



# Understanding Clinical Trials and Research

A guide for people affected by cancer

Practical  
and support  
information

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A guide for people affected by cancer

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*Understanding Clinical Trials and Research* is reviewed approximately every 3 years. Check the publication date above to ensure this copy is up to date.

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### Note to reader

Always consult your doctor about matters that affect your health. This booklet is intended as a general introduction to the topic and should not be seen as a substitute for medical, legal or financial advice. You should obtain independent advice relevant to your specific situation from appropriate professionals, and you may wish to discuss issues raised in this book with them.

All care is taken to ensure that the information in this booklet is accurate at the time of publication. Please note that information on cancer, including the diagnosis, treatment and prevention of cancer, is constantly being updated and revised by medical professionals and the research community. Cancer Council Australia and its members exclude all liability for any injury, loss or damage incurred by use of or reliance on the information provided in this booklet.

### Cancer Council

Cancer Council is Australia's peak non-government cancer control organisation. Through the eight state and territory Cancer Councils, we provide a broad range of programs and services to help improve the quality of life of people living with cancer, their families and friends. Cancer Councils also invest heavily in research and prevention. To make a donation and help us beat cancer, visit [cancer.org.au](http://cancer.org.au) or call your local Cancer Council.



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# About this booklet

Cancer research is an important part of health care. This booklet has been prepared to help you understand more about clinical research, with a focus on clinical trials.

We hope this booklet helps you make an informed decision about taking part in cancer research. It provides an overview of different types of health research and outlines how to get involved in a clinical trial. It includes practical issues to consider when deciding whether to take part.

We cannot give advice about whether you should join a clinical trial. You need to discuss this with your doctors. However, this information may answer some of your questions and help you think about what to ask the clinical trials team (see pages 54–55 for a question checklist).

This booklet does not need to be read from cover to cover – just read the parts that are useful to you. You may also like to pass this booklet to family and friends for their information.

## How this booklet was developed

This information was developed with help from a range of health professionals and people who have taken part in clinical trials.

If you or your family have any questions, call Cancer Council **13 11 20**. We can send you more information and connect you with support services in your area. You can also visit your local Cancer Council website (see back cover).



**Cancer  
Council**  
**13 11 20**

# Contents

<b>Key questions .....</b>	<b>4</b>
What is health research? .....	4
Why is health research important? .....	4
What are the different types of health research? .....	4
What is a clinical trial? .....	8
Why participate in research? .....	8
Who can participate in research? .....	9
Where does research take place? .....	9
Is research safe? .....	10
Who funds cancer research? .....	10
Who is the trial sponsor? .....	11
<b>Types of clinical research .....</b>	<b>13</b>
Clinical trials .....	13
Behavioural research .....	14
Translational research .....	15
<b>Regulation of clinical research .....</b>	<b>16</b>
Human research ethics committee .....	17
<b>Clinical trials explained .....</b>	<b>21</b>
The phases of a clinical trial .....	21
Health professionals and researchers you may see .....	24
Types of clinical trials .....	26
<b>Deciding to take part .....</b>	<b>32</b>
Frequently asked questions .....	33
A second opinion .....	36

<b>Joining a study.....</b>	<b>37</b>
How to find a study .....	37
Australian clinical trials websites.....	38
Taking part in a study .....	40
Informed consent .....	41
Being part of a clinical trial .....	45
Withdrawing from a study .....	47
Finding trial results .....	48
<b>Seeking support .....</b>	<b>50</b>
<b>Caring for someone with cancer .....</b>	<b>53</b>
<b>Question checklist.....</b>	<b>54</b>
<b>Glossary .....</b>	<b>56</b>
<b>Notes .....</b>	<b>62</b>
<b>How you can help.....</b>	<b>64</b>



## Key questions

### Q: What is health research?

**A:** Health research refers to the many types of scientific investigations that aim to test ideas, answer questions, improve treatment options and increase knowledge about human health.

### Q: Why is health research important?

**A:** What we know about cancer changes over time as more research is done. Health research has led to the medical treatments and health programs available today. These advances have helped the five-year survival rate for all cancers to rise from 52% to 68% over the past 20 years.<sup>1</sup>

The search for better ways to prevent, diagnose and treat human diseases is ongoing. It requires the active participation of patients, carers and healthy people.

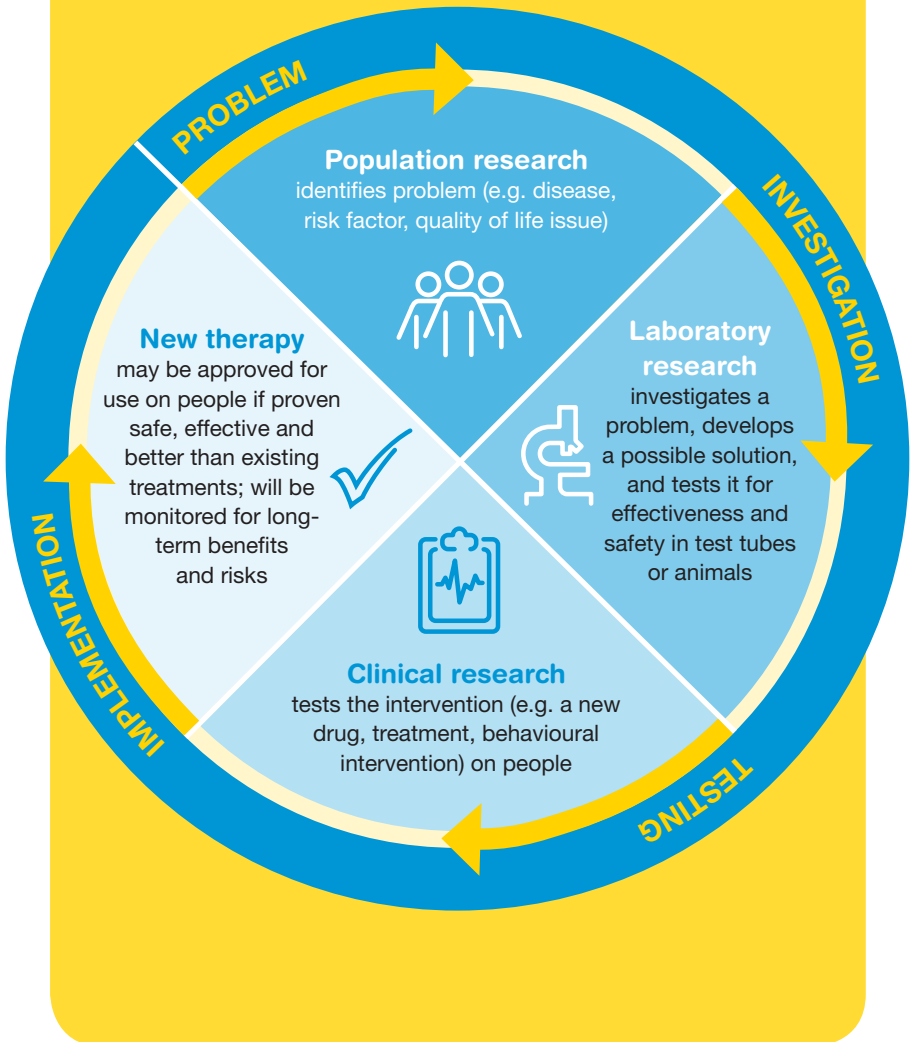
### Q: What are the different types of health research?

**A:** There are three main types of health research: population research, laboratory research and clinical research (see table on pages 6–7). People affected by cancer mainly take part in clinical research.





Population and laboratory research are often the starting point for clinical research. The diagram opposite describes the research cycle.

