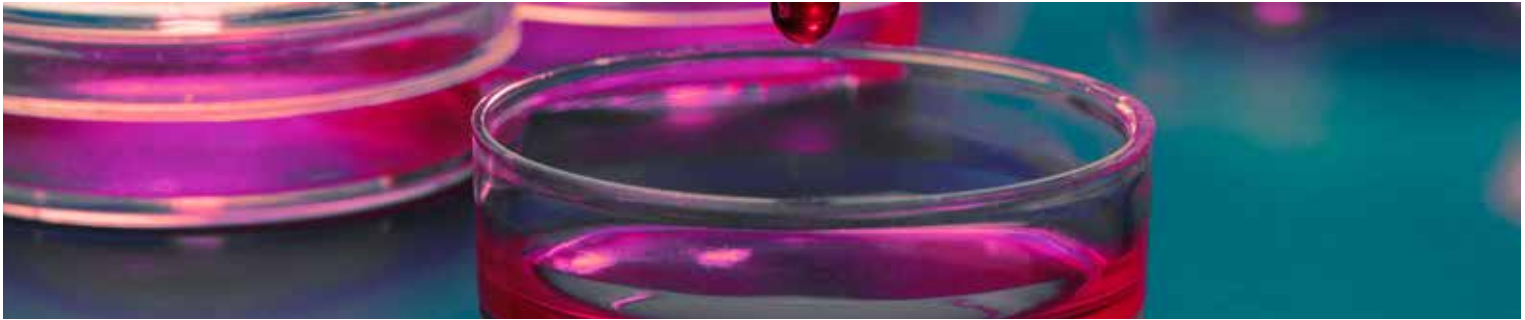




Stem Cell Treatments – Frequently Asked Questions

DECEMBER 2013 | Limited Review 2019



A limited review of this resource has been undertaken to take account of changes to the regulation of autologous human cell and tissue products, commonly referred to as ‘stem cells’, that came into force on 1 July 2019. It was updated in December 2019 to reflect the impact of those amendments. This resource has not undergone a full review, and up-to-date information about the regulation of autologous human cells and tissues products can be found via <https://www.tga.gov.au/autologous-human-cells-and-tissues-products-regulation>.

Are you considering stem cell therapies to treat your condition?

If so, this document may assist you to:

- tell the difference between proven and unproven treatments
- understand the risks involved in undergoing treatments that have not been established as safe and effective.

This resource has been adapted from existing patient handbooks^{1,2}, and provides some frequently asked questions and answers regarding stem cell treatments.

1. What are stem cells?

Our bodies are made up of about 200 different types of cells, such as muscle cells, skin cells and nerve cells. Each cell type specialises in performing a certain function. Stem cells are cells that are not specialised. Their role is to replace specialised cells

that have been lost through injury, disease, or the normal course of events (such as regeneration of the lining of our bowel every few days).

Stem cells have two major features, as illustrated in Figure 1. First, they have the potential to become a range of different cell types. The process of stem cells becoming particular cell types is called ‘differentiation’. Second, stem cells can also replace themselves, or ‘self-replicate’.

The ability of stem cells to differentiate and self-replicate is what has stimulated such enormous interest in the use of stem cells for the treatment of disease.

Stem cells can be recovered from embryonic, fetal and some adult tissues and have different properties depending on their source.

For more information on stem cells visit <http://www.closerlookatstemcells.org>

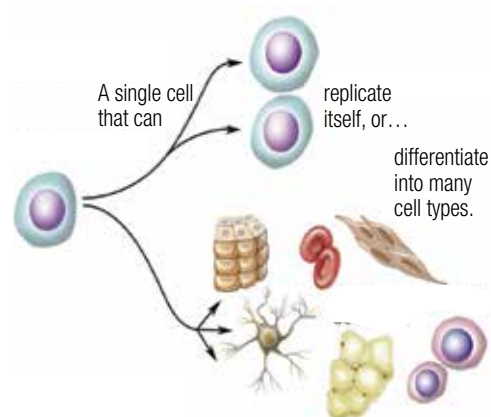


Figure 1: Simplified diagram demonstrating the features of a stem cell

Used with permission from Understanding Stem Cells: An Overview of the Science and Issues from the National Academies by the National Academy of Sciences, Courtesy of the National Academies Press, Washington, D.C.

2. What stem cell treatments are available, and are they safe and effective?

Not all stem cell treatments have been demonstrated as safe and effective.

