Phase 3: Comparison

Phase 3: Comparing the new treatment or intervention with standard of care.

Once a treatment or intervention shows effectiveness and safety in a specific type of cancer in a Phase 2 trial, the next step is a Phase 3 trial. The basic principle of a Phase 3 trial is to compare the new treatment or intervention given to one group of patients against the current standard treatment or intervention given to another group of patients. Sometimes the new treatment or intervention is added to the standard of care to see if the new combination is better when compared to the standard.

The goals of a Phase 3 clinical trial are to:

- Determine if the new treatment or intervention is more effective than the standard of care
- Compare how long the response to treatment lasts in each group of patients
- Compare how long patients live with their cancer in each group
- Compare the side effects, tolerability, and safety of the new treatment or intervention versus the standard of care
- Compare if there are any differences in the day-to-day quality of life for each group

A large number of patients, hundreds to several thousands, will take part from many cancer centres and often many countries around the world.

Patients are assigned to either the new treatment or intervention the experimental group) or the standard of care (the control group) by a process called randomization. A computer or a random number chart will determine if a patient is assigned to the experimental group or control group.

Randomization makes sure that all patients have an equal chance of being in either group and that patients are not chosen for either group in a way which could affect the clinical trial results. This process ensures the clinical trial is well done and leads to more reliable results.

When no standard treatment exists for comparison, a placebo might be used. Using a placebo allows the oncologist and clinical trials research team to fairly assess the trial results. We know that assessment of response and side effects can be affected simply by knowing if a patient is getting the actual treatment or placebo.

Patients are always told what their chances are of getting a placebo. Usually the patient, the oncologist, and the clinical trials research team do not know if the patient is receiving the actual treatment or placebo. This is called blinding. It is important to know that, in certain circumstances, such as a medical emergency, patients can be "unblinded" to reveal whether a patient is taking the actual treatment or placebo.

If the actual treatment shows positive results, the patients who were given the placebo may be allowed to switch over to the actual treatment. At this point, the clinical trial may proceed to compassionate release programs; this may allow further patients to receive the new treatment as part of an expanded access clinical trial until the approval process is complete. Once the treatment or intervention is approved, it may proceed to Phase 4 testing.