## Cycle of health research



## Types of health research

	What it is
<p><b>Population research (epidemiology)</b></p> 	<p>The study of how and why diseases occur in groups of people (populations). Scientists working in this field are called epidemiologists. They look for patterns and trends in illness to work out why certain diseases, such as cancer, occur in some people but not in others. Their findings often lead to recommendations for ways to reduce or prevent disease. This type of research focuses on groups of people rather than individuals.</p>
<p><b>Laboratory research</b></p> 	<p>Scientists conduct laboratory experiments with the building blocks of disease to try to understand how a disease works. They study cells, proteins and DNA from humans and animals, or disease-causing agents such as chemicals, bacteria and viruses. Scientists also study and develop new drugs and treatments in the laboratory. Laboratory research is often the starting point for clinical research.</p>
<p><b>Clinical research</b></p> 	<p>Research conducted on people to better understand, diagnose, prevent and treat diseases. It is usually carried out in a clinical setting such as a hospital or outpatient clinic, and it often requires patient participation.</p>



## Key areas

- **Health services research** – investigates the quality, cost and ease of access to services, such as hospitals, specialists and allied health professionals.
  - **Medical data research** – examines medical records, often from hundreds or thousands of people, to understand what causes cancer and how it might be prevented.
  - **Mathematical modelling** – uses information from the past to estimate what might happen in the future, e.g. modelling can work out how many people are likely to be diagnosed with cancer in 10 years' time.
- 
- **Basic research** – looks at the body's 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 helps to show whether a treatment is likely to be safe and effective.
  - **Animal research** – helps scientists understand how a treatment works, problems it might cause, and whether it might be useful in humans.
  - **Stem cell research** – looks at how stem cells develop, their role in causing disease and treatment resistance, and their possible use as a treatment.
  - **Genomic research** – looks at the role of genes in the development of disease, and how a person's genetic makeup can be used to help prevent, diagnose and treat disease.
  - **Pharmacogenetics** – studies how genes affect a person's response to drugs, and why some people respond well to a particular drug and others don't.
- 
- **Human participation studies** – require contact with patients and/or healthy volunteers. Examples include clinical trials and behavioural research using questionnaires. For more information, see pages 21–31.
  - **Record-based studies** – access personal data without face-to-face contact, e.g. examining patients' medical records to see if treatment was successful.
  - **Laboratory studies** – examine human material such as blood or tissue obtained during surgery, from tissue sampling (biopsy), or a post-mortem examination (autopsy). Tissue banking (or biobanking) collects and stores groups of cells (tissue) for use in cancer research.
  - **Technology studies** – develop new technology for diagnosis and treatment.

## Q: What is a clinical trial?

**A:** A clinical trial is one type of clinical research. It helps show whether a new approach to prevention, screening, diagnosis or treatment works better than current methods and is safe. Some clinical trials investigate new uses for existing treatments, for example, whether a drug that has been proven to work for one type of cancer is effective against other types of cancer.

Volunteers are recruited to test the new intervention (e.g. a drug, medical device, surgical method or test) to see whether it works and whether any side effects occur. If the new approach is shown to work better than existing tests or treatments, it may be approved for use. See pages 21–31 for more information.

## Q: Why participate in research?

**A:** The involvement of patients, carers and healthy people in research is necessary to help researchers learn more about a disease and ways to treat it. Most people diagnosed with cancer who decide to participate in research do so because they want to help improve outcomes for others in the future, as well as for themselves.

Adults and children can participate in different ways, including:

- consenting to their medical records being accessed
- completing surveys
- trialling treatments
- agreeing to be examined regularly by health professionals
- allowing samples taken during tests or treatment to be used for research.

## Q: Who can participate in research?

**A:** All research studies, including clinical trials, have guidelines setting out who can participate. Both adults and children can take part, but children under the age of 18 need a parent's or guardian's permission. Most cancer research involves current patients, however, some studies target former patients, carers, family members, people at risk of cancer or people who have not had the disease.

It is important that people of all ages and social, economic and racial backgrounds take part so the results reflect Australia's diverse population. *Deciding to take part* (pages 32–36) can help you weigh up the benefits and risks of being in a study, and answer other questions you may have. To find out more about participating in clinical trials or other types of cancer research, see pages 37–49.

## Q: Where does research take place?

**A:** Research is carried out in many places, including hospitals, cancer centres, laboratories and universities. Sometimes you can participate from home – you might have treatment or medicines mailed to you, or you might be asked to fill in a survey or complete a telephone interview.

The Australian Government provides useful information for people considering joining a clinical trial at [australianclinicaltrials.gov.au](http://australianclinicaltrials.gov.au).



## Q: Is research safe?

**A:** Understandably, people want to know if there are any risks to 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 human research ethics committees before they can begin. As part of this process, researchers identify risks that might occur, such as possible side effects. They must also explain how they'll monitor these risks and what will be done if problems occur. For more information, see *Regulation of clinical research* on pages 16–18.

## Q: Who funds cancer research?

**A:** Funding comes from many sources.<sup>2</sup>

### **National Health and Medical Research Council (NHMRC) –**

This is the Australian Government's main funding body for medical research. The NHMRC awards grants to researchers based on their ability to investigate important questions about human health. The NHMRC also administers the government's Medical Research Future Fund.

**Cancer charities** – State and territory Cancer Councils and other charities receive donations from the public, and grants from both public and private sectors. This funds their own research and allows them to financially support research carried out by other institutions.

**Government bodies** – Other Australian, state and territory government agencies offer a competitive grants program to fund research and to employ cancer trials staff.

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

**Private sector (industry funded)** – Companies producing medicines and medical equipment run trials to check safety and effectiveness before applying for licences to sell these products. Private companies may also fund research in partnership with a university or other research institution, or for goodwill (philanthropic) reasons.

## Q: Who is the trial sponsor?

**A:** The organisation, institution or company responsible for the overall conduct of a clinical trial is known as the trial sponsor. Their responsibilities include developing, financing and managing the trial, and ensuring the trial meets all legal and insurance requirements.

### Conflict of interest

A conflict of interest may arise in any research project. This means the interests of an organisation or researcher could influence the outcome of the research. Researchers are required to disclose any possible or actual conflicts of interest. All research projects should include details of how a conflict of interest will be managed.

## Involving consumers in research

Sometimes people want to contribute to research but don't want to participate 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 are now involved in identifying priorities for research and helping to decide what projects should be funded. They may also work directly on research studies by providing an informed consumer review.

Cancer Australia developed the National Framework for Consumer Involvement in Cancer Control. This includes a web-based toolkit designed to support CEOs, managers, health professionals, researchers and policymakers in involving consumers in their

work. For more details, go to **[consumerinvolvement.canceraustralia.gov.au](http://consumerinvolvement.canceraustralia.gov.au)**.

Cancer Council offers an online Cancer Consumer Involvement in Research Training Program to help people learn how to work with researchers. For more details, go to **[cancer.org.au/about-us/consumertraining.html](http://cancer.org.au/about-us/consumertraining.html)**.

Consumer advocacy organisations operate in most states. They focus on improving cancer treatment through active consumer participation. To learn more, visit them online at:

- Cancer Voices Australia **[cancervoicesaustralia.org](http://cancervoicesaustralia.org)**
- Cancer Voices NSW **[cancervoices.org.au](http://cancervoices.org.au)**
- CanSpeak Queensland **[canspeak.org.au](http://canspeak.org.au)**
- Cancer Voices SA **[cancervoices.sa.org.au](http://cancervoices.sa.org.au)**
- Cancer Action Victoria **[canceractionvic.org.au](http://canceractionvic.org.au)**

If you would like to get involved, contact one of these organisations or call Cancer Council **13 11 20**.



# Types of clinical research

The three main types of clinical research involving human participation are clinical trials, behavioural research and translational research.

## Clinical trials

Clinical trials are an essential step that show whether new approaches work better than those currently used, and whether they are safe.

There are several types of clinical trials designed to answer different research questions.