The only stem cell treatment that has been established to be safe and effective is the use of haematopoietic (blood) stem cell transplantation to rebuild the blood and immune system. Haematopoietic stem cell transplantation may be referred to as a bone marrow or peripheral blood stem cell transplant, or a cord blood transplant, depending on the source of the stem cells. Transplantation of these cells is used as a treatment for disorders of the blood and immune system such as leukaemia and lymphoma and as supportive treatment in therapy of several other cancers.

Currently, no other stem cell treatment has been demonstrated to be safe and effective. However, some clinics located both in Australia and overseas offer unproven stem cell treatments. These 'stem cell' treatments are frequently made up of a mixture of different cells, rather than pure stem cells.

3. What are the risks of undergoing unproven stem cell treatments?

An unproven stem cell treatment may be unsafe, posing serious risks to your health. The risks include infection, allergic reaction or rejection of the cells by your immune system, and the development of cancer. These complications can be fatal.

Unproven stem cell treatments may also involve significant financial costs, including treatment and follow-up costs, as well as the cost of emergency medical care in the event that something goes wrong. For treatments offered outside of Australia, there are often additional costs associated with travel such as accommodation, airfares, meals and carer's expenses.

Undergoing an unproven stem cell treatment may interfere with proven and potentially beneficial therapies or treatment plans recommended by your general practitioner or specialist. It can also disqualify you from participation in a registered clinical trial (see question 4).

Stem cell clinics can be located in countries with lower standards of medical care than in Australia, including poor hygiene practices and inadequate infection control procedures. These countries may also accept different standards in the qualifications and insurance coverage required to practice as a medical practitioner. They may not regulate medical practitioners (e.g. by government authorities or medical boards) as rigorously as in Australia, and restrictions on who can perform stem cell treatments (e.g. whether a medical qualification is required) may be limited or not enforced. The laws governing medical practice also vary significantly from country to country, and in some countries where stem cell treatments are offered, there are no legal pathways to follow in the event of negligence or malpractice.

4. What is the internationally accepted standard process for developing a new medical treatment?

A new medical treatment should be tested through clinical trials to show that it is safe and effective before it is made available to the public. Clinical trials are research studies where people volunteer to test new treatments, interventions or tests as a means to prevent, detect, treat or manage various diseases or medical conditions.

Researchers may first test new treatments in the laboratory and in animal studies. The most promising experimental treatments are then moved into clinical trials where they are tested in humans. Many clinical trials to develop new treatments are conducted in phases, with initial testing conducted on a small number of participants to investigate safety and efficacy.^a If the treatment is promising, it may move to later phases of testing where the number of participants is increased to collect more information on effectiveness and possible side effects. Treatments are described as 'unproven' if they have not undergone testing in a clinical trial, or have not reached the final phases of testing, and therefore their safety and effectiveness has not been established.

It can take many years to properly assess whether a treatment is safe and effective through the clinical trial process.

^a Efficacy (or whether a treatment is efficacious) refers to whether a treatment works within ideal conditions, such as a controlled, research setting. Conversely, effectiveness refers to whether a treatment works in the real world.

Key features of clinical trials:

- Clinical trials both within Australia and overseas should be listed on a clinical trials register approved by the World Health Organisation, such as the US National Institute of Health Clinical Trial Database or Australian New Zealand Clinical Trials Registry.
- Clinical trials within Australia must be approved by a Human Research Ethics Committee registered with the National Health and Medical Research Council.
- Patients do not usually pay for treatment in a clinical trial.
- Patients will be asked to read and sign a detailed patient informed consent form before participating, which explains the details of the study along with any potential risks and benefits.
- Some patients may not receive the treatment being investigated (placebo or control group), but all patients will have their health and medical condition monitored throughout the trial.
- Undergoing unproven stem cell treatments in Australia or abroad may disqualify a patient from future participation in a clinical trial.

You can find out for yourself whether any clinical trials are currently investigating treatments for your specific disease. The following online registries are available to search for clinical trials being conducted in Australia, New Zealand and around the world.

