



# Understanding Clinical Trials and Research

A guide for people affected by cancer

Practical and  
support  
information

Cancer Council Helpline

**13 11 20**

[www.cancercouncil.com.au](http://www.cancercouncil.com.au)

## Understanding Clinical Trials and Research

A guide for people affected by cancer

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### Note to reader

Always consult your doctor before beginning any health treatment. This booklet is intended as a general introduction to the topic and should not be seen as a substitute for your doctor's or other health professional's advice. However, you may wish to discuss issues raised in this booklet with them. All care is taken to ensure that the information in this booklet is accurate at the time of publication.

### Cancer Council NSW

Cancer Council is the leading cancer charity in NSW. It plays a unique and important role in the fight against cancer through undertaking high-quality research, advocating on cancer issues, providing information and services to the public and people with cancer, and raising funds for cancer programs.

This booklet is funded through the generosity of the people of NSW. To make a donation to help defeat cancer, visit Cancer Council's website at [www.cancercouncil.com.au](http://www.cancercouncil.com.au) or phone 1300 780 113.



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# Introduction

Cancer research is an essential part of health care. If you have been diagnosed with cancer – or you are the carer of someone with cancer – you might be interested in finding out about a clinical trial or other cancer research you or your family member can participate in.

There are many types of research, including clinical trials of new medicines and population studies about cancer. Trials and research studies increase our knowledge about cancer and help in the search for better outcomes for patients and families.

This booklet aims to help you make an informed decision about participating in cancer research. The first section covers general information about different types of research. The second section is about getting involved in a clinical trial or research. It covers practical considerations if you want to participate and explains how you can be assured that research studies are safe and reliable.

We hope this information will answer some of your questions and help you think about more questions you want to ask your doctors or other health care professionals.

This booklet does not need to be read from cover to cover – just read the parts that are useful to you. Some medical and research terms that may be unfamiliar are explained in the glossary. You may also like to pass this booklet on to your family and friends for their information.



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# What is health research?

Health research refers to the wide range of scientific investigations and experiments that aim to answer questions, prove ideas and increase knowledge about human health, illness and health care.

Without health research, the medical treatments and health programs available today wouldn't exist. However, researchers still have an ongoing challenge to find better methods of prevention, diagnosis and treatment for all the diseases that affect humans.

## What is cancer research?

Cancer is a term for more than 200 diseases that have different causes and methods of treatment. Cancer research focuses on improving our understanding of the different types of cancer, and developing better ways to prevent, diagnose and treat these diseases. Cancer research includes population studies, laboratory studies and clinical trials (see below).

## Types of health research

There are three main types of health research: population research, laboratory research and clinical research. People affected by diseases such as cancer are mainly involved in clinical research.

**Population research** – This is also known as epidemiology. It is concerned with the causes and effects of diseases in groups of people (populations). Scientists working in epidemiology (epidemiologists) look for reasons that people get sick.

This often leads to recommendations for ways to reduce disease. Epidemiologists also compare the health of different groups of people. For example, they look at how diseases affect men and women, the rich and the poor, the young and the old, or people living in different places. See page 10 for more information.

**Laboratory research** – This is often the starting point for clinical research. Scientists work in laboratories where they can observe and experiment with cells, enzymes and DNA from humans and animals, or with disease-causing agents such as chemicals, bacteria and viruses. Scientists also study and develop new drugs in the laboratory. See page 13 for more information.

**Clinical research** – This focuses on the causes, diagnosis, treatment and prevention of illness in individuals. It is usually carried out in a clinical setting such as a hospital or outpatient clinic, and it often requires patient participation. It includes clinical trials, which test new ways of preventing, diagnosing and treating diseases. See page 18 for more information.

## Stages of research

Research can take many years and passes through several stages. For example, new treatments often take 10 years or longer in research and development before they're approved for general use. Before people are asked to participate in a study, researchers do a lot of background work to decide what they will test and how they will reliably test it. Researchers also need to have their study approved by different committees before it can begin.

When a study is over, the researchers examine the results and draw conclusions about their work. They are then able to present their findings at conferences and publish detailed articles in scientific or medical journals. This adds to existing scientific and health information. Eventually, this new knowledge helps to change health policy and the way diseases are treated.

## The importance of participation

People are crucial for health research. Patients, carers, healthy people, adults and children all make an important contribution to our understanding of health and disease. People participate in different ways, including:

- consenting to their medical records being accessed
- filling in surveys
- taking medication that has never been given to people before
- agreeing to be examined regularly by doctors.

The need for human participation in research is ongoing, as it is necessary for scientists and clinicians to continually expand and improve medical knowledge and care. For information about getting involved in cancer research, see pages 43–56.

## Who can participate in research?

Research and clinical trials are open to adults and children, however children under 16 need their parent's or guardian's permission to be involved. While most cancer research is directed at cancer patients, some studies target carers, family members or people not affected by cancer.

It is important that people of all ages and social, economic and racial backgrounds take part so that the results reflect Australia's diverse population.

## How many people participate in cancer research?

The participation rate of adults in cancer clinical trials is low.

Currently in Australia it is about one in 20 cancer patients.

About 85% of children participate in trials even though there are far fewer children than adults who are diagnosed with cancer. This has led to a great improvement in children's survival rates because children have been able to access promising treatments, and the evidence for their effectiveness has been obtained quickly.

The number of people participating in other types of cancer research is unknown, but it is possibly higher than the clinical trial participation rate. This is because other research may need people during or after their treatment, there may be fewer restrictions and risks, and the time commitment is usually shorter.

If more people get involved, researchers can obtain their results and bring new treatments and tools into practice faster.

### Where does research take place?

Research is carried out in different places such as hospitals, laboratories and universities. You may not need to leave home to participate in some studies – medicines might be posted to you, or you might be asked to fill in a survey or have a telephone interview.

## Research safety

Understandably, people want to know if there are any risks to them participating in a study. Researchers must follow strict guidelines to make sure studies are as safe as possible for everyone involved. This is called their duty of care.

All studies need to be approved by specially appointed research and ethics committees before they can begin. As part of this process, researchers describe risks, such as possible side effects, that they predict might occur. They must also explain how they will reduce these risks and what will be done if problems occur.

However, it is not always possible to know exactly how study participants will be affected. Depending on what happens during a study, researchers may stop or change a study to make it safer. For more information, see the chapter *Regulating research* on page 40.

## Who funds cancer research?

Research funding comes from many sources:

### **National Health and Medical Research Council**

**(NHMRC)** – This is the Australian Government’s principal funding body for medical research. Highly competitive NHMRC grants are awarded to researchers on the basis of their ability to answer high priority questions about human health.

**Medical research institutions and clinics** – These often use their own resources to support research.

**Polymakers and government** – The government and their advisers often require scientific information when making decisions about health programs. They sometimes provide funding for independent research on specific policy questions.

**Cancer Institute NSW** – It has a competitive grants program to fund many types of research and to employ cancer trials staff.

**Cancer charities** – Cancer Council and other charities receive donations from the public and grants from both public and private sectors. This funds their own research and supports research carried out by other institutions.

**Private sector** – Companies that produce medicines and medical equipment, such as pharmaceutical companies, need to run trials to establish the safety and effectiveness of their products before they can apply for licences to sell them. Private companies may also fund research in partnership with a university or other research institution, or for goodwill (philanthropic) reasons.



Cancer Council NSW funds Strategic Research Partnerships grants for clinical research into cancers with poor outcomes, such as pancreatic cancer, liver cancer and glioma. The research is carried out by universities or research institutions. The grants run over a five-year period and aim to put new research findings into clinical practice faster. This is called translational research (see page 23).



# Population research

Epidemiology is the study of diseases in different groups of people (populations). It finds patterns and trends of health and illness to work out why certain diseases (e.g. cancers) occur in some people but not in others. For example, it compares health between people who have different occupations, live in different regions or come from different countries.

Three key areas of epidemiology include:

- assessing health care in different places
- finding the causes and risk factors of diseases
- predicting how people will be affected by illness in the future.

Population studies can be retrospective or prospective. A retrospective study gets information about people from the past, for example, from their medical records. A prospective study identifies people and then follows them forward in time to see how their health changes.

## Health services research

One part of epidemiology focuses on examining health services such as hospitals, specialists and allied health care practitioners. It investigates their quality and how easily they are accessed.

Health services research is valuable for improving patients' access to health care at all stages of disease. It also helps to improve patients' safety and their experience of care. Health services research works out how much health care costs and can help identify where funds need to be directed for staffing, medical equipment and therapies.



### Example of health services research

The University of Sydney and Cancer Council NSW are looking at the health care of 2000 men with prostate cancer.

The aim is to show how different ways of managing the disease

can lead to different outcomes and quality of life. The study measures 10- and 15-year survival rates, and will work out how much it costs the health care system to care for men with prostate cancer.

## Research into cancer causes

Cancer prevention is an important area of health care. To understand what causes cancer and how it might be prevented, researchers examine medical data from large numbers of people, often hundreds or thousands of people.