- **Treatment trials** – test new treatments, new ways of giving existing treatments, or new combinations of treatments such as drugs, radiation therapy, surgery, nutrition, physiotherapy and complementary therapies. Most clinical trials in Australia are treatment trials.
- **Prevention trials** – evaluate whether medicines or health programs lower the risk of people developing diseases such as cancer.
- **Screening trials** – look at new methods of detecting diseases before symptoms appear.
- **Diagnostic trials** – identify more accurate or less invasive ways of diagnosing a particular disease in people who have signs or symptoms.
- **Quality of life trials** – evaluate ways to improve the comfort and quality of life of people who have cancer; often assessed as part of a treatment trial.

## Behavioural research

Behavioural researchers try to understand why people behave in the way that they do. They study people's individual characteristics, lifestyles and social circumstances to see how these factors affect the risk of someone developing or surviving cancer. They then try to develop ways to prevent or change risky behaviours.

Behavioural researchers also look at the emotional and social impacts of cancer on the person with cancer, as well as the impact on their family and friends.

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

You may also be asked to participate in a program aimed at positively changing these behaviours. For example, you might be offered free counselling, an exercise class or a session on healthy eating. The aim of the programs may be to reduce cancer risk or to improve the way you cope with cancer.

## Psychosocial research

One area of behavioural research is called psychosocial research. This looks at how cancer impacts people emotionally, psychologically and socially. In cancer care, this is sometimes called psycho-oncology.

Researchers try to understand how patients and carers cope emotionally at different stages of a disease. They develop and test methods to improve people's ability to deal with various issues.



## Translational research

Translational research provides a bridge between basic and clinical research. It aims to get new treatments or medical approaches into practice quickly. It is sometimes called “bench to bedside” research because basic research results are directly used to create new therapies and diagnostic tools.

Findings in the clinic can also influence research in the laboratory. This is called “bedside to bench” research. For example, hospitals and health care professionals give information to researchers about the effectiveness of a treatment to help direct research into the most useful areas.

### The National Health and Medical Research Council (NHMRC)

The NHMRC’s mission is to build a healthy Australia.

It does this by:

- developing health advice for the general community and health professionals
- funding research projects ranging from basic through to clinical, public health and health services research
- supporting the translation of research findings into clinical practice.

The NHMRC also produces guidelines outlining ethical conduct in research.

Ethics committees (see page 17) must follow these guidelines and report to the NHMRC every year.

You can download the *National Statement on Ethical Conduct in Human Research* (2007) (Updated May 2015) from [nhmrc.gov.au/guidelines-publications/e72](http://nhmrc.gov.au/guidelines-publications/e72).



# Regulation of clinical research

Clinical research is regulated to make sure it is conducted to a high professional and scientific standard. In Australia, several committees examine and approve a clinical research study before it begins.

These committees confirm that a study is considered scientifically worthwhile and fair to the participants (ethical). They also check that the study meets any regulatory requirements set out in State and Commonwealth legislation.

- **Research or scientific review committee** – decides whether the study has social and scientific value, and if the way it is going to be conducted will produce valid scientific results. May be a subcommittee of a human research ethics committee.
- **Human research ethics committee (HREC)** – confirms that the interests of participants are protected and that researchers will run the study in a fair, honest and neutral (impartial) way. It ensures researchers won't force people into participating, and that the risks of the research generally don't outweigh the benefits. See the opposite page for more details.
- **Research governance review** – checks every site where the research will take place. This review is done by a research governance officer, who makes sure there are enough resources to carry out the proposed research and that the staff members involved are 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.

## Clinical research standards

All health professionals must follow a set of international standards for designing, conducting, recording and reporting clinical research called Good Clinical Practice (GCP). These standards ensure that people taking part are safe, their privacy is protected, information is collected to the highest standard, and results are reliable. GCP is the same anywhere in the world where clinical research is conducted.

## Human research ethics committee

Once a study has been approved by a research review committee, the HREC 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 HREC 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 pages 42–43)
- how risks to participants will be minimised
- how participants' personal information will be used and stored
- how the study might impact on the participants' quality of life.

Committee members are always independent of the researchers. They come from a variety of backgrounds, including medical, scientific, legal and religious professions, and the general community. To reflect different points of view and provide balance, the HREC will consist of at least eight core members with a range of experiences.

A research study cannot begin until it has received approval from the HREC and the governance office. For more information on how studies are conducted fairly, see *Types of clinical trials* on pages 26–29.

## Changes to research

Sometimes changes need to be made to the research:

- **Before approval** – The HREC may ask the research team to make changes to the proposed research before it can go ahead.
- **After approval** – Any changes that researchers want to make during the study must go through ethics approval again before the change can take place. For example, the researchers may want to expand the size of the study, or some safety information may have become available about the intervention. This is called an amendment.

If there are any changes to the participant information, participants already taking part in the study may need to sign a form to show that they have been told about the changes (amendment) and still agree to be involved.

### Therapeutic Goods Administration

The Therapeutic Goods Administration (TGA) is an Australian Government department that regulates all medicines sold in Australia. Any experimental drug used in a clinical trial must be registered with the TGA. For more information, visit [tga.gov.au](https://www.tga.gov.au).



## Philipa'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 wait 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 wellbeing. 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. 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.



## Key points about clinical research

### Types

There are three main types of clinical research involving human participation:

- Clinical trials test new or modified ways of preventing, screening, diagnosing and treating cancer. Most cancer trials look at new treatments.
- Behavioural research looks at why people behave the way they do and whether there are things that can be done to improve people's ability to cope with cancer.
- Translational research aims to get new treatments into practice quickly by sharing information on treatment back and forth between laboratory and hospital.

### Regulation

- All clinical research is regulated to ensure it is worthwhile, ethical and will produce valid results.
- All research proposals must be approved by a research or scientific review committee, a human research ethics committee and a research governance officer.
- Regulation helps ensure that Australian research studies are safe for participants and that the results are reliable.



# Clinical trials explained

Clinical trials look at new ways to 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 of care for patients in the future. The majority of cancer clinical trials are treatment trials, testing new drugs, devices, radiation therapy regimens or surgical techniques. Most of the information in this chapter relates to treatment trials.

## The phases of a clinical trial



Researchers spend many years developing new treatments or medicines in the laboratory before involving people. They then plan the clinical trial to progress in a series of up to four steps called phases. Information gathered in each phase determines whether the study can move on to the next phase, and whether the drug or treatment is approved for use. See the next page for a description of the different phases.

### What are Phase 0 trials?

These are exploratory studies, sometimes referred to as “microdosing” or “pilot studies”. These trials are less common, and are used to test how the body responds to an experimental drug and whether it is worth moving on to a Phase 1 trial. A small dose of the drug is given once or for a short time to 10–15 people, sometimes to healthy volunteers. These trials do not offer any direct benefit to participants.



To find out how to get involved in a clinical trial or another type of study, see pages 37–49.

