

This factsheet provides you with information and some questions to ask if you are considering participating in a clinical trial.

"I read a lot and ask questions – information is important. You know that old cliché: '*forewarned is forearmed*'." **Mark**

7

What is an HIV/AIDS clinical trial?

Clinical trials are experiments in which new therapies for HIV or new approaches to treatment are tested in people. There are many different types of trials and not all trials are designed to find out the same kinds of information. Whether you're considering entering a trial or trying to make sense of the results of a trial, it's important to understand the way that trials operate.

Who conducts clinical trials?

Clinical trials are conducted, or 'sponsored', by a wide range of organisations. They include:

- Pharmaceutical companies, either solely or jointly with other research institutions;
- Private research organisations under a grant from the government's National Health and Medical Research Council or from public donations; and
- Publicly funded research organisations.

Regardless of the type of organisation sponsoring the trial, it must be approved and monitored under the ethical and good clinical practice guidelines set down by the Australian Government¹.

What is a protocol?

All trials are based on a set of rules called a protocol. A protocol describes who may participate in the trial; the schedule of tests, procedures, medications, and dosages; and the length of the study. While in a trial, participants are seen regularly by the research staff to monitor their health and to determine the safety and effectiveness of their treatment.

clinical trials

What are trial phases?²

Clinical trials are conducted in a series of steps, called phases. There are four different types of trials, each one associated with a different phase in the development of a new treatment. It would be uncommon to find a trial that satisfied specific categories. It is nonetheless a helpful guide.

Phase 1 trials are designed to determine whether a treatment is safe for people to take. Researchers test a new treatment in a small group of people for the first time to evaluate its safety, determine a safe dosage range, and identify side effects.

Phase 2 trials are usually the first time the treatment is given to people experiencing the condition (e.g. HIV, cancer) for which it is intended. The treatment is given to a larger group of people to see if it is effective and to further evaluate its safety.

Phase 3 trials aim to double check the effectiveness of the treatment demonstrated in Phase 2 trials. Phase 3 trials monitor side effects, compare it to commonly used treatments and collect information that will allow the treatment to be used safely. The end result is getting the treatment approved by the government.

Phase 4 trials are done after the treatment has been marketed to gather information on its effect in various populations and any side effects associated with long-term use. Phase 4 trials also study the use of the treatment in a clinical setting, as this may differ from the conditions under which the other trials were run.

Who can participate?

All trials have inclusion criteria and exclusion criteria, based on such factors as age, type of disease, medical history and current medical condition. These criteria are not used to reject people personally. Instead, they are used to identify appropriate participants and keep them safe. The criteria help ensure that researchers will be able to answer the questions they plan to study.

What happens during a trial?

The trial process depends on the kind of trial you participate in. Each trial team is led by a doctor and includes nurses as well as pharmacists and other healthcare professionals who are responsible for checking the health of the participants at the beginning of the trial, monitoring them during the trial, and staying in touch with them for a period of time after the trial has been completed. Some trials involve more tests and doctor visits than you would normally have. Your participation will be most successful if you follow the **protocol** carefully and stay in contact with the trial team.

Your experience of taking trial medications may be very different to that of your friends. Some people experience treatment side-effects and others do not. Trials, by definition, set out to show that something is different from something else (ie. compare things). In order to compare things fairly and rigorously scientists use **randomisation, blinding and controls (sometimes placebos)**.

What is a randomised trial?

In a randomised trial, people who join are randomly assigned to one of these. However, the process is not as random as you may think. Most trials adjust the randomisation so that the participants in each arm have more or less the same characteristics (ie. a similar range of ages). Participants in each arm receive a different treatment regimen. In some cases, people in one arm may receive a **placebo**.

What is a placebo or control?

A placebo is an inactive pill, liquid, or powder that has no treatment value. In trials, experimental treatments are often compared with placebos to assess the treatment's effectiveness. In some studies, the participants in the control group will receive a placebo instead of an active drug or treatment. The control group is there to answer an important scientific question: "If the people we gave this treatment to respond in this way, how does that compare with people we did not give this treatment to?"³

Because of the placebo effect, some people experience real physical changes when they take a placebo. People on the placebo arms of trials occasionally have CD4 increases, viral load reductions, and side-effects just like the people taking the real drug.

