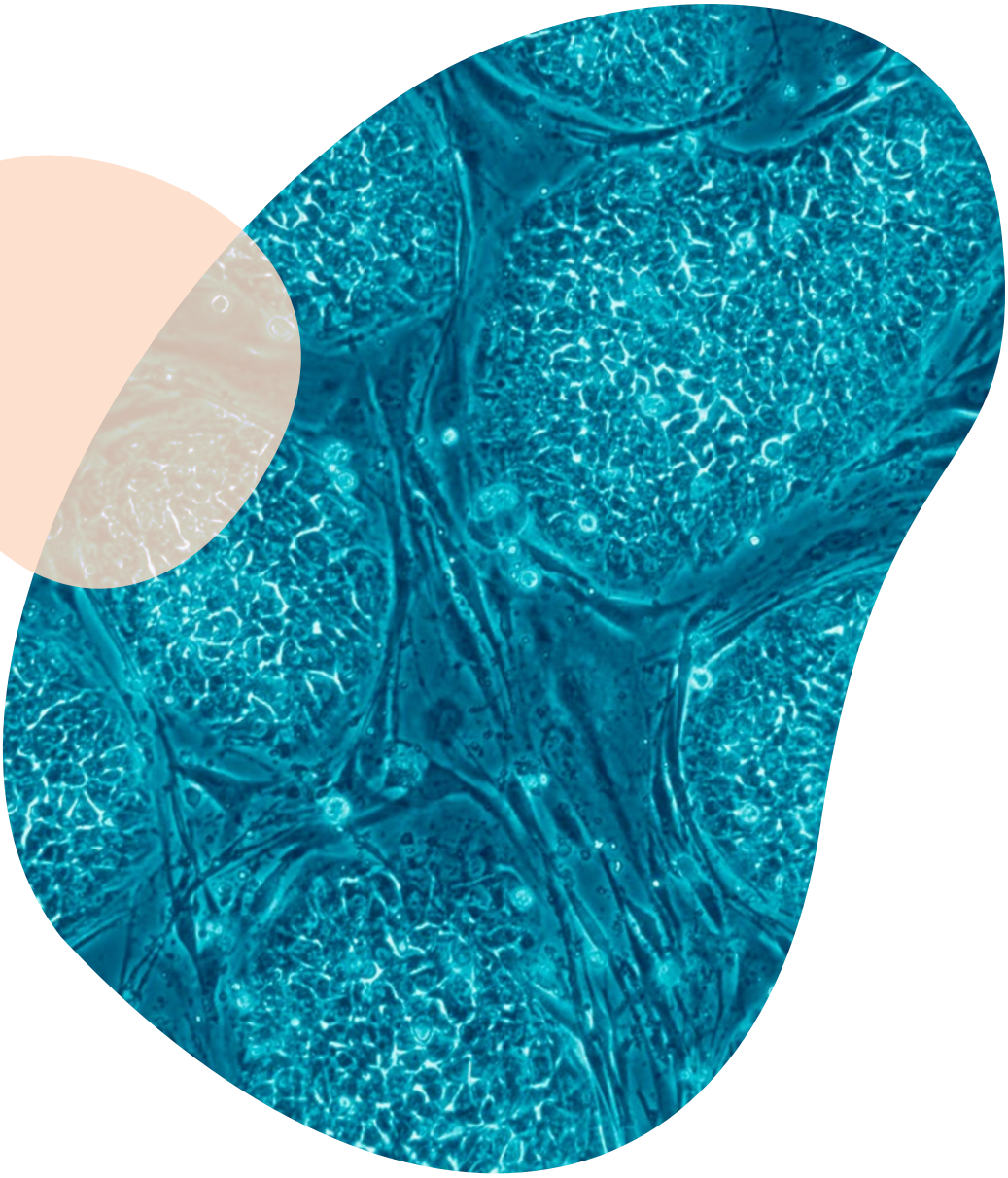
What You Need to Know About Stem Cell Therapies

A resource for people contemplating stem cell-based treatments or clinical trials

National Stem Cell Foundation of Australia



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Introduction

Stem cells are saving lives of people with blood cancers and a small number of other conditions across the globe. They may, one day, save many more lives. However, some treatments on offer today are unproven, expensive for the patient and potentially unsafe.

This short guide to stem cell medicine is written to help you make informed decisions about stem cell treatments that you may be exploring for yourself or someone you care about.

Always talk to your GP and specialist before making a decision about any treatment.

Every type of cell in our body — from brain cells to muscle cells — comes from stem cells. Our first stem cells appeared in the earliest stages of our development, and throughout our lives different kinds of stem cells continue to help grow and repair our organs and tissues. We all have stem cells in our bodies right now, working on tasks like replacing the lining of our gut, making new hair cells, or replenishing white blood cells to fight infection.

Stem cell therapy has been used routinely since the 1970s to treat diseases and conditions of the blood and immune system, such as leukaemia or thalassaemia. Bone marrow or cord blood transplants can replace a patient's faulty blood system with a healthy one that lasts for life, thanks to the properties of blood stem cells.

Stem cells have the potential to replace missing or damaged tissue and repair the effects of disease or injury. There are several different types of stem cells and experimental stem cell therapies are being tested for many diseases including macular degeneration, diabetes, arthritis, Parkinson's disease and heart conditions. But they are not there yet.