	PHASE 1	PHASE 2
PARTICIPANTS	 <p>10–100</p>	 <p>100–300</p>
PURPOSE	<ul style="list-style-type: none"> <li>• first study in people</li> <li>• tests safety of new treatment</li> <li>• finds the safest dose and the best way a treatment can be given</li> <li>• identifies side effects</li> <li>• studies how the intervention works with other medicines or food (interactions)</li> </ul>	<ul style="list-style-type: none"> <li>• builds on the results of Phase 1</li> <li>• continues to test safety of a drug</li> <li>• begins to assess how well a new treatment works on the disease</li> </ul>
HOW IT WORKS	<ul style="list-style-type: none"> <li>• participants are given a fixed dose and watched closely for side effects</li> <li>• if no side effects, dose is increased in next group of participants, this continues until noticeable side effects appear</li> </ul>	<ul style="list-style-type: none"> <li>• often focuses on one cancer type</li> <li>• all participants receive the same experimental treatment</li> <li>• sometimes randomised controlled trial – i.e. participants are put into separate groups and given different treatments, which are then compared to see how well they treat the disease</li> </ul>
MORE INFO	<ul style="list-style-type: none"> <li>• patients sometimes benefit from having the new treatment but great improvements in their condition are uncommon</li> </ul>	<ul style="list-style-type: none"> <li>• people often have treatment as an outpatient, but occasionally need to stay overnight in hospital for monitoring</li> </ul>



PHASE

3



100s–1000s

- tests if the new treatment is better than the standard treatment
  - compares side effects, survival and quality of life
  - assesses whether the risks outweigh the benefits
  - collects information that allows new treatments and existing treatments to be used in new ways or for different diseases
- 
- uses two or more treatment groups – experimental and control
  - usually groups are randomised and sometimes blinded (see page 30)
  - placebo may be used for comparison
  - runs over a long period of time
- 
- Phase 3 trials help the Therapeutic Goods Administration (TGA) assess whether a new drug should be registered for use in Australia

PHASE

4



1000s

- identifies how well treatment works when used more widely
  - monitors the long-term benefits and risks
  - looks for other uses of the drug or treatment
- 
- carried out after a treatment has been registered by the TGA
  - usually run by pharmaceutical companies that make the product
  - may be used as a way to provide early access to a drug after TGA approval but before Pharmaceutical Benefit Scheme (PBS) approval
  - sometimes called expanded access study
- 
- not all treatments go through Phase 4 studies
  - they are less common than Phase 1–3 trials

## Health professionals and researchers you may see

A whole team of people work in clinical trials, and some of their roles overlap. If you join a clinical trial, you may have contact with some of the following people.

### Cancer specialist

- may be a medical oncologist, surgeon, radiation oncologist or haematologist
- supervise your treatment, follow-up and overall care
- usually the coordinating or principal investigator

### Investigator

- also known as researchers
- develops and plans studies, and obtains, analyses and publishes results
- may have a background in medicine, science, psychology, allied health, consumer advocacy or complementary therapies
- the principal investigator has overall responsibility for conducting the trial at their hospital, ensuring patients are safe and that the trial is properly run; is usually a doctor with expertise in the field of research
- the coordinating principal investigator oversees research taking place at more than one study site, e.g. at two or more hospitals



Nurse	Study coordinator	Other professionals
<ul style="list-style-type: none"><li>• may be a clinical trials nurse or a clinical research nurse</li><li>• coordinates recruitment by talking to potential participants, making sure they are eligible and explaining the purpose of the trial</li><li>• arranges appointments for tests, treatments or to see the specialist</li><li>• makes sure all paperwork is completed once you have agreed to join a trial</li><li>• provides emotional support</li><li>• acts as a link between the patient and the researchers or the health care team</li><li>• may also be the main contact person (see page 43)</li><li>• larger clinical trials or hospitals have a dedicated clinical trials nurse, but smaller ones might not</li></ul>	<ul style="list-style-type: none"><li>• may be called a clinical research coordinator or clinical trials coordinator</li><li>• has a science degree or similar</li><li>• ensures the trial meets ethical and legal requirements</li><li>• applies for grants and manages budgets</li><li>• reviews the study protocol and organises study records</li><li>• may be combined with the nursing role</li></ul>	<ul style="list-style-type: none"><li>• a pharmacist will provide advice about medicines and monitor their effect on patients; may conduct laboratory research</li><li>• allied health practitioners and complementary therapists may give treatment or advice in studies investigating the use of non-medical treatments such as nutrition, massage, physiotherapy, counselling, or acupuncture</li></ul>

## Types of clinical trials

Researchers plan and carry out studies in a way that ensures results are accurate and not caused by chance. This means they have to follow strict guidelines. They also need to make sure their own – or the participants’ – ideas or beliefs about the research don’t unfairly influence the results. This chapter describes some methods used to make sure clinical trials are fair and reliable.

### Randomised controlled trials

Randomised controlled trials (RCT) are the best way to test if a new treatment is effective. This is because they help prevent bias. Bias occurs when the results of a trial are influenced by human choice, expectations or other factors not related to the treatment being tested.

Most Phase 3 trials and some Phase 2 trials are randomised. This means participants in the study are randomly assigned into two or more groups, or arms, and the results of the different groups are compared.

**Test or experimental group (or arm)** – This group is given the experimental intervention. Sometimes, the experimental treatment is given in addition to the current standard treatment.

**Control group (or arm)** – This group receives the current standard treatment for the disease.

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, and not to any other factors (see also *Blinded studies*, page 30).

## How randomisation works

Patient information is entered into a computer.



The computer gives each participant a code number.

The code numbers are randomly assigned to the different treatment groups, helping to prevent bias.



The control group receives the standard treatment and the test group receives the new treatment.



## Non-randomised trials

In a single group (arm) trial, all participants receive the same experimental treatment. This method may be used for Phase 1 and Phase 2 trials, or where the cancer being treated is rare and it is hard to conduct a randomised trial.

## Standard treatment and placebos

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

In some cases, active surveillance or watchful waiting is the best currently available standard of care. For example, active surveillance is recommended for some early thyroid cancers that aren't causing any symptoms and are considered to be low risk.

**Placebo** – This is an inactive or mock treatment made to look, taste or feel like the treatment being tested, but that doesn't have any active (therapeutic) ingredients (if a medicine) or beneficial effect (if another type of treatment). Examples of placebos are sugar pills and saline injections.

A placebo is used to compare treatments to see whether the patients' outcome is because of the actual treatment or because of other factors associated with being in the study. If the people given the experimental treatment show more improvement than those given the placebo, this provides stronger evidence that it's the experimental

treatment that is responsible. Participants will be told if a study uses a placebo, but will not be told which treatment they are receiving.

In cancer treatment trials, placebos may be used:

- together with the standard treatment, for example, one group receives the existing standard therapy plus the experimental treatment, and the other group receives the standard treatment plus a placebo
- on their own, when there is no existing standard treatment to compare against an experimental treatment.



### 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. It required an overnight stay in hospital to have the biopsy and treatment, and to be monitored.

I had to fill out questionnaires about my emotional and physical wellbeing on the day of the trial and the following day. This gave me something to do 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.

## Crossover studies

In these studies, participants in each trial arm receive their assigned treatment for a period of time before swapping to the other treatment. This enables all participants to experience all treatments, and helps to confirm which is the most effective. Crossover studies are often used when researchers feel it would be difficult to recruit participants willing to risk not receiving a promising new treatment.

## Blinded studies

In a blinded study, participants don't know which arm of a study they're in. Some randomised controlled trials are called double-blind studies as neither the participant or trial team members know who is receiving the experimental or control treatment. In a double-blind 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.

Blinding is used only when participants can't tell the difference between the two types of treatment. It is not used when the control and experimental treatment are noticeably different – for example, it would be hard to disguise surgery and massage from the participant.

The aim of blinding is to reduce bias in the reporting of benefits and side effects. If you don't know which treatment you're having, the results are less likely to be influenced by your or your doctor's thoughts. For example, if you or your doctor knew you were having the experimental treatment, you might report that you're feeling better than you actually are because you believe you are receiving an effective treatment. If necessary for safety reasons, your doctor can find out what treatment you're having by contacting those running the study.





## Key points about clinical trials

### What they are

- A way to test whether a new intervention is safe and effective in people.
- There may be up to four phases in a clinical trial to test whether the benefits outweigh the risks.
- Many different health professionals and researchers work in clinical trials, including cancer specialists, investigators, clinical trials nurses and research coordinators.

