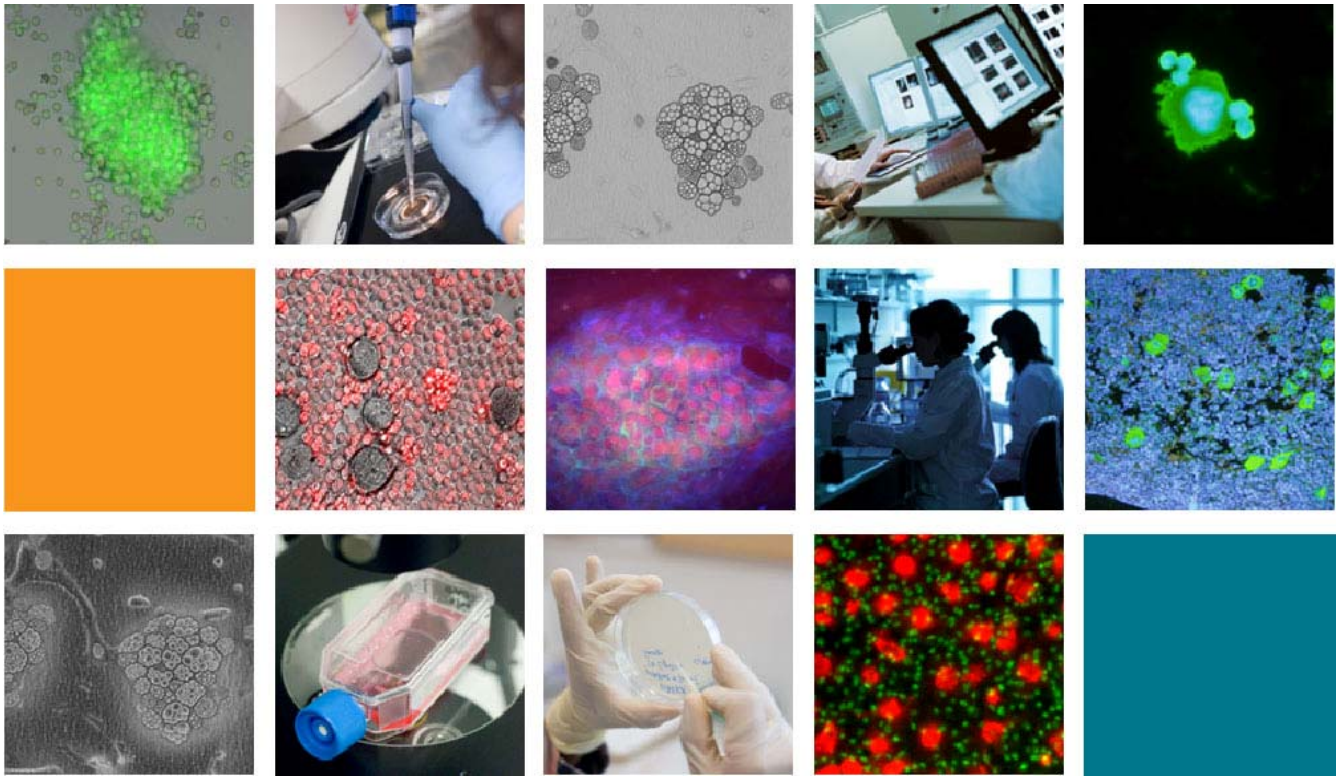
Australian Stem Cell Centre

Stem Cell Therapies: Now and in the Future

The Australian Stem Cell Centre Information Handbook

What you should know about experimental therapies overseas and what is happening in your country

April 2011



Your Quick Guide - Patient Handbook Summary

Stem cell science shows **much promise** for the future treatment of a wide range of diseases and conditions.

Stem cells have had high levels of media and public attention but **much of the research is still in early stages**. There is good cause for hope, but progress is slower than the media often suggests.

Stem cell research is progressing with bone marrow transplants now part of standard clinical practice and growing clinical research and clinical trials encompassing mesenchymal, skin, corneal, cartilage and other stem cell types.

Clinics around the world are offering stem cell treatments but some of these treatments are offered **outside the mainstream medical research environment** - and are marketed directly via the internet. These providers often charge a considerable sum of money for these untested treatments.

The **scientific and medical community is concerned** that some treatments are being offered to patients before they have been proven safe and effective. Some of the experimental therapies on offer may pose **significant health risks** for patients including infection, immune system rejection and possibly cancer later in life.

Stem cell treatments, like any treatment, need to be proven safe and effective before they are accepted into practice. **Testimonials from former patients are not scientific proof** of safety and effectiveness.