Apart from bone marrow and cord blood transplants for conditions affecting the blood and immune systems, and corneal and skin grafting applications, most other stem cell treatments remain experimental, meaning their benefits and safety are still being tested through research. Until this research has been completed, these stem cell therapies are considered unproven and are not recognised medical procedures or products.

Reputable hospitals and biotechnology and university groups are undertaking important research and clinical trials in Australia and around the world to evaluate promising new stem cell-based approaches. However, some private organisations and individual doctors and dentists in Australia and overseas are offering to treat people without first testing in clinical trials that the stem cell therapy they are proposing is safe and effective – bypassing the usual way in which new medicines and treatments are evaluated for safety and efficacy. Circumventing these steps and other regulations can create safety risks and result in adverse outcomes for patients.

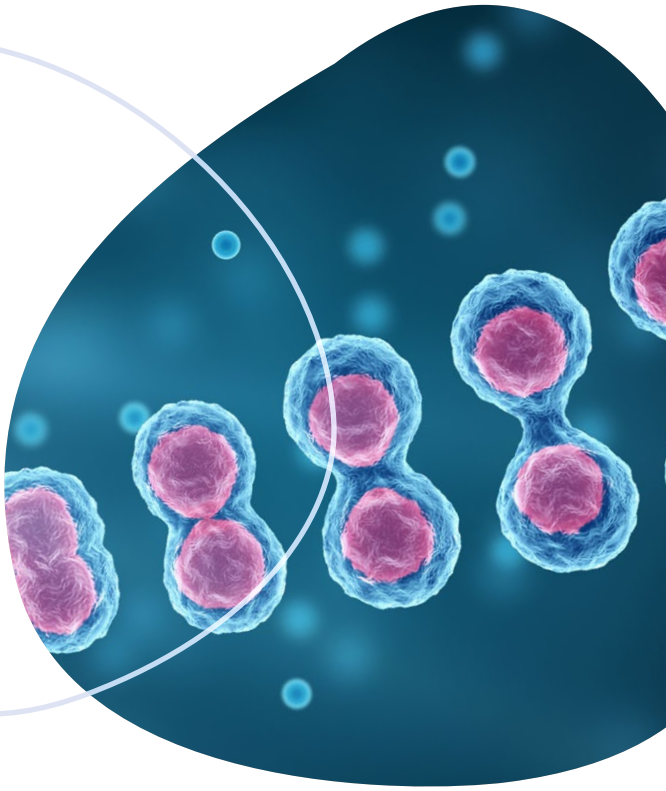
Having heard about stem cells in the media or from well-meaning friends or relatives, people who are living with severe illness or other challenging conditions may consider trying unproven treatments, especially if there seems to be few other options.

Emerging stem cell treatments give us reason for hope, but researchers and doctors are still learning about how to harness the potential of stem cell medicines safely. Every medical treatment has costs, side effects and benefits – it's important to make sure that you are not putting your health at risk.

This guide aims to highlight key information to help you decide what treatment is best for you or your loved one.

The Therapeutic Goods Administration (TGA) has also developed a public education resource to help consumers understand stem cell treatments and risks: **Stem cell treatments and regulation – a quick guide for consumers**.

Above all, please consult with your GP or specialist before making any decisions about your treatment.



What are stem cells and why are they special?

We all start life from a fertilised egg that rapidly develops into a cluster of cells, multiplying to grow ever larger and eventually forming all the parts of the human body.

The human body contains over 200 different kinds of specialised cells, such as muscle cells, nerve cells, fat cells and skin cells. All the specialised cells in our bodies originate from stem cells.

Stem cells are with us through to adulthood and are essential for how we develop and function throughout life. They have the ability to produce the cell types essential for our body's normal growth and development, and to repair many of our tissues and organs. So, when stem cells are not functioning as they should, there can be significant health impacts. It is the ability to grow and repair our bodies that makes stem cells unique, and their potential applications in medicine far-reaching. Apart from the stem cells in our body, researchers have also been able to create primitive stem cells in the laboratory. When given the right signals these primitive stem cells can turn into any of the cells of the body.

Why are stem cells special?

Stem cells are different from other cells in the body in three main ways:

- Stem cells are unspecialised. They have not developed into cells that perform a specific function.
- Stem cells can differentiate. This means they can divide and produce cells that have the potential to become other more-specific cell types. These differentiated or specialised cells are the functional cells within our tissues and organs, for example, skin stem cells give rise to new cells that keep our skin intact and allow it to heal following injury.
- Stem cells are capable of self-renewal, by dividing and producing identical copies of themselves. Once a cell has become specialised (differentiated), it has a very limited capacity to self-renew.

What are the different types of stem cells?

There are three main types of stem cells used in treatments and research:

Tissue stem cells

Tissue stem cells (also referred to as **adult stem cells**) are found in low numbers in most organs in the body throughout life, including during foetal development. They tend to be more limited than other stem cells in the types of cells into which they can develop. These are usually restricted to the specialised cells of the organ in which they are found.

Cord blood stem cells are tissue stem cells harvested from umbilical cord blood after childbirth. They can be frozen in cell banks for future use. These cells have been successfully used to treat children with blood cancers such as leukaemia and certain genetic blood disorders.

Mesenchymal stem cells (MSC) are also cells that can differentiate into multiple cell types. These have been found in the amniotic fluid that surrounds the developing baby inside the mother's womb, as well as in other tissues in the body such as fat, umbilical cord and bone marrow. While not all MSCs are the same, they display properties that may assist tissue repair. MSCs are being investigated in many clinical trials. More research is needed, however, to fully understand their potential therapeutic uses.

