STUDY DESIGN

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Scientific Research

The systematic investigation, with an open mind, to establish novel facts, solve new or existing problems, prove new ideas, or develop new theories, using a scientific method.

Research

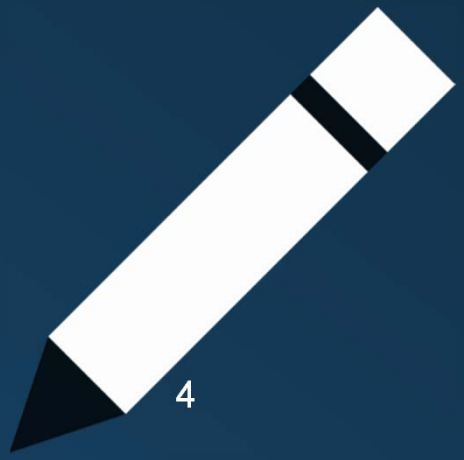
- Qualitative
  - aims to gather an in-depth understanding of human behavior and the reasons that govern such behavior
  - focused samples rather than large ones
  - often precedes quantitative research
- Quantitative
  - aims to test hypotheses with empirical measurement and statistical analysis

Scientific Method

Quantitative Research

Involves gathering empirical and measurable evidence

- systematic observation
- measurement
- formulation, testing, and modification of hypotheses



Research Question

Designing a study always starts with a question.

Research Questions

Quantitative Studies

- Identify key variables or concepts
- Identify potential relationships among the variables
- Identify population of interest

Research Question

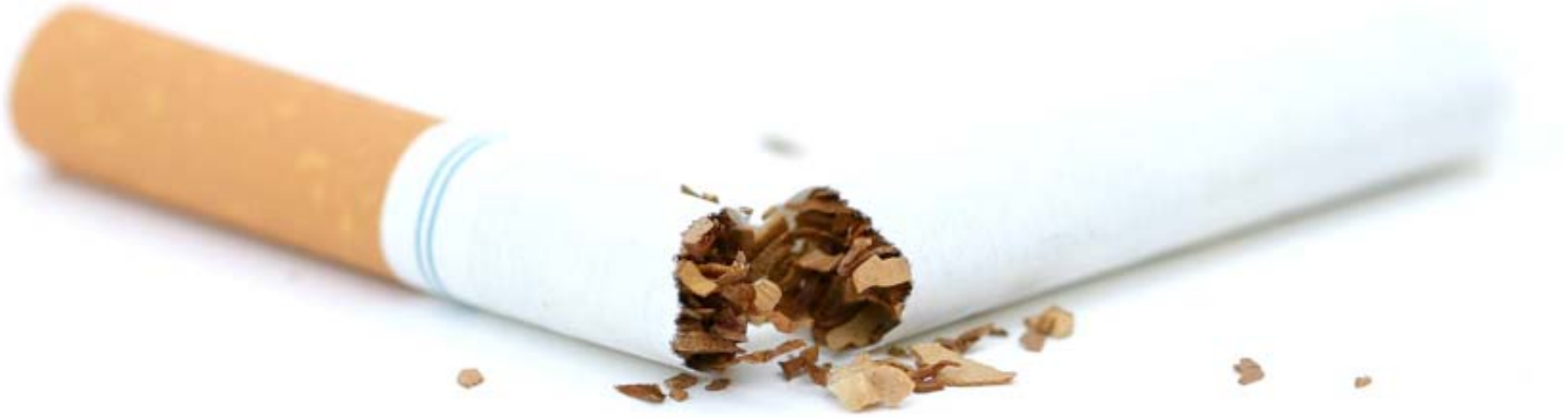
Can use of an improved chimney stove reduce the risk of chronic obstructive pulmonary disease (COPD) in adult women?

Can an improved stove reduce the risk of COPD in adult women?

- Key variables
  - Exposure (independent or predictor)
  - Outcome (dependent)
  - Potential confounders (covariates)

Can an improved stove reduce the risk of COPD in adult women?

