Frequently Asked Questions about Clinical Research

What is clinical research?

Clinical research is research conducted with human subjects, or material of human origin, in which the researcher directly interacts with human subjects. Clinical research helps doctors and researchers to find new and better ways to understand, detect, control, and treat illness. A clinical research study is a way to find answers to difficult scientific or health questions. For example, the study might explore the best ways to treat people with colon cancer. By studying cancer cells from patients, researchers may be able to determine the specific genetic mutations (changes in gene sequence) that caused the normal, healthy cells to become cancerous, and may help doctors decide on the best drugs to prescribe or surgeries to perform. Clinical research today may help other doctors in the future screen their healthy patients before they ever develop cancer.

What is a protocol?

All clinical studies are based on a set of rules or directions called a protocol. A protocol describes what types of people are eligible to participate in the study; determines the schedule of tests, procedures, medications, and dosages; and sets the length of the study.

What is a clinical trial?

If a clinical research study involves testing or studying a drug or medical device to see if it is a safe and effective treatment for people, it is called a "trial." For example, a clinical trial may test the effectiveness of a new drug for treating Parkinson's disease.

Many new medicines and drugs are found to work in the researcher's lab, and to be safe and effective in animal tests. But drugs and devices must be proven to be safe and effective for people before the Food and Drug Administration (FDA) can approve them and doctors can prescribe them to patients. The FDA has strict rules that govern how clinical trials are conducted. These rules are designed to ensure the safety of those who participate.

What are clinical trial "phases?"

Clinical trials of experimental drugs proceed through four phases:

In Phase I clinical trials, researchers test a new drug or treatment for the first time in a small group of normal, healthy volunteers (about 20 to 80) to evaluate its safety, determine a safe dosage range, and identify side effects.

In Phase II clinical trials, the study drug or treatment is given to a larger group of people (about 100 to 300), including patients with the particular disease, to see if the drug or treatment is effective, and to further evaluate its safety.

In Phase III clinical trials, the study drug or treatment is given to large groups of people (from 1,000 to 3,000), including patients, to confirm its effectiveness, monitor side effects, compare it to other commonly used treatments, and collect information that will allow the drug or treatment to be used safely.

Phase IV clinical trials are done after the drug or treatment has been approved by the FDA and marketed for public use. These studies continue testing the drug or treatment to collect information about its effect in various populations and gather data on any side effects associated with long-term use.

What are "blind" or "masked" studies?

In many clinical trials, one group of patients will be given an experimental drug or treatment, while a control group is given either a standard treatment for the illness, or a placebo (a harmless "fake" drug), or no treatment at all.

In a "blinded" or "masked" study, participants do not know whether they are getting the drug being tested, or whether they are in the control group. The goal is to prevent the so-called "placebo effect" from influencing the results of the experiment. The placebo effect is the phenomenon of patients feeling better simply because they think they are receiving a helpful drug or treatment.

Sometimes, clinical trials are "double-blind" or "double-masked." That means that neither the participants, nor the study staff members, know who is receiving the experimental drug and who is in the control group. Studies are performed in this way so that neither the patients' nor the doctors' expectations about the experimental drug can influence the observations and results.

Should I volunteer for clinical research?

Clinical research is a vital part of finding new treatments and cures for diseases. Carefully conducted clinical studies are the fastest way to find treatments that are safe and effective. By volunteering for a clinical study, you would be participating in research that may result in a new treatment for a deadly or debilitating disease.

Before you agree to participate in a study, you must be given complete information about the study, known as "informed consent." Informed consent involves two essential components: a document and a process. The informed consent document gives a summary of the research project (including the study's purpose, research procedures, potential benefits and risks, etc.) and explains the individual's rights as a research participant. This document is part of an informed consent process, which consists of conversations between the research team and the participant, and may include other supporting material such as study brochures. The informed consent process provides research participants with ongoing explanations that will help them make informed decisions about whether to begin or continue participating in the research project.

"Courtesy: National Human Genome Research Institute" https://www.genome.gov/