Some trials do not have a placebo arm. These trials are designed to compare one treatment with another treatment (rather than one treatment with no treatment) and so these trials may have **all active treatment arms**.⁴

What is a blinded study?

A blinded study is one in which participants do not know whether they are in the experimental or control group in a research study. Those in the experimental group get the medications or treatments being tested, while those in the control group get a standard treatment or a placebo.

What is a double-blind study?

A double-blind study is one in which neither the participants nor the study staff know which participants are receiving the experimental treatment and which ones are getting either a standard treatment or a placebo.

These studies are performed so neither the patients' nor the doctors' expectations about the experimental drug can influence the outcome.

What happens if there are side-effects?

By the time a treatment reaches the trial stage it has been extensively tested for likely side-effects. However, part of the purpose of the trials is to see what unexpected side-effects emerge, and how severe or common they are. So as well as experiencing the benefits of new treatment, there may also be side-effects for some people. Problems or side-effects will be carefully recorded and relayed to the principal researcher to ensure that you are kept as safe as possible. Compensation is available for participants who suffer personal injury as a result of their participation in all trials.

What are the benefits and risks?

There are benefits and risks associated with trials. By participating you can:

- Take an active role in your health care.
- Gain access to new treatments.
- Obtain expert medical care.
- Help others by contributing to research.

Clinical trials have risks:

- There may be side-effects or adverse reactions to medications or treatments.
- The treatment may not be effective for you.
- You may be placed in the control group and may not receive the trial treatment until after the trial has finished; and
- The protocol may require a lot of your time for trips to the study site, treatments, hospital stays, or complex dosage requirements.

How am I protected?

The government has strict guidelines and safeguards to protect people who choose to participate in trials. In Australia, trials must conform to the *Ethical Principles of the Declaration of Helsinki* and to *International Good Clinical Practice* guidelines. Before a trial can go ahead, it needs to be approved by independent ethics committees that operate according to the guidelines issued by the National Health & Medical Research Council (NHMRC Guidelines).

What is informed consent?

Informed consent is the process of learning the risks and benefits of a trial before you decide whether to participate. These include:

- Why the research is being done and what the researchers want to accomplish.
- What will be done during the trial and for how long.
- What benefits can be expected and what other treatments are available.
- You have the right to leave the trial at any time.

If you are considering joining a trial, the research staff will give you a patient information statement that includes details about the study and a consent form.

Joining a trial is an important decision. You should ask the research team any questions you have about the study and the consent forms before you make a decision. It is also a good idea to take the consent documents home and discuss them with your partner, friends or family members.

Informed consent is more than signing a form. It is a process that continues through the study. You should feel free to ask the research team questions at any time.

What happens with the results?

As well as being reviewed by the government authorities such as the Therapeutic Goods Administration, the results of the trials may be reported in the medical press and are made available to doctors. The publication of results is done so that doctors can make scientifically valid assessments of the benefits and risks of a new medicine for their patients.

Although the results of the study may be published, nothing that identifies individual patients will be released. All details of a clinical trial participant's treatment are kept confidential and patient anonymity is assured. In addition, your doctor will be notified of the results of the study.

What should I know before I join?⁵

You should know as much as possible about the research study. It is important for you to feel comfortable asking questions and the clinical staff should answer them in a way you can understand. Some questions you might ask about the clinical trial include:

1. What is the trial about?

- Have you or others done this type of trial before? If so, what did you learn?

2. Who put this trial together?

- Who are the researchers? Who do they work for?
- Have they done a trial like this before?
- Is the government part of this trial? Who else is part of this trial?
- Who is paying for the trial?
- Is my doctor accepting a fee for recruiting me into this trial?
- Who will make money from the results?
- Who can I go to with questions or complaints?

3. Who is going to be in this trial?

- What kinds of people are you looking for? Why?
- How are you finding people for this trial?
- Can I quit the study after signing a consent form?
- Is there a support group for trial participants that I can join?

4. What will I get out of the trial?

- What are the benefits? The risks?
- Will I get treated the same as everyone else?
- What kinds of different treatments are offered in this trial?
- Is there a trial and a control treatment?
- Will I continue to get the clinical trial medicine after the study is complete?
- Is payment involved?
- Do I get reimbursed for my fares to and from the clinical trial centre?

