

What You Need to Know about Clinical Studies

Introduction

A clinical study is research that involves human volunteers and seeks to answer questions that will add to our knowledge about a medical condition or how to treat it. There are two main types of clinical studies.

1. **An observational study** collects information on the health of a specific group of people. A natural history study – one that follows participants for many years—is an example of this kind of study.
2. **A clinical trial** tests the safety and benefit of drug treatments, surgical procedures and assistive devices. Ideally, clinical trials should be *controlled* and *randomized*. Controlled means that the information from participants who receive the new treatment is compared to a matching set of people who did not. In a randomized trial the people who are willing to be in the trial are randomly assigned to either receive the new treatment or to be in the control group. Each group is handled in an identical manner so no one knows until the end of the study who is in which group.

Each kind of study must follow specific rules so the information that is gathered is accurate and unbiased. Getting enough people with a rare disorder like OI to participate in clinical studies is often difficult, but the larger the study, the more likely the information will be valid.

A new type of study, **Patient Reported Information**, is being used more often. Developments in the science of writing survey questions make it possible to gather certain types of research information directly from patients. This has expanded the use of electronic and paper based surveys. The **Contact Registry for People with OI** which is part of the Brittle Bone Disease Consortium makes use of these techniques.

How are clinical trials organized?

Each study is led by a principal investigator. The researcher writes a protocol -- a detailed description of every aspect of the proposed study including whether the study will take place at one center or multiple sites. Before people can be recruited as participants, the study must be approved by an Institutional Review Board (IRB). The job of the IRB is to make sure the study is scientifically well designed and participant health and safety are protected. Clinical trials of new medicines usually go through 4 phases before a drug is fully approved by the US Food and Drug Administration (FDA). Each phase looks at safety, side effects, effectiveness, and proper dosage. A phase 1 trial involves only a small group of people. Each following phase involves larger numbers of participants.

When the study is ready to recruit participants, the following information is made public.

- The reason for the study
- Who is eligible to participate
- How many people are needed
- The schedule of tests, procedures, or drugs
- The length of the study
- Location

Choosing to Participate in a Clinical Study

The decision to participate in an observational study or a clinical trial is voluntary and requires a commitment of time and energy by the adult participant and by the family if the study involves children. All study participants go through an **informed consent process**. During this process they receive detailed information about the study and are asked to sign a form. If you are considering participating in a study, be sure that all of your questions are answered during the informed consent process. Here are some sample questions.

- Why do researchers believe the treatment being tested may be effective? Has it been tested before?
- Exactly what kinds of tests or treatments are involved?
- How do possible risks side effects and benefits of the study treatment compare to my current treatment?
- How will this trial affect my daily life?
- Are there any fees that I will be responsible for paying?
- Will hospitalization be required?
- How long is my commitment to this study?
- What are the goals and objectives of the study?
- How will my data be kept confidential?
- Who do I contact if I have a question before or during the study?
- What are the inclusion and exclusion criteria for this study?
- Can I withdraw from the study at any time?

More Information

The web site www.clinicaltrials.gov provides a lot of information about clinical trials in general and lists a large number of studies. In addition, the OI Foundation periodically provides information about studies that are recruiting participants who have OI.

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