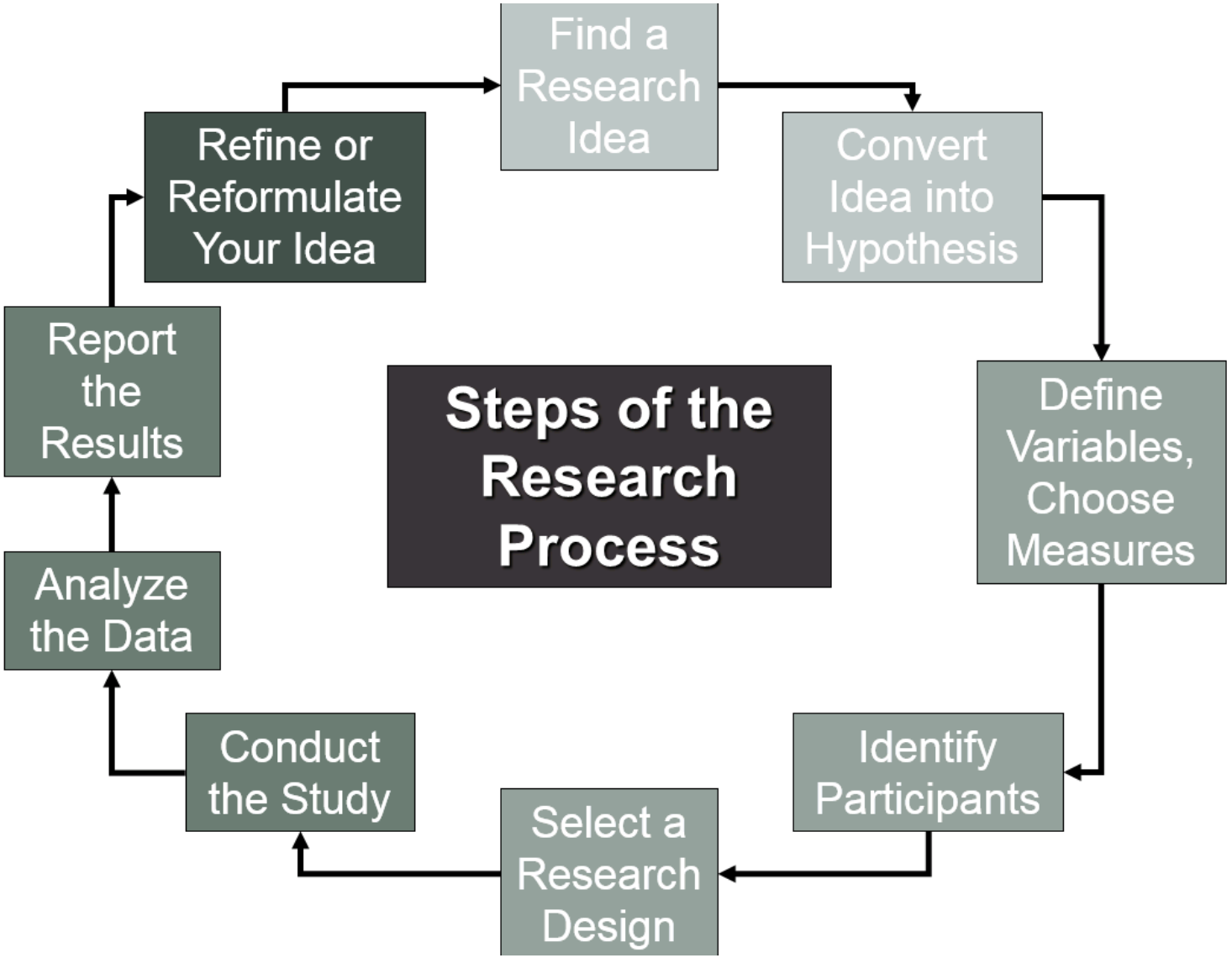
FLORIDA STATE UNIVERSITY
COLLEGE OF MEDICINE

Research Workshop Series #4
Research Design II

The Research Process



Protocol Components

- Research Topic & Question
- Background/ Literature Review
- Research Objectives
- Hypotheses
- Study Designs
- Independent & Dependent Variables
- Subject Selection/Inclusion & Exclusion Criteria
- Study Implementation & Keys to Success
- Data Management & Statistical Analysis
- References

**Additional components required for clinical drug trials*

Study Design

Study Design

- Guides interpretation of study results
- Can be quantitative, qualitative, or mixed methods
- Approach may depend on feasibility

- **Various types of designs**
  - Retrospective
  - Prospective
  - Observational
  - Interventional
  - Longitudinal

Cohort Study

- Type of observational design
  - Participants are treated as a group
  - Share something in common (i.e. disease, injury, prescribed medication)
  - Cohort group compared to control over period of time

* Example:

Evaluating the longitudinal impact of warfarin on drug-drug interactions or drug-disease interactions.