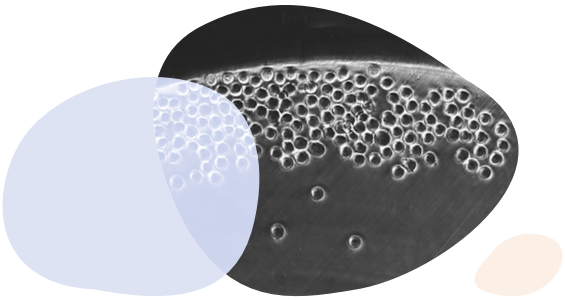
Embryonic stem cells

Embryonic stem cells are derived in the laboratory from donated IVF embryos. Their most important characteristic is their ability, when given the right instructions, to turn into any of the cells of the body (a function called 'pluripotency'). They are being explored as a source of cells to restore function to organs that are damaged by disease.

Induced pluripotent stem cells

Induced pluripotent stem cells (iPSC) are created in the laboratory by modifying skin or other body cells through a process called 'reprogramming'. This changes the pattern of genes expressed by the body cell, making it more like that in embryonic stem cells. Induced pluripotent stem cells can potentially change into any cell type and, when made from a patient's cell, can be used by researchers to understand more about the condition the patient is living with.

How are stem cells currently used as treatments?



Although there is a lot of attention around the potential of stem cells in medicine, the range of conditions for which there are medically recognised or proven stem cell treatments is limited to the following:

- **Blood stem cell (haematopoietic stem cell) transplants** from bone marrow or cord blood have been used for many decades to treat certain blood-related cancers and disorders of the immune system. They are also called 'bone marrow transplants' and now often use blood stem cells that have been released from the bone marrow and collected from the blood stream.

  Depending on the condition, this treatment employs either the patient's own cells or donated cells and is often used in conjunction with chemotherapy and radiation therapy. This type of stem cell therapy is limited to patients with different types of leukaemia, lymphoma, anaemia, thalassaemia, certain immunodeficiencies and autoimmune disorders.
- **Corneal and skin grafts** are forms of surgical transplantation that have been used for many years. The grafts work because of the stem cells that reside within the tissues of the cornea and skin.

  These treatments are medically recognised in Australia, and research into improving them is underway around the world, including Australia.

How one Australian man and his research revolutionised cancer treatment

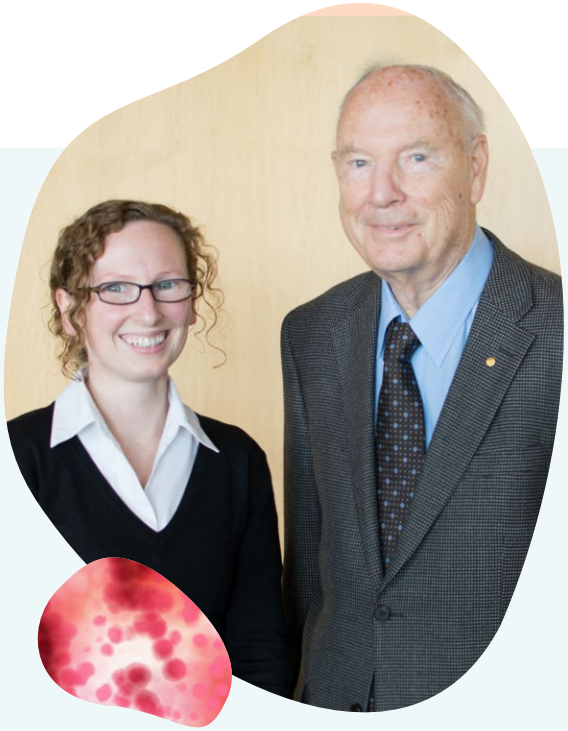
Forty-odd years ago, a life-saving bone marrow transplant or round of chemotherapy treatment often resulted in a severely weakened immune system and a lengthy hospital stay to avoid or treat potentially deadly infections. Bone marrow donation for transplantation was a demanding process that required a painful operation under general anaesthetic.

These days, things are much easier for bone marrow transplant recipients and donors, as well as for some other kinds of cancer patients undergoing treatment, because of the work of the late Emeritus Professor Don Metcalf AC, FRS, FAA.

> Metcalf and his team discovered colony stimulating factors (CSFs), the hormones that coax blood-making stem cells to differentiate into infection-fighting white blood cells. This revolutionised bone marrow transplantation and has helped more than 20 million patients worldwide recover faster after chemotherapy or stem cell transplants.

CSFs greatly shorten the period of low white blood cell counts after bone marrow transplantation and/or chemotherapy. It allows people to be treated with more intensive chemotherapy without long periods of lower protection against bacterial infection. Patients are much less likely to get infections, or if they do, CSFs can be prescribed allowing them to recover sufficiently for their next treatment.

Professor Metcalf and his colleagues also found that CSF treatment was able to coax stem cells to come out of the bone marrow into the peripheral blood (the blood that circulates around the body). This makes them much more accessible for collection, making the donation process less invasive for the donor.



Don Metcalf pictured with inaugural Metcalf Prize winner and brain stem cell researcher Kaylene Young.

The result is a better chance of survival. Millions of people have benefited from the treatments Don Metcalf pioneered. Opera singer José Carreras of 'The Three Tenors' fame credits the CSF treatment he received in 1987 with saving his life from acute myeloid leukaemia.

