CLINICAL TRIALS

IS A TRIAL RIGHT FOR ME?

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Definition of Clinical Trial

- **General Definition:** Evaluate the effectiveness and safety of medications or medical devices by monitoring their effects on large group of people.
- **NIH Definition:** A research study in which one or more human participants are prospectively assigned to one or more interventions (which may include placebo or other control) to evaluate the effects of those interventions on health-related biomedical or behavioral outcomes.

Clinical Trial Design

Blinding: One or more parties to the trial are unaware of the treatment assignment.

Single Blind

The subject is unaware of the treatment assignment.

Double Blind

The subject, the investigator, the monitor, and sometimes the data analysts are unaware of the treatment assignment.

Clinical Trial Design

Randomized

The element of chance is used to assign trial subjects to different treatment or control groups.

vs.

Non-Randomized

All subjects receive the same treatment.

Phase 1

Looking at toxicity

- Include the initial introduction of an investigational drug into humans
- Closely monitored
- Conducted in patients or in normal volunteer subjects
- Designed to determine:
  - metabolism and pharmacologic actions of the drug in humans
  - side effects associated with increasing doses
  - if possible, obtain early evidence on effectiveness
- Total number of subjects is generally 20-80

Phase 2

Looking at activity

- Controlled clinical studies
- Conducted in patients with the disease or condition under study
- Designed to determine:
  - the effectiveness of the drug for a specific indication or indications
  - the common short-term side effects and risks associated with the drug
- Studies usually involve no more than several hundred subjects

Phase 3

Looking at efficacy

- Expanded trials
- Conducted after preliminary evidence suggesting effectiveness of a drug has been obtained
- Designed to gather the additional information about effectiveness and safety needed to evaluate the overall benefit-risk relationship of the drug and to provide an adequate basis for physician labeling
- Studies usually include from several hundred to several thousand subjects

Phase 4

Looking at safety

- Post-marketing studies
  - Delineate additional information about the drug's risks, benefits, and optimal use

Who's Who in Clinical Trials?

Principal Investigator (PI)

- Ultimately responsible for all aspects of the trial

Associate Investigators

- Physicians
- Research Nurse
- Protocol Manager
- Statistician

Other Healthcare Staff

- Floor Nurses
- Laboratory Staff
- Social Workers, Interpreters, etc.

Determining Eligibility

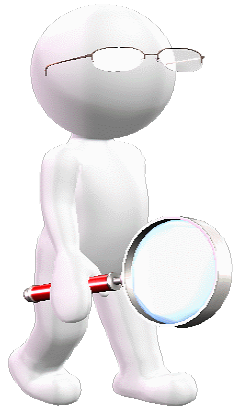
Eligibility for a clinical trial is determined by specific **inclusion/exclusion** criteria.

Some examples of **inclusion/exclusion** criteria:

- Ability to understand and give informed consent
- Ability to perform activities of daily living
- Specific laboratory values
- Disease history
- Prior therapies
- Age

Why do we have eligibility criteria?

- To **identify** appropriate subjects
- To ensure that they are **protected**



Questions Regarding Clinical Trials

- What exactly is a clinical trial?
- Who can participate?
- What is informed consent?
- What is a protocol?
- What are the benefits of participating in a clinical trial?
- What are the risks of participating in a clinical trial?
- Who will oversee my care during the clinical trial?
- Will my safety and privacy be protected?
- How much will it cost me?

What exactly is a clinical trial?

- Clinical trials are carefully designed research studies in which people help doctors find ways to improve health and care related to specific diseases or medical conditions.
- Each study tries to answer scientific questions and to find better ways to prevent, diagnose, or treat that condition.

Who can participate?

- All clinical trials have guidelines about who can participate. The factors that allow someone to participate in a clinical trial are called “**inclusion criteria**” or "**eligibility criteria**" and those that disallow someone from participating are called “**exclusion criteria**”.
- These criteria are based on factors like age, gender, the type and stage of a disease, previous treatment history, and other medical conditions. Before joining a clinical trial, a participant must qualify, or be eligible to participate in the study.

What is informed consent?

- Informed consent is the process of learning the key facts about a clinical trial before deciding whether or not to participate. This process is important to ensure that potential participants understand the study's purpose, potential benefits, and risks in participating as well as their other possible options.
- Informed consent is also a continuing process throughout the study to provide information for participants.
- To help someone decide whether to/not to participate, the doctors and nurses involved in the trial explain the details of the study. Informed consent is not a contract, and the participant may withdraw from the trial at any time.

