SCN2A
ASIA-PACIFIC

Clinical Trials

INFORMATION BROCHURE

What are clinical trials and who can take part?[1,2]

Have you considered taking part in a clinical trial?

Before you take part in a clinical trial, you need to understand what clinical trials are, why we need them, how they work, and who can take part.

This brochure is designed to give you this information. You need to think carefully about taking part in a clinical trial and discuss your decision with your doctor.

What are clinical trials?

Clinical trials are research studies that involve people. They test whether new treatments can improve the health of people and how safe these treatments are. New treatments can include vaccines, medicines, medical devices such as pacemakers, and surgical treatments. Most treatments that we have today, including those for heart disease, diabetes and cancer, have been tested in clinical trials.

Why do we need clinical trials?

All new treatments need to be tested in clinical trials. This helps researchers to learn about how new treatments work in people, how safe these treatments are and what dosage should be used. Clinical trials can also help researchers to compare new treatments with existing treatments in terms of how they work and how safe they are.

Why do people want to take part in clinical trials?

There are many reasons why people want to take part in clinical trials; below are some examples.

- They may want the newest treatment because they hope that it will work better or have fewer side effects than their current treatment.
- They may want the additional medical care they can get during a clinical trial.
- There are people who have a disease for which there is currently no available treatment. Taking part in a clinical trial may be the only way for these people to get access to treatment.

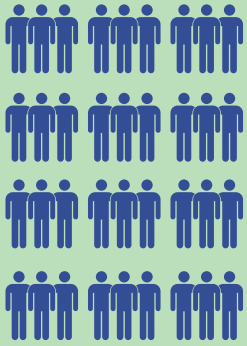
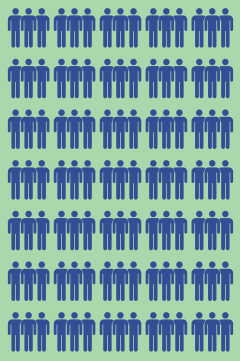
How do clinical trials work?[1,2,3]

Clinical trial phases

Before researchers study how a new treatment works in people during clinical trials, they need to study how it works in a laboratory. Only the treatments that work well in a laboratory can then be studied in people.

There are 4 main types of clinical trials, also called phases. Each phase is a different step in the development of a drug and tests the new treatment in a different way.

The image below shows the main differences between the 4 clinical trial phases.

Phase 1	Phase 2	Phase 3	Phase 4
TRIAL DURATION Months	**TRIAL DURATION** Months to Years	**TRIAL DURATION** Years	**TRIAL DURATION** Years to Decades
Small number of healthy participants	Small number of participants with the disease	More participants than in phase 2 trials who have the disease	More participants than in phase 3 trials who have the disease
			
First test of new treatment in people Treatment is tested for how safe it is and what dose should be used	Treatment is tested for how it works and how safe it is	Treatment is tested for how it works and how safe it is Treatment is compared with existing treatments	These trials are conducted after the treatment is approved Treatment is tested for how safe it is

In rare diseases, such as SCN2A, the clinical trial phases can be combined or may overlap.

If you are thinking about taking part in a clinical trial, it is important to consider what phase the trial is. This is because each clinical trial phase has different benefits and risks. For example, in a phase 1 trial, researchers do not yet know what side effects a treatment may have and what dosage works best. You can find more information about the potential benefits and risks of taking part in a clinical trial later in this brochure.

How do clinical trials work?[1,2,3]

Do all trial participants get the new treatment?

To find out if a new treatment improves participants' health, researchers often give some participants a new treatment and others a ***placebo***. A placebo looks like the new treatment but has no medicine in it. Researchers use a placebo to make sure that any of the effects they see in participants taking a new treatment are actually caused by the new treatment.

In some clinical trials, instead of a placebo, researchers give some participants a treatment that is already available for their disease. This treatment is the one usually given to people to treat their disease and is called a ***standard treatment***. By giving some participants a new treatment and other participants a standard treatment, researchers can study how well the new treatment works compared with the standard treatment.

In many clinical trials, a computer can be used to randomly decide which participants will receive the new treatment and which participants will receive a placebo or a standard treatment. This is done so that the treatments are chosen fairly and so that comparing the results of each treatment is as accurate as possible.

In some clinical trials, neither the participants nor the researchers know which participants got the new treatment and which participants got a placebo or a standard treatment until the end of the study. These types of trials are called ***double-blind*** trials. Researchers use double-blind trials because knowing what treatment the participants are receiving can affect the results of the trial.

