

Parkinson's Disease – Understanding Clinical Trials: What You Need to Know

Progress in treating Parkinson's disease (PD) depends on clinical trials, and the success of clinical trials depends on people like you who volunteer to enroll in a trial.

By participating in a clinical trial, you are helping to improve the understanding of the disease and the best ways to treat it. All of the advances in treating people with PD have come about through clinical trials.

Participation in a clinical trial gives you the chance to feel empowered and more in control of your disease. Participation is voluntary and should only be undertaken with a full understanding of risks and potential benefits.

Clinical Trials Overview

There are two types of clinical trials — interventional and observational. An **interventional clinical trial** tests whether a treatment, such as a new drug, device, or type of surgery, can offer more benefit to patients than current treatments. An **observational clinical trial** does not test a new treatment. Instead, it carefully observes some aspect of the patient's disease, such as tremor or speech, to better understand it, which may lead to better treatments in the future. Observational trials might involve filling out questionnaires and often these trials can be performed virtually.

Both types of clinical trials are crucial for advancing the treatment and ultimately finding a cure for PD.

Eligibility Criteria

Eligibility criteria varies from trial to trial. Inclusion criteria are the defining characteristics of people with PD who are sought after for trial participation. These

criteria might include age, gender, education, or stage of disease. Exclusion criteria are the characteristics of people with PD who cannot be enrolled in the trial. This might include a specific medical co-morbidity or PD symptom. Eligibility criteria might limit your ability to participate in certain clinical trials.

Types of Interventional Clinical Trials

As a new treatment for PD is developed, it must go through several stages of testing before the United States Food and Drug Administration (FDA) considers it for approval.

- A **phase 1 trial** is performed in a small number of healthy individuals to determine if the treatment is safe enough to give to people.
- A **phase 2 trial** tests the treatment in a small number of people with PD. The major goals of a phase 2 trial are to make sure the treatment is safe for patients, to determine the best dose, and to look for initial hints that the drug or device offers some benefit.
- A **phase 3 trial** is performed in hundreds of PD patients at multiple centers, and tests whether the drug or device conclusively provides a benefit. The FDA considers both safety and benefit in determining whether to approve a drug or device for marketing.
- A **phase 4 trial** investigates the treatment after it is approved, to gather more information on its use in a more diverse or complex population of patients.

For many people, receiving a new treatment, whether it's helping or not, can temporarily improve how they feel, even in a disease such as PD. This short-term, psychologically driven

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improvement, called the **placebo effect**, makes it difficult to know whether a new treatment is truly providing benefit.

Therefore, the most rigorous clinical trials are:

- **Placebo-controlled** – One group of people in the trial are not receiving the intervention under study, but rather a placebo - an inactive substance or treatment. This allows results of those receiving the intervention to be compared directly to the results of those not receiving the intervention.
- **Double-blinded** – Neither the participant in the clinical trial nor the doctors/study coordinators are aware of who is receiving active treatment and who is receiving placebo.
- **Randomized** – Study participants are randomly placed in the treatment or placebo groups.

Meeting Current Challenges in PD Treatment Through Clinical Trials

While there are multiple types of drugs or devices that reduce the symptoms of PD, there are none that address all of the symptoms. Researchers are currently developing promising new therapies that aim to better control PD motor symptoms with fewer side effects, and to lengthen the time that the medication works before wearing off. New treatments are also being developed to address the “non-motor” symptoms of PD including cognition, bowel function, and other common issues. Research is also focused on developing drugs or devices that can slow or halt the progression of the disease. Every one of these promising new ideas in PD treatment needs to be studied in clinical trials with PD patients.

Participating in a Clinical Trial

Participation in a clinical trial is voluntary, and you have the right to withdraw from the trial at any time. Participating in an interventional clinical trial may offer you the chance to try a new treatment that may benefit you more than currently available treatments.

Depending on the details of the trial, you may also have the opportunity to receive types of interventions you would not otherwise receive such as new methods of physical therapy or new approaches to dietary advice. However, participation may also expose you to some risks that you would not otherwise be exposed to if you were not in the trial.

Informed Consent

Informed consent is the process of learning about all of the possible risks and benefits of a clinical trial. You have a right to know these in detail prior to participating in a trial. The clinical trial team will discuss these with you, and you will be asked to acknowledge that you understand the information and to sign forms that indicate this. Be sure to ask as many questions as you need in order to fully understand what it will mean for you to participate in the trial. Your care partner should also be made aware of the potential risks and benefits of participating in the trial and have an opportunity to ask questions.

The Clinical Trial Journey

The clinical trial team will describe the different trial visits that will be conducted throughout the trial period. At each visit, different measurements and assessments will be performed to follow your progress. You will be closely monitored throughout the trial for

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side effects as well as potential benefits of the treatment.

Questions to Ask Before Agreeing to Participate in a Clinical Trial

- What have previous studies shown about the safety and effectiveness of the new treatment?
- What are the most common adverse effects seen with use of this treatment?
- How will the treatment team monitor me for adverse effects?
- Is there anything in my medical history that puts me at higher-than-average risk in this trial?
- What will I need to do during the trial, and how long will the trial last?
- Will I have to travel to a different medical center for participation in the trial?
- What are the chances that I will receive a placebo in this trial?
- Who should I contact if my condition worsens during the trial?
- Will I be able to continue on the new medication (or begin it, if I received a placebo) after the trial is over?
- What will it cost me to participate in the trial?

Costs Associated With Participating in a Clinical Trial

There is no cost to participating in a clinical trial. In general, however, patients enrolled in a clinical trial are still responsible for their routine medical costs, such as non-trial-

related physician visits or non-trial medications.

Finding Out About Clinical Trials in Parkinson's Disease

Ask your neurologist if there are clinical trials that you can participate in that are at sites that are close to where you live. It is also important to note that various clinical trials, even certain interventional ones, may conduct some, or even most of the trial visits virtually, making the trial more accessible to people who live far away from a clinical trial site.

You can also search for clinical trials in PD at www.clinicaltrials.gov by entering "Parkinson's disease" into the search box. On the results page, you can refine your search to only show ones that are "open," or still enrolling patients. You can also restrict the results by topic or location.

Clinical trials represent the best hope for finding new treatments to improve the lives of all people with Parkinson's disease. APDA encourages you to consider participating in a trial that is right for you.

Resources

APDA provides information, education, and support to those impacted by Parkinson's disease, and funds scientific research into the causes, prevention, treatments, and ultimately the cure for PD. We provide a nationwide network of programs, activities, and events to facilitate a better quality of life for the Parkinson's community. Through our website, apdaparkinson.org, you can find the full range of resources we offer, as well as links to other important sources of information and support.

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symptoms and manage your care.

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