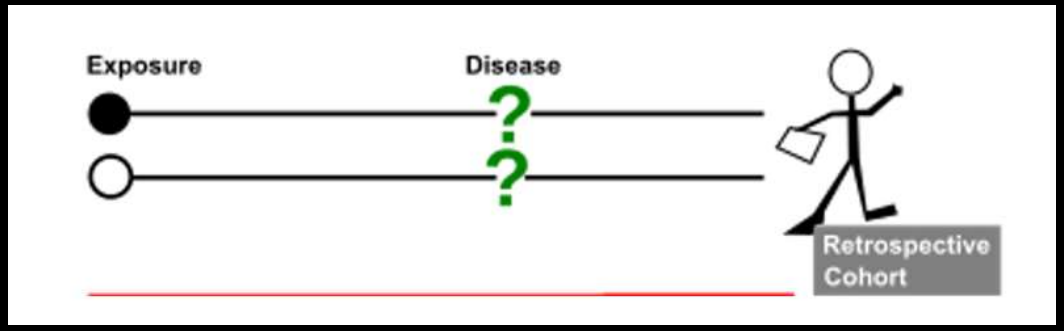
- Observational: seek causes, etiologies, predictors
- Investigator observes nature
  - Case-control
  - Follow-up (cohort)
  - Cross-sectional

Case-Control Study



Cohort Study





The Study Subjects

- Determine which patients/subjects are best suited to answer the research question
- How best to recruit enough subjects
- For example: “ Does taking estrogen after menopause lower the likelihood of developing coronary heart disease?”
  - age, gender criteria

The Variables

- Choose which variables should be measured
- Descriptive: look at individual variables
- Explanatory: measure associations between two or more variables
- One that comes first (presumed to come first) = predictor variable
- The other = outcome variable
- Most observational studies have many predictor and several outcome variables

The Variables

- There are also other variables that may be included, such as possible "confounding" variables or variables that you would want to control for
- For example, in our hormone study, what is the:
  - Predictor variable
  - Outcome variable
  - Possible confounding variables

Comparison of Study Designs

- Timing/duration
- Number of subjects needed (rare vs common diseases)
- Expense
- Bias
- Causation

Clinical Trial

- Timing/duration
- Number of subjects needed (rare vs common diseases)
- Expense
- Bias
- Causation

Clinical Trial

- Timing/duration – varies, can be longer
- Number of subjects needed (rare vs common diseases) – varies, can be large
- Expense – usually more costly
- Bias – least likely, if well designed
- Causation – yes, can determine
- Disadvantage – study one condition at a time, not representative of "real world" conditions

Cross-sectional/Prevalence

- Timing/duration
- Number of subjects needed (rare vs common diseases)
- Expense
- Bias
- Causation

Cross-sectional/Prevalence

- Timing/duration – relatively short
- Number of subjects needed (rare vs common diseases) – may be small, usually yields prevalence and first step for a cohort study
- Expense – usually least costly
- Bias – varies, depends on how you select your sample and who responds
- Causation – no, just associations
- Other – not feasible for rare predictors or outcomes

Cohort Study

- Timing/duration
- Number of subjects needed (rare vs common diseases)
- Expense
- Bias
- Causation