> Each year, the National Stem Cell Foundation of Australia awards and celebrates two exceptional mid-career researchers with the Metcalf Prizes for Stem Cell Research, each worth $55,000. The prizes recognise and honour the exceptional contribution made by Professor Metcalf.

The prizes have supported the careers of researchers working to understand fundamental stem cell science through to developing treatments for leukaemia and other cancers, heart defects and failure, multiple sclerosis, and more. **Meet the Metcalf Alumni.**

How could stem cells be used to develop treatments in future?

There are many registered clinical trials across the world currently exploring the use of stem cells or cells made from them for treating many conditions.

Your specialist is the person who will be most up to date on the proven treatments available for your condition and on the progress of possible new therapies. They will also be able to assist in assessing the suitability of a clinical trial you may be interested in, and whether you would be eligible to participate.

Globally, research scientists are using different types of stem cells to understand better how stem cells 'decide' what to become (differentiate) and how we can influence and control these processes.

Researchers are also using stem cells to speed up the process of developing new drugs and gene therapies, to understand how different diseases develop, and what happens to our cells during illness or after an injury. They are even using stem cells to help understand how cells are affected by bacterial and viral infections, including the SARS-CoV-2 virus that causes COVID-19.

As stem cells can be induced to grow into specific cells, they have great potential to treat conditions where tissue has been damaged or completely lost, such as death of heart muscle following a heart attack, liver failure from the destruction of liver cells by viruses or toxins, or the inability of the pancreas to produce insulin in people with diabetes.

Formal clinical trials of tissue stem cells, as well as differentiated cells made from the different types of pluripotent stem cells, are in progress in several countries to assess whether such treatments are effective and safe, but the treatments themselves might not be available for some time yet.

Could stem cells heal broken hearts?

Cardiologist and researcher Associate Professor James Chong of the University of Sydney has already used human stem cells to repair the damaged hearts of large non-human primates — a world first.

Now he's part way through a **five-year project** that aims to bring the therapy to clinical trial in patients who have had heart attacks.

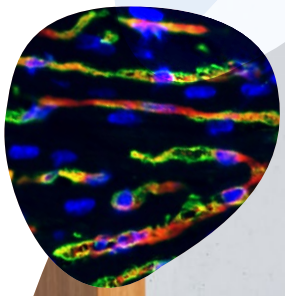
Heart disease is Australia's single leading cause of death, killing more than 18,500 people a year, one every 28 minutes.

James, a researcher at the Westmead Institute of Medical Research and Cardiologist at Westmead Hospital, wants to change those numbers.

He is working on two of the challenges of trials in humans: producing stem cell-derived heart muscle in large enough quantities to be useful, and understanding and preventing any abnormal heart rhythms that might appear in the repaired heart.

> 'The patients I see affect my thinking every day,' he says. 'What I'm working towards is to be able to provide new treatments to extend the quality and length of their lives.'

James's project is being supported under the National Stem Cell Foundation of Australia's Matched Funding Program. **Read more about his work on the Foundation website.**



Clinical trials

What are clinical trials? How do they lead to treatments that are safer and more effective?

Before a new medicine can be provided to patients as part of routine care, it must be rigorously tested to ensure it is safe and effective – this includes stem cell medicines. The internationally accepted way to do this is through clinical trials.

Clinical trials take promising potential treatments and evaluate their effectiveness, figure out the proper dose, and uncover any potential side effects. There are multiple phases of testing. Each phase of the clinical evaluation of a new treatment or drug relies on the findings of the previous phase. And each is designed to answer specific questions, which include:

- Is the new treatment safe?
- How well does it work?
- Is the new treatment potentially better than the treatments we have now?
- What are the side effects and risks of the new treatment?

Many clinical trials, however, do not show clear benefit for the patient or reveal complications that were not anticipated, even though years of laboratory and animal testing had shown promise. That is why trials are so important. They help to identify risks and side effects and to refine and develop ideas for future clinical trials.

Government agencies such as the TGA in Australia and the Food and Drug Administration (FDA) in the USA are responsible for approving new medicines for use.

Developing new medicines is rarely straightforward and the process can take many years.

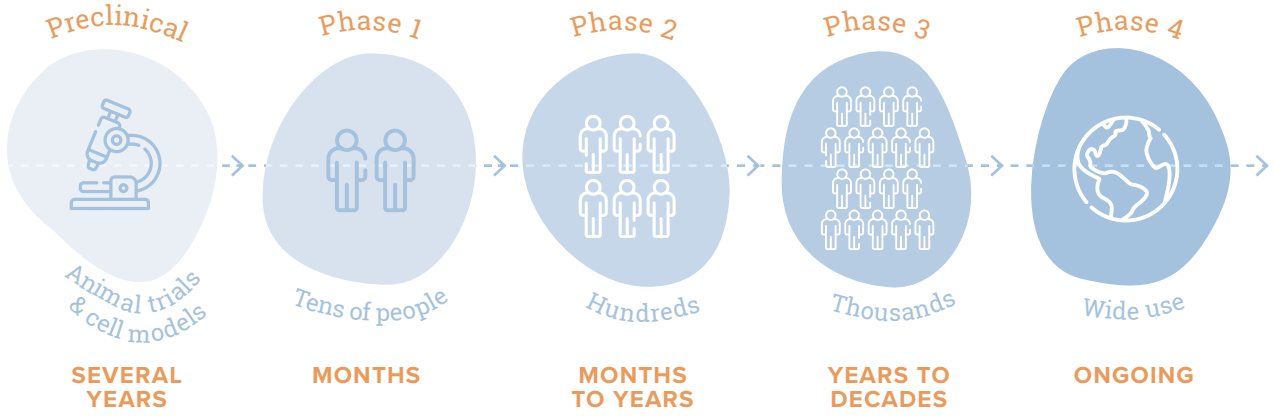
> It is important that clinical trials are conducted ethically to ensure that the interests of participants are protected. In Australia, and most other countries around the world, clinical trials must be reviewed by a government-accredited independent committee before starting. This is usually undertaken by an ethics committee at the hospital or facility where the trial is being conducted.