### Example of cancer causes research

Australia has the world's highest incidence of non-melanoma skin cancers (NMSC), basal cell carcinoma (BCC) and squamous cell carcinoma (SCC).

Cancer Council NSW's Skin Health Study aims to improve our understanding of how sun exposure, viruses and lifestyle combine to cause NMSC.

Two groups of people are taking part: people newly diagnosed with BCC or SCC, and people who have not had skin cancer.

The research will compare the two groups for sun sensitivity, sun exposure, alcohol and tobacco use, and exposure to other factors such as human papillomavirus (HPV).

## Modelling research

Another area of epidemiology is called modelling. This is a mathematical way of using information from the past to estimate what might happen in the future. For example, researchers might try to work out how many people are likely to be diagnosed with cancer in 10 years time or how much funding will be needed to run a cancer screening program.



### Example of modelling research

Cancer Council NSW is looking at different ways to run screening programs to help prevent cervical cancer or detect it earlier.

The aim of the research is to find the most efficient way of screening women, taking into account the latest information about how the disease develops, the different screening tests available, and how frequently tests need to be used.

At first the work will focus on Australia and England where a growing number of women have a lower risk of cervical cancer due to vaccination programs.

Results from the research will help many countries around the world – both those that already have vaccination and screening programs for cervical cancer, and those considering setting up programs in the future.



# Laboratory research

Laboratory research – which is carried out by scientists in laboratories using equipment such as microscopes – provides the basis for clinical research. There are different types of laboratory research.

## Basic research

The more knowledge scientists have about the human body, the better they can understand cancer. Basic research looks at the body's tiniest parts – its cells and molecules – to find out how they function. This helps scientists work out why cancer starts or spreads and how it might be prevented or treated more effectively.

Basic research is sometimes called test tube research because of the equipment used in the laboratory. The main focus of basic research in cancer includes investigating:

- the role of molecules, such as enzymes or hormones, in starting or stopping cancer
- the role of genes in cancer
- new drugs and treatments.

Basic researchers need to show that treatments they are developing are likely to be safe and effective before they are trialled in humans. At first they test new drugs in cells in the laboratory. The cells are from samples of living tissue (a cell culture).

If the treatment has the desired outcome in the cell culture, it will be tested on animals. This gives scientists more understanding of how the treatment works, problems it might cause, and whether it might be useful in humans.



## Example of basic research

The Adelaide Prostate Cancer Research Centre is investigating a particular substance in the blood. It is known as a marker called miR-375. The aim is to see whether miR-375 can be used to predict aggressive prostate cancer at diagnosis,

and whether it helps cause the disease to develop. The researchers will examine samples of blood donated by men with prostate cancer. If the results seem promising, then drugs targeting this pathway will be developed for further testing.

## Animal research

Before any drugs can be approved for use in people, they are tested in animals. Mice are commonly used because they have similar genes to humans, breed quickly, have a relatively short life span, and are easy to look after.

Some people ask whether it is fair to test medicines on animals. There are regulations to ensure that animal testing is only carried out if there's no alternative and it's done in the kindest way possible. For more information, see [www.nhmrc.gov.au](http://www.nhmrc.gov.au).



'In vitro' means 'in glass' and refers to experiments conducted in laboratory equipment such as test tubes and dishes. This is different to an 'in vivo' experiment, which means 'in a living thing' and refers to experiments involving live humans or animals.

## Stem cell research

Stem cells are the first cells that are formed when a person develops. At first they appear the same, but they divide and change to become many different cell types. These eventually form the many tissues and organs in the body.

Because stem cells potentially can change into any kind of cell, researchers are investigating them in the laboratory for their possible use in cancer care.

For more information about stem cell research, see the website [www.biotechnologyonline.gov.au/human/researchsc.html](http://www.biotechnologyonline.gov.au/human/researchsc.html).

## Pharmacogenetics

Pharmacogenetics is the study of how genes affect a person's response to drugs. It is sometimes called pharmacogenomics.

This new branch of research combines the study of drugs (pharmacology) and the study of characteristics passed down from biological parents (genetics). It investigates why some people benefit from having a particular drug while others do not. It also considers why some people get side effects or have serious reactions to drugs, yet others are not affected.

Pharmacogenetics may eventually help doctors choose specific treatments for individual patients based on their genes. Because more will be known about how and why certain medications affect different people, it will also help to improve drug safety.

## Tissue banking

Tissue banking is when groups of cells (tissue) from the body are prepared and kept in safe long-term storage for use in scientific research. Tissue can be from different parts of the body, for example, the bone, an organ such as the liver, or the blood. A tissue sample taken for research is also called a biospecimen.

Tissue banking can provide researchers with material to study cells, cancers and treatments in the laboratory. Researchers must seek appropriate permission from a human research ethics committee (see page 41) before using human tissue. The person donating tissue will be asked to give their consent if their tissue sample could ever be identified. Tissue is only sent to a tissue bank if there is enough available.

If you agree to donate tissue, collection will usually happen in some of the following ways:

- You may need to have your tissue sample collected specifically for the tissue bank. For example, you may need an appointment so some tubes of blood can be taken.
- The tissue sample may be collected during a test or treatment. For example, you may be booked in for a blood test, so an extra tube of blood will be taken for the tissue bank, or you may be asked to consent before scheduled surgery for tissue to be kept.
- A tissue sample may be obtained some time after surgery if it is available and you agree.



## Example of tissue banking

Cancer Council NSW is running the Cancer Lifestyle and Evaluation of Risk (CLEAR) study, which aims to find out what lifestyle and genetic factors influence the development of cancer.

People diagnosed with cancer within the last 18 months, and their partners, can participate.

As well as filling out a detailed questionnaire about their health, lifestyle and diet, all participants can give an optional blood sample.

Participants with cancer can also opt for a sample of tissue from their surgery to be sent to the tissue bank.

These biospecimens will be kept confidentially in freezers until they are ready to be examined. Results will be compared with those of other participants.

The CLEAR study is recruiting 10,000 people in NSW. For more information about the study, including how to participate, see [www.clearstudy.org.au](http://www.clearstudy.org.au) or call **1800 500 894**.

“ When I was diagnosed with cancer, a friend told me about the CLEAR study. Getting involved was simple. Some of the questions are very personal, but the information is kept private. Participating in cancer research is about giving to other people, and I think that’s a very valuable thing. ” Wendy (breast cancer)



# Clinical research

The primary focus of clinical research is people – patients, former patients and people who are well. The main goal of clinical research is to better understand, diagnose, prevent and treat diseases.

## The different areas of clinical research

### Human participation



These studies require contact with patients and/or healthy participants; examples include clinical trials and surveys of people using questionnaires.

### Records-based studies



These require access to personal data without involving any face-to-face contact; an example is an examination of patients' medical records.

### Clinical samples



These are laboratory studies of human material such as blood or tissue which has been obtained from surgery, tissue sampling (biopsy) or in a post-mortem (autopsy).

### Technology development



Researchers develop or adapt technology for diagnosis and therapies.



People taking part in clinical research are not necessarily patients. Many people are healthy or have never been diagnosed with a serious illness. However, most people in cancer clinical trials have been diagnosed with cancer.

## Clinical trials

Clinical trials are an essential step from laboratory research to making improvements in health care. They are part of the final stages of a long and careful research process. Trials show whether promising approaches to prevention, screening, diagnosis and treatment are possible, safe and effective.

- **Prevention trials** – test whether medicines or health programs lower the risk of getting diseases like cancer.
- **Screening trials** – look at new methods to detect diseases before people have symptoms.
- **Diagnostic trials** – seek more accurate or less invasive ways to diagnose disease in people who have signs or symptoms of a particular illness.
- **Treatment trials** – aim to determine the best treatments and support options, such as drugs; radiotherapy; surgery; nutrition; physiotherapy and complementary therapies.

See *Clinical trials explained* on page 25 for more information.

## Behavioural research

Behavioural researchers study people's individual characteristics, lifestyles and social circumstances to see how they affect the risk of someone developing cancer or surviving cancer. Researchers try to understand why people behave in the way that they do, even if

### Some key areas of behavioural research

Research area	Reducing inequality in cancer care	Behavioural causes of cancer
Aim of research	To learn more about social and environmental factors that increase cancer risk, particularly in certain population groups.	To find out why people act the way that they do, even though there's a known risk of getting cancer.
Research example	Looking at why people with low incomes have higher smoking levels and testing ways to change this problem.	Finding out reasons young people sunbake despite the known risks of too much sun exposure.

their lifestyles may lead to cancer or poor health. Researchers also look at how cancer impacts on people emotionally and socially.

Behavioural research does not focus on what causes cancer. This is studied in the field of epidemiology (see page 10).

Nutrition and physical activity	Barriers to screening and diagnosis	Communication
To understand how best to support healthy eating and exercise in the community to reduce cancer risk and improve health.	To investigate why people don't use free cancer screening programs or delay seeking medical advice if they have symptoms.	To see what impact cancer-related information has, and to assess how effectively information is delivered and used.
Assessing the influence of junk food ads and television on obesity in children.	Working out what the barriers are to cervical cancer screening and vaccination so that the high levels of cervical cancer in Aboriginal women can be reduced.	Giving men information about prostate cancer via the internet and following up how useful this is.