### Types of trials

Researchers use different methods to ensure that the results are accurate:

- Randomised controlled trials test different treatments by randomly dividing participants into two more separate groups. They are the best way to prevent bias and assess which treatment is better.
- The test group will receive the experimental treatment and the control group will receive the current standard treatment.
- In a blinded study, the participant will not know whether they are receiving the experimental or standard treatment.
- In a double-blind study, neither the participants nor the research team know what treatment is being applied to each group.
- All participants in a non-randomised (single group) trial receive the same experimental treatment. This type of trial is less common.



# Deciding to take part

You may have many questions when deciding whether to join a clinical trial. As well as talking to your doctor and clinical trials nurse, it is recommended that you talk to your family or carer. This is because your involvement may also affect them (see page 53). 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 may 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. You should usually be given a few days to consider the participant information. If you would like to take more time to think about your participation, ask if this is likely to affect your treatment outcomes. If you decide not to join a study, you will still receive the standard treatment for your disease.

## Weighing up the benefits and risks

- Consider what is most important to you. Some people want to be certain of which treatment they will receive, others prefer the opportunity to try something new.
- Think about the possible impacts of the study on your wellbeing and lifestyle. What is the chance of any serious side effects? Will any requirements of the study be too difficult, e.g. having to have additional tests or extra trips to the hospital. Weigh up these risks and drawbacks against the possible benefits, such as a possibly longer survival time or not having to experience certain side effects.
- Everybody's situation is different – what is right for someone else may not be right for you.

## Frequently asked questions

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

**A:** There are many advantages of 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 treatment for the disease
- accessing expensive drugs that are not currently covered by the Pharmaceutical Benefits Scheme (PBS)
- seeing your treatment team, including specialists, more often
- taking an active part in your health care
- learning new ways to improve your lifestyle
- 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.

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

In a clinical trial, you may be in the control arm and not given the experimental treatment (see page 26). If this is the case, you will receive the current standard treatment, and be monitored more frequently and closely than usual. Either way, your doctor and the clinical trials or research nurse will discuss the possible advantages and disadvantages for you before you join a study.

## **Q: How long will a study last?**

**A:** From start to finish, a study may take several months, years or even, in some cases, decades. However, you may only need to be involved for some 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. The study is then closed to new recruits.

Once the study is over, there may be a follow-up phase. People may be followed up at set intervals for months or years after their treatment is over. Make sure you ask how long you need to be involved for.

The participant information and consent form will set out the expected time frame.

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

**A:** You may be interested in joining 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. It is not usually possible to join more than one clinical trial at a time.

### **Q: Is participation free?**

**A:** The cost of trial-related treatment, tests and check-ups will be paid for by the organisation that is funding or conducting the research. You will still have to pay for any treatments or tests you have as part of your standard care. The participant information statement (see pages 42–43) will outline any costs.

### **Q: Will I be paid?**

**A:** People participating in cancer research and trials don't usually receive payment. It is not acceptable for researchers to offer participants money to encourage them to join a clinical trial. Out-of-pocket costs (e.g. travel or parking) may be refunded. The participant information statement (see pages 42–43) will outline any payments.

### **Q: Can I have other treatment?**

**A:** Check whether the study will have an 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 delay these other treatments, or whether they need to be modified (for example, by changing the dose).



It is important to let your doctor know about any other medicines or supplements you are taking, as they can interact with the experimental treatment and cause harmful 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
- help you to consider all the advantages and disadvantages of being on the trial
- reassure you that you have thought about the different issues that might affect you.

Ask your general practitioner (GP) for a referral to another cancer specialist, but keep in mind that you may have to wait several weeks for an appointment and it may cost you extra money. You can also ask your cancer specialist if it is possible to talk to another specialist on the hospital ward or in the clinic. Some people also like to discuss the possibility of a trial with their GP.

““ A clinical trials nurse accompanied me at every stage of the process. She explained what was happening and answered any questions I had. ””

*Marg (breast cancer)*



# Joining a study

This chapter explains how to find out about a study and what happens once you've decided to participate.

## How to find a study

There are many ways to find out about a clinical trial or other study. Most specialists know about current studies and may recommend a suitable study to you. If your hospital has a clinical trials or research nurse, you can also ask them whether there are any studies that might be appropriate for you.

Hospital and treatment centre waiting rooms often have information about current studies. You can also search clinical trials websites or you might hear about a study through patient support groups or 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 the next two pages for a list of websites where you can find or register for clinical trials.

If you find a trial you're interested in joining, ask your doctor if you meet the eligibility criteria and, if so, whether they could coordinate your involvement or put you in touch with the research team.



You don't have to join a study at your treatment centre – your doctor can refer you to a more suitable trial at another centre.

## Australian clinical trials websites

### To find current trials

Australian Cancer Trials	<a href="http://australiancancertrials.gov.au">australiancancertrials.gov.au</a>
Australian New Zealand Clinical Trials Registry	<a href="http://anzctr.org.au">anzctr.org.au</a>
Cancer Council SA	<a href="http://cancersa.org.au/research/current-clinical-trials">cancersa.org.au/research/current-clinical-trials</a>
Cancer Institute NSW	<a href="http://cancerinstitute.org.au/data-research/clinical-trials/clinical-trials-in-nsw-list">cancerinstitute.org.au/data-research/clinical-trials/clinical-trials-in-nsw-list</a>
ClinTrial Refer	<a href="http://clintrial.org.au">clintrial.org.au</a>
Victorian Cancer Trials Link	<a href="http://trials.cancervic.org.au">trials.cancervic.org.au</a>
Western Australia Cancer Clinical Trials Registry	<a href="http://cancerwa.asn.au/patients/making-decisions-about-treatment/clinical-trials/wa-clinical-trials-registry">cancerwa.asn.au/patients/making-decisions-about-treatment/clinical-trials/wa-clinical-trials-registry</a>

### National Cancer Cooperative Trials Groups

Australasian Gastro-Intestinal Trials Group	<a href="http://gicancer.org.au">gicancer.org.au</a>
Australasian Leukaemia & Lymphoma Group	<a href="http://allg.org.au">allg.org.au</a>
Australasian Lung Cancer Trials Group	<a href="http://altg.com.au">altg.com.au</a>
Australasian Sarcoma Study Group	<a href="http://australiansarcomagroup.org">australiansarcomagroup.org</a>
Breast Cancer Trials	<a href="http://breastcancertrials.org.au">breastcancertrials.org.au</a>



## National Cancer Cooperative Trials Groups – continued

Australia and New Zealand Children's Haematology/Oncology Group	<a href="http://anzchog.org">anzchog.org</a>
Australia and New Zealand Melanoma Trials Group	<a href="http://anzmtg.org">anzmtg.org</a>
Australian and New Zealand Urogenital and Prostate Cancer Trials Group	<a href="http://anzup.org.au">anzup.org.au</a>
Australia New Zealand Gynaecological Oncology Group	<a href="http://anzgog.org.au">anzgog.org.au</a>
Cooperative Trials Group for Neuro-Oncology	<a href="http://cogno.org.au">cogno.org.au</a>
Palliative Care Clinical Studies Collaborative (PaCCSC)	<a href="http://uts.edu.au/research-and-teaching/our-research/IMPACCT/paccsc/what-paccsc">uts.edu.au/research-and-teaching/our-research/IMPACCT/paccsc/what-paccsc</a>
Primary Care Collaborative Cancer Clinical Trials Group	<a href="http://pc4tg.com.au">pc4tg.com.au</a>
Psycho-oncology Co-operative Research Group (PoCoG)	<a href="http://pocog.org.au">pocog.org.au</a>
Trans Tasman Radiation Oncology Group (TROG)	<a href="http://trog.com.au">trog.com.au</a>
<b>To register for studies</b>	
Breast Cancer Network Australia	<a href="http://bcna.org.au/get-involved/participate-in-research">bcna.org.au/get-involved/participate-in-research</a>
Center for Analysis of Rare Tumors (CART-WHEEL)	<a href="http://cart-wheel.org">cart-wheel.org</a>
Register4	<a href="http://register4.org.au">register4.org.au</a>

## Taking part in a study

Before a trial begins, researchers develop the protocol. This detailed plan describes the study's design, reasons for the study and who can join (the eligibility criteria).