You can find information about current clinical trials on the websites of the **Australian New Zealand Clinical Trials Registry**, **Australian Cancer Trials**, **NHMRC Clinical Trials Centre**, and **Australasian Leukaemia & Lymphoma Group**.

Registering a clinical trial with an appropriate registry is mandatory for accredited clinical trials. Some commercial clinics marketing unproven stem cell treatments, however, have begun using these platforms as a way to lend legitimacy to their practices and the unsubstantiated claims they promote. Such studies will often describe themselves as 'patient-funded', 'self-funded', or 'patient-sponsored', and require patients to pay for participation in the trial. This would be highly unusual for an accredited clinical trial.

Be especially cautious of clinical trials that require payment for participation.

Stages of clinical trials



The pathway from promising discovery in a laboratory to approved treatment that we can routinely access in a clinic or hospital takes many years and happens in different stages:

- Basic laboratory research
- Preclinical research in animal models
- Clinical trials
- Approval for use by regulators and government authorities.

There are four phases for clinical trials:

- **Phase 1 clinical trials** usually involve a small number of volunteers (10-60). The trials aim to determine the safety of the medicine, how it works and how well it is tolerated. These clinical trials identify preferred methods of administration (tablet, liquid, or injection) and help determine appropriate doses for later studies.
- **Phase 2 clinical trials** are normally the first use of the medicine in patients suffering from the condition for which it is intended. Phase 2 clinical trials mainly seek to determine effectiveness and safety. They are undertaken in a small number of patients and conducted by specialists of the disease or condition and its treatment.
- **Phase 3 clinical trials** involve greater numbers of patients and determine whether the medicine offers clinical benefit in the disease/s for which effectiveness was demonstrated in Phase 2. They also determine the nature and likelihood of any side effects. A key feature of Phase 3 is usually a comparison against the currently accepted treatment, and more than one Phase 3 trial may be required before regulatory approval is granted for clinical use of the treatment.
- **Phase 4 clinical trials** occur after the medicine has been approved by regulators such as the TGA or FDA for treatment of a particular disease. Phase 4 clinical trials continue to monitor the effectiveness and safety of the new treatment and compare new medicines to a wider range of existing medicines/ therapies and establish how the new medicine is best used.

> The timeframe for these various stages can be quite lengthy and sometimes the trials reveal challenges that are not anticipated. There is no guarantee that research will result in a proven product.

What makes a clinical trial worth considering?

If you are thinking about participating in a clinical trial it is important to take your time and find out as much as possible about the study before you decide to join.

In 'controlled' trials, a participant will either receive the treatment being evaluated or a placebo that mimics the therapy but does not contain any of the active ingredients. In such 'double blind' trials, neither the trial participant nor the supervising medical staff know if the participant is in the treatment or the placebo group. This is often an important factor for removing bias from the trial and provides greater certainty about the test treatment's safety and effectiveness.

If you are invited to participate in a clinical trial, you must give **informed consent** to take part. To help you decide whether or not to give consent, you should be given clear information explaining:

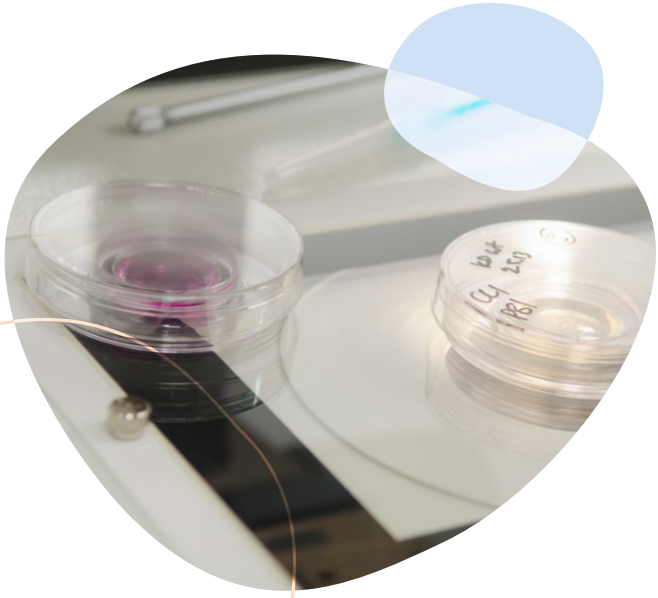
- the purpose of the trial, the kinds of tests and treatments, and possible side effects;
- whether the trial is 'blind' and includes a control group – if a prospective patient may receive a placebo treatment, they should be aware of this possibility;
- who is eligible to participate (clear inclusion and exclusion criteria);
- the timeframe for the study;
- any costs or payments. As a participant you are not usually expected to pay to participate. In fact, your costs, and those of any complications, should be covered by those conducting the trial;
- what support you will receive if there are any complications;
- the steps involved in the review and regulatory oversight of the trial; and
- whether the trial has been registered with the Australian New Zealand Clinical Trials Registry or clinicaltrials.gov (for overseas trials).

