Research and Statistics

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Randomized Controlled Trials

- Has high internal validity
- Reduced risk of confounding variable
- Reduced external validity
- Expensive, time-consuming variables

Cohort Studies

- Useful for sequential events
- Can study multiple outcomes from exposures
- Retrospective: less expensive
- Require large sample size
- Risk of confounding variables
- Difficult to study rare outcomes
- Prospective: expensive

Case-Control Studies

- Useful for rare outcomes
- Can study several exposures
- Inexpensive
- Risk of confounding variables

Cross-Sectional Studies

- Can study multiple outcomes and exposures
- Cannot infer causality
- Risk of confounding variables
- Less useful for rare exposures or outcomes

Case Studies

- Useful for rare outcomes
- Convenient and inexpensive
- Lack of a comparison group
- Cannot infer causality
- Risk of confounding variables

Systematic Reviews

- Summarize existing studies descriptively
- A descriptive results section summarizing the findings and addressing the qualities of the included studies

Meta-analyses

- **Use statistics to combine the results from each included study and generate a single summary statistic**
  - Compilation of evidence that potentially has greater power to inform clinical decisions than would an individual study in the systematic review or meta-analysis
  - If the quality of the studies included in the systematic review or meta-analysis is poor, the summary conclusions are similarly inadequate

Case Reports

- Aid in recognizing and describing new disease processes or rare manifestations
- Describe the disease in the context of comorbidities and individual characteristics
- Identify drug adverse effects
- Help to illustrate the diagnostic process and help students apply the literature to an individual patient
- Help identify emerging health conditions
- Show how exposures and disease outcomes are related

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- Can stimulate important research questions and help guide hypotheses
- Are purely descriptive and one of the weakest forms of evidence
- Cannot be used to make inferences about the broader population
- Cannot prove causality

Anecdotal Evidence

- A clinician's personal experience
- Shares some characteristics with case reports
- Lacks the strength of data collected via rigorous methodology that also involves significant numbers
  anecdotal evidence can suggest hypotheses and leads to the creation of credible studies

Descriptive Epidemiologic Studies

- Follow up on case reports
- Used to describe patterns of disease in the population according to person, place, and time
- Do not test a predefined hypothesis or determine a cause-and-effect relationship
- Used to develop hypotheses for subsequent analytic studies
- Use a variety of tools, including surveillance reports, cross-sectional analyses, and surveys

Validity

- Addresses whether an instrument or test actually measures what it is intended to measure
- **Criterion validity is the degree to which the measurement correlates with an external criterion or another instrument or test that is considered valid**
  - Convergent validity is the degree to which independent measures of the same construct are highly correlated
  - Predictive validity is the ability of an instrument or test to predict some future criterion
  - Discriminant validity requires that an instrument or test shows little or no correlation with measures from which it differs
- **Content validity refers to the extent to which aspects of items that make up an instrument or test are representative of a particular construct**
  - Face validity is a judgment about whether elements of an instrument make intuitive sense
  - Sampling validity refers to whether the instrument incorporates all of the aspects under study

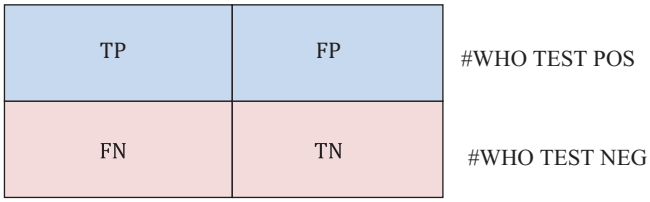

Fig. 1 Foursquare: *TP* true positive, *FP* false positive, *FN* false negative, *TN* true negative

Reliability

- The consistency or repeatability of scores
- Test–retest reliability assesses whether an instrument or test yields the same results each time it is used with the same study sample under the same study conditions
- Internal consistency reliability is a measure of the consistency of the items within a test
- Interrater reliability is the degree to which two raters independently score an observation similarly

Sensitivity: Screening

- Probability of correctly identifying those who truly have the disease
- True positives/disease
- TP/(TP+FN) (Fig. 1)

Specificity: Confirmation

- Probability of correctly identifying those who do not have the disease
- True negatives/disease
- TN/(FP+TN)

Positive Predictive Value (PPV)

- Probability of correctly identifying those who truly have the disease amongst those whose tests are positive
- True positives/test
- TP/(TP+FP)

Negative Predictive Value (NPV)

- Probability of not having the disease given a negative test
- True negatives/test
- TN/(FN+TN)

Predictive values are dependent on the prevalence of the disease. The higher the prevalence of a disease, the higher the PPV of the test.

p Value

- The p value is the probability of obtaining a test statistic result at least as extreme as the one that was actually observed, assuming that the null hypothesis is true.
- A researcher will often "reject the null hypothesis" when the p value turns out to be less than a predetermined significance level, often 0.05 or 0.01. Such a result indicates that the observed result would be highly unlikely under the null hypothesis.
- Many common statistical tests, such as chi-square test or Student's t test, produce test statistics which can be interpreted using p values.
- **An informal interpretation of a p value, based on a significance level of about 10 %, might be:**
  - $p \leq 0.01$: very strong presumption against null hypothesis
  - $0.01 < p \leq 0.05$: strong presumption against null hypothesis
  - $0.05 < p \leq 0.1$: low presumption against null hypothesis
  - $p > 0.1$: no presumption against the null hypothesis

False Positive

- A false positive occurs when the test reports a positive result for a person who is disease free.

False Negative

- A false negative occurs when the test reports a negative result for a person who actually has the disease.

Odds Ratio (OR)

- Calculates the relative risk (RR) if the prevalence of the disease is low. It can be calculated for case-control study (retrospective study)
- The OR can be used to determine whether a particular exposure is a risk factor for a particular outcome and to compare the magnitude of various risk factors for that outcome

Relative Risk (RR)

- Disease risk in the exposed group divided by disease risk in unexposed group. It can be calculated for cohort study (prospective study)
- The 95 % confidence interval (CI) is used to estimate the precision of the OR
- If the 95 % CI for OR or RR includes 1, the study is inconclusive
- A large CI indicates a low level of precision of the OR, whereas a small CI indicates a higher precision of the OR
- For a rare disease, OR approximates RR

Suggested Readings

1. Perry-Parrish C, Dodge R. Research and statistics: validity hierarchy for study design and study type. Pediatr Rev. 2010;31:27.
2. Hernandez RG, Rowe PC. Research and statistics: cohort studies. Pediatr Rev. 2009;30:364.
3. Moore EM, Johnson SB. Research and statistics: case reports, anecdotal evidence, and descriptive epidemiologic studies in pediatric practice. Pediatr Rev. 2009;30:323.
4. Copeland-Linder N. Research and statistics: reliability and validity in pediatric practice. Pediatrics in Review. 2009;30:278.
5. Palaia A. Research and statistics: study design and data sources. Pediatrics in Review. 2013; 34:371.