Learn the Types and Phases of Clinical Trials

Description

Clinical trials are studies that are conducted to test novel medications, already approved drugs, technologies, or other forms of treatment. Many clinical trials investigate novel methods for detecting, diagnosing, or measuring the extent of disease.

Some are even looking into ways to prevent diseases from occurring. Human volunteers are still used by researchers to evaluate these technologies, and the same regulations apply. Clinical trials are used by doctors to determine whether a new drug, treatment, or combination works and is safe for people to use.

Clinical trials are critical in the development of novel medicines for serious diseases such as cancer. Clinical trials are required for all novel treatments before they can be authorized by the Food and Drug Administration (FDA). Cancer clinical studies might last for years. It can take months, if not years, to determine whether a cancer treatment is effective.

Why do clinical trials exist?

Clinical trials demonstrate what works (and does not work) in medicine and health care. They are the

most effective technique to learn what works in the treatment of diseases such as cancer. Clinical trials are intended to provide answers to the following critical questions:

• Is the new treatment effective in humans? If it does, doctors will examine how effectively it functions. Is it superior to the current treatment? If not better, is it at least as good and has fewer side effects? Or does it aid folks who aren't benefited from existing treatments?

• Is the new treatment risk-free? No therapy or procedure, no matter how common, is without danger. But do the new treatment's advantages outweigh the risks?

• Is this treatment superior to the normal treatment for this disease? Clinical trials can assist in determining whether a new drug or treatment or a novel treatment combination, is more effective than what is now available.

Answering these concerns while exposing as few patients as possible to an unknown medication frequently necessitates multiple clinical trials in different "phases." Each phase is meant to answer specific questions while keeping participants as secure as possible. The results of these phases indicate if the new drug or treatment is reasonably safe and effective.

Pre-clinical (or laboratory) research

Clinical trials are only conducted once pre-clinical studies indicate that the new medicine or treatment is likely to be safe and effective in humans. Pre-clinical investigations, commonly known as laboratory studies, comprise the following:

• Cell studies: These are frequently the initial tests performed on a novel treatment. To see if it works, researchers examine the effects of the new medication on cancer cells cultivated in a lab dish or test tube. This research could be conducted on human or animal cancer cells.

• Animal research: Treatments that show promise in cell studies are then evaluated in live animals on malignancies. This allows researchers to determine how safe the new medicine is for a living being. Pre-clinical studies provide a lot of helpful information, but not all of it. Humans and mice absorb, process, and eliminate medications and therapies in fundamentally different ways.

A cancer treatment that works in mice may or may not work in humans. There could potentially be adverse effects and other issues that did not manifest in mice but could manifest in humans. If the pre-clinical investigations are done, and the treatment still appears promising, the US Food and Drug Administration (FDA) must approve the treatment before it may be tried on humans.

The application for an investigational novel drug (IND)

The research must be approved before a clinical trial may begin. When researchers want to examine medicine in humans, they must file an investigational new drug (IND) application or request with the FDA. The IND application must include particular details, such as • Study results, so that the FDA can determine whether the treatment is safe for human testing.

• Who makes the drug, what's in it, how stable it is, and other details.

• Study protocols, which are detailed outlines for planned clinical research, are assessed to check if participants are being exposed to unnecessary hazards.

• Information about the clinical trial team to determine if they have the knowledge and skills to conduct clinical studies.

The research sponsor must commit to obtaining informed permission from all clinical trial participants. They must also agree to have the study examined by an institutional review board (IRB) and to follow all of the rules for researching investigational new medications.

Clinical trial phases

Clinical trials are typically carried out in stages that build on one another. Each phase is intended to provide answers to specific questions. Knowing the clinical trial phase is crucial because it might give you an indication of how much is known about the treatment under study.

Each stage of a clinical trial offers advantages and disadvantages. Although clinical trials for gadgets and other diseases and treatments exist, medications for cancer patients are used in the clinical trial phases detailed here.

Clinical studies in phase 0: Investigating whether and how a novel medication might function

Even though phase 0 investigations are conducted in humans, they are not the same as the other phases of clinical trials. This phase's goal is to help expedite and streamline the medication approval process. Phase 0 studies may assist researchers in determining if the medications perform as intended. This could save time and money that would otherwise be spent on later phase trials.

In phase 0 investigations, only a few modest doses of a new medicine are administered to a limited number of people. They may investigate whether the drug reaches the tumor, how the drug operates in the human body, and how cancer cells respond to the drug in the human body.

As part of the process, participants in these studies may require further testing, such as biopsies, scans, and blood samples.

Unlike earlier rounds of clinical studies, persons in phase 0 trials have absolutely no chance of benefiting. The benefit will accrue to others in the future. Furthermore, because the medicine doses are minimal, participants participating in the trial face less danger.

Phase 0 studies are not extensively performed, and they would be ineffective for several medications. Phase 0 studies are typically small, with fewer than 15 participants, and the medicine is administered for a brief period of time. They are not essential in the testing of new medicine.

Is the treatment safe in phase I clinical trials?

People are usually involved in the earliest phase of a new drug's development. Phase I trials are conducted to determine the greatest dose of a new medication that can be administered safely without generating serious negative effects.

Although the medication has been evaluated in laboratory and animal trials, the negative effects in humans are unknown. These studies also aid in determining the optimum technique to administer the new therapy.