- The Australian New Zealand Clinical Trials Registry (<http://www.anzctr.org.au/>)
- US National Institute of Health Clinical Trial Database (<http://clinicaltrials.gov/>)

For information on stem cell clinical trials currently underway around the world for various conditions, visit <http://www.stemcellsaustralia.edu.au/about-stem-cells/stem-cell-clinical-trials2.aspx>

For more information on clinical trials, visit <http://www.australianclinicaltrials.gov.au/>

5. What should I be aware of when researching stem cell treatments?

There are a number of factors to consider when researching stem cell treatments. To ensure that a stem cell treatment is safe and effective it is important to know whether the final phases of independent clinical trials have been completed (see question 4) and if the treatment has been assessed as safe and effective by regulatory authorities.^b Studies demonstrating the effectiveness of the treatment should also have been published in independent, peer-reviewed scientific journals. Peer review is the process whereby scientific work is checked and agreed by independent experts.

You should be cautious when researching health information online. It is important to look at websites that provide reliable evidence that the treatment has been tested for safety and effectiveness. Government websites and disease alliance websites are often more trustworthy sources than personal blogs or websites sponsored by commercial entities. It is also important to seek information from a source other than the clinic that is offering the treatment.

Remember that anyone can publish information on the internet, whether or not the information is true. Personal testimonies from other patients do not mean that the treatment will work for you. You should also think twice about statements describing the therapy as a 'quick fix', 'scientific breakthrough', 'miracle cure', or other similar claims. If it sounds too good to be true – such as a claim that the treatment can cure a disease or treat a variety of conditions – it usually is.

6. What questions should I ask when considering stem cell treatment?

It is important to understand all the risks associated with unproven stem cell therapies before undertaking treatment. This includes financial risks, as well as the potential risks to your health and future entitlements to participate in approved clinical trials (see question 3).

The following questions may help guide you in your discussions with stem cell treatment providers in Australia and overseas.

^b In Australia, the Therapeutic Goods Administration (TGA) is responsible for the regulation of biological items including stem cell treatments. In the USA this is the role of the Food and Drug Administration (FDA).

Key questions to ask the treatment provider:

- What will the treatment do for me? Is there scientific evidence that the treatment could work?
- What are the risks involved in the treatment?
- Is there scientific evidence identifying and examining these possible risks?
- Have there been any clinical trials performed?
- Where is the information/evidence about risks and benefits published?
- If the provider refers to studies that have been conducted, you may want to ask:
 - How many patients were included in the study?
 - Have the effects been measured and verified by someone outside of the clinic?
 - Is the benefit short or long-term? How was this tested?
 - Is it better than existing treatments? How was this tested?
- Is the treatment only for my specific condition?

NB: diseases that are not related (e.g. heart disease and osteoarthritis) would usually have very different treatments.
- What is the cost for the entire procedure and what is included in the price? How much do I have to pay upfront? (e.g. travel, accommodation, meals, insurance, medications, hospital bed costs).
- How many treatments do I need? Will I need follow up assessment/s? If yes, how many, and how often? If receiving treatment abroad, can the follow-up care be provided by someone in Australia? What will be done if complications or serious side effects develop? What would the potential costs be?
- Who is the doctor performing my treatment? What are their qualifications? Is he/she a specialist in treating my condition?

You should also discuss with your general and specialist medical practitioner/s any information that you uncover in your research, or information provided to you by the institution offering the stem cell treatment.

7. Where can I get further information?

In order to make a well informed choice regarding the stem cell treatment you are considering, you should seek additional information from a source other than the treatment provider. You should take time to consider the risks and benefits of the treatment and should not feel rushed or pressured into making a decision.

The following resources may also be useful for patients considering stem cell treatments:

- National Stem Cell Foundation of Australia and Stem Cells Australia – *The Australian Stem Cell Handbook*: http://www.stemcellfoundation.net.au/educational_resources
- International Society for Stem Cell Research – *Patient Handbook on Stem Cell Therapies*: <http://www.closerlookatstemcells.org/patient-resources>
- International Society for Stem Cell Research *A Closer Look at Stem Cell Treatments* website: <http://www.closerlookatstemcells.org/>
- Stem Cells Australia website: <http://www.stemcellsaustralia.edu.au>

References

- 1 Australian Stem Cell Centre. *Stem Cell Therapies: Now and in the Future*. Melbourne: ASCC, 2011
- 2 International Society for Stem Cell Research. *Patient Handbook on Stem Cell Therapies*. Skokie: ISSCR, 2008. Viewed 12 September 2019 update: <https://w.closerlookatstemcells.org/patient-resources/>
- 3 Australian Clinical Trials. *What is a clinical trial?* Canberra: NHMRC. Viewed 25 June 2013 <http://www.australianclinicaltrials.gov.au/whatclinical-trial>