



Understanding a Cancer Research Study

Some information about cancer and its treatment can be very detailed and hard to understand. There are many clinical trials and research papers about cancer and you may be reading those to learn more. There are many abbreviations and terms used in these studies that can be hard to understand. Most providers are taught how to interpret and write these articles at some point during their education and still may have trouble explaining them. This article describes what a cancer research study is and lists terms often seen in studies. This may help you better understand what you are reading.

What is a cancer research study?

Cancer research studies are research done to learn more about cancer or its treatments. One of the most common cancer research studies is called a **clinical trial**. A clinical trial is a study that compares group(s) of patients receiving a new or experimental intervention or treatment (called the experimental group) with group(s) receiving the standard of care or a placebo (the control group). The intervention can be a medication, support group, some type of education, a vaccine, or any treatment. The trial is explained to each participant by a provider. All people in the trial must receive and sign a consent form. All trials are reviewed by an impartial group called the Institutional Review Board (IRB) for safety and quality control.

A clinical trial cannot test everyone in the population. The group of patients being studied is an estimate of how the entire population would respond to the therapy. Statistical tests are used to analyze the study results, which can include survival rates, survival in months/years, time to disease progression, and quality of life measures, among other things. Researchers use different measures and tests depending on what is being analyzed.

One important fact to remember is that a trial can only truly answer the question(s) it was designed to answer. Sometimes researchers will see something else interesting happening in a trial. These findings would then need to be proven in another trial specifically designed to look at that finding.

Terms Used in a Cancer Research Study

Clinical Benefit Rate: Also called CBR, this value is the total number (or percentage) of patients who achieved a complete response, partial response, or had stable disease for 6 months or more. Basically, this is the number of patients who had any benefit from the intervention.

Complete Responders: This is the number of patients whose tumors disappeared after the intervention. This might also be called "complete remission."

Confidence Interval (CI): Any result in a clinical trial is only an estimate of the entire population. A study gives a confidence interval (CI) as a range that would reflect the true effect on the entire population. This value tells us how precise our statistical calculation is and gives us an estimate of the amount of error involved in our data. For example, in "overall survival of 81% (95% CI 78%-83%)": 81% is the mean overall survival of the group, with a 95% likelihood that the population's result will fall into the range of 78%-83% (the size of the range is called the standard error). A 95% CI is equivalent to a p-value of 0.05, while a 90% CI is equivalent to a p-value of 0.01 (see p-value definition below).

Control Group: This is the group of patients that is compared to the experimental group. In some studies, the control group might be a group of healthy individuals who are similar in age and other demographic characteristics; in others, it may be a group of patients with the same disease, receiving the standard therapy. Placebos are not often used in cancer clinical trials.

Double-Blind: A trial uses double blinding to prevent the patient or his/her healthcare provider from knowing what treatment the patient is getting. This prevents any "placebo effect," or the chance that the result is due to the patient or provider thinking they are getting the medication or not. An example of double-blinding would be giving the control group a sugar pill (the

placebo), while the experimental group gets the new medication. In this case, the pharmacist would be the only one who knows who gets the placebo or the new medication (and he or she isn't telling!). A study can be blinded (i.e. single blinding) by allowing the provider to know who is getting the experimental drug but the patient does not know what they are getting. Double-blind studies are the best and most reliable type of study. It allows for honest, unbiased reporting from patients in the study. The above example of a sugar pill as a placebo is just to give you an idea of how double-blind studies work. Often in cancer clinical trials, the control group is given the standard treatment, which has already been tested and approved for treatment. Your safety, whether you are in the control group or the experimental group, is always the highest priority in any research study.

Experimental Group: This is the group of patients that gets the intervention being tested. It is compared to a control group, which may get the standard therapy or placebo.

Hazard Ratio (HR): The HR is a way to summarize the difference between two "survival curves." These survival curves are shown on a graph during and after a trial. Survival is shown with these two curves: one representing the experimental group, and one the control group. The rate of survival changes over time. The HR is a numerical representation comparing the two survival curves. If the two survival curves are the same, the HR is 1 and there is no survival difference between the two groups. An HR over 1 means the experimental treatment is less effective than the control treatment and therefore does not contribute to survival; the higher the value, the less effective the experimental therapy is in regard to survival. If the HR is below 1, survival is greater with the experimental therapy; the lower the value, the more survival benefit from the experimental therapy.

