# clinical trials answers

## Clinical trial enrollment common terms

Are you or someone you love currently enrolled in or considering a clinical trial of cancer immunotherapy? Understanding your options, trial types, and terms can help in your search for the answer to cancer.

## Three phases of clinical trials

| phase | what it is | why it matters |
|---|---|---|
| **phase I trial** | • Very early trials, usually composed of fewer than 50 patients<br>• The primary purpose of a phase I trial is to determine whether a treatment is safe<br>• Often, all patients in a phase I trial receive the same treatment<br>• May be used to determine preliminary information about a treatment: find a safe dose, decide route of administration (by mouth, intravenous, etc.) | • Therapies in phase I trials are still in the very early stages of testing<br>• While phase I trials can sometimes show that a treatment is effective, they are used primarily to determine if a treatment is safe |
| **phase II trial** | • Intermediate-stage trials, usually composed of fewer than 100 patients<br>• Purpose is to determine if a treatment is effective for a particular type of cancer<br>• May also be used to determine how the body processes a drug and/or the effects a drug has on different organs or systems of the body | • Phase II trials are more advanced than phase I trials<br>• Phase II trials may enroll patients with a specific disease (e.g., type of cancer) to see if the treatment being studied has a positive effect in that disease |
| **phase III trial** | • Large-scale trials, usually composed of >100 to several thousand patients<br>• Purpose is to compare a new treatment (or suggested treatment) with either a placebo product or the current standard of care | • Phase III trials form the basis for the approval of a treatment by the FDA<br>• Patients who are receiving the new experimental treatment in a phase III trial may see benefit from their treatment |
| **phase I/II or phase II/III combined trial** | • Some researchers design trials that combine phases I/II or phases II/III into a single trial. This happens in order to simplify or shorten the trial design | • Patients may be allowed to move directly from one phase of a trial to the next<br>• This transition between trial phases helps to quickly answer important questions about the safety and efficacy of a given treatment |

## Types of clinical trials

| term | what it is | why it matters |
|---|---|---|
| **randomized trial** | • A type of clinical trial in which patients are assigned to groups on a random basis where they receive either the new experimental treatment or the established standard of care (or a placebo) | • In a randomized trial, patients have an equal chance of receiving treatment with the new experimental therapy or the established standard of care (or a placebo)<br>• Neither you nor your doctor can choose which treatment you receive, which helps to ensure the trial produces valid results |
| **double-blind trial** | • Trials in which neither you (the patient) nor the study researchers will know if you are receiving the experimental treatment or the placebo | • Double-blind trials are considered a "gold standard" of clinical research because they offer unbiased assessment of a treatment's effectiveness |
| **open-label trial** | • Trials in which both the clinicians and the patients know if the patient is receiving the experimental drug or the standard treatment/ placebo | • Following a double-blind portion of a clinical trial, patients may have the option to participate in an open-label portion of the trial where they know they will be getting the experimental treatment |
| **single-blind trial** | • Trials in which you (the patient) will not know if you are receiving the experimental treatment but the study researcher will know | • Single-blind trials are not as common as double-blind trials |

## Clinical trial terms

| term | what it is | why it matters |
|---|---|---|
| **control group** | • The group in a randomized trial that receives either standard of care therapy or placebo—not the experimental treatment being studied | • Neither you nor your doctor can choose whether you are part of the experimental treatment group or the control group. This helps ensure that the trial produces valid results |
| **exclusion criteria** | • Characteristics that disqualify patients from inclusion in a clinical trial<br>• Patients may be excluded because of gender, age, type of disease being treated, previous treatments, and other medical conditions | • If even one exclusion criteria is present, a patient will not be eligible to participate in a given trial<br>• In clinical trials of cancer immunotherapy, certain immune function tests may be used as exclusion criteria |
| **inclusion criteria** | • Characteristics that patients must have in order to be considered for a clinical trial<br>• May include gender, age, type of disease being treated, previous treatments, and other medical conditions | • Inclusion criteria help identify suitable participants for clinical trials<br>• All inclusion criteria must be met in order for a patient to be considered for a trial<br>• In clinical trials of cancer immunotherapy, certain |
| **placebo** | • A "dummy" treatment that is not active but is designed to look like the treatment being tested | • Placebos are rarely used in cancer clinical trials; they are used when there is no standard treatment<br>• You will always be told if the trial you are in uses a placebo |

## How effectiveness is measured in cancer clinical trials

| term | what it is | why it matters |
|---|---|---|
| **complete response (CR)** | • The complete disappearance of a tumor or cancer lesion as a result of treatment | • Patients who achieve a CR may not be completely cured; however, in clinical trials, patients with a CR are considered to be in "complete remission" |
| **durable response (DR)** | • Length of time that a patient experiences a complete or partial response as a result of treatment | • In clinical trials, a durable response suggests that a treatment may have a lasting effect |
| **median overall survival (OS)** | • The time point at which 50% of patients in a trial are still alive | • This time point provides a direct assessment of benefit for patients in the trial |
| **overall survival (OS)** | • The length of time from either the date of diagnosis or the start of treatment that patients are still alive | • Demonstrating that a treatment improves overall survival is a "gold standard" of clinical research |
| **objective response (OR)** | • An OR denotes either a partial or a complete response based on the criteria outlined in the study<br>• The criteria may include the amount that the tumor is reduced and the minimum time period | • In a clinical trial, OR can be used to show if a treatment is effective |
| **partial response (PR)** | • A partial decrease in the size of a cancerous tumor or lesion or a decrease in the total number of cancer lesions | • Although the PRR does indicate that some disease remains, it also shows that the treatment may be working and should be studied further |
| **progression-free survival (PFS)** | • The length of time during and after the treatment of a disease (e.g, cancer), that a patient is alive with disease that has not progressed or gotten worse | • In cancer clinical trials, measuring PFS is an important way to see how well a new treatment works |

## How effectiveness is measured in cancer immunotherapy clinical trials

Immunotherapies for cancer treatment work differently than chemotherapy or radiation. With immunotherapy, your body must first build its immune response before acting on your cancer. Because of this, patients receiving immunotherapy may go through an initial period where their cancer stays the same, or even progresses (gets worse), before it improves. Different criteria are therefore used in cancer immunotherapy trials to assess if a treatment is working.

| term | what it is | why it matters |
|---|---|---|
| **immune-related complete response (irCR)** | • The complete disappearance of all tumors identified at the beginning of the trial, as well as any new tumors | • The irCR allows for a continuous, and therefore more complete, assessment of how cancer responds to treatment over the course of the clinical trial |
| **immune-related partial response (irPR)** | • A decrease of ≥50% in the total tumor size and/or total number of tumors since the start of the trial | • Patients may still have an irPR even if they develop new lesions or tumors, as long as their disease is improving overall |
| **immune-related stable disease (irSD)** | • A response that does not meet the criteria for irCR or irPR but also does not indicate that cancer is getting worse | • Stable disease where cancer is no longer progressing can indicate that a given immunotherapy is effective |
| **immune-related progressive disease (irPD)** | • An increase of ≥25% in total tumor size and/or number of tumors since the start of the trial | • During immunotherapy treatment, patients may see a benefit from treatment after a period of stable disease—or even temporary worsening<br>• Some experts therefore recommend that assessments of progression be performed at least twice, with each assessment at least 4 weeks apart |

**Access this clinical trial terms list to help stay informed on TheAnswerToCancer.org**

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