What is a Protocol?

- A protocol is a study plan on which all clinical trials are based. The plan is carefully designed and reviewed by administrators and other researchers to safeguard the health of the participants as well as answer specific research questions.
- A protocol describes what types of people may participate in the trial; the schedule of tests, procedures, medications, and dosages; and the length of the study.
- While in a clinical trial, participants following a protocol are seen regularly by the research staff to monitor their health and to determine the safety and effectiveness of their treatment.

What are the benefits of participating in a clinical trial?

- Play an active role in their own healthcare.
- Gain access to new research treatments before they are widely available.
- Obtain expert medical care at leading healthcare facilities during the trial.
- Help others by contributing to medical research.
- Some clinical trials offer monetary compensation or reduced treatment costs as an incentive for participation.

What are the risks of participating in a clinical trial?

- There may be unpleasant, serious, or even life-threatening side effects to experimental treatment.
- The experimental treatment may not be effective for the participant.
- The protocol (medical guidelines for the study) may require more of their time and attention than would a non-protocol treatment, including trips to the study site, more treatments, hospital stays, or complex dosage requirements.

Who will oversee my care during the clinical trial?

- Participants continue to work with their own healthcare doctors, but often the healthcare providers work with the research team to ensure that other medications or treatments will not conflict with the protocol.
- Participants in clinical trials are often more closely monitored than patients treated outside clinical trials. This is because trial protocol requires detailed collection of health data and frequent patient checkups to assess how patients are doing.
- The research doctors typically come from among the clinicians who are most knowledgeable about the disease or condition under study — it's the focus of their work.

Will my safety and privacy be protected?

- The ethical and legal codes that govern medical practice also apply to clinical trials. In addition, most clinical research is federally regulated with built in safeguards to protect the participants.
- The trial follows a carefully controlled protocol, a study plan which details what researchers will do in the study. As a clinical trial progresses, researchers report the results of the trial at scientific meetings, to medical journals, and to various government agencies.
- Individual participants' names remain undisclosed and are not mentioned in these reports.

How much will it cost me?

- Patients need to ask for a detailed description of what costs are covered and what costs are not covered. When asking about costs, distinguish between the pre-trial or evaluative period and during the trial.
- Lodging (short term vs. long term)
- Travel
- Subsistence
- Non-trial related medical costs

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Who conducts clinical research trials ?

- Government Health Agency – NIH
- Researchers affiliated with a hospital or university medical program
- Independent researchers
- Private industry

Who approves/participates in clinical research trials?

- Government agencies-based on clinical results for example the FDA.
- Participants in clinical research trials are:
  - Volunteers-may get paid
  - Subjects

Clinical Trials

What are the four possible outcomes from a clinical trial?

Positive Trials

- Shows that the new treatment has a large beneficial effect and is superior to standard treatment.

Negative Trials

- Shows that a new treatment is inferior to standard treatment.

Inconclusive Trials

- Shows that the new treatment is neither clearly superior nor clearly inferior to standard treatment.

Non-inferior Trials

- Shows that the new treatment is equivalent to standard treatment.

Clinical Trials.gov

COVID-19 is an emerging, rapidly evolving situation.
Get the latest public health information from CDC: https://www.coronavirus.gov.
Get the latest research information from NIH: https://www.nih.gov/coronavirus.

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ClinicalTrials.gov is a database of privately and publicly funded clinical studies conducted around the world.

Explore 343,915 research studies in all 50 states and in 216 countries.

See listed clinical studies related to the coronavirus disease (COVID-19)

ClinicalTrials.gov is a resource provided by the U.S. National Library of Medicine.

IMPORTANT: Listing a study does not mean it has been evaluated by the U.S. Federal Government. Read our disclaimer for details.

Before participating in a study, talk to your health care provider and learn about the risks and potential benefits.

Find a study (all fields optional)

Status

- ○ Recruiting and not yet recruiting studies
- ○ All studies

Condition or disease (For example: breast cancer)

Aplastic Anemia

Other terms (For example: NCT number, drug name, investigator name)

Country

Search | Advanced Search

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Resources

- **Clinical Trials Information**
  - http://clinicaltrials.gov - Comprehensive registry of federally and privately supported clinical trials conducted in the United States and around the world.
- **Patient Support Organizations**
  - http://www.aamds.org - The Aplastic Anemia & Myelodysplastic Syndromes International Foundation.

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NHLBI Hematology Branch
National Institutes of Health

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