Cohort Study

Advantages

- Provides clarity of sequence of events
- Aids incidence calculation
- Can study rare exposures (e.g. Agent Orange)
- Examine multiple effects of a single exposure
- Avoid selection bias

Limitations

- May be difficult to follow subjects longitudinally
- Expensive & time consuming
- Not optimal for rare diseases

Cross-Sectional Study

- Type of observational design
  - Observational study at a point in time
  - Data collected from a population subset and is analyzed

* Example:

  Effect of childhood trauma on cognitive functioning as adults

Cross-Sectional Study

Advantages

- Less time-consuming
- Inexpensive
- Can examine prevalence of exposure and outcomes

Limitations

- Lacks element of time- just a snapshot

Case-Control Study

- Type of observational design
  - Retrospectively investigates whether or not frequency of exposure is associated with a particular outcome
  - Cases are compared to those with little or no exposure

*Example:

Exposure to second hand tobacco smoke to asthma in children

Case-Control Study

Advantages

- Inexpensive
- Less time-consuming than cohort design
- Efficient for studying rare outcomes

Limitations

- Subject to recall bias
- Not optimal for rare exposures
- Difficult to establish timing of exposure and outcome

Crossover Study

- Type of interventional design
  - Two treatments given consecutively to participants
  - Each group serves as their own control

* Example:

  Evaluating the effects of glucose and sucrose on mood

Crossover Study

Advantages

- Minimizes influence of confounding variables
- Statistically efficient
- Requires fewer subjects

Limitations

- May not always be feasible or ethical
- Can have "order effects": order of administration of treatment may affect outcomes
- "Carry-over" between treatments can confound treatment effects

Randomized Control

- Type of interventional design
  - People allocated at random to intervention or control groups (standard of care/placebo)
  - Considered the gold standard of clinical trial designs

* Example

Drug trials with study drug vs. placebo

Randomized Control

Advantages

- Can investigate cause-effect relationships with minimum bias and confounding variables
- Easier to generalize your findings
- Controls for selection bias
- Crossover design can be used

Limitations

- Expensive and time consuming
- Follow up can be difficult to complete with patients

Independent & Dependent Variables

Independent & Dependent Variables

- What factors will be measured
- Independent Variable
  - Stable and unaffected by other variables measured
  - Predictor variable
- Dependent Variable
  - Depends on other factors that are measured
  - Expected to change
  - Outcome variable

Exercise

- Opioid doses and acute care utilization outcomes for adults with sickle cell disease: ED versus acute care unit

- **Independent Variable:**
  - SCD Pain-related care received at Emergency Department or Acute Care Unit

- **Dependent Variable(s):**
  - Dosage of opioids
  - Pain ratings
  - Hospital admission rates
  - Length of stay

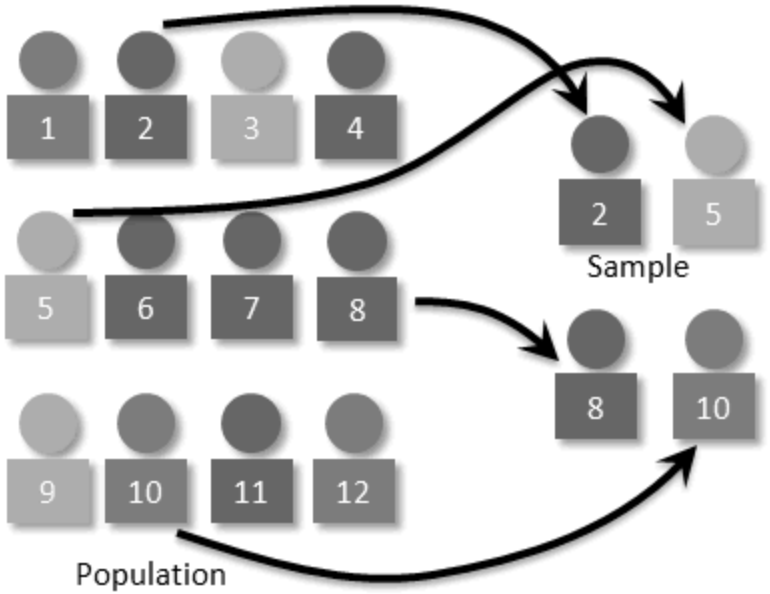
Subject Selection & Inclusion/Exclusion Criteria

Subject Selection

- Define the target population
  - Determine population of interest
  - Identify patients best suited for the research question
- Determine Sample Size
  - Consider number of subjects needed
  - Is this number achievable?
  - Specify the inclusion/exclusion criteria
- Items to consider
  - Cost per patient
  - Controlling for random error
  - Generalizability to the population of interest
  - Clarity and realism of inclusion & exclusion criteria

Study Population: Sampling

- Who is your target population?
- How many participants are needed?
- What sampling method will you use?
- How can you maximize the representation of your sample?