If you take part in a behavioural research study, you may need to fill in questionnaires or be interviewed about your lifestyle, including your eating, drinking, smoking, communication and exercise habits.

You may also need to participate in a program aimed at positively changing these behaviours. For example, you might be invited to have some free counselling, attend an exercise class or go to a session on improving communication skills. The aim of the programs may be to reduce cancer risk or improve the way you cope with cancer.

## Psychosocial research

Psychosocial research is an area of behavioural research. It looks at how cancer impacts on people emotionally (psychologically) and socially. In cancer care this is sometimes called psycho-oncology.

Researchers try to understand how both patients and carers may cope emotionally at different stages of disease. They develop and test methods to improve people's ability to deal with the different issues cancer brings up.



### Example of psychosocial research

In a study being run by the University of Western Sydney, researchers are looking at the best ways to help couples cope with changes to their intimate relationship after a

cancer diagnosis. Couples are randomly put into either a group receiving professional support or a group given resources for self-directed help. The study will compare the two groups.

## Translational research

It can take many years for research findings to be adapted into standard health care. Due to the limits on research funding, there is pressure for basic research (see page 13) to be used for patients' needs as fast as possible.

Translational research describes studies that get new treatments or medical approaches into practice quickly. It is sometimes called 'bench to bedside research' because results from basic research are directly used to create new therapies and diagnostic tools.

Findings in the clinic can also influence research in the laboratory. This is called 'bed to benchside research'. For example, information from hospitals and health care professionals is given to researchers to help direct research into the most useful areas.

Often one institution oversees all aspects of a translational research project, from its beginnings in the laboratory to the roll-out of a clinical trial and commercial development of a medicine.

### Example of translational research

The Cooperative Trials Group for Neuro-oncology is finding out whether chemotherapy given at the same time as radiotherapy, or after radiotherapy, will improve survival in brain tumour patients.

The research will show the effects of chemotherapy, and whether patients most likely to benefit from the treatment can be identified from markers in their tumour tissue samples.





## Ann's story

After I had completed my treatment for breast cancer, I found out about a study assessing the effects of chemotherapy on brain function and memory. Sometimes this is called 'chemo brain'. After chemotherapy, my memory really worsened, so I was keen to participate to help researchers understand more about this condition.

I had to do a computer-based questionnaire that assessed how well my brain was working. This included tests such as

spotting the differences between two pictures.

The next day, the researcher interviewed me over the phone about the computer test. Unfortunately my memory was pretty bad so I couldn't remember much about the test, but I guess that is useful information in itself for the researchers to record.

It was a simple way for me to get involved in research and it didn't take up much of my time at all.



# Clinical trials explained

Clinical trials help improve the diagnosis, treatment and management of people with cancer. If a trial proves that a test or treatment is better than existing options, it may become the new standard care for patients in the future.

The majority of cancer clinical trials test anti-cancer treatments, in particular drugs, but also radiotherapy and surgery. Most of the information in this chapter relates to trials of treatments.

## Clinical trials as a first line of treatment

In the past, clinical trials were seen as a last resort for people who had no other treatment choices. Today, people often have the option of receiving their first cancer treatment in a clinical trial. If a new treatment proves effective or more effective than the current standard treatment, trial patients who receive it will be among the first to benefit.

## The stages of a trial

Before involving patients in a clinical trial, researchers spend many years doing basic research (see page 13) to investigate and develop new medicines in the laboratory.

The researchers then plan the clinical trial to progress in a series of steps called phases. There are usually four phases. These phases allow researchers to build up reliable information about the treatment over a long period of time. Information gathered in each phase determines whether the study can move on to the next phase.

## Phase 1 trial

**Aim** – A phase 1 trial is the first step in testing a new treatment in people. Before this, the new treatment has only been tested in the laboratory. The main aim of a phase 1 trial is to show that a new treatment can be given safely. Researchers try to work out the best way a treatment can be given (e.g. by mouth or injection), as well as the safe dose.

Sometimes a phase 1 study tests how the drug works with other medicines or food. These are called interactions. This lets researchers know whether the drug should be taken with or without food, or if the patient might have problems if they're taking other medication.

**Number of participants** – Because less is known about the possible risks and benefits in a phase 1 trial, usually only 10–40 people participate. This helps protect patients in case the drug needs further work in the laboratory. Participants in phase 1 trials of anti-cancer drugs are usually patients who have already had all the other proven treatments for the type of cancer affecting them.

**How it works** – In the phase 1 trial, small groups of recruited patients (called cohorts) receive a fixed dose of the new treatment and are watched closely for any side effects. If there are no major side effects, the dose is increased in the next cohort of patients, and so on, until significant side effects start to appear. This indicates that the dose should go no higher and establishes a safe dose for future studies.

**Other information** – Participants may need to stay overnight in a hospital or clinical trials unit so that they can be closely monitored. Patients sometimes benefit from having the new treatment, but great improvements in their condition are uncommon. This is because only a small number of new drugs being tested prove to be major advances.

## Phase 2 trial

**Aim** – A phase 2 trial continues to test the safety of a drug and begins to assess if a new treatment shows signs of working on the disease. The aim is to discover if a new treatment seems to work well enough to justify a larger study (phase 3 trial). Patients may benefit from the experimental treatment but this depends on the type and stage of the cancer, the type of treatment used, and other factors, some of which may be unknown.

**Number of participants** – Usually 30–300 people with the same type of cancer are involved.

**How it works** – Although the treatment may be effective for many types of cancer, a phase 2 trial focuses on one cancer type only. In most phase 2 trials, all patients receive the same experimental treatment. However, patients may be put into separate groups and given different treatments, which are then compared. This is called a controlled trial. (See *Phase 3 trial* on the next page for more information.)

**Other information** – People often have treatment as outpatients, but occasionally they need to stay overnight for monitoring.

## Phase 3 trials

**Aim** – A phase 3 trial is designed to determine whether a new treatment is better than the best available standard treatment by comparing their effects on survival, quality of life and side effects. These trials can be used to assess new treatments, or to improve how existing treatments are used in different ways for different diseases.

**Number of participants** – A phase 3 trial recruits large numbers of patients, usually several hundred, and sometimes thousands, to reliably detect differences between the tested treatments. People from different countries often participate.

**How it works** – Patients are usually put into two treatment groups. One group receives the experimental treatment (e.g. a new drug or a new dose of an existing drug) – this is the experimental group. The other group receives the best standard treatment available – this is the control group. Sometimes when there isn't an existing treatment for the cancer type or its symptoms, a treatment without any medical effect (a placebo) is used for comparison (see page 39). Trials usually run over a long period of time, and the outcomes of both groups are compared.



In phase 3 trials, the experimental and control groups are always randomised and sometimes blinded. This means that patients are randomly put into one of two groups, and they do not know which treatment they are receiving. See pages 35–38 for more information on why studies are conducted this way.

**Other information** – Phase 3 trials of new treatments are sometimes called registration trials because if they are successful, the next step will be for the manufacturer of the treatment (often a pharmaceutical company) to apply for the treatment to be registered and made available to the public. Researchers need to show that a new drug is safe and effective in two independent phase 3 trials before they can apply for registration with the Therapeutic Goods Administration in Australia.

### Approving and subsidising drugs

The Australian Department of Health and Ageing's Therapeutic Goods Administration (TGA) approves drugs that are safe and effective for sale in Australia. See [www.tga.gov.au](http://www.tga.gov.au).

Decisions about the funding of new drugs require additional information, some of which is gathered in phase 4 studies.

Manufacturers of new drugs that have been shown to be better and/or more cost effective than existing treatments can apply to list them in the Pharmaceutical Benefits Scheme (PBS) so that the cost of the treatment is largely funded by the Government. This process generally takes several months.

“ Although I wasn't accepted for the trial – as the lead researcher suggested I have other treatments first – I found the thorough disclosure of both the trial and the possible side effects reassuring. ” Piers (*chronic lymphocytic leukaemia*)

## Phase 4 study

**Aim** – A phase 4 study is conducted after a treatment has been registered for use by the TGA and doctors are able to prescribe it outside of clinical trials. These studies are usually run by the pharmaceutical companies that make the product. This helps the companies more fully understand how their treatment compares to other treatments that are available for the same condition. They may also discover that the new treatment is beneficial for other conditions.

**Number of participants** – Usually several thousand people from many countries take part in phase 4 studies.

**How it works** – Companies monitor the long-term effects of the drug, particularly unexpected side effects. They may also investigate other uses of their drug – for example, its effects in medical conditions other than the condition for which it was originally registered.

**Other information** – Not all treatments are put through phase 4 studies. They are much less common than trials in the earlier phases.



To find out how to get involved in a clinical trial or another type of study, see page 43. For information on what you need to do if you join a clinical trial, see page 48.