### Eligibility criteria

All clinical trials have guidelines for who can participate. These are called the eligibility criteria and they outline the characteristics that must be shared by all participants to ensure that the people taking part are as similar as possible.

Eligibility criteria can be broken down into:

- **inclusion criteria** – the characteristics a person needs to have to join a study
- **exclusion criteria** – the factors that stop someone from participating.

The eligibility criteria also make sure people are safe while on a trial by taking into account any characteristics that might make your participation more risky. For example, you may be excluded from a trial if you are pregnant, have high blood pressure, or have some other condition that significantly increases the risks of the treatment.



Although there are thousands of different clinical trials occurring around the world at any one time, eligibility requirements can be very specific, and there may not be a trial suitable for your particular situation. For example, if you have metastatic cancer, the disease may have to be active or progressing for you to be eligible to join a trial.

Depending on the research, eligibility criteria may include:

- age or sex
- cancer type
- stage of the cancer
- symptoms or side effects experienced
- length of time since diagnosis or treatment
- previous treatments for the cancer
- previous medical conditions.

## Informed consent

Before becoming part of a clinical trial or other research study, all participants have to give “informed consent”. This means you will be asked to confirm you have read and understood the purpose, risks and possible outcomes of the research before deciding whether to join. Informed consent involves two steps:

- **Written information** – You will receive written information about the study, explaining its purpose, what is expected of the participants, what the possible risks are and how the results will be presented. This is called participant information (see next page). You can also talk to your doctors and/or clinical trials or research nurse about any aspect of the study.
- **Agreement in writing** – You agree to participate by signing the informed consent form. For people under 18, a parent or guardian has to give legal consent. Signing the form is not a contract and you can withdraw at any time (see page 47). You will receive a copy of the consent form signed by you and the researcher.



Sometimes you may need to consent to 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, which may also require you to consent again.

The process of providing informed consent continues throughout a study. If the study changes or new information becomes available while you are involved, you will be given updated participant information and you will need to sign an updated version of the consent form if you are willing to continue.

## Participant information

Researchers must provide written information about the 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
- who is running the study (institution and researchers)
- who has approved the research
- who is funding the study
- how the study will be run and what you need to do
- how the study will be monitored
- whether you will need to have tests or other procedures
- 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
- whether you will be reimbursed for any related expenses
- information about possible side effects or other risks
- information about possible benefits
- any restrictions on things you can do while you are on the study, e.g. other treatments you can have
- who to contact for further information or if you have any problems or complaints during the study (see *Contact person* below)
- information about your rights, such as keeping your records private (see page 44) and your ability to withdraw from a study.

The participant information can help people decide whether they want to join or continue participating in a study. You can also ask the research team any questions you have about the study. It's a good idea to mark any questions on the information sheet so that you can raise them with the doctor or study coordinator.

## Contact person

All studies have a contact person. You can talk to this person before you decide to participate and at any stage during the study if you have questions or concerns. The contact person is often a clinical trials nurse or study coordinator (see page 25).

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.

## Privacy

Medical records are private and confidential, including those relating to your involvement in a trial or study. Health professionals directly involved in your care or study can access your personal and medical information, but only if it's necessary for their work. They can't disclose 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.

Information collected during the study is often de-identified. This means that it won't have your name on it so the results cannot be linked to you. Sometimes not even the researchers know which results belong to which individuals. When the results are published in journal articles and discussed at conferences, you will not be named.

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



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

## Communicating with the treatment team

- Keep contact information handy if you have questions before and during the study.
- Sometimes a trial is run at a different hospital to the one where you are 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 ask who your main contact person is. It may also help to take your own record of test results to the hospital.
- If you are in a clinical trial and develop significant problems, go to your hospital's emergency department and/or contact the oncology registrar. Make sure to tell them you're in a clinical trial. If you go to hospital, let your trial team know.

## Being part of a clinical trial

What you need to do when you agree to join a clinical trial depends on what kind of research it is. Generally, only treatment trials require preparation or ongoing follow-up, but it depends on what the study is testing and what phase it is in.

Your participation is usually organised by one person (often a clinical trials or research nurse), but you may come into contact with different members of the research team (see pages 24–25). Your overall care will probably continue to be coordinated by your cancer specialist.

### Before the trial starts

- Discuss the trial with a member of the clinical trials team, your oncologist or other cancer specialists.

- Read the participant information (see pages 42–43). You may want to discuss the information with family, friends or your GP.
- Ask your doctors or the clinical trials or research nurse any questions you have about the study. For some suggested questions, see pages 54–55.
- Have any medical tests, such as a CT scan and blood test, to check that the trial is suitable for you.

### **During the trial**

- Follow the instructions you are given about the trial to help ensure that the trial results are as reliable as possible. That means going to all appointments, having the required tests, taking medicines at the specified time, and completing logs or questionnaires.
- Be prepared for more tests and visits to your doctor than you would normally have. 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 emotionally and physically.

### **After the trial is over**

- Researchers may stay in contact with you and collect follow up information for some time after the trial so they can gather 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.



## Continuing access to medicines

Many people wonder whether they're able to continue receiving the experimental treatment after a trial is over.

This depends on several factors including the trial phase and results, how effective the treatment was for you, what the recommended course of treatment is, and whether the trial sponsor is prepared to continue providing the treatment.

Some people join clinical trials to access treatments that would

otherwise not be available. It can be frustrating to not be able to continue with a promising treatment after the study ends.

In many cases when the trial shows that the experimental treatment is effective and has no significant side effects, treatment may be continued long-term even after the trial is over.

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

## Withdrawing from a study

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

- no longer have the time or energy to commit to it
- don't feel it is helping
- are having side effects or your health is worsening
- move further away from where you are receiving treatment
- change your mind.

If you do decide to withdraw from a study, you will not be penalised, and you will receive the standard treatment that is currently the best option for you.

## Finding trial results

It can take a while to get trial results. Usually results are available 2–5 years after the study finishes, but sometimes it can take 10 years or more. The results of most clinical trials will be published in medical journals and presented at conferences and scientific meetings.

If you'd like to know the results of the study you participated in, start by asking your doctor or clinical trials coordinator. The participant information you read at the beginning of the trial and the informed consent document you signed often say how the results will be available.

You may want to ask your doctor what the results mean. When research teams analyse clinical trial results, they look at end points. Examples of end points are whether a cancer has shrunk (response to treatment) or how long it is before the cancer starts to grow again (duration of response).

“ My doctor suggested I take part in a study and I thought it sounded beneficial. I found the thorough disclosure of both the trial and the possible side effects reassuring. ” Piers (*chronic lymphocytic leukaemia*)



## Key points about participation

### Deciding to take part

- There are lots of things to consider when deciding whether to join a clinical trial, such as the impact of the study on your wellbeing and lifestyle.
- You need to weigh up the benefits and risks to decide if the study is right for you.

### Joining a study

- There are different ways to find out about current studies, including your specialist, your hospital's clinical trials nurse, clinical trials websites, and patient groups.
- Participants will need to meet eligibility criteria to ensure everyone in the group has similar characteristics.
- You have to give informed consent to join a trial. Before joining, you will be given written information about the study and will need to sign a consent form.
- It is recommended that you talk to your doctors or the clinical trials nurse about your participation in the study.

### During the trial

- It is important to follow the instructions from the clinical trials team to ensure the trial results are valid.
- Your clinical trials team will monitor your emotional and physical health.
- You can leave a clinical trial at any time without any penalty.
- Once the trial is over, it may take a couple of years before the results are available.