Sampling Strategies

Your sampling strategy is crucial in ensuring you have adequate representation of the entire study population.

Probability Sampling

- Simple random sampling
- Systemic sampling
- Stratified sampling
- Cluster sampling
- Multistage sampling

Nonprobability Sampling

- Quota sampling
- Convenience sampling
- Purposive sampling
- Snowball sampling

Additional information on sampling strategies

How many subjects do I need?

- **Quantitative Data: Power Analysis**
  - Plan for estimating study scope
  - Aids in determining sample size required to show an effect of a given size with a specified degree of confidence (i.e. 95% CI)
  - Determines the number of participants needed to help generalize study findings
  - Probability of finding an effect that is really there
  - Basis for testing the statistical significance of findings
- **Qualitative Data: Data/Theoretical Saturation**
  - Goal: depth of data, not a specific number of subjects
  - Saturation: when incoming data becomes repetitive and contains no new information

Inclusion & Exclusion Criteria

- Inclusion criteria - attributes or characteristics of subjects that are necessary for study participation.

  - BMI between 18-40
  - Current tobacco use
  - Presents with chronic pain
  - 1000 mg Tylenol daily for 1 year

- Exclusion criteria - attributes or characteristics that exclude the subject from study participation

  - Positive urine drug screen
  - Cancer treatment in past 5 yrs
  - Uncontrolled HTN
  - IM of Haldol in past 60 d

Evaluating Eligibility Criteria

- Subjects must have hypertension.

  - Too vague, needs quantifiable parameters

- Subjects who have a major medical condition will be excluded from the study.

  - Too general, it is better to specify a list of excluded conditions within a specific time frame (five years)

- Subjects aged 18-64 will be included in the study.

  -Good, clear, and precise

Practice-based Study Implementation & Keys to Success

Study Implementation

- Planning is key
- Develop logistical processes
- Recruit site(s) and determine physician and staff roles
- Regulatory processes, Research Advisory Committees, IRBs
- Form development
  - Recruitment materials
  - Enrollment and other tracking logs
  - Data collection templates
  - Develop interview/focus group guides
- Data storage platform
- Site level training

Keys to Success

- Acknowledge clinic time constraints
- Develop processes with minimal workflow disruption
- Flexible protocol to achieve project goals
- Engaging entire clinic in the study concept
- Creating collaborative professional relationships

Data Management & Analysis

Quantitative Research

- Quantifiable data:

  Based on quantities obtained using an objective measurement process

  - Surveys with closed-ended questions
  - Lab results

- Benefits:

  - Data can be collected and analyzed quickly
  - Can generalize to population
  - Reliable
  - Repeatable

Common Statistical Tests

(Handout)

- **Correlational**
  - Pearson correlation
  - Spearman correlation
  - Chi-square
- **Comparison of Means**
  - Paired T-test
  - Independent T-test
  - ANOVA
- **Regression**
  - Linear or Logistic
  - Simple regression
  - Multiple regression
- **Non-parametric**
  - Wilcoxon rank-sum test
  - Wilcoxon sign-rank test
  - Sign test

Statistical Tests Example

- Opioid doses and acute care utilization outcomes for adults with sickle cell disease: ED versus acute care unit

- Descriptive statistics
  - Mean; standard deviation, count, frequency

- Fisher's Tests & ANOVA
  - Compare demographics of patients visiting ED; ACU; Both

- Regression
  - Compare patient outcomes between ED and ACU

Qualitative Research

- Descriptive in nature

Data Collection:

- Interviews
- Focus groups
- Ethnography
- Open ended questions on surveys, etc.

Benefits:

- Depth of data
- Focuses on key issues of participants from their perspective
- Investigate sensitive and complex topics

Qualitative Analysis

- Qualitative research generates large amounts of text data
  - Just one transcript can generate easily generate 20 pages of text
- Analysis is labor intensive and time consuming
- Goal is to extrapolate meaning from subjects' words
  - Emic perspective: represents the subjects' views and perspectives, not the researchers'
  - Grounded theory: building theories that are grounded in/based on the data

Qualitative Analysis

- Analysis Steps
  - Document (e.g., field notes, transcripts of recorded interviews)
  - Define concepts and categories
  - Code data
  - Explore relationships, themes
- Analytical software: 2 most common
  - Atlas.ti
  - Nvivo

Group Activity

You want to conduct a study comparing patients taking antihypertensive medications after a stroke and identify subsequent ER visits and hospital admissions for recurrent stroke. How might you address the following key questions for the study design?

Please reference handout: "Small Group Exercise for Workshop Research Design 2"

Thank you!

Questions & Discussion