By giving some participants a new treatment and other participants a standard treatment, researchers can study how well the new treatment works compared with the standard treatment.

Taking part in clinical trials[1,2]

Potential benefits and risks

Before you can join a clinical trial, you need to understand all the possible benefits and risks of and alternatives to the trial. To make sure that you understand this, the trial researchers will explain the trial, and give you information to read and possibly questionnaires to complete.

If you have any questions about the clinical trial, ask the trial staff.

The table below shows some of the potential benefits and risks of taking part in a clinical trial.

POTENTIAL BENEFITS	POTENTIAL RISKS
You have free access to a new treatment that is otherwise not available	The new treatment may be uncomfortable or cause side effects
You get additional medical care that is available during the trial	The new treatment may not work or may not be better than your current treatment
You may have information and access to support groups and resources	You may not be part of the group that gets the new treatment but may instead be part of the group that gets a placebo or a standard treatment
You help people to learn more about a disease	The clinical trial may be time consuming because of extra appointments and you may need to travel to the trial site
You help others to get better treatment for a disease in the future	

Clinical trials are run by researchers and a medical team that includes doctors, nurses, pharmacists and other healthcare professionals. The clinical trial medical team is responsible for checking the health of trial participants during and for a period after the trial. If participants have serious or unexpected side effects from the trial treatment, the trial doctors will decide if the treatment dosage needs to be changed or if the participants need to stop taking their treatment.

Eligibility

The aim of each clinical trial is to answer a research question. Researchers choose clinical trial participants carefully to be able to best answer the research question.

For each clinical trial, researchers decide what traits the participants need to have or cannot have to take part in a clinical trial. The traits the participants need to have are called ***inclusion criteria***. The traits the participants cannot have are called ***exclusion criteria***.

These traits can include being a certain age or having a particular type or stage of disease.

If a person meets all inclusion criteria and none of the exclusion criteria for a clinical trial, they are considered ***eligible*** to take part in that trial.

Informed consent

Before you take part in a clinical trial, you will be asked to sign an ***informed consent form***. An informed consent form has information about the trial, such as its purpose, duration, and potential benefits and risks of taking part in the trial.

It is important that you read it carefully and make sure that you understand how the trial works and what is expected of you. You can take this form home and discuss it with your doctors, family and friends. By signing this form, you confirm that you have understood all information in the form. If you have any questions, ask the trial researchers.

Even if you have signed the informed consent form and joined a clinical trial, you can leave the trial at any time and for any reason! The trial researchers may remove you from the trial if they think that your health is getting worse.

Even if you have signed the informed consent form and joined a clinical trial, you can leave the trial at any time and for any reason!

Key considerations[1,2]

It is important to understand the clinical trial that you wish to take part in.

Researchers do many tests on new treatments in laboratories before they test how these treatments work and how safe they are in people during clinical trials. During clinical trials, researchers learn about possible side effects that some people may have with the new treatment. It is important that you understand that the new treatment may or may not help your condition and may or may not cause side effects.

Taking part in a clinical trial is voluntary. You should never feel pressured to take part and you can leave the trial at any time and for any reason.

You should never have to pay for the trial treatment.

Any clinical trial you consider taking part in should be led by healthcare professionals at a clinical site and monitored by an institutional review board.

You can ask the clinical trial staff about their qualifications and experience.

Clinical trial results may be published and made publicly available, but the identity of the participants will always be protected.

This brochure is for you to learn about clinical trials. It does not give you medical advice, and it does not encourage you to participate in a clinical trial. Always ask your doctor if you have any questions.

Where to find more information and support

The following resources may be useful.

The Center for Information and Study on Clinical Research Participation: information about clinical trials and search function for clinical trials

https://www.ciscrp.org

National Institutes of Health Clinical Research Trials and You: information about clinical trials and how to find appropriate trials

https://www.nih.gov/health-information/nih-clinical-research-trials-you

SCN2A ASIA-PACIFIC

@SCN2AAsiaPacific

@scn2aaustralia

@scn2aaustralia

SCN2A Asia-Pacific

Scan the QR code to access the SCN2A Australia landing page

References

1. Australian Government. Australian Clinical Trials. Available from: https://www.australianclinicaltrials.gov.au/ (Accessed 15 September 2022).
2. Medicines Australia. Clinical Trials. Available from: https://www.medicinesaustralia.com.au/policy/clinical-trials/ (Accessed 15 September 2022).
3. Akobeng AK. Understanding randomised controlled trials. *Arch Dis Child* 2005;90:840–4.