## Philippa's story

When I was diagnosed with non-Hodgkin lymphoma in 2006, the drug rituximab wasn't available for a patient's first line of treatment. However a worldwide trial was being conducted to compare the outcomes of patients who no longer had active lymphoma after receiving rituximab.

My haematologist suggested I join this trial to get rituximab immediately rather than waiting until my other treatment options had been exhausted. I got a second opinion from another haematologist and he also recommended the trial. There was a lot of patient information to read, but my brother read it too, which was helpful.

The clinical trials nurse looked after me every time I went in for treatment. I had chemotherapy and then rituximab, and after a few months I went into remission. Then I began the experimental part of the trial.

One group of patients was given a maintenance dose of rituximab and had check-ups every three months. The control group just had check-ups every three months. I was in the control group so was observed until the cancer came back. At that point I came off the trial.

During treatment and for the check-ups, I had to have a physical examination, blood tests and an interview about my general well-being. It was good to be monitored so often, especially as I didn't have to pay for any of these tests. I had to travel overseas at one point, and as it was a worldwide trial, I was able to attend a clinic in the UK to have my regular three-monthly check-up.

It was worthwhile going on the trial because I was able to have the rituximab straightaway. I also felt by participating in the trial I was contributing to finding a cure for this particular cancer.

## Who works in clinical trials?

Cancer research is a large part of the health industry and offers many varied roles. If you join a study you may have contact with some of these people:

Health professionals	
<b>medical specialists, such as an oncologist, haematologist or surgeon</b>	Supervise your treatment, follow-up and overall care.
<b>clinical trials nurse or research nurse</b>	Coordinates recruitment and participation for a clinical trial or other type of study and liaises with other health professionals. They may also be the contact person (see next column).  See page 34 for more information.
<b>clinical trials or study coordinator</b>	Similar to a clinical trials or research nurse, except that they do not have nursing qualifications.
<b>allied health practitioners or complementary therapists</b>	Give treatment or advice in studies that investigate the use of non-medical treatments such as nutrition, physiotherapy, counselling, acupuncture or massage.
<b>pharmacist</b>	Involved in laboratory research and during a clinical trial to provide advice about medicines and monitor their effect on patients.

## Researchers

<b>coordinating investigator</b>	Oversees research taking place at more than one study site, e.g. at multiple hospitals.
<b>lead researcher or principal (chief) investigator</b>	Has overall responsibility for the scientific, administrative, financial and ethical aspects of a study, and reports the study results.
<b>researcher or investigator</b>	<p>Develops and plans studies, and obtains, analyses and publishes results.</p> <p>Health researchers come from backgrounds such as medicine, science, consumer advocacy or complementary therapies.</p>
<b>contact person</b>	<p>Point of contact for all research participants if they have any queries or problems that arise during a study.</p> <p>See page 46 for more information.</p>

## The clinical trials nurse

Not all clinical trials or hospitals have a dedicated clinical trials nurse, but the larger ones do. The clinical trials nurse plays a key role for trial participants. Most patients find it easier to communicate with one person about a trial, so the clinical trials nurse is usually their main point of contact.

- Nurses may help to recruit patients by talking to potential participants, making sure they are eligible and explaining the purpose of the trial. They also let the patient and their family know what to expect and what to do if there are any problems during the trial.
- Once you have agreed to join a trial, the nurse arranges appointments for tests, treatment or to see the specialist. They also make sure all paperwork is filled in.
- Nurses provide emotional support and act as a link between the patient and the researchers or health care team.

Sometimes, this role is done by someone who is not a nurse. In this case, they are called the clinical trials or study coordinator.



A research nurse has a similar role to a clinical trials nurse except that they coordinate other types of research. Depending on the study that you're involved in, you may be in contact with a research nurse rather than a clinical trials nurse.



# Planning research

Researchers plan and carry out studies in a way that ensures results are accurate and not caused by chance. They also have to reduce the possibility that their own – or participants’ – ideas or beliefs about the research might unfairly influence (bias) the results. This chapter describes some methods used to make sure studies are fair and reliable. Some information applies to clinical trials only.

## Study size

For study results to be reliable, a large number of people usually need to take part. This is because the higher the number of people who get involved, the less likely the results will be due to chance.

In the early stages of clinical trials (phase 1), only small numbers of people participate because so little is known about the drug and its safety in humans. Once safety is established, a larger number of people (30–300) is needed for phase 2 trials to see if the new treatment can work in humans. If these trials are successful, then often many hundreds or thousands of people are needed to establish if the new treatment is better than the best available treatment.

Participant numbers vary for other types of health research, depending on the aim and type of study.

## Randomised controlled trials

Most phase 3 trials, some phase 2 trials and many other studies are randomised controlled trials. This means that participants are randomly assigned to either a ‘test’ group or a ‘control’ group.

## Test and control groups

If patients in a trial are all given a new treatment and do better than other people having standard treatment outside of the trial, researchers don't know whether it is because the new treatment is more effective or because the people chosen for the trial were going to do better than average regardless of their treatment. This is because people who are offered and accept participation in trials tend to do better regardless of the treatment they are given (see page 53). The only way to get around this problem is to randomly divide trial participants into two or more groups and compare them directly.

The group that has the experimental treatment is called the test or experimental group (or arm). The group that has the comparison treatment is called the control group or arm. The control treatment is the current best available standard treatment for the disease.

## Randomisation

Randomisation means that participants are randomly assigned to the different arms of a trial. A computer usually does the sorting so that human choices do not affect the selection of treatment or the trial results. This method also makes sure that the groups are as similar as possible, both for characteristics that are important and known – such as age and gender – and for characteristics that are important but not yet known.

When randomly allocated groups are compared with each other, it is possible to reliably work out which treatment is better. This is because researchers can be certain that the results are related to the treatment or chance, and not to any other factors.

It is easy to check and account for imbalances in characteristics that are known to be important, for example age and gender. However, there are always many other characteristics that affect the outcomes of treatment which are not yet known or measured. Randomisation is the only way to account for these important unknown characteristics.



### Marg's story

After I was diagnosed with breast cancer I asked if there were any clinical trials I could go on. One was assessing how effective a pain-relieving inhaler was for women having a sentinel node biopsy, which was the procedure I needed.

I felt that the trial was low risk and I had the potential to receive some useful, extra treatment, so I agreed to participate. It required an overnight stay in hospital to have the biopsy and treatment, and be monitored.

A clinical trials nurse accompanied me at every stage of the process. She

explained what was happening and answered any questions I had, which was reassuring.

I had questionnaires to do about my emotional and physical well-being on the day of the trial and the following day. This gave me something to do with my time and I felt that I was actively participating in my own health care.

The study was blinded and we were randomly assigned to get either the experimental treatment or a placebo. I don't think I was given the experimental drug, but it was still a positive experience to participate in the trial.

## Cross-over studies

In these studies the two arms of the trial have their assigned treatment for a period of time before swapping and having the other treatment. This enables all participants to experience all treatments, which helps confirm which is the most effective.

## Blinded studies

When people don't know which arm of a study they're in, this is called blinding. Many trials are blinded so that neither you, nor your doctor, know whether you're receiving the experimental treatment or the control treatment. These studies are known as double-blinded.

Blinding aims to reduce bias in the reporting of benefits and side effects. For example, if you or your doctor knew that you were having standard treatment, you might feel disappointed that you didn't get the experimental treatment and this may affect your well-being. On the other hand, if you or your doctor knew you were having the experimental treatment, then you might report that you're feeling better than you actually are because you want the treatment to work. If you don't know which treatment you're having, the results are less likely to be influenced by you or your doctor's thoughts.

In a double-blinded trial, even the lead researchers only discover who is in each arm of the study at the end of the trial when the results are being analysed. If necessary, for safety reasons, your doctor can find out what treatment you're having by contacting those running the study.

## Standard treatment and placebos

**Standard treatment** – This refers to the current most effective treatment or care given to people for their disease or condition. For example, standard treatment for newly diagnosed early breast cancer is removal of the breast tumour, often followed by radiotherapy, hormone therapy and/or chemotherapy.

In some cases, there is no known useful medication or treatment, but patients will be monitored regularly. That is then the currently available best standard of care. This would be the case for women who have completed standard surgery, radiotherapy, hormone therapy and/or chemotherapy for early breast cancer.

**Placebo** – This is not a real treatment. It is made to look, taste or feel like a real treatment but doesn't have active (therapeutic) ingredients (if a medicine) or a remedial

effect (if another type of treatment). Placebos are used in addition to best standard treatment to prevent people from knowing which treatment they are getting in a trial.

A placebo is used when comparing treatments to see whether the patients' outcome is because of the actual treatment or because of other things associated with being in the study. If the people given the experimental treatment do better than those given the placebo, this provides strong evidence that it's the experimental treatment that is responsible rather than other factors.

Trials that include a placebo are only approved if the placebo is used together with the best standard therapy for the cancer or condition being studied.

Participants must always be told if a study is using a placebo.



