In clinical trials, both research concerns and patient wellbeing are important. To help protect patients and produce sound results, research with people is carried out according to strict scientific and ethical principles. These include:

Each clinical trial has an action plan (protocol) that explains how it will work.

The study's investigator, usually a doctor, prepares an action plan for the study. Known as a protocol, this plan explains what will be done in the study and why. It outlines how many people will take part in the study, what medical tests they will receive and how often, and the treatment plan. The same protocol is used by each doctor that takes part.

For patient safety, each protocol must be approved by the organization that sponsors the study (such as the National Cancer Institute) and the Institutional Review Board (IRB) at each hospital or other study site. This board, which includes consumers, clergy, and health professionals, reviews the protocol to try to be sure that the research will not expose patients to extreme or unethical risks.

Each study enrolls people who are alike in key ways.

Each study's protocol describes the characteristics that all patients in the study must have. Called eligibility criteria, these guidelines differ from study to study, depending on the research purpose. They may include age, gender, the type and stage of cancer, and whether cancer

patients who have had prior cancer treatment or who have other health problems can take part.

Using eligibility criteria is an important principle of medical research that helps produce reliable results. During a study, they help protect patient safety, so that people who are likely to be harmed by study drugs or other treatments are not exposed to the risk. After results are in, they also help doctors know which patient groups will benefit if the new treatment being studied is proven to work. For instance, a new treatment may work for one type of cancer but not for another, or it may be more effective for men than women.

Cancer clinical trials include research at three different phases.

Each phase answers different questions about the new treatment. Phase I trials are the first step in testing a new treatment in humans. In these studies, researchers look for the best way to give a new treatment (e.g., by mouth, IV drip, or injection? how many times a day?). They also try to find out if and how the treatment can be given safely (e.g., best dose?); and they watch for any harmful side effects. Because less is known about the possible risks and benefits in Phase I, these studies usually include only a limited number of patients who would not be helped by other known treatments.

Phase II trials focus on learning whether the new treatment has an anticancer effect (e.g., Does it shrink a tumor? improve blood test results?). As in Phase I, only a small number of people take part because of the risks and unknowns involved.

Phase III trials compare the results of people taking the new treatment with results of people taking standard treatment (e.g., Which group has better survival rates? fewer side effects?). In most cases, studies move into Phase III testing only after a treatment shows promise in Phases I and II. Phase III trials may include hundreds of people around the country.

In Phase III trials, people are assigned at random to receive either the new treatment or standard treatment.

Researchers assign patients by chance either to a group taking the new treatment (called the treatment group) or to a group taking standard treatment (called the control group). This method, called randomization, helps avoid bias: having the study's results affected by human choices or other factors not related to the treatments being tested.

In some studies, researchers do not tell the patient whether he or she is in the treatment or control group (called a single blind study). This approach is another way to avoid bias, because when people know what drug they are taking, it might change the way they react. For instance, patients who knew they were taking the new treatment might expect it to work better and report hopeful signs because they want to believe they are getting well. This could bias the study by making results look better than they really were.

Why Do Phase III Clinical Trials Compare Treatment Groups?

Comparing similar groups of people taking different treatments for the same type of cancer is another way to make sure that study results are real and caused by the treatment rather than by chance or other factors. Comparing treatments with each other often shows clearly which one is more effective or has fewer side effects.

Another reason Phase III trials compare the new treatment with standard treatment is so that no one in a study is left without any treatment when standard treatment is available, which would be unethical. When no standard treatment exists for a cancer, some studies compare a new treatment with a placebo (a look-alike pill that contains no active drug). However, you will be told if this is a possibility before you decide whether to take part in a study.

Your Doctor Can Tell You More

If you have any questions about how clinical trials work, ask your doctor, nurse, or other health professional. It may be helpful to bring this booklet and discuss points you want to understand better.