- Identify potential interactions between variables
  - Effect modification
  - Confounding



Can an improved stove reduce the risk of COPD in adult women?

Identify population of interest

- Feasibility
- Generalizability

Can an improved stove reduce the risk of COPD in adult women?

Operational Definitions

- Clarifiy and define variables under investigation
  - Exposure
  - Outcome

- Specifies how variable will be observed and measured

Can an improved stove reduce the risk of COPD in adult women?

- Identify population of interest
- What study design to use?

Study Designs

- Cohort Studies
  - Prospective or retrospective
    - Experimental
- Cross-sectional Studies
- Case-control studies

Study Design

- Key Questions:
  - What is the research question?
  - Will there be an intervention?
  - What types of comparisons will be made?
  - What is the time frame of the study?
  - How many times will data be collected?

What is the Research Question?

Study Design	Descriptive	Analytic
Cohort	Incidence	Causation
Cross-sectional	Prevalence, associations	No causation
Case-control		Identify predictors
Experimental	Treatment effect	Causation

Cohort Study Design

- The word cohort was the ancient Roman term for a group of soldiers that marched together into battle.

- In epidemiological research, a cohort means a group of subjects followed together over time.

Why Conduct a Cohort Study?

Descriptive

- To describe the incidence of certain outcomes over time.
- Incidence is the number of NEW diseases/outcomes during a specified time period.

Analytic

- Analyze associations between risk factors and those outcomes.
- Includes independent and dependent variables.

Prospective Cohort Study

- Compares groups of people on risk of getting a disease
- Independent variables measured before outcome is measured.
- Compares groups within the sample on characteristics measured as independent (predictor) variables.
- Comparison are made between groups, based on outcome, within one cohort.

Prospective Cohort Study

Strengths

- Can determine incidence of disease.
- Can identify potential causes of disease (time sequence).
- More accurate data

Weaknesses

- Expensive
- Need large samples.
- Inefficient, if study is of rare disease
- Loss of subjects on follow-up

Cross-sectional Study Design

- All measurements for each subject are conducted at the same time.
- No follow-up period
- Describes variables and their distribution within a sample.

Cross-sectional Study: Associations

- There is an association between solid fuel cook stove use and asthma.
  - An association does not distinguish predictors from outcome measures:
- Women who cook with solid fuels become asthmatic. OR
- Asthmatic women are more likely to cook with solid fuels.

Cross-sectional Studies

Advantages

- Short duration, inexpensive, no problem with loss to follow-up
- Can study several outcomes
- Control subject selection and measures
- Good first step for a cohort or experimental study
- Yields prevalence (no. of people with disease at one point in time)

Cross-sectional Studies

Disadvantages

- Does not establish sequence of events
- Potential bias in measuring predictors
- Potential survivor bias (no information about patients that died)
- Not feasible for rare conditions (need large sample size)

Case-control Study Design

- Studies of the causes of rare diseases/conditions
- Retrospective study design
- Cases - people with the disease
- Controls - people without the disease (matched w/ cases)

Steps: Case-Control Studies

- Develop a research question
- Select a sample from a population of people with the disease (cases)
- Select a sample from a population at risk without the disease (controls)
- Measure predictor variables

Case-Control Studies: Strengths and Weaknesses

Strengths

- Inexpensive
- Small sample size
- Short duration
- Study rare diseases

Weaknesses

- One outcome
- No causal relations
- Bias
  - Sample two groups
  - Measurement
  - Survivor bias

Bias in Case-control Studies

- Match cases and controls (age, sex)
- Sampling bias - Is the sample representative?
  - Random sample of all people with the disease
  - Disease group does not include undiagnosed, misdiagnosed, dead

