



stem cell interventions for spinal cord injury

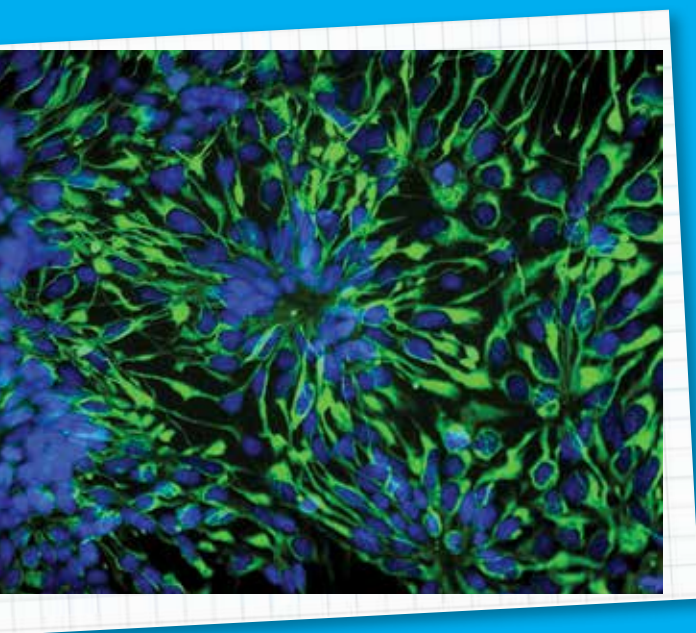
Stem cell interventions for spinal cord injury

Stem cell research is in its relatively early stages. While laboratory and animal research to date holds great promise for treating a range of illnesses in the future, there are currently **no stem cell therapies** that are recommended for people with spinal cord injuries. Translation of laboratory-based stem cell research into treatments for people is likely to take many years and must be conducted with scientific rigour and strict controls in clinical trials. This includes ethical oversight and safeguards to protect the welfare of those volunteering to participate in such research. Paramount is ensuring that a new intervention, including those involving stem cells, reliably provides benefit and does not cause further harm. Findings from clinical trials are usually shared with colleagues and the broader community through peer-reviewed scientific publications.

Recent publicity resulting from the Australian of the Year awards has focussed attention on the use of specialised cells from the nose (olfactory ensheathing cells, or OEC) to potentially treat spinal cord injury. Whilst some basic science on these cells shows that they have features that make them potentially useful, there are as yet no reliable clinical results to show that they are effective in restoring function following spinal cord injury. The dramatic claims in the media about the man that 'walked again' after receiving OEC are yet to be substantiated. While this early study showed that it appeared safe, the researchers themselves recommended caution in interpreting the results. A much more rigorous study will be needed to show if there is long-term benefit from the use of OEC, and a group in London is in the planning stages of such a study.

There is growing concern that stem cell therapies are already being marketed and sold around the world before they have been shown safe and effective in clinical trials. There are controversial treatments being offered in some parts of China, India and other countries that have not been reported in respectable peer-reviewed scientific journals and have not been replicated by other scientific groups. The doctors providing these treatments only highlight the positive outcomes, have no, or very limited, pre- and post-treatment assessments, and often charge large sums of money to vulnerable individuals. They rely on testimonials from people who say that they have had improvement. We rarely hear about the people who have not improved. We know little about the possible side-effects and long-term complications related to these procedures. Some serious side effects including meningitis, tumours and death have been reported. In addition to compromising the health and wellbeing of those who decide to pursue these unproven interventions, such unethical and poor scientific practices could in fact hinder the advancement of this important area of research.

In summary, people with spinal cord injury should be extremely careful at this stage about participating in experimental procedures outside of registered clinical trials. Alleged stem cell treatments marketed on the internet are expensive, and have not been proven to be safe and effective. Individuals should find out all they can about what is involved in experimental stem cell procedures and any side-effects of such treatments. Many of the ways the cells are administered such as lumbar puncture carry risk of serious complications such as bleeding, infection, headache and even death. Seek advice from your spinal cord injury unit or specialist if you are considering participating in these types of stem cell procedures or any other research trials.





Frequently asked questions (FAQ's)

What are stem cells?