## Seeking support

When you are first diagnosed with cancer, 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, some people may want to take part in research or they 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. Although people who participate in trials generally report a positive experience, you may find that the extra appointments are stressful or that dealing with a different health care team is unsettling or confusing. The treatment you're having may cause side effects, or you may worry that you're not getting the best treatment.

After the trial ends, you may be relieved because you no longer have this commitment. You may be happy with the outcome and feel ready to put the cancer behind you. On the other hand, you may feel worried because your health won't be monitored as frequently or disappointed that the cancer has not gone into remission.

It's important to discuss any worries with your doctor or clinical trials or 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.

## Support from Cancer Council

Cancer Council offers a range of services to support people affected by cancer, their families and friends. Services may vary depending on where you live.

### Cancer Council 13 11 20

Trained professionals will answer any questions you have about your situation and link you to services in your area (see inside back cover).



### Information resources

Cancer Council produces booklets and fact sheets on over 25 types of cancer, as well as treatments, emotional and practical issues, and recovery. Call **13 11 20** or visit your local Cancer Council website (see back cover).

### Practical help

Your local Cancer Council can help you find services or offer guidance to manage the practical impact of a cancer diagnosis. This may include access to transport and accommodation services.



### Legal and financial support

If you need advice on legal or financial issues, we can refer you to qualified professionals. These services are free for people who can't afford to pay. Financial assistance may also be available. Call Cancer Council **13 11 20** to ask if you are eligible.

### Peer support services

You might find it helpful to share your thoughts and experiences with other people affected by cancer. Cancer Council can link you with individuals or support groups by phone, in person, or online. Call **13 11 20** or visit [cancercouncil.com.au/OC](http://cancercouncil.com.au/OC).



## Useful websites

You can find many useful resources online, but not all websites are reliable. These websites are good sources of support and information. See also pages 38–39 for a list of Australian clinical trials websites.

### Australian

Australian Clinical Trials	<a href="http://australianclinicaltrials.gov.au">australianclinicaltrials.gov.au</a>
Cancer Council Australia	<a href="http://cancer.org.au">cancer.org.au</a>
Cancer Council Online Community	<a href="http://cancercouncil.com.au/OC">cancercouncil.com.au/OC</a>
Consumer Involvement in Cancer Cooperative Trials Groups	<a href="http://consumerlearning.canceraustralia.gov.au">consumerlearning.canceraustralia.gov.au</a>
Healthdirect Australia	<a href="http://healthdirect.gov.au">healthdirect.gov.au</a>
National Health and Medical Research Council (NHMRC)	<a href="http://nhmrc.gov.au">nhmrc.gov.au</a>
NHMRC Clinical Trials Centre	<a href="http://ctc.usyd.edu.au">ctc.usyd.edu.au</a>
Pharmaceutical Benefits Scheme	<a href="http://pbs.gov.au">pbs.gov.au</a>
Therapeutic Goods Administration	<a href="http://tga.gov.au">tga.gov.au</a>

### International

ClinicalTrials.gov (US)	<a href="http://clinicaltrials.gov">clinicaltrials.gov</a>
EU Clinical Trials Register	<a href="http://clinicaltrialsregister.eu">clinicaltrialsregister.eu</a>
International Clinical Trials Registry Platform	<a href="http://apps.who.int/trialsearch">apps.who.int/trialsearch</a>
ISRCTN registry	<a href="http://isrctn.com">isrctn.com</a>
NHS Choices – Clinical Trials	<a href="http://www.nhs.uk/conditions/clinical-trials">www.nhs.uk/conditions/clinical-trials</a>
World Health Organization	<a href="http://who.int/topics/clinical_trials">who.int/topics/clinical_trials</a>



## 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 taking part 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 or research nurse.

While the decision to participate lies with the person who has cancer (unless they're under 18), it's important that you, as the carer, know what impact the study might have on the participant, and on you and your family. For example, you may 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 to 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 very stressful. Try to look after yourself by giving yourself some time out, and sharing your worries and concerns with a counsellor or your doctor. To find local support services, information and resources contact the Carer Gateway (call 1800 422 737 or visit [carergateway.gov.au](http://carergateway.gov.au)), Carers Australia (call 1800 242 636 or go to [carersaustralia.com.au](http://carersaustralia.com.au)) or Cancer Council 13 11 20.

➤ See our *Caring for Someone with Cancer* booklet.



# Question checklist

Asking questions of your doctor or the clinical trials team will help you make an informed choice. You may want to include some of the questions below in your own list.

## Practical questions

- What are my chances of benefiting from this research?
- What are the risks to me?
- Will I experience any side effects? How will they be treated?
- Are there any extra tests involved?
- Do I need to stay overnight in hospital?
- Will I need to take time off work? Will my day-to-day life be affected?
- Can I receive any reimbursement of out-of-pocket expenses?
- Can I still participate if I need to travel interstate or overseas?
- Can I be involved in more than one study at the same time?
- How much time do I have to think about whether or not to join this trial?

## Other treatments

- Who will oversee my cancer care while I'm participating?
- If I join this study, will I miss out on other treatment opportunities later?
- Can I still take other medicines or complementary therapies while I'm involved in the trial?
- Who will look after my treatment if I leave the trial early?

## Study background

- What is being tested in the trial or study and why?
- Is this the first time the intervention has been tested? If not, what were the results of the previous studies?
- How many other people will be involved in this research?
- How long does the research last? For how long do I need to be involved?
- If I take time to decide, will delaying the treatment affect how well it works?
- Will the trial use a placebo?
- If it is a random control trial, will I have the option to switch treatment arms if the cancer advances or I have bad side effects?



## 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?
- Who will cover the treatment costs for any side effects or complications?
- 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?

## After the trial

- How will I receive feedback on the trial results?
- Will I have follow-up care through the clinical trials team?
- If I respond to the experimental treatment, will I still be able to get it after the trial is over?

“ My naturopath suggested I take zinc and vitamin B supplements. Because I'm on a clinical trial, I checked with the nurses. They were very encouraging and said it would be okay. ” Alan (*multiple myeloma*)



# Glossary

## **active ingredient**

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

## **adverse event**

An unintended and possibly harmful occurrence related to taking a medicine or using a medical device.

## **animal research**

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

## **arm**

Group of people who receive the same treatment in a randomised trial. Most randomised trials have two arms, but some have three or more arms.

## **baseline**

A phase during a study when 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 tiny components of the body, including cells, compounds and molecules. Sometimes called test tube or laboratory research.

## **behavioural research**

Research into how people's behaviours affect their chances of getting or recovering from cancer.

## **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/study**

A trial in which participants do not know

if they are receiving the control or the experimental treatment.

## **blood test**

A test to look for abnormalities in a person's blood.

## **bone marrow**

The soft, spongy material inside bones, which produces red blood cells, white blood cells and platelets.

## **cancer**

Uncontrolled growth of cells that may result in abnormal blood cells or grow into a lump called a tumour. These cells may spread throughout the lymphatic system or bloodstream to form secondary (metastatic) tumours.

## **case control study**

A type of study that compares people who have a particular disease (the cases) with people who are healthy (the controls) and looks back over time to see if they have anything in common, such as their history of smoking or exposure to asbestos. Also called retrospective studies.

## **cells**

The basic building blocks of the body. A human is made of billions of cells that 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 research**

Research that focuses on people's health and medical care.

## **clinical trial**

A research study that tests new approaches to prevention, screening,

diagnosis or treatment, to see if they are better than current treatments.

### **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. A cohort study also has a control group. Also called prospective studies.

### **control group**

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

### **controlled trial**

A trial that compares two or more treatments to find out which one is more effective.

### **control treatment**

The existing 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.

