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NED 2020
Networking to Enhance Development
A Conference By and For Research Coordinators
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Basic Knowledge of Study Design and Methods

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Learning Objectives

1. Introduce Clinical Research Coordinators to different types of study designs

2. Discuss how knowledge of different study designs can benefit coordinators in their day to day work

What to Expect Today

1. Overview: Study Design Types

2. How does knowledge about study design affect research coordination work?

3. Group Activity and Discussion

Research Study Designs

Research Study Design Types

Basic Terminology

First, some general terminology!

- Population: Group of individuals who are under study
  - Population is defined by inclusion / exclusion criteria
  - Study participants are enrolled or sampled from the existing population of interest
- Research Question: What hypothesis will be tested with the data collected?
  - Each research question should be distinct and clearly defined
  - Research question dictates what data should be collected
  - May need to refine research question if the data needed are too vast or complicated to collect – need to be realistic!
- Exposure: Any factor that may be associated with an outcome of interest
  - Important to define and measure correctly
  - Example: Smoking (Current smoker? Ever smoker? Smoke > 1 pack/day?)
- Outcome: Defined disease or condition – outcome is usually subject of research question
  - Important to define and measure clearly (e.g. physician diagnosis, score on test)
  - Example: Quality of life score 1 year after cancer diagnosis; Diagnosis of ADHD by 5 years of age

Clinical Trial

An experimental study designed to test the effect of an intervention on an outcome of interest within a population

- Involves an intervention delivered over a specified period of time
  - Can be a drug, a device, or a behavioral intervention
- Can be randomized or non-randomized
  - Randomized clinical trials ("RCTs") typically involve 1 or more treatment groups as well as a control or placebo group
    - Often "double blind", indicating that neither the patient nor the study team knows whether patient received drug or placebo
  - Subjects may be randomized based on strata (e.g. gender, age, race) to help ensure balance between groups
  - Non-randomized clinical trials are common when it is not practical or ethical to randomize patients to a placebo or control group
    - Example: Early stage efficacy trials with small number of patients

Clinical Trials, continued

- Outcomes of interest are defined at the start of the trial and are measured at specific timepoints
  - Example: Difference in 5 year mortality rate among patients receiving Drug X vs. Drug Standard-of-Care for cancer treatment
- New drugs and devices will typically go through several clinical trial stages in order to receive FDA approval in the US
  - Phase 0 (First in human) through Phase 4 (post-approval, monitoring side effects)
- Population definition is key – efficacy results cannot extend to patients who weren't included in the trial
  - Example 1: Drug may be tested in patients with stage 2 cancer, but not stage 3
  - Example 2: Drug may be approved in adults, but not children

Observational Cohort

A group of individuals are followed over time (weeks, months, years!)

- Can be retrospective or prospective
- Cohort is defined by characteristics (“exposures”) of interest
- Patients are observed for the development of outcomes of over time
- Can be used to assess incidence of an event in a population, or to assess the association of an exposure with an outcome
- Often much easier and less expensive to conduct than RCTs, but can be harder to control for confounding

Research Study Design Types

Cross - Sectional

Observational study in which patients are observed at a single point in time

- Can measure prevalence of a disease or condition in a population at a given time
- **Cannot** answer questions about incidence or cumulative incidence (e.g. how many patients at the CF clinic will develop an infection during their first 5 years of treatment at SCH?)
- Quick and easy to do – no follow-up needed
- May or may not involve looking at the association of an exposure and outcome

Case – Control Studies

Observational study in which patients with an outcome of interest ("cases") are compared to patients without the outcome ("controls") to assess association of the outcome with an exposure

- Patients have already developed the outcome prior to the study
- Cases and controls should come from the same population and should be fairly similar to one another outside of case/control status
- Many case-control studies are "matched"
- Analyses often look at "odds ratios", which compare odds of having an exposure (e.g. smoking, radiation, high fiber diet, etc.) for cases vs. controls

Other types

Other types of research studies include:

- Case reports
  - Often used for rare disease reporting, when insufficient number of patients are available for statistical comparisons
- Meta-analyses
  - Aggregate the results of many different, related trials to systematically assess the results of previous research
- Systematic reviews
  - Summary review of the existing literature

How does this affect my work?

How does knowledge about study design affect research coordination work?

It's critical for many reasons!

- Enrollment of eligible patients
  - Facilitates understanding and importance of inclusion/exclusion criteria
- Informed consent process
  - Knowledge of study design can help RCs to better explain purpose of study to interested families
- Data collection
  - Knowledge of how data will be analyzed facilitates accurate data collection and creation of appropriate data collection forms
- Literature reviews
  - Can search for relevant studies in the literature to prepare for an upcoming grant
- Summary tables
  - Guides creation of basic summary tables for data reporting

Group Discussion: Design your own study!

Choose one of the types below, and design your own study (does not need to be based on any real data or drugs!)

- Clinical trial
- Observational cohort
- Cross-sectional
- Case-control
- Other?

Include the following:

- Research question (with exposure and outcome defined, when applicable)
- Description of population under study
- What data you will collect, as well as how and when you will collect it

Questions?

Thank You


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