5. What do I have to do?

- How much of my time will be needed?
- Will I need to take extra time off work?
- What extra tests or procedures will I be subjected to?

6. How will I be protected?

- Do I stand a chance of being harmed in the trial? In the future?
- Does the trial protect me from all types of harm? If I get harmed in any way, will I get all needed treatment and compensation?
- Who pays for the treatment or tests?

7. How will my privacy be protected?

- Who is going to see the information?
- Will my name be used?
- What are you going to do with the results of the trial?
- What happens to the information I give if I quit the trial?
- Is there a written guarantee of privacy?

8. What progress has been made?

- When did you start this clinical trial? How long will it last?
- How much of the clinical trial have you already done?
- Have there been any problems so far?

You can leave a trial at any time. If you plan to stop participating, let the research team know why. If you decide not to participate in the trial it will not affect the services provided to you by any health services.

Getting information and support:

- Talk to your doctor.
- **Albion Street Centre** ☎ (02) 9332 9600. HIV/AIDS, Hep C clinical treatment and research centre. Trials, nutrition, counselling, antibody and viral load testing. NSP and pharmacy. Counsellor and doctor on call 24hrs (hours vary).
- **ACON's Treatment Information Officers** ☎ (02) 9206 2013 or 9206 2036 or outside Sydney Freecall 1800 816 518. Call for up-to-date information about treatments for HIV.
- **ACON's Women's HIV Support** ☎ (02) 9206 2012. Information, education, support and referral services to women living with HIV/AIDS.
- **People Living with HIV/AIDS (NSW) Inc.** ☎ (02) 9361 6011; Freecall 1800 245 667. A non-profit community organisation representing the interests of people living with HIV/AIDS in New South Wales.
- **Multicultural HIV/AIDS Service** ☎ (02) 9515 3098 or outside Sydney Freecall 1800 108 098. Mon-Fri 9am-5pm. Bilingual/bicultural co-workers providing emotional support, advocacy and information to people living with HIV/AIDS from non-English speaking backgrounds.
- **Heterosexual HIV/AIDS Service (Pozhet)** ☎ (02) 9361 6011 Freecall 1800 812 404 (national) or visit www.pozhet.org.au Men and women living heterosexually with HIV/AIDS.
- **Consumers' Health Forum of Australia** ☎ (02) 6273 5444 or visit www.chf.org.au
- **Medicines Australia** ☎ (02) 6622 4453 or visit www.medicineau.net.au
- **Health Care Complaints Commission (HCCC)** ☎ (02) 9219 7444 or outside Sydney Freecall 1800 043 159 or visit www.hccc.nsw.gov.au Monitors, resolves and investigates complaints about health care providers and health care services in NSW.

For regional NSW HIV/AIDS and related services:

- **Contacts** A directory of services for people living with HIV/AIDS. Available from People Living With HIV/AIDS (NSW) Inc. ☎ (02) 9361 6011; Freecall 1800 245 667 or visit www.plwha.org.au

References

- 1 These are: *Access to Unapproved Therapeutic Goods – Clinical Trials in Australia*, Therapeutic Goods Administration, 2000; *The National Statement on Ethical Conduct in Research Involving Humans*, National Health and Medical Research Council, 1999; *Guidelines for the Ethical Review of Research Proposals for Human Somatic Cell Gene Therapy and Related Therapies*, National Health and Medical Research Council, 1999.
- 2 See: *Access To Unapproved Therapeutic Goods – Clinical Trials In Australia*, Therapeutic Goods Administration May 2001 at: <http://www.health.gov.au/tga/docs/pdf/unapproved/clintrials.pdf> & Medicines Australia www.medicineau.net.au
- 3 Medicines Australia www.medicineau.net.au
- 4 See: *Access To Unapproved Therapeutic Goods – Clinical Trials In Australia*, Therapeutic Goods Administration May 2001 at: <http://www.health.gov.au/tga/docs/pdf/unapproved/clintrials.pdf> and Medicines Australia www.medicineau.net.au
- 5 See Medicines Australia www.medicineau.net.au

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