Independent clinical **trials approved by government** regulators, **publication** and **peer review** assessment in international scientific journals, and replication of results by other laboratories are the only **sources of evidence** that can be relied upon to confirm a treatment is **safe** and **effective**.

Direct internet marketing is criticised by the general medical and scientific community as it allows providers to make claims that are not substantiated by scientific evidence.

Many of these practitioners **guard their treatment methods and processes tightly which is contrary to the mainstream scientific premise** of peer review which encourages transparency.

Sources of cells can be aborted foetal tissues, cord blood, embryonic stem cells or adult stem cells. **Knowing the source of the stem cells** or tissues being used in the treatment is vital.

Stem cells from another person are likely to be rejected if they are not matched. To reduce the risk of rejection, patients may be instructed to take drugs that suppress the immune system. **Suppression of the immune system** can make patients susceptible to disease and infection.

Embryonic stem cells are of great interest to scientists because in their undifferentiated state they are **pluripotent**, meaning they can **become any cell in the body**. Therefore, embryonic stem cells introduced to a patient, in an undifferentiated state, may also have the potential to become tumours or pre-cancerous cells.

Before embarking on any treatment individuals are encouraged to **discuss** all options with their **doctor** or **specialist**.

Introduction: Why have we written this booklet?

The Australian Stem Cell Centre (ASCC) is often asked about the status of stem cell research in Australia and about stem cell research and treatments overseas.

This Handbook has been written to help patients gain a greater understanding of:

- **what stem cells are**
- **currently available stem cell treatments**
- **unproven stem cell treatments on offer, both in Australia and overseas**
- **the current status of stem cell research in Australia and overseas**

The ASCC Patient Handbook (Handbook) aims to help patients critically analyse stem cell treatments before considering taking part in them. As with all medical treatments, there are risks involved. Our aim is to give patients the information needed to evaluate these risks. This Handbook does not seek to advise or evaluate the treatments, or an individual's reasons for travelling for treatment. Instead it aims to provide the patient with as much information as possible prior to considering any therapy. It also outlines where Australia and the stem cell field are positioned today with respect to stem cell research and clinical trials, in order to provide more insight into the development of stem cells and their future potential.

This Handbook will summarise the pros and cons of medical travel, potential risks and benefits, and help patients evaluate their options when considering travelling overseas for unproven treatments. In addition, we will suggest some questions to ask of practitioners and providers who market these treatments, with the ultimate aim of helping you to make the best informed decision possible.

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Some Common Acronyms

ASCC Australian Stem Cell Centre
ES Embryonic Stem Cell
iPS Induced Pluripotent Stem Cell
IVF In Vitro Fertilisation
TGA Therapeutic Goods Association

1 How to Recognise an Unproven Stem Cell Treatment

Figure 1: characteristics of unproven stem cell therapies

Distinguishing characteristic	Why this is a problem
Direct to consumer marketing. Patients generally learn about these treatments via the Internet, either in direct marketing or as a result of blogs, Facebook pages, etc.	Success rates and benefits can be claimed without any corroboration or peer review. Claims of success are unsubstantiated and cannot be verified. Often very persuasive language is used.
Claims of success based on anecdotes and patient self-reporting.	Individual reports of success may be real, or may be due to the placebo effect, or due to a desire to report a positive outcome because of the amount of time and money invested in obtaining the treatment. Without proper medical scrutiny of these claims they remain unproven and are not valid 'evidence' of success.
No scientific rationale is offered. Scientific methods are not described, and are often kept as a 'trade secret'. Patients are sometimes told that this is due to patents pending.	Many treatments use completely unproven techniques which have not been tested for their safety in humans or animals. Without a scientific rationale to critique, the rest of the scientific community are unable to comment whether a treatment is likely to be safe or effective. 'Patents pending' is not an excuse to conceal scientific methods from the scrutiny of respected colleagues.
Multiple diseases are offered for treatment with the same cells.	Unless the diseases are related, different diseases such as cerebral palsy, diabetes and heart disease would usually have very different treatments. In addition you would most likely not be treated by a doctor who is a specialist in your disease.
The operator claims there is no risk associated with the treatment.	All medical procedures carry risk.
Lack of after care and follow up once the procedure is completed.	Patients have often undergone a significant medical procedure and are at risk of complications. If no responsibility is taken for the management of complications, or if the patients' funds run out as a result of complications, they can be left with large bills and even expensive medical evacuations to get them back to their home country.
Lack of protection via insurance and medico-legal coverage of the practitioner.	Most travel insurance will not cover a pre-existing medical condition. Many of these treatments are provided in countries where there is no legal pathway to follow for medical negligence claims, should they arise.
No evidence that the treatment has undergone review by a recognised ethics committee.	Ethics committees exist to ensure the proper conduct of doctors and scientists, and to protect patients from unscrupulous use of unproven treatments. Without ethics review, there is no such protection for the patient.
Data is not collected. Results are not published in peer reviewed journals.	Without international peer review, practitioners promoting unproven stem cell treatments are unaccountable for their actions. They also fail to contribute to the 'greater good' by publishing their results for the benefit of other patients and researchers. Medical journals are keen to publish results on novel therapies but only if they have been carried out in an ethical manner.