### **CT scan**

A computerised tomography scan. This scan uses x-rays to create a detailed, cross-sectional picture of the body.

### **diagnosis**

The identification and naming of a person's disease.

### **DNA**

A tiny molecule in every cell of the body that carries instructions for how that cell behaves and functions. Also called deoxyribonucleic acid.

### **double-blind trial**

A trial in which neither the patient

nor their research team know what treatment the patient is receiving. This is done to reduce bias.

### **eligibility criteria**

Characteristics of the people who are suitable for a particular trial.

### **end point**

What a clinical trial is trying to measure or find out. It is important that the goals for clinical trials are clearly defined in advance. Typical end points include measurements of toxicity, response rate and survival.

### **epidemiology**

The study of how and why diseases occur in different populations.

### **ethics**

The study of moral values or principles, including 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 treatment**

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

### **first-line treatment**

The initial treatment used to target cancer.

### **genes**

The microscopic units that determine how the body's cells grow and behave. Genes are found in every cell of the body and are inherited from both parents.

**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.

**informed consent**

A legal process by which a patient is given detailed information about a study before they agree to become involved.

**investigator**

Another term for a researcher. Can be a coordinating principal investigator (over multiple sites) or a coordinating investigator at one site.

**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.

**laboratory**

Place where experiments are carried out and new medicines developed.

**laboratory research**

Research that is carried out in a laboratory.

**literature review**

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

**longitudinal study**

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

**lymphatic system**

A network of tissues, capillaries, vessels, ducts and nodes that removes excess fluid from tissues, absorbs fatty acids, transports fat, and produces immune cells. Includes the bone marrow, spleen, thymus and lymph nodes.

**medical device**

A device placed in or on a person's body to help treat a disease.

**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 drug therapies such as chemotherapy, targeted therapy and immunotherapy (systemic treatment).

**medical science**

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

**molecular research**

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

**molecules**

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

**MRI scan**

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

**oncology**

The study, diagnosis and treatment of cancer.

**palliative care**

The holistic care of people who have a life-limiting illness, their families and carers. It aims to maintain quality of life by addressing physical, practical, emotional, spiritual and social needs. Also known as supportive care.

**participant information**

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

**peer review**

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

**pharmacogenetics**

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

**pharmacology**

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

**Pharmaceutical Benefits Scheme (PBS)**

A government-funded scheme that subsidises some prescription medicines.

**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 feasible.

**placebo**

A dummy pill, injection or other treatment that looks like the new treatment being tested but doesn't contain the active ingredient.

**prevention trial**

A trial that tests a new approach 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. Also called cohort studies.

**protocol**

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

**psychosocial research**

Research looking at the emotional, psychological and social 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 radiation therapy.

### **radiation therapy**

The use of targeted radiation to kill or damage cancer cells so they cannot grow, multiply or spread. The radiation is usually in the form of x-ray beams. Also called radiotherapy.

### **randomisation**

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

### **randomised controlled trial (RCT)**

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

### **remission**

When the signs and symptoms of the cancer reduce or disappear. This may not mean that the cancer is cured.

### **research governance officer**

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

### **research grant**

Money given by an institution to fund research, usually allocated through a competitive process.

### **response**

A decrease in the size of tumours as a result of treatment.

### **retrospective study**

Research that looks at what has happened in the past to gain an understanding about why something is occurring in the present. Also called case control studies.

### **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 effect of a drug or treatment.

### **single-blind trial**

A trial in which only the research team know whether patients are receiving the standard treatment or the new 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 used to help treat diseases.

**stem cells**

Unspecialised cells from which various types of mature cells can develop. Stem cells are found in the bone marrow.

**supportive care**

Care extending 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 of similar type that make up an organ or structure in the body. When removed from the body, tissue is sometimes called a biospecimen.

**tissue bank**

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

**tissue banking**

When people donate their tissue for research in the future.

**toxicity**

See side effect.

**toxicology**

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

**translational research**

Research that fast-tracks results from basic research with the aim of getting new treatments into clinical practice.

**treatment trial**

A trial that tests a new or modified treatment.

**x-ray**

A type of high-energy radiation that shows solid areas in the body such as bone. X-rays are used to diagnose different conditions.

**Can't find a word here?**

For more cancer-related words, visit:

- [cancercouncil.com.au/words](http://cancercouncil.com.au/words)
- [cancervic.org.au/glossary](http://cancervic.org.au/glossary)
- [cancersa.org.au/glossary](http://cancersa.org.au/glossary)

**References**

1. Australian Institute of Health and Welfare (AIHW), *Cancer in Australia 2017, Supplementary tables: Chapter 5 Survival and survivorship after a cancer diagnosis*, AIHW, Canberra, 2017.
2. Cancer Australia, *Cancer Research in Australia: an overview of funding to cancer research projects and research programs in Australia 2006 to 2011*, Cancer Australia, Surry Hills, 2014.









## 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 other Pink events, or hold your own fundraiser or become a volunteer.

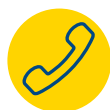
**Make a donation:** Any gift, large or small, makes a meaningful contribution to our work in supporting people with cancer and their families now and in the future.

**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 13 11 20

Being diagnosed with cancer can be overwhelming. At Cancer Council, we understand it isn't just about the treatment or prognosis. Having cancer affects the way you live, work and think. It can also affect our most important relationships.

When disruption and change happen in our lives, talking to someone who understands can make a big difference. Cancer Council has been providing information and support to people affected by cancer for over 50 years.

Calling 13 11 20 gives you access to trustworthy information that is relevant to you. Our cancer nurses are available to answer your questions and link you to services in your area, such as transport, accommodation and home help. We can also help with other matters, such as legal and financial advice.

If you are finding it hard to navigate through the health care system, or just need someone to listen to your immediate concerns, call 13 11 20 and find out how we can support you, your family and friends.

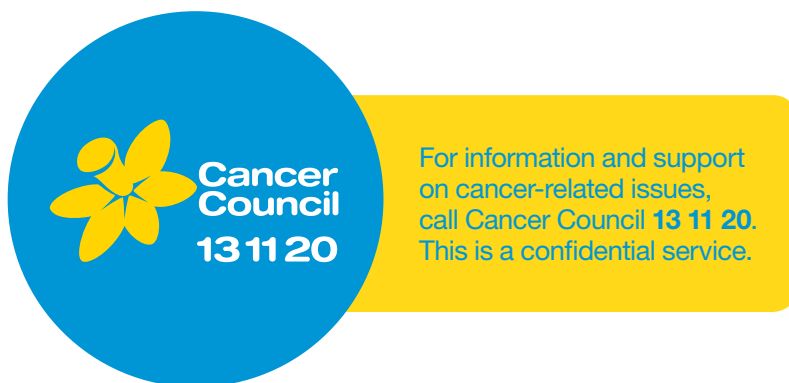
Cancer Council services and programs vary in each area.

13 11 20 is charged at a local call rate throughout Australia (except from mobiles).



**If you need information in a language other than English, an interpreting service is available. Call 13 14 50.**

**If you are deaf, or have a hearing or speech impairment, you can contact us through the National Relay Service. [www.relayservice.gov.au](http://www.relayservice.gov.au)**



## Visit your local Cancer Council website

**Cancer Council ACT**  
actcancer.org

**Cancer Council NSW**  
cancercouncil.com.au

**Cancer Council NT**  
nt.cancer.org.au

**Cancer Council Queensland**  
cancerqld.org.au

**Cancer Council SA**  
cancersa.org.au

**Cancer Council Tasmania**  
cancertas.org.au

**Cancer Council Victoria**  
cancervic.org.au

**Cancer Council WA**  
cancerwa.asn.au

**Cancer Council Australia**  
cancer.org.au

*This booklet is funded through the generosity of the people of Australia.  
To support Cancer Council, call your local Cancer Council or visit your local website.*