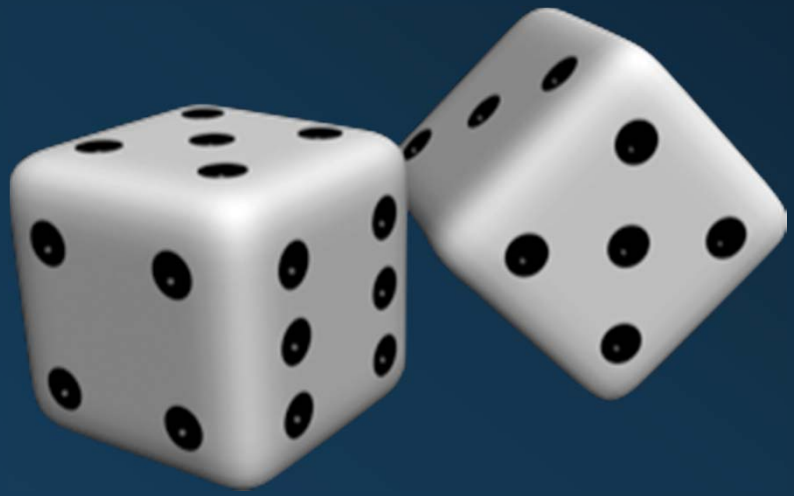
Selecting Controls

Find an accessible population with the following characteristics:

- At risk of developing the disease
- Represent same population
- Match cases on age, sex, ethnicity

Experimental Study Design

- Cohort study
- Investigator controls predictor variable (intervention)
- Controls influence of confounding variables



Experimental Study Design

- Basic descriptive characteristics are known based on previous studies
- Answers different questions
  - Why (etiology)?
  - How prevent or treat?
- Strength in showing causation

Experimental Study Characteristics

- Manipulation
  - Experimenter does something to at least some of the subjects in the study
- Control
  - Sample includes a control group
- Randomization
  - Experimenter assigns subjects to a control or experimental group on a random basis
- Note: Quasi-experimental studies LACK randomization or a control group

Designing a Randomized Controlled Trial (RCT)

1. Select sample from population
2. Measure baseline variables
3. Randomly assign subjects to intervention and control groups
4. Apply interventions
5. Follow-up the cohorts
6. Measure outcome variables (blindly)

Random Assignment to Treatment

- What is random assignment?
  - One group receives a treatment and the other group receives NO treatment and serves as a control group.
- Why randomize subjects to different groups?
  - To test the effect of a treatment by comparing the treatment group with the control group.

Baseline Characteristics

Why is it important for the two groups to be similar?

- If age/gender is related to the etiology of the disease, then the group comparison is affected by age/gender and not only the treatment.
- The two groups should be similar on baseline characteristics, such as age, gender, and ethnicity.

Random Allocation Procedures

Purpose: No intentional or unintentional biases can influence the allocation process.

- Random numbers table
- Coin flip
- Establish criteria for group assignment
  - ID#s (even vs. odd)
  - Day consent form is signed

