Use + Share + Adapt

{ Content the copyright holder, author, or law permits you to use, share and adapt. }

- **Public Domain – Government**: Works that are produced by the U.S. Government. (17 USC § 105)
- **Public Domain – Expired**: Works that are no longer protected due to an expired copyright term.
- **Public Domain – Self Dedicated**: Works that a copyright holder has dedicated to the public domain.
- **Creative Commons – Zero Waiver**: Creative Commons – Attribution License
- **Creative Commons – Attribution License**: Creative Commons – Attribution Share Alike License
- **Creative Commons – Attribution Noncommercial License**: Creative Commons – Attribution Noncommercial Share Alike License
- **GNU – Free Documentation License**: GNU – Free Documentation License

Make Your Own Assessment

{ Content Open.Michigan believes can be used, shared, and adapted because it is ineligible for copyright. }

- **Public Domain – Ineligible**: Works that are ineligible for copyright protection in the U.S. (17 USC § 102(b)) *laws in your jurisdiction may differ

{ Content Open.Michigan has used under a Fair Use determination. }

- **Fair Use**: Use of works that is determined to be Fair consistent with the U.S. Copyright Act. (17 USC § 107) *laws in your jurisdiction may differ

Our determination **DOES NOT** mean that all uses of this 3rd-party content are Fair Uses and we **DO NOT** guarantee that your use of the content is Fair.

To use this content you should **do your own independent analysis** to determine whether or not your use will be Fair.
Research Design

Emily Sagalyn, MD, MPH, FAWM
University of Utah
Health research is the process of systematically investigating a single well-defined aspect of physical, mental, or social well-being.
Examples of Health Research

- Is an 8-week physical therapy program effective at reducing the risk of anterior cruciate ligament tears in high school athletes?
- What are the most common signs and symptoms associated with multiple sclerosis?
- How much does the risk of severe hearing loss increase with age?
- Which factors predict binge drinking behavior in college and university students?
Examples of Research Goals

- Identifying and classifying new health problems
- Determining risk factors for disease
- Developing and testing new interventions for preventing or treating illness
- Evaluating the impact of health policies on health outcomes
- Synthesizing existing knowledge so that it can be applied by others
Research Process

1. Identify study question
2. Select study approach
3. Design study & collect data
4. Analyze data
5. Report findings
<table>
<thead>
<tr>
<th>Types of Study Designs</th>
</tr>
</thead>
<tbody>
<tr>
<td>Reviews &amp; meta-analyses</td>
</tr>
<tr>
<td>Correlational (ecological)</td>
</tr>
<tr>
<td>Case series</td>
</tr>
<tr>
<td>Cross-sectional</td>
</tr>
<tr>
<td>Case control</td>
</tr>
<tr>
<td>Cohort</td>
</tr>
<tr>
<td>Experimental &amp; quasi-experimental</td>
</tr>
<tr>
<td>Qualitative</td>
</tr>
</tbody>
</table>
Epidemiology Terms

- **Prevalence**: Amount of a disease in a population at a point in time. Unit-less data.
  - Example: Prevalence of HIV in Ghana is X%.

- **Incidence**: Number of new cases in a defined period of time.
  - Example: Incidence of HTN in 1999 was 150 cases per 100,000 persons (not actual data).

- **Rate Ratio**: Rate in the exposed/rate in unexposed
  - Calculated in cohort studies

- **Odds Ratio**: Approximates the rate ratio
  - Calculated in case-control studies

- **95% Confidence Interval**: There is a 95% change that the true value measured falls within the boundaries.
  - Can not include 1.0. If it does it is not significant

- **P value**: measure the statistical significance of a study is less than 0.05
# Goals of types of studies

