Cancer Clinical Trials: The Power of Participation

Clinical trials offer access to cutting-edge treatment and the opportunity to help others while helping yourself.

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The remarkable advances in cancer CARE AND treatment are largely attributable to the countless patients who have chosen to take part in clinical trials over the years. Participating in studies can be a good way to gain early access to the newest therapies, contribute to science and help other people living with cancer. But it's important to weigh the pros and cons.

Despite the benefits, less than 10% of adult cancer patients in the United States participate in treatment trials, and groups such as Black and Latino people, those with lower incomes and rural residents are underrepresented.

"How precise can medicine be when the data showing whether a drug works or not excludes entire populations?" asked Yehoda Martei, MD, a medical oncologist at the University of Pennsylvania, in a recent STAT opinion piece. "Our failure to enroll truly representative populations in clinical trials not only harms patients, it threatens the foundations of innovation and undermines the promise of personalized medicine."

The most familiar clinical trials assess the safety and effectiveness of new therapies in a formalized, stepwise manner (see "Clinical Trial Stages," below). The process of developing new medications is lengthy and expensive, and only a small fraction ever make it from the laboratory to pharmacy shelves. Many drug candidates that show promising activity in a test tube or a mouse don't end up working in humans—but the only way to know is to try.

The gold standard for testing new therapies is the double-blind randomized controlled trial. Randomization means participants could be randomly assigned to any of the study groups, or arms. A controlled trial means patients receive either the experimental therapy or a comparison intervention—for example, an existing standard-of-care treatment or an inactive placebo. Double-blind means neither the researchers nor the participants know who is in which group.

This is all done to reduce bias. Randomization aims to make sure the study groups are as similar as possible—for example, including people of the same ages and disease severity. Using a control intervention gives a better idea of the real effect of an experimental therapy, since some people will naturally improve, worsen or stay the same even without treatment. Blinding minimizes the influence of expectations on the part of researchers and patients—including the well-known placebo effect.

Trial endpoints are prespecified, measurable outcomes used to determine whether a new therapy is effective. Thanks to the efforts of advocates in the early years of the AIDS epidemic, the Food and Drug Administration (FDA) introduced new pathways to speed up access to promising treatments.

The FDA can grant accelerated approval based on surrogate markers deemed likely to predict clinical outcomes, such as overall response rate (tumor regression) or progression-free survival (time to disease worsening), rather than waiting for patients to die. But such therapies are expected to undergo further testing to confirm their clinical benefit—most important, overall survival—and the agency can rescind approval if they fail to measure up.

Other types of trials evaluate new diagnostic methods and treatment strategies for different patient populations. Prevention trials look at screening methods or biomedical interventions to halt the development of cancer or reduce the risk of disease progression or recurrence. Sometimes—as with therapeutic cancer vaccines—the distinction between prevention and treatment can be blurry. Some trials aim to help patients manage symptoms or side effects, improve their quality of life or optimize their health, such as studies of exercise regimens, nutrition and complementary therapies.

For ethical or practical reasons, some studies can’t use randomization, controls or blinding. Observational studies, which don’t randomly assign interventions but instead follow patients to see what happens over time, can also yield valuable information.

Trial Barriers

A good study design is essential to ensure that research yields reliable and relevant data. In particular, trials should enroll enough people and last long enough to produce statistically significant results, meaning the findings are unlikely to be due to chance alone.

It’s also important to enroll participants who reflect the population that will use a new intervention in the real world, including people of both sexes, a range of ages, all racial and ethnic groups and those with coexisting health problems. But the status quo is falling short.

An analysis by the Commission on Cancer and the American Cancer Society Cancer Action Network found that only 7% of patients participated in cancer treatment trials. The enrollment rate rose to 22% at National Cancer Institute–designated comprehensive cancer centers but fell to just 4% at community cancer centers. This is a concern, as around 80% of cancer patients in the United States are diagnosed and treated in community settings.

Lack of awareness is one challenge. Some people don't understand the clinical trial process or may not know how to find relevant studies—and all too often, care providers fail to inform them. One small study that asked women with breast cancer why they declined to participate in trials found that the three main reasons were fear of clinical trials, mistrust of the medical establishment and logistical barriers.