Experimental Design

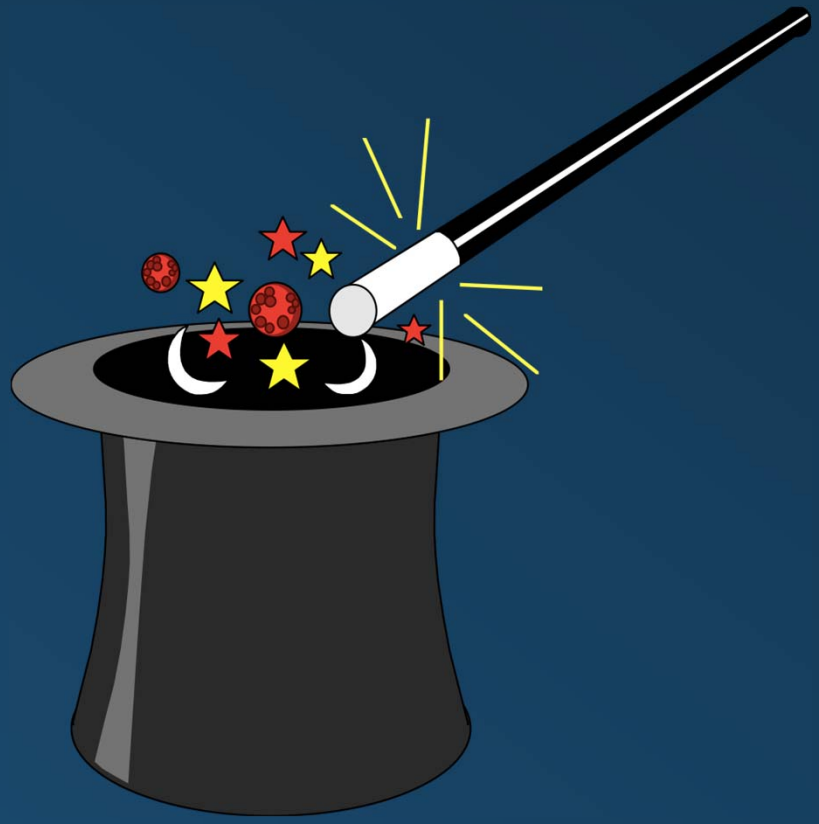
Advantages

- Produce strongest evidence for cause and effect
- Only design from some questions
- Sometimes cheaper and faster than observational studies

Disadvantages

- Costly in time and money
- Narrow research questions
- Standard interventions increase generalizability

Limitations: Experimental Studies



- Ethical problems
  - Clinical trials that show effect in early stages of a study
- Hawthorne Effect
  - Change occurs regardless of intervention
  - Placebo effect

RCT: Gold Standard of Epi Design: Site Selection Criteria for RESPIRE

1. High child morality
2. High rate of pneumonia
3. High pollution exposure from cooking fuel
4. Experienced local partners in conducting field research
5. Available intervention that is well used.
6. Intervention reduces exposure in field conditions.

- With WHO funding, we searched nine sites in three continents to choose highland Guatemala that met #s 1-5
- Then spent 8 years doing pilots studies to show #6
- Unfortunately, focused that work mostly on IAP, not actual exposure

Gold Standard, perhaps, but 24-carat or Fool's?

- Only way to show strict causality, but
- Is causality really in question anymore?
- But rather effectiveness and exposure-response?
- We do not doubt causality for a wide-range of pollutants, even with no RCTs (e.g., outdoor air, SHS, ATS, lead, arsenic, etc.)
- For policy, however, we need Exp-Resp – how much benefit at how much reduction
- Solutions are highly varied and change with time and place, but an RCT deals with one solution in one time in, usually, one small population – does not translate.
- Exposure translates – there is a reason that it is the central metric of environmental health

Gaps in HAP Endpoints

- Cardiovascular disease – biggest gap, because biggest burden
- Intervention trials for cleanest options: gas
- Large-scale cluster randomized effectiveness trials needed to convince health funders: adverse birth/neonatal outcomes
- Cognitive effects in children, potentially with interventions
- Birth defects: cleft, etc.
- Other cancers, upper airway, cervical, leukemia, etc.
- TB: data still unclear
- Non-air-pollution impacts: burns/scalds, hygiene, and women's time
- Interactions with other household risk factors: combined interventions with water/sanitation, concrete floors, etc.
- There are now 3 major RCTs for child pneumonia: one finished, one underway, and one starting, which cover three continents. Not highest priority to start another at present.

Strategic Epidemiology

- Main purpose is to inform policy
- Focus on known diseases if possible; less on signs, symptoms, and biomarkers, except as support
- Focus on diseases with major burdens
- Consider also charismatic diseases – those with special public/policy impact
- Fill in the gaps in knowledge, i.e. exposure levels, age groups, disease endpoints
- Provide translation across populations
- Study interventions where at all possible
- Scalable interventions are even better

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