<table>
<thead>
<tr>
<th>Study Approach</th>
<th>Goal</th>
</tr>
</thead>
<tbody>
<tr>
<td>Review/meta-analysis</td>
<td>Synthesize existing knowledge</td>
</tr>
<tr>
<td>Correlational (ecological)</td>
<td>Compare average levels of exposure and disease in several populations</td>
</tr>
<tr>
<td>Case series</td>
<td>Describe a group of individuals with a disease</td>
</tr>
<tr>
<td>Cross-sectional survey</td>
<td>Describe exposure and/or disease status in a population</td>
</tr>
<tr>
<td>Case-control study</td>
<td>Compare exposure histories in people with disease (cases) and people without diseases (controls)</td>
</tr>
<tr>
<td>Cohort study</td>
<td>Compare rates of new (incident) disease in people with different exposure histories or follow a population forward in time to look for incident diseases</td>
</tr>
<tr>
<td>Experimental study</td>
<td>Compare outcomes in participants assigned to an intervention or control group</td>
</tr>
<tr>
<td>Qualitative study</td>
<td>Seek to understand how individuals and communities perceive and make sense of the world and their experiences</td>
</tr>
</tbody>
</table>
Carefully gathers all prior publications on a specific topic and summarizes them to provide a big-picture analysis.

- Systematic reviews attempt to locate ALL the articles on a well-defined topic
  - Key words
  - Inclusion criteria

- Meta-analysis combines the results of several high-quality articles that used similar methods to collect and analyze data into one summary statistic.
Analysis

- Systematic Review – summary table of studies and findings
- Meta-analysis

http://www.healthstrategy.com/meta/meta/.pl

http://www.healthstrategy.com/meta/meta-analysis.jpg
Study Design: Ecological

- AKA: cross-sectional, correlational, aggregate
- These studies use **population-level data** to examine the relationship between exposure rates and disease rates
  - Associations
  - Does the percentage of adults with multiple sclerosis tend to be higher in countries farther from the equator?
Analysis

Correlation, linear regression, rate adjustment

**FIGURE 8-3  Correlation**

<table>
<thead>
<tr>
<th>Positive slope</th>
<th>Negative slope</th>
</tr>
</thead>
<tbody>
<tr>
<td>$r^2 = 0.92$</td>
<td>$r^2 = 0.29$</td>
</tr>
<tr>
<td>$r = 0.96$</td>
<td>$r = -0.54$</td>
</tr>
</tbody>
</table>

$\quad$  

$\quad$  

$\quad$  

Source Undetermined
<table>
<thead>
<tr>
<th>Objective</th>
<th>Compare average levels of exposure and disease in several populations</th>
</tr>
</thead>
<tbody>
<tr>
<td>Primary study question</td>
<td>Do populations with a higher rate of exposure have a higher rate of disease?</td>
</tr>
<tr>
<td>Population</td>
<td>Existing population-level data are used; there are no individual participants.</td>
</tr>
<tr>
<td>When to use this approach</td>
<td>The aim is to explore possible associations between an exposure and a disease using population-level data.</td>
</tr>
<tr>
<td>Requirement</td>
<td>The topic has not been previously explored using individual-level data.</td>
</tr>
</tbody>
</table>
| First steps                       | 1. Select the sources of data that will be used.  
                                        2. Decide on the variables to include in the analysis. |
| What to watch out for             | The ecological fallacy  
                                        Limited publication venues |
| Key statistical measure           | Correlation |
Study Design: Case Studies

- Case report – 1 patient
- Cases series – 2 or more patients with the same disease or the same procedure
  - Describe characteristics or similarities
  - Identify new syndromes; refine case definitions
  - Clarify typical disease progression
  - Develop hypotheses for future research
  - THINK: person, place, time
Analysis

- Descriptive statistics
  - Numeric summary (mean, median, mode)
  - Count or frequency
<table>
<thead>
<tr>
<th><strong>Objective</strong></th>
<th>Describe a group of individuals with a disease</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Primary study question</strong></td>
<td>What are the key characteristics of the cases in this study population?</td>
</tr>
<tr>
<td><strong>Population</strong></td>
<td>All individuals in the study must have the same disease or be undergoing the same procedure.</td>
</tr>
<tr>
<td><strong>When to use this approach</strong></td>
<td>A source of cases is available, and no comparison group is required or available.</td>
</tr>
<tr>
<td><strong>Requirement</strong></td>
<td>An appropriate source of cases is available.</td>
</tr>
</tbody>
</table>
| **First steps** | 1. Specify what new and important information the analysis will provide.  
                              2. Identify a source of cases.  
                              3. Assign a case definition.  
                              4. Decide on the characteristics of the study population that will be described. |
| **What to watch out for** | A lack of generalizability |
| **Key statistical measure** | Only descriptive statistics are required. |
Study Design: Prevalence