Cohort Study

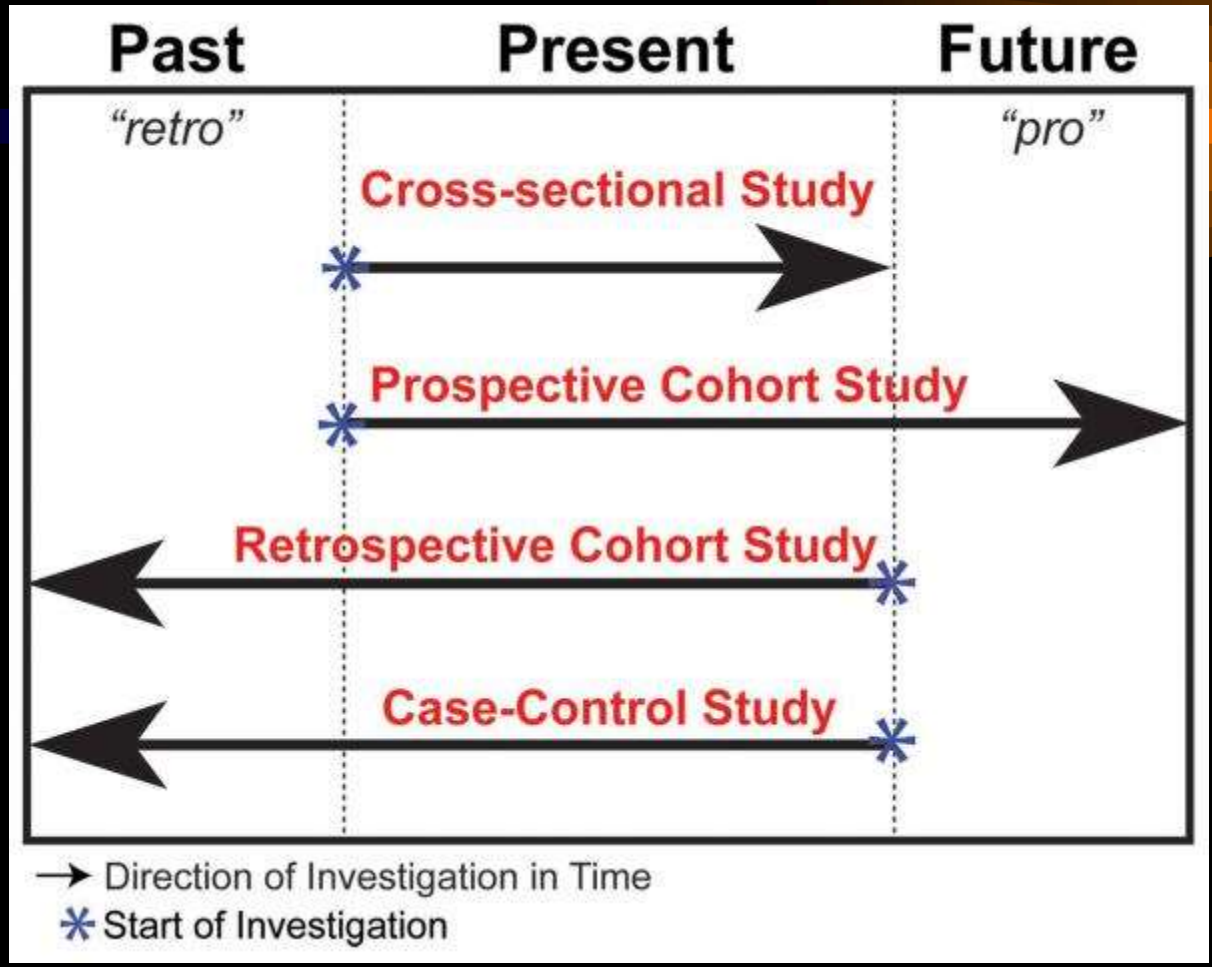
- Timing/duration – longer
- Number of subjects needed (rare vs common diseases) – large number of subjects, especially if rarer outcome; generally use when have outcomes that are relatively common
- Expense – can be very costly
- Bias – varies, depends on how you select your sample and who responds and stays in the study
- Causation – can infer since start with population without the disease of interest

Case Control Study

- Timing/duration
- Number of subjects needed (rare vs common diseases)
- Expense
- Bias
- Causation