Participating in a clinical trial can be rewarding. While it may not help you, it's helping other people who will benefit from what is being learnt. Findings from clinical trials will assist people with your condition in future.

You can find out more about your rights and the consent process at the **Australian Clinical Trials website**. Discuss your options with your GP or specialist and ask their help to determine if you are eligible to participate in the trial and if it is being conducted by a reputable clinic, hospital or organisation. Some clinics may claim to run clinical trials or research studies, but may not fully comply with relevant laws and ethical standards.

> If you are considering participating in a clinical trial, the best place to start is with your regular hospital doctors, specialists, and your GP. We strongly advise you to visit your doctor/s and not seek medical advice from the internet. If it sounds too good to be true, be wary.

The best protection for patients and families in this situation is to be fully informed. It is a good idea to have a written list of questions and write down or obtain the answers in writing from the provider. This means you can discuss these answers with your treating doctors and make an informed decision.



Unproven treatments and why you should be cautious

What are unproven stem cell treatments?

Many people facing severe illness or injury may consider trying unproven treatments, especially when there seem to be few other options. Unproven stem cells treatments are those that have not been evaluated through registered clinical trials and have not received government approval for their use. Such treatments will not be recognised by Medicare and private health insurers. Furthermore, there is a risk that unproven stem cell treatments will be advertised and promoted online by clinics and doctors who are not recognised specialists or experts in the diseases they are proposing to treat, and who have a strong financial interest in getting you to buy the unproven treatment.

The sale of unproven treatments is problematic because:

- people may gain no benefit or experience complications which further compromise their health;
- there may not be any follow-up care or monitoring to detect side effects after treatment;
- people may discontinue seeing their current specialists and avoid other recognised treatments that could provide benefit; and
- we do not learn from the experience of people who participate, as there is no systematic record keeping or sharing of information about outcomes, as unproven treatments do not involve clinical trials.

Unproven stem cell treatments are sold by commercial clinics and in doctor's surgeries in Australia and overseas. It can be difficult to determine if the treatments are legitimate, as the claims of benefit and expertise made online and in other marketing material can be convincing, even though they have little reputable evidence to support them. In reality, the treatments that have been shown to work are very specific – a particular type of stem cell used in a highly-defined procedure to treat one specific condition.

There is a growing trend where patients and their carers travel overseas to receive treatments. It is important to remember that Australia has a high standard of care and oversight of its healthcare system. Other countries may have dramatically different medical systems, regulations, consumer protection laws, healthcare standards, and costs compared to Australia. Notwithstanding this, there are clinics and doctors in Australia offering what they claim to be stem cell treatments that are unproven.

> With any unproven treatment offered overseas or locally, you may be putting the health and the quality of life you do have at risk, as well as spending a lot of money on potentially unsafe and/or ineffective treatments.

The medical research literature has many cautionary tales:

- A 72-year-old American woman with **age-related macular degeneration** travelled interstate to have her own stem cells harvested from fat tissue and injected into her eyes to restore her lost vision. She found the clinic through its listing on ClincalTrials.gov and thought she was participating in a clinical trial. She paid $USD 5,000. Over time both of her retinas detached. She was completely blind within months.
- A 66-year-old American man sought stem cell therapies in China, Argentina, and Mexico for **stroke-related complications**. Following these treatments he developed lower back pain, paraplegia, and urinary incontinence. Doctors at a Boston hospital found he had developed a dense tumorous mass around his lower spinal cord. The tumour left him far worse off than the stroke.

Almost all unproven treatments share a number of characteristics, which are listed in the next section.

Signs that a stem cell treatment is likely to be unproven

- The experimental treatment is not part of a registered clinical trial.
- Patient testimonials are used to support claims of benefit with no independent verification.
- There are no published scientific papers to support the claims made for the treatment.
- The treatments are advertised directly to you rather than via a referral from your treating doctor.
- The one type of treatment is claimed to treat a wide range of conditions and illnesses.
- The clinic or doctor claims there is no risk involved.
- You are asked to pay for a treatment that is not yet accepted in medical practice or is an authorised product.
- There is no Medicare or private health insurance rebate for the treatment.
- The stem cells are prepared on-site at the doctor's surgery.

If you are considering a stem cell treatment, you should ask the following important questions:

- Is the treatment offered by an established hospital or medical research institution? If you are not sure, talk to your doctor;
- Do your GP and specialist both recommend the treatment for you?; and
- Is the treatment recognised by Medicare or your private medical insurance provider?

Questions to ask about stem cell treatments

If you have satisfied yourself with answers to these questions and booked a consultation with a private clinic to find out about a stem cell treatment, here are some more detailed questions to ask the clinic that can help you with your final decision.

The treatment

- Is this an approved medical procedure? If yes, who approved it?
- Is the treatment considered 'experimental'? If so, is it offered as part of a clinical trial?
- If part of a clinical trial, is the trial registered with ANZCTR, NIH Clinical Trials or any other recognised registry?
- Has this treatment been reviewed by an ethics committee? Which committee?
- Who is funding this trial?
- What is the evidence that this treatment will work for my condition?
- Can you provide me with peer-reviewed publications that can support the likely benefit of exactly this treatment?
- What are the risks or side effects associated with the treatment? Are there any risks of the formation of tumours or problems with my immune system?
- What is the cost and exactly what does this include?
- How many visits are required? Is there a separate fee for each visit?