# Regulating research

Research is regulated to make sure it is conducted to a high professional and scientific standard and doesn't put participants at unnecessary risk. In Australia, several committees thoroughly examine and approve a study before it begins. These committees confirm that a study is considered both scientifically worthwhile and fair (ethical).

- **Research review committee** – decides whether the study has social and scientific value, and if the way it is to be conducted will produce valid scientific results.
- **Ethics review committee** – confirms that the interests of participants are protected and that researchers will run the study in a fair, honest and neutral (impartial) way; they won't force people into participating; and the risks of the research generally don't outweigh the benefits.
- **Research governance review** – checks every site where the research will take place. This review is done by a research governance officer who makes sure that there are enough resources to carry out the proposed research and that the staff members involved are adequately qualified. The governance officer authorises the research to begin at each site.

The study may also be monitored by outside agencies such as pharmaceutical companies, research institutions and auditors. These bodies ensure that the research is carried out properly.

For information on how studies are conducted fairly, see pages 35–39.

## Human research ethics committee

Once research has been approved by a research review committee, the researchers put together an application for a human research ethics committee. This ethics committee assesses the impact of the proposed research on participants, researchers and the general community. The impact is then weighed up against the benefits of conducting the research. The committee makes sure that the study will be carried out in a way that protects participants.

The committee reviews a number of aspects of the study, including:

- the qualifications of the researchers conducting the study
- the way participants are recruited
- the quality of the participant information (see page 44)
- how risks to participants will be minimised
- how the study might impact on the participants' quality of life.

Members of an ethics committee are always independent of the researchers. They come from a variety of backgrounds, including medical, scientific, legal and religious professions, and the general community. There are usually at least seven core members, both men and women. They have a range of professional and personal experiences so that the reviews are balanced and come from different points of view.

“ I had no thought about research until the day of my operation when someone asked me to sign a form to say that I agreed to having the parts removed handed over for a study. ” Terry (prostate cancer)

## National Health and Medical Research Council (NHMRC)

NHMRC is the national research funding and development body representing the views of the government, the health industry, the research community and consumer advocacy groups.

It produces Australian guidelines about ethical conduct in research. Ethics committees must follow these guidelines and report to NHMRC every year. You can download the guidelines from [www.nhmrc.gov.au/guidelines/publications/e72](http://www.nhmrc.gov.au/guidelines/publications/e72).

## Changes to research

Sometimes changes need to be made to the research:

- The ethics committee may ask the research team to make changes to the proposed research before they approve it.
- Once a study has been approved, any subsequent changes that researchers want to make must be submitted for ethics approval again before the change can take place. If there are any changes to the participant information, then participants already taking part in the study may need to sign a form to show that they have been informed of the change and still agree to be involved.

“ I was informed that a participant died whilst on the same trial as me. I had to consent again to show I understood the risks and benefits of staying on the trial. ” *Kristin (ovarian cancer)*



# Getting involved

## Finding a study

If you want to join a clinical trial or other study, let your doctors know. Most specialists know about current studies and may recommend them to you. If your hospital has a clinical trials or research nurse, you can also ask them about any studies that might be suitable for you.

Hospital and treatment centre waiting rooms often have information about current studies. Other opportunities may be advertised on websites, through patient support groups and in the general media. If there isn't a suitable study now, you can register with some organisations to be informed of studies that come up in the future. See pages 60–61 for a list of websites to find or register for studies.

If you hear about a trial you're interested in, speak to your doctor who can find out more about it and either coordinate your involvement or put you in touch with the research team.

## Informed consent

Before agreeing to participate in a study, you need to understand what it involves. Informed consent is the legal term for the requirement that all participants are told about the purpose, risks and possible outcomes of the research before their involvement begins.

If you decide to join a study, you must sign a consent form. This may need to be witnessed. You should only sign the form if you understand what being in the study means for you. For people under 16, the form must be signed by a parent or legal guardian.

You will be given written information so you can read about the study. This is called participant information (see below). You can also talk to your doctors and/or clinical trials or research nurse about any aspect of the study.

If there are changes to the study or new information becomes available while you are involved, you will be told about this and you may need to sign an updated version of the consent form.



Sometimes you may need to consent for each aspect of a study. For example, you might agree to take part in a trial of a new surgical procedure, and then need to consent for your tissue to be collected and banked during that surgery. You might be given an extra questionnaire – this may also require you to consent again.

## Participant information

Researchers must provide written information about their study to anyone thinking about getting involved. This is called participant (or patient) information. It answers a range of questions about a study, including:

- the purpose of the study
- if it is a clinical trial, and what phase it is in
- who can participate in the study (see the opposite page)
- who is running the study (institute and researchers)
- who has approved the research
- who is funding the study

- how the study will be run and what participants need to do
- how long you need to be involved for
- where you need to go for appointments, treatments or meetings
- whether your medical records need to be accessed
- information about possible side effects or other risks
- who to contact for further information or if you have any problems or complaints during the study (see the next page)
- information about your rights, such as keeping your records private (see page 47) and your ability to withdraw from a study (see page 50).

## Eligibility criteria

Researchers create strict eligibility criteria to make sure the people in a study have certain characteristics. The criteria help produce trustworthy results because the participants will have many similarities. Depending on the study, criteria might include:

- age
- type and stage of cancer
- symptoms or side effects experienced
- length of time since diagnosis or treatment
- previous medical history
- which treatments you have had in the past.

Potential participants must be able to satisfy the inclusion criteria. If any exclusion criteria apply to them, they won't be able to participate. Doctors use the criteria to assess the suitability of a new treatment or test on particular patients. The eligibility criteria also protect people. This is because the criteria take into account any characteristics that might increase your risk of participating.

For example, you may be excluded from a trial if you are pregnant, have high blood pressure, or some other condition that significantly increases the risks of the treatment.

## Contact person

All studies must list details of a contact person. You can talk to this person before you decide to participate and at any stage of the study if you have questions or concerns. The contact person is often a clinical trials nurse or research coordinator (see page 34).

You will also be given details of who to contact if you have a complaint about the study, for example how it was run or how you were treated. This person is independent of the research team. Complaints about research are rare but it is one of your rights as a participant to have your concerns heard if you have a problem.

### tips

- Sometimes a trial will be run at a different hospital to where you're having treatment. This means you may have two treatment teams. If this is the case, make sure your medical information and any relevant test results are available to both treatment teams, and find out who your main contact person is. Taking your own record of test results to hospital can also be helpful.
- If you are in a clinical trial and develop significant problems, then you should go to the hospital's emergency department and/or contact the hospital's oncology registrar. If you go to hospital, let your trial team know.

## Privacy

Medical records are private and confidential, including those relating to your involvement in a trial or study. Only health professionals directly involved in your care or the study you're on can access your personal and medical information if it's necessary for their work. They can't reveal anything about you to others, unless it is relevant to your health care or the study.

The participant information may mention who will and won't have access to your personal data. For example, it might state that your regular medical team won't have access to your questionnaire responses but the researchers will. You might be asked to consent to the research team accessing your existing medical records or particular test results.

Often, information collected during the study is de-identified. This means that it won't have your name on it. Not even the researchers know which results belong to which individual people. When studies are discussed at conferences or in journal articles, no individual participants are identified.

For information and resources about privacy issues in health care, talk to the social worker at your hospital or call Cancer Council Helpline 13 11 20.

At the end of a study, all personal information is stored for at least 15 years before it can be destroyed. This is a legal requirement.



## Being part of a clinical trial

What you need to do when you agree to join a study depends on what kind of research it is. Most studies that aren't clinical trials don't require preparation or ongoing follow-up. A clinical trial may be more involved, but it depends on what it is testing and what phase it is in.

### Before the trial starts

Before you agree to participate, a member of the clinical trials team will discuss the trial with you and your oncologist or other cancer specialist. You will be given participant information to take away and read (see page 44). You should take time to read the information and ask any questions you have. You may want to discuss the information with family, friends or your GP. See pages 62–63 for a list of questions you might like to ask your doctors or the clinical trials or research nurse.

You will probably need to have medical tests, such as a CT scan and blood test, beforehand to check that the trial is suitable for you.

### Continuing access to medications

Many people wonder whether they're able to continue receiving the experimental treatment after a trial is over. This depends on the phase of the trial, the study results,

how effective the treatment was for you, what the recommended course of treatment is, and whether the trial sponsor is prepared to provide the treatment.

## During the trial

Once you've agreed to participate, you will be given specific instructions about the trial. For example, you may need to go to hospital for treatment or you may have to take medication at home. It is important to follow these instructions both for your safety and so that the trial results are as reliable as possible. This means going to all appointments, having the required tests, taking medicines on time, and completing logs and questionnaires, including surveys about your quality of life.

Being in a trial may mean that you have more tests and visits to your doctor than you normally would. This is to monitor your health and to see if and how the treatment is working. The research team will also ask about how you are feeling and doing.

You may come into contact with different members of the research team (see pages 32–33), but your participation is usually organised by one person (often a clinical trials nurse). Your overall care will probably continue to be coordinated by your cancer specialist.

