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A Cancer Patient’s Guide to Clinical Trials

If you’re considering whether to enroll in a trial of an experimental drug, here’s what you need to know.

by Caroline Chen, Sept. 19, 4:59 a.m. EDT

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Clinical trials are a crucial step in getting new treatments to market. Before a drug can be approved by the U.S. Food and Drug Administration and released widely, manufacturers are required to carry out studies in humans to document that it is effective and to discover any side effects.

Fewer than 5 percent of adult cancer patients enroll in clinical trials. ProPublica has found that the vast majority of participants in these studies are white, even when minorities have a similar or higher risk of getting the cancer that the drug treats.

Most trials are run at academic medical centers and conducted by researchers there. Patients outside those centers often aren't aware that clinical trials are an option, or they may wonder what joining a study entails. For patients who might consider a clinical trial, here are answers to some common questions.

Why should I join a clinical trial?
Drugs being tested in a trial often reflect the most cutting-edge technology and most current understanding of the disease. In the past decade, the cancer field has seen rapid advances in treatments, dramatically changing outcomes for some patients. Participating in a trial gives you early access to a drug that may relieve your symptoms or extend your life.

By participating in a trial, you're also helping scientists find a treatment for your disease. It may take researchers months or even years to recruit the hundreds or thousands of patients needed for a large study. The faster the trial is fully enrolled, the sooner scientists can learn how well the drug works.

Are clinical trials a last resort?
Patients may be eligible to join a trial at any stage of their illness. Some trials seek patients who are newly diagnosed, and others are for people who have already tried other therapies.

Typically, treatments that haven't been approved are tested first in patients who have exhausted all other options. Once approved, those drugs may then go into another round of trials for patients who are newly diagnosed.

Not all trials involve new drugs. Some test approved drugs in new combinations. Those trials may also limit eligibility based on the disease's progression.

What are the risks of joining a clinical trial?
All trials come with risks. If a drug has not been approved, researchers may not fully understand its side effects. It's possible that the treatment could worsen your condition or, in very rare cases, prove fatal.

Testing is usually done in three phases. The first trial is usually the smallest, and its main goal is to make sure the drug is safe. Risks of unexpected side effects may be higher in phase one trials than in phases two or three.

You should ask your doctor to discuss the possible benefits and risks of the study with you to help you decide whether to enroll.

How do I join a clinical trial?
Not every trial may be right for you. Studies have enrollment criteria, which may include your age, what stage your cancer is at and whether you are newly diagnosed or have already taken other treatments.

You don't have to wait for your doctor to recruit you for a trial. You can ask your oncologist what studies you may be eligible for. If you are being treated at a community hospital, your doctor may need to refer you to an academic hospital that can help you enroll in a trial.

Some foundations that support the fight against certain diseases, like the Leukemia and Lymphoma Society, have specially trained staff to connect patients with an appropriate trial. You can also use online search tools, such as this one from the National Cancer Institute or clinicaltrials.gov.

Will I be given a placebo?
A placebo is a "dummy" treatment that has no effect. It's unethical to give patients a placebo if they could benefit from further treatment, so placebos are rarely used in cancer trials. If they are, the researcher who is running the study is supposed to explain that to you.

Studies that use placebos typically are comparing a new drug to what's known as "standard of care," which is the typical treatment that the patient would get outside of a study. One group of patients receives the standard of care plus a placebo, and another gets the standard of care plus the experimental drug.

If there are no available treatments for a disease or patients have exhausted all options, a study may compare an experimental drug to a placebo to see if it is better than no treatment at all.

Will I be paid to join a trial?
Many trials reimburse patients for expenses like travel and parking. Most do not cover lost earnings or family expenses such as day care. Each trial is different, so talk to the trial coordinator to find out what will be covered. Some foundations may provide financial support to cover costs associated with trials.

The manufacturer always provides the experimental drug for free. You have to pay for any already-approved treatments you may take as part of the study, as well as any related care such as hospital stays, but your health insurance may cover part or all of that cost.

How much time does it take to participate in a trial?
Every trial is different. Some can run for just a few weeks, others can last for years, especially if they are measuring a drug's impact on survival.

The impact on the patient's daily life also varies. Some studies may require you to make extra trips to the doctor or undergo additional medical tests, such as a new biopsy.

Some trials are reducing patients' travel by sending nurses to their homes for checkups and measurements.

What is informed consent?
Informed consent is a process that gives you information about the study you are about to participate in. Before the trial starts, researchers should explain to you what the purpose of the study is, what procedures will take place, the possible benefits and risks, and the number of visits or medical tests required. They should also answer any questions you have about the study.

After you understand what the trial will entail, you'll be asked to sign an informed consent form before taking part in the study.

Will my privacy be maintained?
As part of the informed consent process, the study coordinators should explain to you what type of information will be gathered about you, who will be able to view it, how it will be shared and how long it will be kept. When the results of the study are published, no personally identifying information, like your name, will be printed.

What if I cannot find a trial that will accept me?
It's possible that there won't be a trial with enrollment criteria that match your profile. In that case, keep checking with your doctor, as new trials may start in the months ahead. Also, as your circumstances change, new opportunities may arise. For example, you may not be eligible for a trial when you are first diagnosed, but you may become eligible if your cancer relapses.

Can I quit a clinical trial?
Yes, you can end your participation any time, for any reason.

What happens if the FDA doesn't approve the drug?
If the FDA rejects the treatment, that means the manufacturer didn't provide sufficient evidence that the drug is safe and effective. In that case, the manufacturer may stop making the drug, unless it's approved to treat another condition. Sometimes the FDA requests more information, and the drugmaker may extend the trial or start a new one.