Is a Clinical Trial Right for You?

Joining a trial can be a good way to get promising experimental therapies, but you should carefully weigh the potential risks and benefits.

June 18, 2018 By Liz Highleyman

The process of developing new medications is complex, lengthy and expensive. Only a tiny fraction of compounds make it from the laboratory to clinical trials, and less than 10 percent of drugs that enter Phase I studies are ever approved, according to the Food and Drug Administration (FDA).

Preclinical studies: Drug candidates first undergo in vitro (Latin for “in glass”) testing in a laboratory. For example, researchers may test whether a compound kills cancer cells in a petri dish. If these tests are promising, the drug will often be tested in mice or other animals. But activity in a test tube or a mouse does not mean that a drug will work in people.

Phase I: If a compound still looks promising, researchers submit an application to the FDA for designation as an Investigational New Drug. The first human trials usually include 10 to 100 participants, starting with healthy volunteers. These trials look for side effects and collect information about how a drug is processed in the body, known as its pharmacokinetics. Researchers also try to determine which dose will provide the best balance of activity and safety.

Phase II: Mid-level trials typically include a few dozen to a few hundred participants. They are designed to test whether a drug still appears safe in a larger group of people with cancer and to gather preliminary data on efficacy, or how well the therapy works under ideal conditions.

Phase III: The largest and longest trials, usually enrolling several hundreds or thousands of people, aim to determine how well a drug works in the population that will ultimately use it. This often involves comparing the new drug against existing therapies. Data from major Phase III studies—known as pivotal trials—may be submitted to the FDA to support a New Drug Application for approval.

Phase IV: After a drug has been approved and made commercially available, post-marketing studies are done to see how well it works under real-world conditions and to determine its long-term safety and effectiveness. This is important because uncommon side effects may show up only after a drug is used by many people over a long period.

An experimental drug can take years to move from the laboratory to pharmacy shelves, but there

are ways to speed up access to promising therapies for life-threatening diseases.

People who are unable to enroll in a trial and have no satisfactory alternatives can request expanded access, or compassionate use. The FDA grants most expanded access requests, but pharmaceutical companies don't have to provide their investigational drugs outside trials and insurers don't have to pay for them.

Trial Design

A good trial design is critical to ensuring that a study can provide reliable information about how well a drug works. In particular, trials should include enough participants and last long enough to produce statistically significant results, meaning that the findings are unlikely to be attributable to chance alone.

A study's enrollment criteria describe who may participate. Cancer trial inclusion criteria specify the type of cancer, stage of disease and prior treatments. Today, study criteria often include genetic characteristics that predict whether a drug will work. As inclusion criteria become more specific, it gets harder to enroll enough people.

Researchers may be tempted to select participants who are most likely to do well on a new therapy. But it is important to include a range of participants who reflect the population that will actually use the drug, such as people with advanced disease, those with other health problems and people of all ages and from all racial and ethnic groups.

The gold standard for testing new drugs is the randomized controlled trial. In these studies, participants are randomly assigned to receive either the experimental therapy or a comparison intervention, which could be a competing new drug, the currently available standard treatment or a placebo (an inactive mock treatment, such as a sugar pill or saline injection).

Randomization—meaning any trial participant has an equal chance of ending up in any treatment arm—helps ensure that all the groups are otherwise similar. But this also means that not everyone who joins a trial will receive the experimental drug under study.

Another way to reduce bias is double-blind studies, where neither the investigators nor the participants know who is assigned to which treatment arm. In open-label studies, everyone knows who is getting which treatment.

Study endpoints are milestones that must be met for the experimental therapy to be considered a success. The ultimate endpoint for cancer treatment is overall survival. But in some trials—especially those for early-stage or curable cancers—most participants will not die during the course of the study. Progression-free survival (meaning participants are still alive without worsening of disease) and overall response (meaning complete or partial tumor shrinkage) are endpoints that can be measured sooner. Cancer trials increasingly also include patient-reported outcomes, such as quality of life and ability to perform daily activities.

Joining a Trial

When considering a trial, learn all you can about the study and the treatment being tested. Although they are governed by regulations to ensure that they are as ethical and safe as possible, trials of new therapies—especially novel types of drugs—can't offer guarantees. Researchers don't yet know how effective the treatment will be and can't rule out unforeseen side effects.

Before agreeing to join, you should be given information about all aspects of the trial, including its potential risks, and must sign an informed consent document. But informed consent is not a contract—participants have the right to withdraw from a trial at any time for any reason.

Joining a clinical trial may be an obvious choice for people with advanced cancer who have exhausted all available treatment options. Those with less advanced disease may be motivated by altruism and a desire to help advance medical science for future patients.

Trial Pros and Cons

Pros:

- Early access to promising new therapies
- Free drugs and health monitoring
- Expert doctors and leading medical centers
- Satisfaction of helping others
- Advancement of medical knowledge

Cons:

- Inconvenient and time-consuming study visits
- Need to stop or forgo other treatment
- Might not receive experimental therapy
- Experimental therapy might not work
- Risk of side effects

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