Those barriers include financial considerations. While the experimental therapies tested in clinical trials are free, associated costs can add up.

Weichuan Dong, PhD, of Houston Methodist, and colleagues reviewed electronic medical records from more than 12,000 adult cancer patients in Ohio; only about 5% enrolled in treatment clinical trials. Using a machine learning algorithm to analyze over 400 variables, they found that financial factors, such as income and homeownership—rather than demographics—were the strongest predictors of trial participation.

"Clinical trials save lives, but financial barriers prevent too many patients from participating," Dong says. "Addressing the real-world costs patients face, like transportation, child care and lost wages, can make trials more equitable and ensure advances in cancer care benefit everyone."

Many other studies, however, have found that race and ethnicity matter, with Black people being especially underrepresented. In one recent Phase III trial that led to the approval of a new drug for breast cancer, only 0.6% of participants were Black, even though Black women are 40% more likely than white women to die of breast cancer.

"When the trial participants do not reflect the diverse populations in the real world, the evidence that informs universally adopted cancer treatment guidelines is incomplete," Martei wrote. "This exclusion is not just a moral failure—it is a scientific one."

But expanding trial access and representation requires resources, and recent federal funding cuts for cancer research could make matters worse. Federally funded research fills essential gaps not

covered by pharmaceutical companies, including basic science, early-phase trials and studies of rare and childhood cancers.

Joining a Trial

Whether to take part in a clinical trial is an important decision in your cancer journey. Trial participation can offer many benefits, including early access to promising new therapies and care provided by leading experts (see “Trial Pros and Cons,” below). But there are also potential drawbacks. These include time-consuming study visits, frequent blood draws and scans, the risk of side effects and the need to forgo other therapies. And remember, in a randomized trial, you might not be assigned to the experimental treatment.

If you’re thinking about joining a trial, your cancer care team, support groups and advocacy organizations can be good sources of information about available studies. The National Institutes of Health’s ClinicalTrials.gov website lists open studies for all conditions, including cancer.

When considering a trial, learn all you can about the treatment or other intervention being tested, what other options are available and the potential risks and benefits. Find out the frequency of study visits, the types of testing and monitoring that will be done and whether the trial provides reimbursement for travel and other expenses. Don’t be afraid to ask questions! Before agreeing to join a trial, participants must sign an informed consent document, but this is not a contract—you have the right to withdraw at any time for any reason.

Clinical trials of new cancer therapies can’t offer guarantees. Researchers don’t yet know how effective an experimental intervention will be, and they can’t rule out unforeseen adverse events. But despite this uncertainty, trials can be a gateway to better treatment for yourself and other people facing cancer now and in the future.

Clinical Trial Stages

- Preclinical: Experimental therapies first undergo testing in a laboratory, followed by animal studies. Promising activity at this stage does not necessarily mean a drug will work in humans.
- Phase I: Early feasibility trials evaluate safety, look for common side effects and early signs of antitumor activity and collect information about pharmacokinetics, or how a drug is processed in the body. Dose-ranging studies aim to determine an optimal dose that balances activity and tolerability.

- Phase II: Mid-level trials test whether a new therapy appears safe in a larger group of patients and gather preliminary information about effectiveness, such as tumor shrinkage or changes in biomarkers.
- Phase III: The largest and longest trials further evaluate safety and effectiveness, typically comparing an experimental therapy against other options, such as the existing standard of care. The goal is to show clinically meaningful benefits, such as delayed disease progression, better quality of life or improved survival.
- Phase IV: After a treatment has been approved and is commercially available, post-marketing studies evaluate how well it works in the real world. Less common side effects, for example, may show up only when a therapy is used by more people over a longer period.

Doriot Kim

Trial Pros and Cons

Pros

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

Cons

- Time commitment
- Associated unreimbursed costs
- May need to stop or forgo other treatment
- Might not receive experimental therapy
- Therapy might not work
- Potential side effects

Doriot Kim