- AKA cross-sectional
- Measures the proportion of a population with a particular exposure or disease at one point in time, based on a representative sample
  - Survey or questionnaire
Analysis

- Prevalence
- Prevalence ratios (association)
<table>
<thead>
<tr>
<th>Objective</th>
<th>Describe the exposure and/or disease status in a population</th>
</tr>
</thead>
<tbody>
<tr>
<td>Primary study question</td>
<td>What is the prevalence of the exposure and/or disease in the population?</td>
</tr>
<tr>
<td>Population</td>
<td>The study participants must be representative of the population from which they were drawn.</td>
</tr>
<tr>
<td>When to use this approach</td>
<td>Time is limited and/or the budget is small.</td>
</tr>
<tr>
<td>Requirement</td>
<td>The exposures and outcomes are relatively common, and the likelihood of being able to recruit several hundred participants is strong.</td>
</tr>
</tbody>
</table>
| First steps | 1. Define a source population.  
               2. Develop a strategy for recruiting a representative sample.  
               3. Decide on the methods to be used for data collection. |
| What to watch out for | Nonrepresentativeness of the study population |
| Key statistical measure | Prevalence |
Study Design: Case-Control

1. What is the individual’s current disease status?
   - Disease (case)
   - No disease (control)

2. Did the individual have the exposure at some point in the past?
   - Exposure
   - No exposure

Efficient for rare diseases
Analysis

- Odds, odds ratio, 95% confidence interval
- Matched odds ratio (individual matching)
- Sensitivity analysis to assess potential effect of bias
  - Misclassification
  - Recall
| **Objective** | Compare exposure histories in people with disease (cases) and people without diseases (controls) |
| **Primary study question** | Do cases and controls have different exposure histories? |
| **Population** | Cases and controls must be similar except for their disease status. |
| **When to use this approach** | The disease is relatively uncommon, but a source of cases is available. |
| **Requirement** | A source of cases is available. |
| **First steps** | 1. Identify a source of cases.  
   2. Assign a case definition.  
   3. Decide what type of control population will be appropriate for the study.  
   4. Decide whether cases and controls will be matched. |
| **What to watch out for** | Recall bias |
| **Key statistical measure** | Odds ratio (OR) |
Study Design: Cohort

- Follows participants through time to calculate the rate of incident disease & identify risk factors
  - Baseline measurement
  - Follow-up measurement(s)
Thinking about time

A longitudinal cohort recruits participants based on their membership in a well-defined source population (prospective studies don’t necessarily rely on source populations)
Analysis

- Incidence rate
- Attributable risk – absolute difference in incidence rate between exposed & unexposed
- Attributable risk % (attributable fraction among the exposed)
Analysis, cont.

- Rate ratio & 95% confidence interval
  - AKA: relative rate, risk ratio, relative risk
<table>
<thead>
<tr>
<th>Approach</th>
<th>Prospective or Retrospective Cohort</th>
<th>Longitudinal Cohort</th>
</tr>
</thead>
<tbody>
<tr>
<td>Objective</td>
<td>Compare rates of new (incident) disease in people with different exposure histories.</td>
<td>Follow a population forward in time to look for new (incident) diseases.</td>
</tr>
<tr>
<td>Primary study question</td>
<td>Is exposure associated with an increased incidence of disease?</td>
<td>Is exposure associated with an increased incidence of disease?</td>
</tr>
<tr>
<td>Population</td>
<td>Participants must be similar except for exposure status.</td>
<td>Participants must be available for follow-up months or years after enrollment.</td>
</tr>
<tr>
<td></td>
<td>Because the goal is to look for incident disease, no one can have the disease of interest at the start of the study.</td>
<td>The study participants must be reasonably representative of the population from which they were drawn.</td>
</tr>
<tr>
<td>When to use this approach</td>
<td>An exposure is relatively uncommon, but a source of exposed individuals is available.</td>
<td>The goal is to examine multiple exposures and multiple outcomes, and time is not a concern.</td>
</tr>
<tr>
<td>Requirements</td>
<td>A source of individuals with the exposure is available.</td>
<td>There is adequate time and money for the study.</td>
</tr>
</tbody>
</table>
| First steps | 1. Identify a source of individuals with the exposure.  
2. Decide what type of unexposed individuals will be an appropriate comparison group. | 1. Select a source population.  
2. Select the exposures and outcomes that will be assessed.  
3. Decide how often data will be collected.  
4. Develop a strategy for minimizing the burden of participation and maximizing benefits and incentives. |
| What to watch out for | Participant drop-outs (prospective studies) or missing records (retrospective studies) | Participant drop-outs |
| | Information bias, in which the exposed participants are more thoroughly examined for disease than unexposed participants | Potential data management challenges if lots of information is collected at many points in time. |
| Key statistical measure | Relative risk (rate ratio, RR) | Relative risk (rate ratio, RR) |
Study Design: Qualitative Methods