Phase I clinical trial highlights

• The first few participants in the research are given a very modest dose of the medication and are closely monitored. If the side effects are modest, the following few individuals are given a greater dose. This technique is repeated until doctors identify a dose that is most likely to work while causing an acceptable level of adverse effects.

• Phase I studies look at what the medicine does to the body as well as what the body does with the drug.

• The primary priority is safety. The research team maintains a careful check on the participants and looks out for any serious adverse effects. Due to the small number of participants in phase, I research, unusual side effects may not be observed until later stages of trials, when more people take the medication.

• While some patients may benefit from being on one, the primary goal of a phase I trial is not illness response.

• Placebos (inactive therapies) are not utilized in phase I trials.

• Phase I trials typically include a modest number of participants (up to a few dozen).

• Phase I trials often include people with various forms of cancer, and these studies are typically conducted in large cancer centers.

Phase I trials are the most dangerous. However, some patients benefit from phase I research.

For those suffering from life-threatening conditions, properly balancing the potential dangers and benefits is critical. When all other therapeutic options have been exhausted, people may choose to participate in phase I trials.

Clinical studies in phase II: Is the treatment effective?

If a new medication is determined to be safe in phase I clinical trials, it is next tested in phase II clinical trial to evaluate if it works in specific forms of cancer. The benefit sought by doctors is determined by the treatment's purpose. It is possible that cancer will shrink or disappear.

Or it could suggest that cancer does not grow for an extended length of time or that it takes longer for the disease to return. According to certain studies, the benefit could be enhanced quality of life. Many clinical studies are conducted to determine whether patients who get the new medication survive longer than the general population.

Phase II clinical trial highlights

• In a phase II study, 25 to 100 individuals with the same form of cancer receive the new treatment.

• In phase II clinical trials, everyone receives the same dose and procedure that were shown to be the safest and most successful in phase I investigations. However, in certain phase II research, patients are assigned to different therapy groups at random. These groups may receive different doses or treatments in order to determine which gives the optimal balance of safety and response.

• In phase II trials, no placebos (inactive medicines) are employed.

• Phase II research can be conducted at major cancer centers, community hospitals, or even doctors' offices.

Because phase II trials enroll a larger number of patients, less common adverse effects may be observed. Phase III clinical trials are initiated if enough patients benefit from the medication and the side effects are not too severe.

Is it superior to what's already available in Phase III clinical trials?

Treatments that have been shown to be effective in phase II clinical trials must also be successful in a third phase before they can be licensed for general use. Phase III clinical studies assess the novel treatment's safety and effectiveness compared to that of the current standard treatment.

Because doctors do not yet know which treatment is superior, study participants are frequently assigned at random (referred to as randomized) to either the traditional or innovative treatment.

When feasible, neither the doctor nor the patient is aware of which of the treatments is being administered. This is referred to as a double-blind study. Randomization and blinding are covered in further depth later.

Phase III clinical trial highlights

• The majority of phase III clinical trials involve a high number of patients, usually several hundred.

• These studies are frequently conducted in multiple locations across the country (or even around the

world) at the same time.

• Phase III clinical trials are more likely to be available in community hospitals and doctor's offices, and they endure longer than phase I and II studies.

• Placebos may be employed in some phase III studies, but they are never utilized alone if a medication that works is available. A patient who is randomly allocated to the placebo for part of the study may be offered the usual treatment at some point. Patients in phase III clinical trials, like those in other trials, are continuously monitored for side effects, and therapy is halted if they are too difficult to control.

An application seeking FDA approval: New medication application (NDA)

In the United States, a new drug application (NDA) is submitted to the Food and Drug Administration (FDA) for approval when phase III clinical studies (or sometimes phase II trials) prove that a new drug is more effective or safer than the present treatment. The FDA examines clinical trial outcomes as well as other pertinent information.

The FDA chooses whether to approve the medication for use in patients with the ailment for which the drug was evaluated based on the review. If the new treatment is approved, it often becomes the standard of care, and other medications may be tested against it before they are approved.

If the FDA believes that more evidence is required to demonstrate that the new treatment's benefits outweigh its hazards, it may request additional information or even demand additional research.

What else do we need to know about Phase IV clinical trials?

In phase IV research, drugs approved by the FDA are frequently monitored over a lengthy period of time. Even after testing a new therapy on thousands of patients, not all of the treatment's side effects may be known. Some questions may remain unanswered.

For example, a medicine may be approved by the FDA if it has been shown to minimize the chance of cancer recurring following treatment. But does this suggest that those who obtain it will live longer?

Are there any uncommon side effects that have yet to be observed or side effects that appear only after a person has taken the drug for a long time? These types of concerns, which are frequently addressed in phase IV clinical trials, may require many more years to solve.

Phase IV clinical trial highlights

• Phase IV trials focus on medications that have already received FDA approval. The medications are now ready for doctors to give to patients, although phase IV research may be required to answer critical questions.

• These studies may involve thousands of participants.

• This is often the safest sort of clinical trial because the treatment has already been extensively researched and is likely to have been administered to a large number of people.

• Phase IV trials examine long-term safety. Other features of the treatment, such as quality of life or cost-effectiveness, may be examined in these studies.

You can obtain the medications used in a phase IV trial without participating in the study.

And the care you would receive in a phase IV study is quite similar to the care you would receive if you received the treatment outside of a trial. However, in phase IV studies, you are assisting researchers in learning more about the treatment while also benefiting future patients.

Category

1. Lifestyle

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