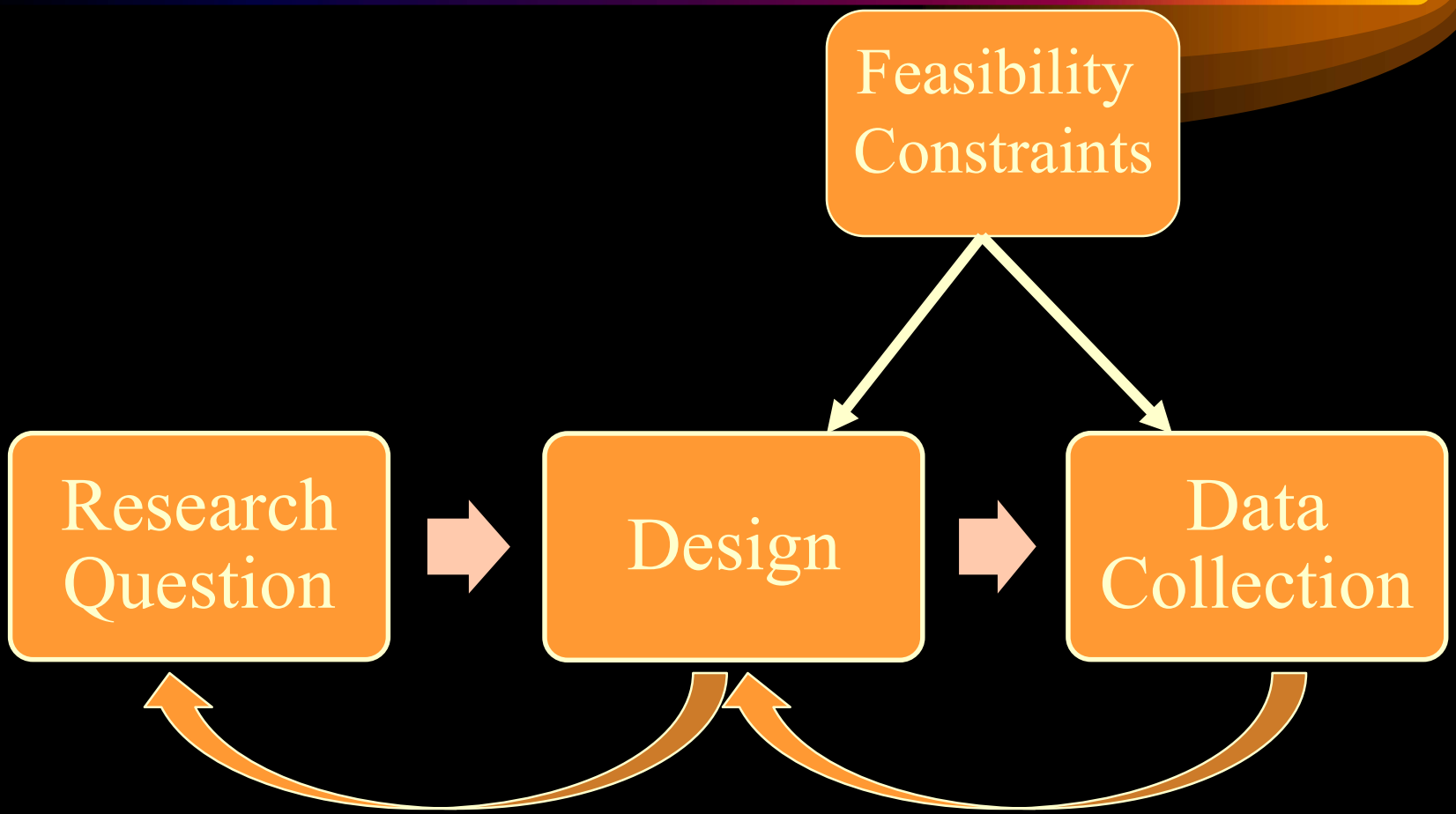
Case-Control Study

- Timing/duration – shorter (but could be longer if you are collecting the data prospectively)
- Number of subjects needed (rare vs common diseases) – relatively small, good when the disease is rare, can gain power by having more controls
- Expense – less costly
- Bias – varies, depends on how you select your cases and controls and the participation rates; greater potential for bias, such as recall bias
- Causation – inferred


Past
Present
Future
"retro"
"pro"
Cross-sectional Study
Prospective Cohort Study
Retrospective Cohort Study
Case-Control Study
Direction of Investigation in Time
Start of Investigation

Considerations

Considerations



Why is this important?

- Saving time and effort
- IRB application
- Grant proposals
- Other support for study

TriNetX

- **FEASIBILITY:** Determine if a sufficient patient population matches a protocol
- **DESIGN:** Analyze inclusion / exclusion criteria and the impact of protocol changes
- **COHORT EXPLORATION:** Investigate attributes and comorbidities of the eligible cohort
- **TRENDS:** View the rate of incidence of your query criteria over time in the specified population
- **RECRUITMENT:** Find eligible patients for an active clinical trial by submitting a re-identification request

iTHRIV - Resource/Event Details

Resources - Data Services and Resources - HSL at University of Virginia-Claude Moore Health Sciences Library

Thank you!

Questions or comments?