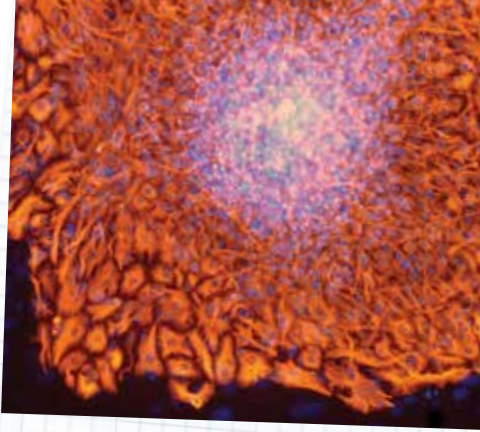
Stem cells are unspecialised cells that can multiply and produce new, specialised cells that are necessary for the body to function. They also have an important role to help the body repair as a result of injury or disease. There are several different types of stem cells. Some can be found in early embryos, others in the placenta and umbilical cord, as well as in adult tissues and organs throughout the body such as the bone marrow. In the laboratory, stem cells have been shown to reproduce themselves through cell division and under certain conditions can give rise to specialised cells. Different types of stem cells have different abilities and potential applications.

Embryonic stem (ES) cells

Embryonic stem cells are produced from a very early stage of embryo development called a blastocyst (around a week after fertilisation). Cells are removed from the embryo and grown in the laboratory. The embryos from which these cells are derived are “left over” fertility clinic (IVF) embryos that would otherwise be discarded and are obtained only with the donor's informed consent. The cells are not from naturally conceived embryos. Embryos cannot be specifically created for research purposes. These cells, called ES cells, can be relatively easily grown in large numbers in the laboratory and given the correct signals they can make specialised cells such as blood, nerves and muscle. The ability to grow into many different types of cells is called pluripotency. While this ability to grow readily is very valuable, it also means that, in some circumstances, these ES cells may form tumours and may trigger immune rejection after they are transplanted. Embryonic stem cell research has been legal in Australia since 2002 (please see link to legislation at the end of this section). Cells made from ES cells are starting to be tested in clinical trials for diabetes and a specific type of blindness.

Tissue or adult stem cells

Tissue stem cells are found in body tissues such as fat, heart and bone marrow and from discarded umbilical cords and placentas. These cells have the potential to make the type of specialised cells found in their host tissue, but usually not other types of cells. Their main job in the body is to maintain and repair the tissue in which they are found. Often called adult stem cells, these stem cells are often considered less controversial than ES cells because there is no destruction of



embryos and when isolated from the patient's own tissues may avoid problems with immune rejection. However, a current limitation is that these cells usually occur in very small numbers and are difficult to grow in the laboratory. They are also more specialised than ES cells and therefore have limited ability to produce different cell types. Haematopoietic or blood stem cells are already used to treat diseases of the blood and immune system such as leukaemia.

Induced pluripotent stem (iPS) cells

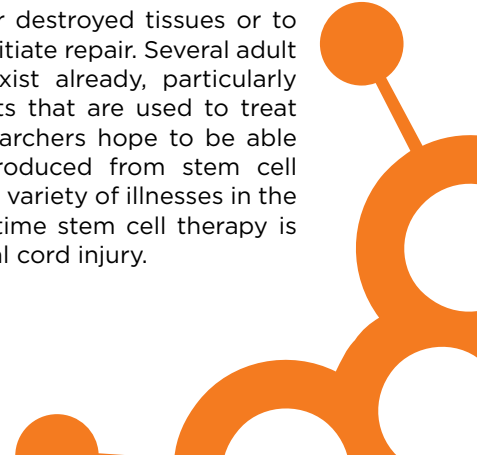
More recently scientists have been able to make a new type of stem cell. Using advanced laboratory techniques, induced pluripotent stem cells or iPS cells can be produced from any specialised cell in the body, for example a skin cell. Like ES cells they can be turned into any cell type and iPS cells are patient specific and thus avoid problems related to immune rejection. However, like ES cells, these stem cells carry a risk that they could form tumours if not properly treated. Only discovered just over 10 years ago, these cells are used extensively in research and cells made from them are being to be used in clinical trials in Japan.

More information on stem cells

- Stem Cells Australia has produced a very informative and thorough review of stem cell treatments. Their publication 'Australian Stem Cell Handbook' is available at: www.stemcellsaustralia.edu.au/about-stem-cells.aspx
- National Institutes of Health – follow-link “Learn more about stem cells” www.stemcells.nih.gov

What is stem cell therapy?