Some people go on clinical trials because it is the only way they can access treatments that would otherwise not be available. It can be frustrating and disheartening to come to the end of the study and not

be able to continue with the promising treatment.

Ask your doctor or clinical trials nurse whether it's possible and advisable to continue the experimental treatment.

## When the trial is over

Researchers may stay in contact with you for some time after the trial so that they can collect long-term information on how you are doing. You will return to having the standard care and/or check-ups that are appropriate for you, depending on the stage of the cancer and what your cancer specialist recommends.

## Support during and after a trial

Although most people say that being in a trial is rewarding you may have mixed emotions during or after it. There are people who can support you throughout a study – see page 57 for more information.



You can ask the study's contact person to send you updates and the final study results if you're interested. Sometimes, however, it takes many years before the results are ready.

## Withdrawing from a study

Participating in research is voluntary and you can stop being involved at any time. You may want to withdraw because you:

- no longer have the time or energy to commit to it
- don't feel it is helping
- get side effects or your health is worsening
- move further away
- change your mind.

You will not be penalised, and you will continue to receive the type of treatment that is the best option for you.

## Consumer involvement in research

Sometimes people want to contribute to research in a different way to being a participant in a study. They may have been affected by cancer in the past – either directly or indirectly – so they want to offer their knowledge to help shape future cancer research. Researchers refer to these people as ‘consumers’ to distinguish them from patients or others recruited to a study.

The role of consumers in research has increased over the years. Consumers can now identify priorities for research and help to decide what projects should be funded. They may also work directly on research studies, providing an informed consumer view.

Cancer Voices is a consumer advocacy organisation that operates in most states. It focuses on improving cancer treatment through active consumer participation.

For example, Cancer Voices NSW provides a matching service for researchers wanting to include the informed consumer view in their work.

Cancer Voices NSW and Cancer Council NSW have a process that enables trained consumers to rate research proposals being assessed for Cancer Council funding. The consumer-only panel judges research on its expected benefit, the likelihood the results will be put into practice, its fairness, and the extent of consumer involvement. When Cancer Council decides whether or not to fund a project, it considers both the consumer rating and the outcome of the research review.

If you would like to get involved, contact your state Cancer Voices group (see page 62 for websites or call Cancer Council Helpline **13 11 20** for contact details).



# Making decisions

You may have many considerations when deciding whether or not to join a study, particularly if it is a clinical trial. As well as talking to your doctor and clinical trials (or research) nurse, it's usually recommended that you talk to your family or carer. This is because your involvement may also impact on them. Ultimately, though, it's your decision to participate in research or not.

You shouldn't feel pressured to take part in research, and you should not be rushed into making any decisions that affect your health or treatment. Ask your doctor or nurse how much time you have to think about whether or not to join a study. If you take some time, ask if this is likely to affect your treatment outcomes.

## Weighing up the advantages and disadvantages

You need to think about the possible benefits, inconveniences and risks to you if you join a study. Everybody's situation is different – what is right for someone else may not be right for you.

Participating in general cancer research, such as doing a survey or participating in a focus group, probably poses little risk or trouble. Many clinical trials are also very straightforward.



Most people diagnosed with cancer decide to participate in research because they want to help improve outcomes for others in the future.

## Q: Will I be better off in a study?

- A:** There are many advantages to being involved in research or a clinical trial. Depending on the study, these may include:
- accessing programs, medicines or other treatments that are not available outside of the study and may be better than the current standard care for the disease
  - accessing expensive drugs that are not covered by the Pharmaceutical Benefits Scheme
  - seeing your treatment team, including specialists, more often
  - being motivated to take an active part in your health care
  - learning new ways to improve your lifestyle and communication
  - improving your quality of life
  - feeling that you've tried all treatment possibilities
  - knowing you've made a valuable contribution to helping others in the future.

If you join a study, it doesn't always mean you will be better off than before or better off than other people in a similar situation to you. This is because although researchers may predict and hope that the outcomes of their study will be positive, not everyone will respond in the way that they want.

In a clinical trial, you may be in the control arm and not given the experimental treatment (see pages 35–36). However, you will then receive the best standard care available and be monitored more frequently and carefully than usual. Either way, your doctor and the clinical trials or research nurse will discuss the possible advantages for you before you join a study.

## Q: How long will a study last?

**A:** From start to finish, a study often takes years or even decades. Your personal commitment may only be for part of this time. It may be a one-off couple of hours, or you may need to give a bit of time every few weeks, months or years.

Some studies require people to be surveyed at regular intervals for several months or years. This allows researchers to understand the long-term effects of treatments, monitor the general health of study participants and collect data about long-term survival.

Studies have what is known as a recruitment phase. This usually occurs over a few months or years until the required number of people have agreed to take part. However, the individual participants are only involved in the study for the period stated in the participant information.

## Q: Will it cost me to participate?

**A:** The costs of research, such as treatment, tests and patient check-ups, are paid for by the organisation that is funding or conducting the research. You may get travel or out-of-pocket costs refunded. Ask your doctor if this is available.

“ The trial I was on ran initially for three years, but my involvement only lasted a year and a half. I had to have check-ups every three months – a physical examination and blood tests. ” *Philippa (non-Hodgkin lymphoma)*

**Q: Will I be paid?**

**A:** People participating in cancer research and trials don't usually receive any payment, apart from the reimbursement of out-of-pocket expenses if this applies to the study.

**Q: Can I be involved in more than one study?**

**A:** You may be asked to join multiple studies. Check with the contact officers of each study whether you can participate in more than one study at the same time. If you can, think about whether you'll be able to commit to all their requirements.

**Q: If I go on a clinical trial can I have other treatment?**

**A:** Check whether the study will impact on any other treatment you're having or planning to have. This includes medicines for symptoms or side effects of cancer or other conditions, or complementary therapies, such as herbal or nutritional supplements or massage.

Ask your doctor if you need to stop or put off these other treatments, or whether they need to be modified (for example, the dose).

“ I was using Chinese herbs all through a clinical trial of a drug that was being tested for its ability to improve immunity during and after chemotherapy. Although I told them I was taking herbs, I was still allowed to participate. ”

*Julie (breast cancer)*

## tips

- Consider what is most important to you. Some people want to be certain of which treatment they will receive, whereas others prefer the opportunity to try something new.
- Think about the possible problems of being in a study and how they might affect your well-being and lifestyle.
- Weigh up any problems with the potential benefits, such as a possibly longer survival time or not having to experience certain side effects.

## A second opinion

Some people like to get a second opinion about whether they should join a study, particularly if it is a clinical trial. A second opinion can confirm or clarify your doctor's recommendations and reassure you that you have thought about the different issues that might affect you.

Ask your GP for a referral to another cancer specialist, but be mindful that you may have to wait some weeks for an appointment and it will cost you extra money. You may be able to talk to another specialist on the hospital ward. Ask your cancer specialist if this is possible.



## Seeking support

Being diagnosed with cancer can turn your life upside down. You may feel that you don't have enough time or energy to think about research. For most people, their key goal will be to start treatment as soon as possible and then concentrate on getting better.

However, you may want to take part in research, or you may be invited to take part. Being involved may give you an opportunity to feel more supported during or after cancer treatment. You may also find the experience rewarding.

If you agree to participate, you may have mixed emotions during or after the study. This is not unusual for people having cancer treatment, whether they are in a trial or not. However, you may find the extra appointments stressful or dealing with a different health care team unsettling. The treatment you're having may cause side effects or you may worry that you're not getting the best treatment.

Afterwards, you may be relieved because you no longer have the commitment. You may be happy with the outcome and feel ready to put the cancer behind you. However, you may feel less certain because your health won't be monitored as frequently, or you may be disappointed that the cancer has not gone into remission.

It's important to discuss any worries with your doctor or clinical trials/research nurse. They can help you understand information about the research so that you feel reassured and positive about your involvement. Make sure you understand the aims of the research before you participate so you have realistic expectations.

It can also be helpful to talk to your friends or family about how you're feeling, but sometimes you might want to speak to somebody neutral. You can talk to:

- a counsellor, social worker or psychologist
- members of a cancer support group
- a peer support mentor who's had a similar experience to you.

To find out more about these services and to get free copies of resources about coping with emotions during and after cancer treatment, call Cancer Council Helpline 13 11 20.

## Caring for someone with cancer

You may be reading this booklet because you are caring for someone with cancer. The person you're caring for may be interested in or invited to participate in research. Depending on the type of research, it is usually recommended that carers read about the study themselves and talk it over with the person who has cancer. You can also discuss the study with the clinical trials/research nurse.

While the decision to participate lies with the person who has cancer (unless they're under 16), it's important that you, as the carer, know how the study might impact on the participant and on you and your family. For example, it may mean you have to take extra time off work to drive the person you're caring for to appointments, or you may be worried about how the treatment will affect them. Check with the clinical trials or research nurse whether you can be reimbursed for costs associated with helping the person participate in the study.

Being involved in research may offer the person with cancer an opportunity to have a promising new treatment or other useful supportive care options. It may be satisfying to know that their participation will help others in the future.