Travelling overseas

- Will my travel insurance cover my treatment or help me get home if I have a reaction or get sicker?
- Are there likely to be additional costs or are these included in the fee (e.g., travel, accommodation, meals, insurance, medications, hospital bed costs, consumables used during the treatment, cell processing costs)?

The procedure

- What type of cells are you using, mine or someone else's?
- If using donor cells, where do they come from? Are they human or from another species? If from another species, which one?
- Are the donor cells screened for viruses (HIV, Hepatitis B, Hepatitis C, HTLV-1, HTLV-2)?
- Are the cells prepared in a licensed laboratory or using an approved device? If so, who prepares them? Who were the devices approved by?
- Do you use animal products to grow the cells and how do you guarantee there is no risk of contamination or infection?
- How are the cells delivered? Are they injected? If so, how and what are the possible complications?
- Do I need to take any medication before or after the treatment? Is there an additional charge for this medication?

Care and cost

- Who is the doctor performing the treatment? Are they a specialist in treating my condition? (Note: 'stem cell specialist' or 'regenerative medicine specialist' are not recognised qualifications.)
- How much does the treatment cost and is there a rebate from Medicare or my private health insurance?
- Will I receive any other treatments or therapies when I am at the clinic? Does this attract an additional cost?
- What ongoing care will I receive? Does this attract an additional cost?
- How will my health be evaluated after the treatment, for how long and by whom?

My rights

- What happens if something goes wrong? Who do I contact?
- Who will cover the costs of any medical complications?
- To what compensation am I entitled if I am injured?
- What are my rights as a participant, such as confidentiality or my right to be informed of new information gained about my health or condition?
- Do I have the right to withdraw from the treatment process?

Regulating stem cell treatments

In Australia, the TGA is responsible for the regulation of products for therapeutic use including human cells and tissues (termed 'biologicals'). This includes human stem cell treatments. Regulated products are listed on the Australian Register of Therapeutic Goods (the Register).

> In recent years, the TGA has strengthened the regulation of biologicals including stem cell products in Australia. The new regulations maintain consumer access to established medicines and cell therapies while protecting consumers from unproven and potentially harmful treatments.

Previously, the TGA excluded doctors who used a patient's own cells from its regulatory requirements. Such products are referred to as autologous. In response to concerns about an increasing number of commercial clinics offering unproven stem cell treatments in Australia, the TGA has expanded its oversight.

Under the new regulations, all doctors and dentists providing autologous cell and tissue products who operate outside of Australian hospitals will need to meet the TGA's requirements for safety, quality and effectiveness. Providers who fail to meet these requirements may face civil or criminal penalties.

Patients who have not found relief from the symptoms of a health condition through conventional means are especially vulnerable to claims about emerging treatments. Due to the vulnerability of these people, penalties can apply when a person misleadingly advertises or promotes the use of cell products to the public. All advertising of unproven stem cell-based therapies in Australia is banned, unless authorised or required by a government authority. To date, no authorisations have been granted.

The TGA has developed a public education resource: **Stem cell treatments and regulation - a quick guide for consumers.**

About the National Stem Cell Foundation of Australia

This guide was produced by the National Stem Cell Foundation of Australia.

We promote the study and use of stem cells in the prevention or control of disease in human beings and enhance public education in this field.

We aim to:

Assist Australian stem cell researchers to pursue cures for as-yet untreatable diseases using stem cell technology and regenerative medicine. This is achieved by supporting research activities in these areas.

Provide resources for the Australian public on stem cell technology and regenerative medicine including its risks, achievements, benefits and overall technical progress.

We achieve these aims by pursuing charitable purposes only, using donations received to promote these activities.

You can find out more about stem cell research and our work at **www.stemcellfoundation.net.au**

We welcome your support for our work.
Visit **www.stemcellfoundation.net.au/donate**

www.twitter.com/AusStemCell

www.facebook.com/stemcellfoundation

Useful links

Making sense of stem cell science:
www.stemcellsaustralia.edu.au

A Closer Look at Stem Cells:
www.closerlookatstemcells.org

Patient support organisations

Cancer Council: **www.cancer.org.au**, and stem cell transplants for cancer:
www.canceraustralia.gov.au/impacted-by-cancer/treatment/stem-cell-transplant

JDRF (Type 1 diabetes):
www.jdrf.org.au

Kidney Health Australia:
www.kidney.org.au

Leukaemia Foundation:
www.leukaemia.org.au

MS Australia (multiple sclerosis):
www.msaustralia.org.au

Parkinson's Australia:
www.parkinsons.org.au

Stroke Foundation:
www.strokefoundation.org.au

Vision Australia:
www.visionaustralia.org

Glossary

Some of these terms appear in this guide and others you may come across in your research or consultations.

Adult (tissue-specific) stem cell: An unspecialised stem cell found in a tissue or organ that can renew itself and differentiate (specialise) to develop mainly into the cell types of the tissue from which it originated.

Allogeneic transplantation: Cell, tissue, or organ transplants from one person to a genetically different person (i.e. transplantation from a donor).

Australian Research Council (ARC): A statutory agency under the Industry, Innovation, Climate Change, Science, Research and Tertiary Education portfolio within the Australian Government. Its mission is to deliver policy and programs that advance Australian research and innovation globally and benefit the community.

Autologous transplantation: Cell, tissue, or organ transplants from a person back into the same person (i.e. transplantation which involves your own cells). Such transplants, which are often performed with blood products or bone marrow, do not induce an immune response and are not rejected. However, these transplantations still involve potential risks to the patient.

