Schizophrenia™ & Psychosis Action Alliance

Clinical Trials 101

Disclaimer: Content prepared by Neurocrine Biosciences, Inc.

What Is a Clinical Trial?

Clinical trials, also called clinical research studies, are studies in which participants are asked to take an investigational drug under the supervision of a physician and other research professionals. These trials are the primary way that researchers find out if potential treatments, such as medications, drugs, or medical devices, are safe and effective in people. Often a clinical trial is used to learn if a potential treatment is more effective and/or has fewer side effects than the standard treatment.[1]

Clinical trials must be approved by an institutional review board (IRB). An IRB is a group that is responsible for helping to protect the rights and welfare of study participants. In addition, every trial participant is monitored with trial-related medical tests and exams before, during, and sometimes even after the trial.

How Do Clinical Trials Work?[1,2]

There are 4 phases of clinical trials. In order for a new treatment or drug to be approved for use by the Food and Drug Administration (FDA), it must complete 3 phases. The fourth phase happens after an investigational drug has been FDA approved.

Phase 1

This is the first stage of human testing. In this phase, an investigational drug is tested in a small group of people (often healthy volunteers) to look at possible risks and side effects.

Phase 2

While the emphasis in phase 1 is on safety, phase 2 emphasizes effectiveness. This phase can last several years and usually enrolls up to a few hundred volunteers from different populations who have the condition the investigational drug is designed to treat. Phase 2 trials usually provide more information about safety and how the investigational drug affects different populations, and they also help determine the best dosage level.

Phase 3

Phase 3 trials usually enroll several hundred to thousands of participants, and they study different dosages as well as how the investigational drug acts in different populations or in combination with other drugs. This phase provides the evidence for safety and effectiveness that the FDA will consider when deciding whether to approve the investigational drug for use by the general public.

Phase 4

After the FDA approves a drug, its safety and effectiveness may continue to be monitored in large, diverse populations.

What Should I Expect as a Participant in a Clinical Trial?[1]

Regardless of which phase of a clinical trial you participate in, here's what you can expect:

- The trial doctors will explain the trial and gather more information about you.
- Once you've had a chance to ask all your questions, if you decide you want to participate, you will sign an informed consent form.
- Next, you will attend the screening visit(s) for initial tests and assessments to make sure you are eligible to participate in the clinical trial. After all necessary tests and assessments have been completed, you will enter the trial. Some, but not all, clinical trials are double-blinded, which means neither the participants nor the researchers know which treatment or intervention participants are receiving until the clinical trial is over. If you take part in a double-blinded clinical trial, you will be assigned at random to receive either the investigational drug or a placebo, which is made of a substance that looks just like the investigational drug being tested but contains no active ingredients. Placebos allow researchers to fully understand the effects of the investigational drug. You will receive the same care whether you are assigned to receive the placebo or the investigational drug.
- Throughout the trial, you (and possibly your family members) will follow the trial procedures and attend scheduled trial visits at the research site so that the trial team can collect information about the effects of the investigational drug and your health and safety.
- Because every clinical trial has different eligibility criteria, not all people are appropriate for every clinical trial. Often, this could be because of other conditions a person has, or current or past treatments. If you are interested in a clinical trial but are not eligible, talk to your doctor about your interest in clinical research so that together you can evaluate future studies.
- You will continue to see your regular doctor for any other healthcare needs throughout the clinical trial.
- Participation in a clinical trial is completely voluntary, and you may choose to leave the trial at any time for any reason.

Why Should I Participate in a Clinical Trial?

Different people choose to participate in clinical trials for a variety of reasons. Some people participate because there is no approved treatment for their condition, or the treatments they have tried did not work, or they want access to expert medical care at leading healthcare facilities. Some people participate because they want to help researchers learn more about certain medical conditions and know that their contributions could help future generations lead healthier lives.

How Do I Join a Clinical Trial?

If you are interested in joining a clinical trial, you should talk to your doctor. Your doctor can help you determine if a clinical trial would be right for you, and they can help you identify what questions might be important to ask before you decide to participate in one. To learn more about clinical trials for schizophrenia, visit the Schizophrenia & Psychosis Action Alliance's website.

References:

1. nia.nih.gov/health/what-are-clinical-trials-and-studies
2. alz.org/alzheimers-dementia/research_progress/clinical-trials/how-trials-work