The Cancer Health Basics on Clinical Trials

Study participation can be a gateway to better cancer care.

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Developing more effective and better tolerated cancer treatment depends on clinical trials of experimental therapies. Participating in a study can be a good way to access cutting-edge treatment and contribute to science, but it's important to weigh the risks and benefits.

Clinical trials involve multiple steps:

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 trials evaluate tolerability, look for early signs of antitumor activity and collect information about pharmacokinetics, or how a drug is processed in the body.

Phase II: Mid-level trials test whether a new therapy appears safe in a larger group and gather further information about efficacy, such as tumor shrinkage.

Phase III: The largest and longest clinical trials test the safety and effectiveness of a new therapy compared with other options, with the goal of delaying disease progression and improving survival.

Phase IV: After a therapy has been approved, post-marketing studies evaluate how well it works in the real world. Less common side effects may show up only when a therapy is used by more people over a longer period.

The gold standard for testing new medications is the randomized controlled trial. Randomization means that trial participants have an equal chance of being assigned to the different study arms. This reduces bias by making sure that the groups are as similar as possible. A controlled trial means that participants are randomly assigned to receive the experimental therapy or a comparison intervention—for example, the current standard of care or a placebo.

A good study design is essential for ensuring that clinical trials provide reliable and relevant data. It is important for trials to enroll the full range of patients who will use a new therapy in the real

world, including both sexes and people from diverse racial and ethnic groups.

Joining a Clinical Trial

Your cancer care team, support groups and advocacy organizations can be good sources of information about available trials. The National Institutes of Health’s ClinicalTrials.gov website lists open studies for all diseases and conditions.

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

Benefits of trial participation include early access to promising new therapies, care delivered by leading experts and altruism—knowing you are contributing to science and helping others. Drawbacks can include time-consuming study visits, the need to forgo other therapies and the risk of side effects. Remember that in a randomized trial, you might not get the experimental treatment.

Clinical trials of new cancer therapies can’t offer guarantees. Researchers don’t yet know how effective an experimental medication 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.