2 I am considering going overseas for an unproven stem cell treatment. What questions should I ask?

There are a number of questions that we would recommend you ask, outlined in Figure 2 below. It is a good idea to have a written list of questions and to write down the answers or preferably obtain the answers in writing from the provider of the treatment. Take the answers away and spend some time thinking about them and discussing them with your physician.

Figure 2: Checklist – Questions to ask the provider of the treatment

The cells

- ❑ What type of cells are you using (my own, someone else's, cells from umbilical cord blood, foetal tissue or embryos)?
- ❑ Do you use animal products (particularly bovine, or cow-derived*) to grow the cells?
- ❑ Do you test the cells for viruses (HIV, Hepatitis B, Hepatitis C, HTLV-I and HTLV-II)?
- ❑ Could the cells harm me? Could they form tumours or could they cause autoimmune problems?
- ❑ Will my immune system reject the cells?

The procedure

- ❑ How are the cells delivered? Are they injected**?
- ❑ How many visits are required?
- ❑ What are the potential complications of injection?
- ❑ Do I need to take any medications afterwards?
- ❑ If so, what are their side effects?
- ❑ What chance is there of the treatment working? What evidence are you basing this on?

Transparency and accountability

- ❑ Did this treatment undergo ethics committee review?
- ❑ Are you collecting data to publish?
- ❑ Have you published data already?

Personnel

- ❑ Who is the doctor performing the treatment?
- ❑ Is he/she a specialist in treating my condition?

Medical Care and Practicalities

- ❑ Will my travel insurance cover my treatment?
- ❑ Who covers the cost of any medical complications?
- ❑ Who looks after me if I become unwell overseas?
- ❑ What happens if I become unwell back at home?
- ❑ Cost: what is included in the price (travel, accommodation, meals, insurance, medications, hospital bed costs, consumables used during treatment, cell processing costs)?

*Many cell culture techniques use products derived from cows or calves. This carries a theoretical risk of variant Creutzfeldt Jacob disease (mad cow disease).
** Injections into brain, spinal cord or pancreas carry risks of damage to these structures.

3 Stem cells – the science explained

What are stem cells?

The human body is made up of about 200 different kinds of specialised cells such as muscle cells, nerve cells, fat cells and skin cells. All specialised cells originate from stem cells. A stem cell is a cell that is not yet specialised. The process of specialisation is called differentiation and once the differentiation pathway of a stem cell has been decided, it can no longer become another type of cell.

Different types of stem cells have different levels of *potential.* A stem cell that can become every type of cell in the body is called **pluripotent** and a stem cell that can become only some types of cells is called **multipotent**.

Where are stem cells found?

Stem cells are found in the early embryo, the foetus, amniotic fluid, the placenta and umbilical cord blood. After birth and for the rest of life, stem cells continue to reside in many sites of the body including skin, hair follicles, bone marrow and blood, brain and spinal cord, the lining of the nose, gut, lung, joint fluid, muscle, fat, and menstrual blood, to name a few.

In the growing body, stem cells are responsible for generating new tissues, and once growth is complete, stem cells are responsible for repair and regeneration of damaged and ageing tissues.

Stem cells can be divided into two broad groups: **tissue specific stem cells** (also known as **adult** stem cells or multipotent stem cells) and **pluripotent stem cells** (including embryonic stem cells and induced pluripotent stem cells). Tissue specific stem cells are derived from, or resident in, adult tissues, and can usually only give rise to the cells of that tissue, and are considered multipotent. Embryonic stem (ES) cells, derived from a small group of cells in the early embryo (five-seven days), are undifferentiated and are considered pluripotent as they can become every type of cell in the body. In 2007, scientists discovered that a mature fully specialised cell, for example a human skin cell, in the right conditions could be induced to mimic the characteristics of an ES cell. These are known as induced pluripotent stem cells (iPS cells).

Why are stem cells so different?