Weighing up these options – and just being a carer in general – can be stressful. Try to look after yourself by giving yourself some time out and sharing your worries and concerns with a counsellor or your doctor. You can also call Carers Australia on 1800 242 636 or the Cancer Council Helpline to obtain other resources and support.

Some studies are also open to carers or partners of people with cancer. You may be interested in taking part in a study yourself.



## Cancer Council library\*

Following a cancer diagnosis many people look for information about new types of treatment, the latest research findings and stories about how other people have coped. Cancer Council has a range of books, CDs, DVDs, medical journals and information about research that may be helpful for you. The librarian can also help you find research papers relating to your area of interest.

Call the Cancer Council Helpline to find out how to access the library services.

*\*This service is not available in Victoria and Queensland.*



# Useful websites

The internet has many useful resources, although not all websites are reliable. The websites listed below are good sources of information.

## Australian

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### To find current trials

Australian Cancer Trials..... [www.australiancancertrials.gov.au](http://www.australiancancertrials.gov.au)  
Australian New Zealand Clinical Trials Registry ..... [www.anzctr.org.au](http://www.anzctr.org.au)  
Australian Cancer Research Foundation..... [www.acrf.com.au](http://www.acrf.com.au)  
Cancer Institute NSW..... [www.cancerinstitute.org.au](http://www.cancerinstitute.org.au)  
Cancer Trials Australia..... [www.cancertrialsaustralia.com](http://www.cancertrialsaustralia.com)  
Trans-Tasman Radiation Oncology Group ..... [www.trog.com.au](http://www.trog.com.au)  
Trial Spotting ..... <http://trialsplotting.com.au>  
Cancer Trials Westmead  
and Blacktown Hospitals, NSW ..... [www.cancertrials.com.au](http://www.cancertrials.com.au)

### To register for studies

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Breast Cancer Network Australia Review  
and Survey Group ..... [www.bcna.org.au/speak-out#reviewSurvey](http://www.bcna.org.au/speak-out#reviewSurvey)  
Cancer Council NSW ..... [www.cancercouncil.com.au/joinastudy](http://www.cancercouncil.com.au/joinastudy)  
CLEAR Study (NSW residents only) ..... [www.clearstudy.org.au](http://www.clearstudy.org.au)  
Register4 ..... [www.register4.org.au](http://www.register4.org.au)

### For consumer involvement in research or advocacy

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Cancer Voices Australia..... [www.cancervoicesaustralia.org.au](http://www.cancervoicesaustralia.org.au)  
Cancer Voices NSW ..... [www.cancervoices.org.au](http://www.cancervoices.org.au)  
Cancer Voices Queensland ..... [www.cancervoicesqld.org.au](http://www.cancervoicesqld.org.au)  
Cancer Voices South Australia..... [www.cancervoicessa.org.au](http://www.cancervoicessa.org.au)  
Cancer Voices Victoria ..... [www.cancervoicesvic.org.au](http://www.cancervoicesvic.org.au)

## Other national websites

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Cancer Council Australia..... [www.cancer.org.au](http://www.cancer.org.au)  
 Health Insite..... [www.healthinsite.gov.au](http://www.healthinsite.gov.au)  
 National Health and  
 Medical Research Council..... [www.nhmrc.gov.au](http://www.nhmrc.gov.au)  
 NHMRC Clinical Trials Centre..... [www.ctc.usyd.edu.au](http://www.ctc.usyd.edu.au)  
 Pharmaceutical Benefits Scheme ..... [www.pbs.gov.au](http://www.pbs.gov.au)  
 Therapeutic Goods Administration..... [www.tga.gov.au](http://www.tga.gov.au)

## International

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ClinicalTrials.gov..... [www.clinicaltrials.gov](http://www.clinicaltrials.gov)  
 International Standard Randomized  
 Controlled Trial Number ..... [www.controlled-trials.com/isrctn](http://www.controlled-trials.com/isrctn)  
 James Lind Library ..... [www.jameslindlibrary.org](http://www.jameslindlibrary.org)  
 Macmillan Cancer Support..... [www.macmillan.org.uk](http://www.macmillan.org.uk)  
 NHS Choices – Clinical Trials and  
 Medical Research..... [www.nhs.uk/conditions/clinical-trials](http://www.nhs.uk/conditions/clinical-trials)  
 World Health Organization..... [www.who.int](http://www.who.int)  
 WHO International Clinical Trials  
 Registry Platform..... <http://apps.who.int/trialsearch/>



# Question checklist

You may find this checklist helpful when thinking about the questions about cancer research or clinical trials you want to ask your doctor, clinical trials or research nurse, or the research contact person.

## Practical questions

- What are my chances of benefiting from this research?
- What are the risks to me?
- Will I get any side effects? How will they be treated?
- Are there any tests involved?
- Do I need to go into hospital?
- Will I need to take time off work? Will being involved affect my day-to-day life?
- Can I receive any reimbursement of out-of-pocket expenses?
- Can I still participate if I need to travel interstate or overseas?
- Who will oversee my cancer care while I'm participating?
- Can I be involved in more than one study at the same time?
- If I join this study, will I miss out on other treatment opportunities later?
- Can I still take other medication or complementary therapies while I'm involved in the trial?

How much time do I have to think about whether or not to join this trial or study?

Can I still take other medication or complementary therapies while I'm involved in the trial?

### Study background

- What is being tested in the trial or study and why?
- How many other people will be involved in this research?
- How long does the research last? How long do I need to be involved for?
- How much time do I have to think about whether or not to join this trial or study?
- If I take some time to decide, will delaying the treatment affect how well it works?

### Legal and ethical questions

- Has the study been approved by an ethics committee?
- Can you go through the participant information with me?
- Can I have the participant information in a different language?
- Will I be covered if anything happens to me while I'm on this study?
- How will my identity be protected while I'm participating and who will have access to my information?
- What will happen with the results of the research?
- Who can I contact if I have a problem?



# Glossary

You may come across new terms when reading this booklet or talking to health professionals. You can check the meaning of other health-related words at [www.cancercouncil.com.au/words](http://www.cancercouncil.com.au/words) or [www.cancervic.org.au/glossary](http://www.cancervic.org.au/glossary).

## **active ingredient**

The compound in a medicine that has a therapeutic effect on the body.

## **animal research**

Research using animals to check the safety and efficacy of a treatment before it is tested on humans.

## **baseline**

A phase during a study when the participants are not receiving any treatments. This is usually at the beginning of a trial before treatment is started.

## **basic research**

Scientific research carried out in a laboratory to study the tiniest components of the body, including cells, compounds and molecules. Sometimes called test tube or laboratory research.

## **behavioural research**

Research that looks at people's behaviours and how these affect their chances of getting cancer or recovering from it.

## **bias**

Human choices or other factors not related to the treatments being tested that might affect a study's results.

## **biospecimen**

See tissue.

## **blinded trial**

A trial in which participants do not know which treatment they are getting.

## **blood test**

A test that examines a sample of blood. It assesses levels of different substances in the blood to help diagnose disease.

## **cancer**

A disease of the body's cells that

starts in the genes. Damaged genes cause cells to behave abnormally, and they may grow into a lump called a tumour.

### **case control study**

A type of study that matches people who have a particular disease (the cases) with people who are healthy (the controls), for example using age or birthplace. The groups are compared to see if they have anything in common, such as their history of smoking or exposure to asbestos.

### **cells**

The basic building blocks of the body. A human is made of billions of cells, which are adapted for different functions.

### **chemistry**

The study of matter (such as atoms and ions) and how it changes and reacts to other matter.

### **clinical**

Relating to medical or nursing care.

### **clinical research**

Research that focuses on

people's health and medical care.

### **clinical trial**

Research that tests new and better ways of improving health in people.

### **cohort**

A group of individuals sharing a similar characteristic such as age, sex, disease or exposure to a pollutant or drug.

### **cohort study**

A study to determine risk factors for a disease by tracking a group of healthy people who share a similar characteristic, such as their type of work, and seeing whether they develop the disease in question. A cohort study also has a control group.

### **control group**

A group of patients that is compared with people in the experimental group. In a clinical trial, the control group receives the control treatment.

### **control treatment**

The treatment that is being compared with the experimental treatment. The control is generally

the best standard treatment available. In some cases, a placebo is used.

### **controlled trial**

A controlled trial compares two or more treatments to find out which one is the best.

### **CT scan**

A computerised tomography scan. This scan uses x-rays to create a picture of the inside of the body.

### **DNA**

A tiny molecule in every cell of the body that carries instructions for how those cells behave and function. Also called deoxyribonucleic acid.

### **double-blind trial**

A trial in which neither the patient nor their doctor knows what treatment the patient is receiving, to reduce bias.

### **eligibility criteria**

List of characteristics that a person must have to be suitable for a trial.

### **enzymes**

Proteins that are essential for the normal functioning and performance of the body.

### **epidemiology**

The study of health on a population level.

### **ethics**

The study of responsible conduct and what is fair.

