



# Clinical Trials 101 – For Caregivers

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.<sup>1</sup>

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?<sup>1,2</sup>

There are 4 phases of clinical trials. In order for a 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 While My Loved One Participates in a Clinical Trial?<sup>1</sup>

Regardless of which phase of a clinical trial your loved one participates in, here's what you can expect:

- The trial doctors will explain the trial and gather more information about your loved one.
- Once you and your loved one have had a chance to ask all your questions, if your loved one decides they want to participate, they will sign an informed consent form (ICF). If they are under the age of 18, they will sign an assent form and you will sign the ICF.
- Next, your loved one will attend the screening visit(s) for initial tests and assessments to make sure they are eligible to participate in the clinical trial. After all necessary tests and assessments have been completed, your loved one 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 your loved one takes part in a double-blinded clinical trial, they 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. Your loved one will receive the same care whether they are assigned to receive the placebo or the investigational drug.
- 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 your loved one is interested in a clinical trial but is not eligible, they should talk to their doctor about their interest in clinical research so that together, they can evaluate future studies.

### References:

1. [nia.nih.gov/health/what-are-clinical-trials-and-studies](https://www.nia.nih.gov/health/what-are-clinical-trials-and-studies)
2. [alz.org/alzheimers-dementia/research\\_progress/clinical-trials/how-trials-work](https://www.alz.org/alzheimers-dementia/research_progress/clinical-trials/how-trials-work)

- Throughout the trial, you and your loved one 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 loved one's health and safety.
- Your loved one will continue to see their regular doctor for any other healthcare needs throughout the clinical trial.
- Participation in a clinical trial is completely voluntary, and your loved one may choose to leave the trial at any time for any reason.

## Why Should My Loved One 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 Can My Loved One Join a Clinical Trial?

If your loved one is interested in joining a clinical trial, they should talk to their doctor. Their doctor can help them determine if a clinical trial would be right for them, and they can help you both identify what questions might be important to ask before your loved one decides to participate in a clinical trial. To learn more about clinical trials for schizophrenia, visit the [Schizophrenia & Psychosis Action Alliance's website](#).