Median: The median is the "middle of the pack." For example, when looking at the number of years since treatment, the median is the time when half of the patients have had more years since treatment and half have less. For instance, if the patients were 2, 4, 6, 10.8, 12, 12 and 14 years since treatment, 10.8 is the midpoint or the median. This is different from the mean, which would be the average time since treatment.

Mean: The mean is the average of the group.

Mode: The mode is the value (number) that appears the most. A set of data can contain more than one mode, or it can contain no mode at all.

Objective Tumor Response Rate: This is the total number of partial responders (the number of patients whose tumors decreased in size) and complete responders (the number of patients whose tumors disappeared after the intervention) combined.

Overall Survival (OS): Also called OS, this refers to a percent, number, or time of survival of all patients, regardless of whether they are disease-free or have active disease.

P-Value: P-value is a difficult term to understand, and requires some understanding of the null hypothesis. The null hypothesis states that there is no difference between the two groups being studied and the p-value is used to prove or disprove this. P-value is a statistical test used to measure how much evidence there is against the null hypothesis and prove statistical significance. Researchers use a p-value of 0.05 or less to say that the intervention had an effect and is statistically significant. So a p-value of <0.001 would be statistically significant. But, you can remember that the lower the p-value, the more convincing the result (See "power" for another caveat).

Partial Responders: This is the number of patients whose tumors decreased in size. Some studies will dictate what percentage of decrease is required to count as a partial response.

Placebo-Controlled: A placebo-controlled trial compares the experimental intervention to a placebo. In this method, everyone gets something; for example, the experimental group would take 2 doses a day of the medication being tested. The placebo group would take 2 doses of a sugar pill a day, although both groups would be "blinded," meaning they do not know if they are getting the sugar pill or the real medication. Placebos are rarely used in oncology trials.

Power: The power of a study is its ability to detect a real difference in the results of the study groups. It is used in planning a study and dictates the number of participants needed to detect a predetermined difference in the results. If a study is "underpowered" because it has too few participants, it may not be able to detect a difference. It can be a reason that the resulting p-value is not statistically significant.

Progression-Free Survival (PFS): Also called PFS, this refers to the number, percent, or time of survival (in days, months, or years) before the patient's disease progresses/recurs.

Randomized: In a randomized trial, participants are randomly assigned to one of the treatment arms. This is often done by a computer, with the process being similar to the flip of a coin. The patients or their providers do not have any choice or control over which treatment group they are assigned to.

Retrospective Study: A retrospective study looks back at what effect something had on a group. For example, if I want to know how symptoms of lung cancer predict stage at diagnosis, I could look back at the records of people with lung cancer to determine what symptoms they reported when they were diagnosed, and correlate this with their stage. This is not as reliable as a trial that follows people going forward over time.

Stable Disease: This is the number of patients whose tumors did not grow or shrink. It is typically used with a defined time period; for example, the trial may specify "stable disease for 6 months or more."

Statistically significant: This term is used to determine whether the intervention (drug, support group, vaccine, etc) is the cause of the statistical difference in outcomes between the two studied groups, or if the outcomes could have differed just by chance. Researchers use a p-value of 0.05 or less to say that the intervention had an effect and is statistically significant. It is not enough to compare the result of one group versus the result of the other group. Many other variables are taken into account, including the number of participants, follow-up time, and how the outcome applies to all the participants. Having a statistically significant value is crucial in order for a clinical study to determine whether or not the intervention is effective. You will see many studies report results that "trend towards statistical significance." While this trend can help generate further research questions (called hypothesis generating), it does not mean an intervention was effective. Having said that, just because an intervention does not meet the criteria for statistical significance, does not automatically mean it doesn't carry some clinical importance for a given patient or group of patients.

These are some of the most common terms used in cancer research studies, especially in a clinical trial. It is important for you and your caregiver to talk with your care team about any questions or concerns you might have about being involved in a clinical trial. The treatments that we use today are a result of patients taking part in trials in the past. We are always trying to find better treatments to treat or cure cancer. There are also studies to find less toxic treatments, which can cut down on side effects. Research studies and trials are very rewarding for both the patient and the provider. The patient may benefit from new treatment options and, at the same time, help future cancer patients in their fight against this disease. Learn more about clinical trials at [OncoLink](#).

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