

# Understanding Clinical Trials



## What is a clinical trial?

Every new treatment must be thoroughly tested to make sure it is safe and effective at treating cancer. A clinical trial is a carefully controlled medical research study with people. Clinical trials find new ways to prevent, diagnose or treat disease. Clinical trials test new treatments or devices in people with cancer to make sure they are safe, and they work. They also compare different treatments. All new treatments must go through several phases of clinical trials before their benefits and risks can really be known. This process can take many years from start to finish.

## Pre-clinical studies



Pre-clinical studies are the experiments that are done before the drug is given to people. They are usually done in a laboratory. These experiments are used to discover new cancer medicines and test them before they are tested in people. The researchers look to see if the new medicine kills cancer cells and how it might work in people. When a medicine has passed pre-clinical testing, it might be tested in a clinical trial.

## Types of clinical trial

There are two main types of clinical trials:

**Interventional clinical trials** aim to find out more about an intervention like a new medicine, medical treatment, or device.

**Observational studies** aim to observe what happens to patients in different situations. There are no treatments or interventions.

## Interventional clinical trials

There are 4 phases of interventional clinical trials, which are described below:

### Phase I – Safety and dose



Phase I clinical trials are also called ‘first in human trials’. They involve small numbers of patients. They are used to see whether the new treatment is safe for people. Phase I trials also look at how the new

medicine works in people, how the body breaks down the medicine, how much medicine is needed, and if there are any side effects.

### Phase II – Effectiveness and dose



Phase II clinical trials test the new medicine in a larger group of patients. They look at how well the medicine works at different doses, and its safety in patients. When phase II clinical trials are completed, the decision will be made whether phase III clinical trials can start. A new medicine will only start phase III clinical trials if there is a very good chance of it meeting the strict guidelines of the regulatory authorities.

### Phase III – How does the treatment compare to standard care?



Phase III clinical trials compare the effectiveness and safety of the new medicine with either the standard existing treatment or a dummy treatment (placebo). Phase III clinical trials may also look at the new medicine in different patient populations and different doses and combinations of medicines.

These trials are usually randomised and sometimes blinded so that the patients (and the doctor) do not know which treatment they are receiving to avoid bias when looking at the results. Information from phase III clinical trials is used to show the benefits of the new medicine over the current standard of care to see if it is better.

Information from phase III clinical trials is used by pharmaceutical companies to apply to government agencies for a license to sell the new medicine.

### Phase IV – Real world data collection

Phase IV is the collection of information on the use of the new treatment in routine patient care (i.e., after the medicine has been given a license). Patients in the real world often have other illnesses, take other medications, and have real-life issues that may not have been seen in clinical trials.

**Basket trials** involve a single new medicine or a combination of medicines that is studied across different types of cancer.

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## From smaller to larger clinical trials

Phase	I	II	III
	The first studies in humans	Looks at the safety and effectiveness of the treatment	Compares the safety and effectiveness of the new treatment to existing treatment
No. of patients	10s	100s	1000s
Pluses	Early access to new treatments	The new treatment is effective and safe	The new treatment is effective and safe and better than existing treatment
Minuses	Safety and effectiveness unknown; could be toxic and not work	Best dose of medicine not known	If the trial is randomised, you might only have a 50% chance or less of getting the new medicine
Location	A phase I research clinic	Few hospitals in a few countries	Many hospitals world-wide

## Observational studies

There are two main types of observational study:

- Case control studies look at two groups of people. One group has cancer (the cases), the other does not (the controls). The groups are compared with a certain characteristic, e.g., being overweight, to see if the characteristic is more frequent in the cases compared to the controls.
- Cohort studies follow a group of people over time to assess the incidence of a disease and the effect of risk factors.

For more information about the treatment options for kidney cancer, read

[My Treatment My Choice clinical trial basics](#) on the IKCC website.

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## Good to know!

### Placebo

A dummy treatment that is designed to be harmless and to have no effect. It looks, smells, and tastes like the treatment being tested, so that the patients in the trial do not know if they are taking the dummy treatment or the treatment itself, i.e., they are blinded to the treatment they are taking. The effects of the new treatment are compared to the effects of the placebo.

If you are thinking of taking part in a trial with a placebo group, you need to think about how you will feel if you find out at the end of the trial that you were given the placebo. Some trials give the new treatment to the placebo group after the trial has ended or swap the treatment and placebo groups during the trial. So even if you are in the placebo group at first, you might still get the new treatment later. When you ask about a clinical trial, be sure to ask about whether any patients will receive a placebo.

There are many clinical trials for kidney cancer around the world. For information on finding a clinical trial, contact your local patient organisation or see: [www.ikcc.org](http://www.ikcc.org).

