A&D Resource Directory

Alzheimer's and Dementia Resource Center of NYC

# NEWSLETTER #4 – Apr 2021

## Clinical Trials

### Introduction

Clinical trials continue to be an area of interest to many of our readers. To help answer your questions the folks at Antidote have prepared the following for us.

**Part One: What is a clinical trial?**

A clinical trial is a research study that tests whether a new potential therapy is safe and effective for patients. It evaluates new drugs, behaviors, or devices, and reveals whether these potential therapies work for particular diseases or particular groups of patients. Clinical trials can provide the best available data for the approval of new disease treatments.

There are two main types of clinical trials: interventional studies and observational studies:

- Interventional studies. These are clinical trials testing whether a specific intervention (such as a drug, device, or behavioral change) affects health-related outcomes. Different groups of people are assigned at random to receive and not receive the intervention in a process called randomization. Typically, the group that does not receive the intervention — also known as the **control arm** — receives either the current standard of care or a placebo (a fake version of the intervention), depending on the condition. Interventional trials are typically blinded, meaning that the volunteer is not aware if they are in the control group or receiving the intervention, or double blinded, meaning that both the researcher and the volunteer are not aware.
- Observational studies. These studies are ones in which participants are put in groups based on their characteristics, and an intervention is tested in each of these groups. The difference is that the groups are assigned based on volunteer characteristics, rather than at random. Both types of studies follow a protocol, a detailed plan for the study written by the trial sponsor and approved by the FDA.

A **trial sponsor** is an organization which initiates, funds, and conducts a clinical trial. This is often a pharmaceutical company, but it can also be a university or another type of research organization. When selecting a clinical trial, it's important that potential volunteers are fully aware of and comfortable with who is sponsoring the trial.

Potential volunteers for trials can be anyone. While many people view clinical trials as a last resort only to be relied upon when all other options have been exhausted, this is not the case. Research studies need a variety of participants to enroll. Some trials are looking for volunteers who have been recently diagnosed, while others may only need those who have

been living with a condition long-term. Certain studies may require patients who have had different treatment experiences in the past. Every trial is different, but with thousands of studies recruiting, there is likely a trial that is right for you.

**Part Two: What are the phases of clinical trials?**

Clinical trials begin with an idea in a lab. Researchers thoroughly test potential treatments in the lab and in animals before ever beginning trials in humans. If these lab and animal tests are successful and the therapy looks promising, the researchers will begin to conduct research studies beginning at Phase I and moving through to Phase IV. Potential new treatments typically move through at least three clinical trial phases before they can be approved by the FDA.

- Phase I clinical trials test whether drugs are **safe to use in humans**.
- Phase II clinical trials test the **effectiveness of a drug or medical device**. This stage can last from several months to a few years, and generally involves several hundred participants.
- Phase III clinical trials are typically the largest. They involve anywhere from several hundred to several thousand participants in a randomized, blind study. This testing phase can last several years as the FDA gathers thorough data about the drug's effectiveness and **potential side effects**.
- Phase IV trials, also known as Post Marketing Surveillance Trials, take place after a drug or device has been approved. In this phase, pharmaceutical companies monitor a drug's **long-term effectiveness and impact**, compare it to existing trials in the market, and determine the cost effectiveness of the new treatment.

**Part 3**

It's a big decision to take part in a clinical trial, and it's important that patients and caregivers fully understand all of the benefits and risks before enrolling.

**Why join a clinical trial**

There are many reasons that people choose to volunteer for clinical research, but it's important to weigh these against potential risks. Some of the top benefits of taking part in research include:

**Access to care.** People who take part in a clinical trial receive access to the latest treatments in development, and for many patients looking for options, this is a real benefit. In fact, according to a CISCRP survey of 2,194 former clinical trial participants, 44% of people surveyed cited "obtain better treatment" when asked to share their top reasons for participation. In some cases, patients are able to continue taking the drug after the trial is over, as well. Also, in many cases, patients receive either the standard of care or the investigational drug, removing the risk of receiving a placebo.

**Quality of care.** Patients in clinical trials report a high level of personalized care while participating in research. While trials may require more office visits than normal, or more tracking of symptoms, this extra time spent on health can mean a higher quality of care.

**Financial benefits.** Treatments administered during a clinical trial are often given at no cost to patients; in fact in many cases, volunteers are compensated for their time, travel, or participation in general. The amount of payment has to do with the phase of the trial - earlier trials such as Phase I pay more (nearly $2,000 on average) because the treatments being studied are not as well-understood. To find a paid clinical trial, you'll likely need to find a few trials for which you may qualify, then contact the sites to learn about potential payment.

**Helping advance science for future generations.** The goal of clinical trials is to discover new, better treatments (and cures) for the condition being studied. This means that, if successful, patients with that condition in the future will have better options than current patients. This is an important motivator for many patients today. In the CISCRP survey referenced above, nearly half of participants cited "help advance science, treatment of disease/condition" as one of their top reasons for participation.

On the other hand, of course, clinical trials also come with some degree of risk. There is a chance that the treatment being studied might not work as expected or be a better option than the standard of care. The study drug could also cause an unexpected side effect, especially in earlier phase trials.

With that said, there are several protections in place for patients who take part in clinical trials:

- An **Institutional Review Board** (IRB) ensures that trials are ethical and that participant rights are protected, and the FDA provides oversight for all clinical trials testing new drugs or devices.
- Participant rights include informed consent, meaning that volunteers are given all the facts about a trial and can ask any questions they'd like before enrolling. These questions might include:
    - How long will the study last?
    - What is the goal of the study?
    - Will I be reimbursed for my expenses?
    - Does the study include a placebo?
    - How will I receive the treatment?
    - Can I continue taking the study drug after the trial if it works for me?
    - How will my privacy be protected?
    - What can I expect at each study visit?
    - What happens if I leave the study early?
    - What happens if my condition gets worse or I am injured during the trial?
    - Who will be conducting the study?
    - What did previous studies find out about the treatment? Have the results been published?
    - What are the potential risks and benefits of the study drug?
    - Will I receive follow-up care after the study?
    - Will the results of the trial be provided to me?
- After signing an informed consent, a participant can still leave a clinical trial **at any time, for any reason**. In addition, if unexpected risks emerge during the trial,

patients have a right to be informed about them and be removed from the study if appropriate.

- Special protection is also given to children who participate in trials as well. Often, both parents must give legal consent, and children over age six need to agree to take part as well.

If you are interested in participating in a clinical trial go to the following on our website:

https://www.adrcnyc.org/dir-clinical-trials.htm

**Note:** If you have any questions or concerns please contact Ira at Ira@asherman.com