- Look for themes and meanings emerging from observation on a small, selected group of informants
  - Phenomenology – how do pt understand/interpret
  - Grounded theory – develop general theories based on observation
  - Ethnography – insiders view of a group’s perspective
Qualitative, cont.

- In-depth and semi-structured interviews
  - Open-ended questions (probing is allowed)
  - Possibly supplemented (diary, journal)
- Focus group
  - 4-12 people
  - Moderator led discussion
Analysis

- Coding/classifying observations
  - Derive major & minor themes
  - Quotations often used
  - Possibly minimal use of descriptive statistics (counts)
A retrospective cohort study to investigate the association between smoking and renal artery stenosis compared 71 patients with documented renovascular hypertension and 308 age-matched control patients with essential hypertension. 94% (30/32) of men and 74% (29/39) of women with renal artery stenosis had smoked cigarettes compared with only 43% (64/150) of men and 41% (65/158) of women in the control group. This striking relation was true for both patients with fibromuscular disease (71% smokers; 15/21) and patients with atherosclerotic lesions (88% smokers; 44/50). All renal artery stenosis groups had significantly higher systolic and diastolic blood pressures than the relevant control group. When the groups were stratified according to blood pressure, there were significantly more smokers in the renal artery stenosis group at every level of blood pressure.
Answers

- Population?
  - 71 patients with renovascular htn

- Control Group?
  - Control: 308 age-matched controls

- Disease?
  - Disease: renal artery stenosis

- Exposure?
  - Exposure: smoking

- Time?
  - Identify cases first, then found age-matched controls

- Type of study?
  - Case control study – select the cases (disease) first then look back to exposure

Leukemia is one of the most prominent late effects of exposure to ionizing radiation. We have studied the incidence of leukemia among 46,988 Swedish patients exposed to iodine-131 (131I) for diagnostic reasons or to treat hyperthyroidism or thyroid cancer. The observed number of leukemia’s was compared with that expected based on incidence data from the general population. The mean absorbed dose to the bone marrow was estimated as 14 mGy (range 0.01-2.226). 195 leukemia’s occurred more than 2 years after exposure, and the standardized incidence ratio (SIR) was 1.09 (95% confidence interval 0.94-1.25). Similar, but again not significantly, increased risks were seen for chronic lymphocytic leukemia (CLL) (SIR = 1.08), a malignant condition not found to be increased after irradiation, and for non-CLL (SIR = 1.09). The risk of leukemia did not vary by sex, age, time, or radiation dose from 131I...
### Answers

<table>
<thead>
<tr>
<th>Question</th>
<th>Answer</th>
</tr>
</thead>
<tbody>
<tr>
<td>Population?</td>
<td>46,998 Swedish patients exposed to iodine-131</td>
</tr>
<tr>
<td>Control Group?</td>
<td>General population incidence rates</td>
</tr>
<tr>
<td>Disease?</td>
<td>Leukemia</td>
</tr>
<tr>
<td>Exposure?</td>
<td>Iodine-131</td>
</tr>
<tr>
<td>Time?</td>
<td>First identified exposure the followed and looked for disease</td>
</tr>
<tr>
<td>Type of study?</td>
<td>Cohort study</td>
</tr>
</tbody>
</table>
Resources List