Stem cell therapy involves injection of stem cells or cells made from stem cells into damaged tissues or the blood stream to treat someone with a disease or injury. For this form of therapy to be successful, scientists must be able to produce large numbers of stem cells, turn them into the desired specialised cell types, transplant sufficient numbers of cells to replace the damaged or destroyed tissues or to stimulate the tissue to initiate repair. Several adult stem cell treatments exist already, particularly bone marrow transplants that are used to treat leukaemia. Medical researchers hope to be able to use technologies produced from stem cell research to treat a wider variety of illnesses in the future. At this point in time stem cell therapy is not established for spinal cord injury.



Where are stem cell treatments for spinal cord injury taking place?

There are currently no approved stem cell treatments available. It is important to note that the leap from the laboratory to the clinic is technically difficult and expensive. Many therapies that have been proven to work in animals in the laboratory have failed to work when tested in humans.

There are over 40 registered stem cell research programs that aim to show if there is benefit for spinal cord injury. Of those, 7 have been completed but only two have published their results and these are inconclusive. In recent years two highly publicised, large scale stem cell projects have been prematurely terminated, most likely due a combination of lack of positive results and very high cost. The first study by Geron involved the use of cells made from ES cells and the later one was by Stem Cell Inc., which used neuronal stem cells. Expert comment reveals that in future, more exhaustive and rigorous laboratory and animal testing should be undertaken to avoid public and patient disappointment when a much publicised clinical trial is terminated.

Is stem cell therapy in spinal cord injury effective?

The effectiveness of stem cell therapy is yet to be determined. Experimental studies offered in parts of China, India and other countries have not been scientifically proven to be safe or effective. While sometimes marketed as clinical trials, these interventions are usually outside conventional experimental research. You do not usually have to pay for an experimental treatment. Unfortunately, several people have experienced complications including infections, tumours and even one report of a death following so called stem cell therapy. Although some who seek out these interventions

may claim benefit, it is often unclear whether this was as a result of the cell therapy, the accompanying physio therapy or an improvement that may have occurred without intervention. Only properly conducted clinical trials can provide the necessary evidence to determine whether stem cell therapy can provide real, long-term benefit to those with spinal cord injury.

Where can I get advice?

1. Always consult with your treating doctor. Those selling the interventions have a vested interest in getting you to participate in their program and may not provide impartial advice.
2. The International Campaign for Cures of Spinal Cord Injury Paralysis has published a guide if you are considering participation in a clinical trial 'Experimental Treatments for Spinal Cord Injury: what you should know (version 2)'.
<http://icord.org/wp-content/uploads/2014/01/Clinical-Trials-Document-V2-BL-Final.pdf>.
3. The International Society for Stem Cell Research has compiled a patient handbook and guidelines for the responsible development of safe and effective stem cell therapies.
<http://www.closerlookatstemcells.org/Patient/ISSCRPatientHandbook.pdf>
4. The handbook produced by Stem Cells Australia includes a guide on how to assess the potential value of an offered stem cell treatment.
<http://www.stemcellsaustralia.edu.au/about-stem-cells.aspx>
5. The Stem Cell Treatment – Frequently Asked Questions information sheet from the National Health and Medical Research Council.
<https://www.nhmrc.gov.au/guidelines-publications/rm001a>

This position statement was prepared by the Australasian Spinal Cord Injury Network (Spinal Network) in collaboration with Stem Cells Australia and the Australian and New Zealand Spinal Cord Society (ANZSCoS). Images were kindly supplied by Stem Cells Australia.

The Spinal Network is a bi-national network of leading researchers, clinicians, key stakeholders and people with a spinal cord injury who are committed to a coordinated approach to advancing treatments for spinal cord injury. The Spinal Network is supported by the Office of Health and Medical Research. For more information visit www.spinalnetwork.org.au

Stem Cells Australia brings together Australia's premier life scientists to tackle the big questions in stem cell science. It was established in 2011 by the University of Melbourne, Monash University, the University of Queensland and the University of NSW, CSIRO, the Walter and Eliza Hall Institute, the

Victor Chang Research Institute and the Florey Institute of Neuroscience and Mental Health with Murdoch Childrens Research Institute joining in 2016. For more information visit www.stemcellsaustralia.edu.au

ANZSCoS is an impartial, non-political and non-profit making association whose objective is to promote high quality management of people with spinal cord lesions throughout Australia and New Zealand. It aims to study any and all problems relating to people with traumatic and non-traumatic lesions of the spinal cord including; causes, prevention, basic and clinical research, medical and surgical management, clinical practice, education, rehabilitation and social reintegration.

Disclaimer: This information has been compiled from public data available at the time of production. Neither the Spinal Network, Stem Cells Australia nor ANZSCoS accept any liability for errors.

Date of Production March 2017