

Clinical Trials

Bell Ringer: What is involved in a clinical trial?

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- A clinical trial is a research study using human volunteers, aimed at determining the safety and efficacy of a drug, vaccine, medical device, or behavioral intervention.

- Before an experimental treatment can be tested in a clinical trial, it must have shown benefit in laboratory testing, animal research, or research in a small group of humans.
- Clinical trials are required to follow the same ethical and legal guidelines as all other standards of medical care and interventions to protect patient safety.

- Every clinical trial in the United States must be approved and monitored by an Institutional Review Board (IRB) to protect patient safety.

- Every medical institution that conducts clinical trials has an IRB.
- An IRB is a committee of health care professionals and community members that do not have connections to the specific clinical trial.
- The IRB is designed to facilitate unbiased decisions about the clinical trial so that patient safety is achieved.

Clinical Trials Eligibility

- Every clinical trial has guidelines and requirements for who can participate in the study
- Variables that determine who can participate in a particular clinical trial include age, gender, race/ethnicity, type and stage of disease, and treatment history
 - The factors that allow a subject to participate in a clinical trial are called “inclusion criteria”
 - The factors that disallow a subject to participate in a clinical trial are called “exclusion criteria”
 - These criteria are used solely to ensure that the research can be objectively studied

Clinical Trial Protocol

- A protocol is the clinical trial plan that explains the purpose and process of the study.
- A protocol is designed to protect the health of research participants and ensure that research questions are answered in an objective, empirical manner.

Clinical Trial Protocol

- A research protocol will include information on:
 - Who can participate in the study
 - How many people can participate in the study
 - The treatment plan (or intervention) which includes procedures, medications and dosages
 - Type and frequency of tests to be used in the study
 - How the results will be measured
 - Reasons why the study can be stopped
 - Known and expected effects of the study treatment/intervention
 - Potential benefits of the study

Clinical Trial Informed Consent

- Informed consent is the process of learning about the clinical trial before deciding to participate.
- Informed consent is also a process that continues throughout the entire study – providing participants with information as it may change.

Clinical Trial Informed Consent

- Before someone can participate in a clinical trial, they must review and sign an informed consent form which will include some of the following information:
 - Clinical trial process, including tests that may be conducted
 - Known and expected risks of experimental treatment
 - Length of clinical trial
 - Clinical trial contact information
- Informed consent is not a legal contract; the participant can withdraw from the trial at any time for any reason.

Clinical Trial Process

- The process for a clinical trial depends on the type of clinical trial being conducted.
- At the beginning of a trial, the participant's health is checked and the research team provides information to the participant about the study.
- The participant is then monitored by members of the research team, as determined by the protocol of the study.
- Follow-up after the study is completed is generally part of the protocol.

Clinical Trials Phases

- Phase 1 – Researchers test a new drug or treatment in a small group of people for the first time to evaluate its safety, determine a safe dosage range, and identify side effects
- Phase 2 – The drug or treatment is given to a larger group of people to see if it is effective and to further evaluate its safety
- Phase 3 – The drug or treatment is given to large groups of people to confirm its effectiveness, monitor side effects, compare it to commonly used treatments, and collect information that will allow the drug or treatment to be used safely
- Phase 4 – Studies are done after the drug or treatment has been marketed to gather information on the drug's effect in various populations and any side effects associated with long-term use

Types of Clinical Trials

- There are different types of clinical trials. Deciding which type of investigation to use depends on the type of question the researcher is attempting to answer. In each type of trial, the goal is the same: to safely search for solutions to specific health problems.

Interventional or Treatment Trials

- Designed to test/assess experimental treatments, new combinations of drugs, or new approaches to surgery or radiation therapy

Prevention Trails

- Looks for better ways to prevent disease in people who have never had the disease or to prevent the disease from returning
- Typically involves medicines, vaccines, vitamins and/or lifestyle changes

Diagnostic Trials -- Designed to find better tests or procedures for diagnosing a particular disease or condition

- Screening Trials -- Designed to test the best ways to detect certain diseases or health conditions
- Quality of Life Trials -- Designed to find ways to improve the quality of life and comfort for people with chronic conditions

Sample Size and Clinical Trials

- Sample size is the number of participants in the study and it is one of the first practical steps in designing a clinical trial. If the sample size is too small, the researcher may fail to detect a serious problem with the treatment and if the sample size is too large, money, time and resources are wasted
 - Determining sample size is usually done in the design phase of the clinical trial, before seeking IRB approval
 - Researchers typically consult with a biostatistician to determine sample size

Sample Size and Clinical Trials

- Sample size calculation is used to find the number of research participants needed to ensure that the treatment/intervention/product is effective.
 - Determining sample size depends on four key factors:
 - The specific aim of the study
 - The study design
 - The outcomes and predictors of the study
 - The proposed statistical analysis plan

Methodological Assessment in Clinical Trials Research

- Quantitative and qualitative research are two major paradigms in medical research. Although research studies typically use both approaches, the advantages and disadvantages of which one to use are hotly debated in the medical community.

Qualitative data is information that is descriptive and cannot be measured by numbers and statistical analysis.

- Variables include opinions, experiences, and values
- A subjective form of research that is driven by issues or questions that emerge and unfold during in the data collecting process
- Data is collected in a text or narrative format on the basis of observation and interactions with research participants
- The data is not converted into numerical format and it is not statistically analyzed

Quantitative data is information that is absolute and can be measured.

- The vast majority of clinical trials are designed to be quantitative studies
- Quantitative research is driven by a hypothesis
- Its key characteristics are numerical data that permits a range of statistical analysis which is typically carried out with the aid of sophisticated statistical computer packages

- There are several approaches to quantitative research, including experimental, descriptive, correlational and causal comparison paradigms.

Experimental Studies

- An intervention is made under controlled conditions with the exclusive goal of evaluating its effect
- The gold standard is the ‘Randomized Controlled Trial’ (RTC) which is used to determine if a therapeutic intervention is effective

Descriptive Studies

- Collecting data to test a hypothesis
- Used largely in epidemiological studies to look at the incidence or distribution of disease, or to assess current health problems or needs
- Data is typically collected in the form of questionnaires

Correlational Studies

- Used to discover relationships between two variables, such as determining if there is a link between birth weight and gestational diabetes

Causal Studies

- Usually used in epidemiological research to look at issues associated with cause and effect
- It is assumed that if a cause and effect relationship exist between variables, the independence variable cannot be manipulated
- The gold standard is a P value which is a statistical concept that means probability; it measures the likelihood that a particular finding or observation is due to chance