Blastocyst: An early-stage embryo about seven days after fertilisation and containing about 150 cells. A blastocyst consists of two types of cells: cells of the inner cell mass, which give rise to all the organs and tissues of the future embryo and foetus; and the trophoblast which forms a portion of the placenta. Embryonic stem cells are derived from the inner cell mass of donated IVF embryos.

Cell culture: The growth of cells in a laboratory where nutrients, growth factors and all other requirements for cell survival are provided.

Cell division: The process by which one cell divides into two cells, thereby increasing the cell population.

Cell based therapies: A treatment that administers cells required to repair or rebuild depleted cell populations or tissues. These may include cells derived from stem cells.

Differentiation: The process whereby an unspecialised (undifferentiated) stem cell develops into specialised (differentiated) cells such as those in the liver, brain or heart.

Efficacy: The capacity to produce an effect, i.e. how well it works.

Embryo: A stage of development immediately following fertilisation of an egg by a sperm. Stages of embryonic development can be divided into pre-implantation (early development before the pregnancy is established) and post-implantation (after the embryo has implanted into the lining of the uterus).

Embryonic stem cells (ESCs): Stem cells derived from human embryos (at the blastocyst stage). ESCs are self-renewing (can replicate themselves) and have the potential to differentiate into all cell types in the body.

Food and Drug Administration (FDA): An agency of the United States Department of Health and Human Services, which is responsible for protecting and promoting public health through the regulation and supervision of food safety, tobacco products, dietary supplements, prescription and over-the-counter pharmaceutical drugs, vaccines, biopharmaceuticals, blood transfusions, medical devices, electromagnetic radiation emitting devices and veterinary products.

Foetal stem cells (foetal tissue): These are stem cells derived from donated foetal tissue and share many of the characteristics of the adult stem cells.

HTLV-1 - Human T-Lymphotropic Virus Type I: A transmissible human RNA retrovirus that causes T-cell leukaemia and T-cell lymphoma in adults and may also be involved in certain demyelinating diseases, including tropical spastic paraparesis.

Haematopoietic stem cell (HSC): The parent stem cell or 'precursor' of mature blood cells. They are found in adult bone marrow, umbilical cord blood, peripheral blood, and foetal liver.

Human Research Ethics Committee (HREC): Assists Australian institutions in meeting their obligation for the effective governance of research involving humans and is the Australian equivalent of an Institutional Review Board in the USA.

Induced pluripotent stem cells (iPS cells): Stem cells which resemble pluripotent embryonic stem cells. They are derived from mature, fully differentiated cells of the body that have been reprogrammed through genetic manipulation and other techniques to restore developmental potential.

In vitro fertilisation (IVF): An assisted reproduction technique where fertilisation is achieved outside the body. Sperm and eggs are brought together in the laboratory (i.e. in a glass dish) to achieve fertilisation before being transferred to the uterus (or womb) to attempt pregnancy.

International Society for Stem Cell Research (ISSCR): An independent, non-profit organisation formed in 2002 to foster the exchange of information on stem cell research. With more than 3500 members worldwide, the ISSCR has become the voice of the stem cell research community.

Mesenchymal stem or stromal cells (MSCs): A type of adult stem cell found in several tissues of the body including bone marrow, umbilical cord blood, placenta, endometrium (lining of the uterus), amniotic fluid and adipose tissue. Those from the bone marrow can give rise to a number of tissue types such as bone, cartilage, fat tissue, and connective tissue. Most MSC types display immunosuppressive and anti-inflammatory properties.

Multipotent: The potential of an individual stem cell to develop into a restricted number of (but not all) types of cells. Many adult stem cells are multipotent.

National Health and Medical Research Council (NHMRC): Australia's peak body for supporting health and medical research; for developing health advice for the Australian community, health professionals and governments; and for providing advice on ethical behaviour in health care and in the conduct of health and medical research.

National Stem Cell Foundation of Australia (NSCFA): A not-for-profit company limited by guarantee and an Australian Tax Office endorsed charity. Established in 2011 as the follow-on organisation from the Australian Stem Cell Centre.

Peer review: The process of subjecting an author's scholarly work, research, or ideas to the scrutiny of others who are experts in the same field.

Pluripotent: The ability of a stem cell to develop into any type of cell in the body. Embryonic stem cells and induced pluripotent stem cells are examples of pluripotent stem cells.

Progenitor cell: A transitional form of stem cell that can differentiate but cannot renew itself indefinitely. Progenitor cells are restricted to generating a few types of specialised cells.

Regenerative medicine: A treatment in which stem cells are induced to differentiate into a specific cell type to repair damaged tissues or replenish a depleted cell population. They are then used to treat disease.

Stem cell: An unspecialised or undifferentiated cell with the ability to self-renew, and to differentiate to produce specialised cell types in the body.

Stem cell line: Stem cells that have been established and propagated in culture and which maintain consistent characteristics and developmental potential.

Stem Cells Australia (SCA): An ARC-funded special research initiative in stem cell science that ran from 2011 to 2021.

Therapeutic Goods Administration (TGA): Australia's regulatory authority for therapeutic goods. It carries out a range of assessment and monitoring activities to ensure therapeutic goods available in Australia are of an acceptable standard with the aim of ensuring that the Australian community has access, within a reasonable time, to therapeutic advances. The TGA is the equivalent of the FDA in the USA.

National Stem Cell
Foundation of Australia