### **ethics committee**

A committee that reviews the plans and other paperwork relating to a research study to make sure it is safe and ethical.

### **experimental group**

The group of patients that receive the experimental treatment in a randomised controlled trial. Also called the test group.

### **experimental treatment**

The new or modified treatment that is being tested in a clinical trial.

### **gene**

A small part of DNA that causes people to have particular characteristics relating to their physical appearance or health.

**genetic marker**

A gene or DNA sequence associated with a particular characteristic.

**haematologist**

A doctor who specialises in studying and treating diseases of the blood, bone marrow and lymphatic system.

**hypothesis**

An explanation or guess based on limited evidence that serves as a starting point for research.

**immunology**

A branch of medicine that studies the immune system, which helps fight off disease in the body.

**in vitro**

Laboratory experiments that are done using scientific equipment, such as test tubes and dishes.

**in vivo**

Experiments that are done using a living organism, such as an animal or human.

**informed consent**

A process in which someone

agrees to participate in research, after being given information about the study that they understand completely.

**investigator**

Another term for a researcher.

**laboratory**

A place where scientists carry out experiments and formulate new medicines.

**laboratory research**

Research that is carried out in a laboratory.

**literature review**

A review of previous research that has been done in a particular area and which relates to a current problem being investigated.

**longitudinal study**

A study done over a long period of time – often decades – with the participants being asked the same questions or having the same tests periodically to assess how their health changes.

**medical intervention**

Medical tests, procedures or

treatments that are aimed at relieving illness or injury, or curing disease.

### **medical oncologist**

A doctor who specialises in treating cancer with chemotherapy.

### **medical science**

An area of study focusing on maintaining health and preventing and treating disease.

### **meta-analysis**

A process in which the results of a number of studies researching the same questions are combined and compared to see whether the results have more weight when analysed together.

### **molecular research**

Laboratory research that focuses on discovering which genes are responsible for certain diseases and how the disease develops.

### **molecule**

Very small particles in the body that can join with other molecules to form larger substances. A gene is a type of molecule.

### **MRI scan**

A magnetic resonance imaging scan. A scan that uses magnetism and radio waves to take detailed cross-sectional pictures of the body.

### **multi-centre research**

Research that is conducted at more than one site.

### **oncology**

The study, diagnosis and treatment of cancer.

### **outpatient**

A person who receives medical treatment without being admitted into hospital.

### **palliative care**

Any form of medical care or treatment that reduces disease symptoms and helps improve quality of life.

### **participant information**

An information sheet that explains everything a participant needs to know about the trial and treatment.

**peer review**

A process in which independent experts check research to make sure it is accurate and reliable.

**pharmacogenomics**

A branch of pharmacology that examines both drugs and genes to see why certain people react positively or negatively to different treatments. Also called pharmacogenetics.

**pharmacology**

The study of drugs and how they can be used to treat diseases.

**phase**

A stage of a clinical trial. There are usually four phases of testing.

**pilot project**

A small project that is carried out to see whether a similar large-scale study is realistic to run.

**placebo**

A dummy pill, injection or other treatment that is made to look, taste or feel like a real treatment but doesn't have any active ingredients (if a medicine) or any remedial effect (if another type of treatment).

**prevention trial**

Trials that test new approaches that researchers and doctors believe may lower the risk of getting cancer.

**prospective study**

Research that looks at what happens to different groups of people from the start of the study up to a point in the future.

**protocol**

A plan that describes all the details about a study, including its aims and methods and reasons for being conducted.

**psychosocial research**

Research into the emotional and psychological effects of disease and how people can be helped through supportive care measures.

**qualitative study**

Research that focuses on individual responses rather than numerical data to obtain the results.

**quality of life**

Your comfort and satisfaction, based on how well your physical, emotional, spiritual, sexual, social

and financial needs are met within the limitations of your health and personal circumstances.

### **quantitative study**

A study that focuses on collecting numerical data and analysing the results using statistics.

### **radiation oncologist**

A doctor who specialises in treating cancer with radiotherapy.

### **randomisation**

A method used to prevent bias in research. A computer is used to put patients into groups by chance, rather than the researchers or doctors choosing.

### **randomised controlled trial**

A trial in which participants are randomly allocated to receive the experimental (test) treatment or the standard treatment (the control).

### **research governance officer**

The person responsible for the management and approval of applications for research at their particular location.

### **retrospective study**

Research that looks at what has happened in the past to gain an understanding about why something is occurring now.

### **screening**

An organised program to identify disease in people before any symptoms appear.

### **screening trial**

A trial that tests the best way to find cancer, especially in its earliest stages.

### **side effect**

Unintended effects of a drug or treatment.

### **stage**

The extent of a cancer and whether the disease has spread from an original site to other parts of the body.

### **standard treatment**

The best treatment known and in current use, based on the results of past research.

### **statistics**

A type of mathematics used to collect and analyse large

quantities of numerical data.

### **stem cell research**

Research to better understand how stem cells work and how they might be able to be used to help treat disease.

### **stem cells**

Early-stage cells from which mature cells develop. Stem cells are found in the bone marrow.

### **study sponsor**

The company, organisation or institution that is responsible for carrying out a study.

### **supportive care**

Care that extends beyond treating the actual cancer. It covers wider issues that occur due to cancer and includes counselling, practical assistance, physiotherapy, occupational therapy, spiritual care and complementary therapies.

### **surgical oncologist**

A doctor who specialises in the surgical treatment of cancer.

### **survival rate**

The proportion of patients diagnosed with the same disease

who are still alive after a particular period of time.

### **tissue**

A collection of cells that make up a part of the body. When removed from the body, tissue is sometimes called a biospecimen.

### **tissue bank**

A secure place with freezers where body tissue, such as blood, is stored for future research.

### **tissue banking**

When people donate their tissue for use in research in the future.

### **toxicology**

The study of poisonous substances. It is a branch of pharmacology.

### **translational research**

Research that fast-tracks results from basic research into studies that focus on a clinical situation.

### **x-ray**

A type of scan that shows solid areas in the body such as bone. It is used to diagnose different conditions.



## How you can help

At Cancer Council we're dedicated to improving cancer control. As well as funding millions of dollars in cancer research every year, we advocate for the highest quality care for cancer patients and their families. We create cancer-smart communities by educating people about cancer, its prevention and early detection. We offer a range of practical and support services for people and families affected by cancer. All these programs would not be possible without community support, great and small.

**Join a Cancer Council event:** Join one of our community fundraising events such as Daffodil Day, Australia's Biggest Morning Tea, Relay For Life, Girls Night In and Pink Ribbon Day, or hold your own fundraiser or become a volunteer.

**Make a donation:** Any gift, large or small, makes a meaningful contribution to our work in supporting people with cancer and their families now and in the future.

**Buy Cancer Council sun protection products:** Every purchase helps you prevent cancer and contribute financially to our goals.

**Help us speak out for a cancer-smart community:** We are a leading advocate for cancer prevention and improved patient services. You can help us speak out on important cancer issues and help us improve cancer awareness by living and promoting a cancer-smart lifestyle.

**Join a research study:** Cancer Council funds and carries out research investigating the causes, management, outcomes and impacts of different cancers. You may be able to join a study.

To find out more about how you, your family and friends can help, please call your local Cancer Council.



# Cancer Council Helpline 13 11 20

Cancer Council Helpline is a telephone information service provided by Cancer Council NSW for people affected by cancer.

For the cost of a local call (except from mobiles), you can talk about any concerns confidentially with oncology health professionals. Helpline consultants can send you information and put you in touch with services in your area. If you need information in a language other than English, an interpreting service is available.

You can call the Helpline, Monday to Friday, 9am to 5pm.

If you have difficulty communicating over the phone, contact the National Relay Service ([www.relayservice.com.au](http://www.relayservice.com.au)) to help you communicate with a Cancer Council Helpline consultant.

For more information, go to [www.cancercouncil.com.au](http://www.cancercouncil.com.au).

## Regional offices

### **Central and Southern Sydney**

Woolloomooloo  
Ph: 02 9334 1900

### **Central Coast**

Erina  
Ph: 02 4336 4500

### **Far North Coast**

Alstonville  
Ph: 02 6627 0300

### **Hunter**

Broadmeadow  
Ph: 02 4923 0700

### **Mid North Coast**

Coffs Harbour  
Ph: 02 6659 8400

### **North West**

Tamworth  
Ph: 02 6763 0900

### **Northern Sydney**

Crows Nest  
Ph: 02 9334 1600

### **South West**

Wagga Wagga  
Ph: 02 6937 2600

### **Southern**

North Wollongong  
Ph: 02 4223 0200

### **Western**

Orange  
Ph: 02 6392 0800

### **Western Sydney**

Parramatta  
Ph: 02 9354 2000



Cancer Council  
**Helpline**

**13 11 20**

For support and information on cancer and cancer-related issues, call Cancer Council Helpline. This is a confidential service.

For further information and details please visit our website: [www.cancercouncil.com.au](http://www.cancercouncil.com.au)