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

1. **Stem cells are unspecialised.** They have not developed into cells that perform a specific function.
2. **Stem cells can differentiate.** They can divide and produce cells that have the potential to become other more specific cell types, tissues or organs. These new cells and tissues are used to repair or replace damaged or diseased cells in the body. Once cells have differentiated, they have less capacity to form multiple different cell types, and become 'committed' to becoming a particular cell type. Skin stem cells, for example, give rise to new skin cells when needed, to assist regeneration after damage and as part of the normal ageing process.
3. **Stem cells are capable of self-renewal.** Stem cells are able to divide and produce copies of themselves which leads to self-renewal. Once a cell has become specialised (has differentiated) to a particular tissue or organ, it has a very limited capacity to self-renew (produce new stem cells) but instead produces only cells relevant to that organ.

For more information on the different types of stem cells please see *Fact Sheet 2: Types of Stem Cells*.

4 What are the main types of stem cells currently used in treatments? What treatments are on the horizon?

Within Australia the only proven treatment available involving stem cells is for the treatment of some blood and autoimmune diseases. These treatments involve either bone marrow or umbilical cord blood transplants. The remainder of treatments are still in the research phase or in clinical trials, and are yet to be proven safe and effective.

Stem cells are already being used to treat a number of disorders in patients around the world. However, while there is a great deal of attention surrounding the potential of stem cells, in reality, the range of diseases for which there are proven treatments using stem cells is quite small. The only established stem cell therapies are those of the blood system involving transplants of haematopoietic (blood forming) stem cells (usually from bone marrow or with cord blood) to reconstitute the blood and immune system. All other medical procedures involving stem cells are still currently considered 'experimental' or 'unproven'. Within the 'experimental' category, there are some promising clinical trials in the tissue specific (adult) stem cell field in areas such as corneal, mesenchymal, skin and cartilage, and the first clinical trial using human ES cells for spinal cord injury has recently begun (see Chapter 6 Clinical trials explained for more detail.)

Stem cells were first used in bone marrow transplants, with the first performed in the USA in 1956. Since the late 1990's when ES cells were discovered in humans, the advances in stem cell science have been rapid. However not all research is looking at specific cellular replacement therapies, much of the work in the field of stem cell science is focused on understanding normal development and disorders of the human body, and for testing new drugs.

Stem cell science is moving towards clinical trials in certain areas with several safety studies having recently commenced, examples of which are provided throughout this document. Figure 3 provides an overview of the accepted diseases for which there is a proven stem cell therapy.

What are bone marrow stem cells and how are they used in therapy or research?

Bone marrow transplants containing blood forming (haematopoietic) stem cells have been used for about 50 years to treat people with a variety of life threatening disorders such as lymphomas, leukaemia and thalassemia. A bone marrow transplant is a procedure carried out to renew the patient's bone marrow and immune system with healthy stem cells that are ideally free of disease. A patient's own bone marrow stem cells may fail either because of an underlying disease or due to the effects of chemotherapy or radiotherapy. Haematopoietic stem cells are either taken directly from the bone marrow or are harvested by 'mobilisation' – using a medication which causes the stem cell to leave the bone marrow, and circulate in the bloodstream. They can be collected from the bloodstream in a process similar to a blood donation.

Haematopoietic stem cells used in therapy can either be autologous or allogeneic. An autologous transplant is when the cells transplanted were taken from and transplanted back to the same individual. Such transplants do not induce an immune response and are not rejected. An allogeneic transplant involves the donor cells being used to treat a different individual. The type of transplant required depends on the condition that is being treated.

The Australian Bone Marrow Donor Registry provides further information on what happens during a bone marrow transplant.

What about umbilical cord blood stem cells?

Umbilical cord blood is a rich source of stem cells that are actually a type of haematopoietic stem cell with the same characteristics of stem cells from bone marrow. With the consent of the parents, blood can be collected from the umbilical cord of a newborn baby shortly after birth. This does not hurt the baby or the mother in any way, and it is blood that would otherwise be discarded as biological waste along with the placenta (another rich source of stem cells) after the birth. Umbilical cord blood also contains cells of the immune system which may also have medical potential and are currently being investigated for use in treating autoimmune diseases.

Are umbilical cord blood stem cells already used in medicine?

Umbilical cord blood stem cells are currently used to treat a range of blood disorders and immune system conditions such as leukaemia, anaemia and certain autoimmune diseases. They are used largely in the treatment of children but have also started being used in adults.

There are many groups now researching the use of cord blood for the treatment of other diseases or injury with some clinical trials underway. For example there is a high profile clinical trial ongoing at Duke University in the United States which is testing the use of cord blood to treat children with cerebral palsy (read more about the trial on the US government clinical trials registry www.clinicaltrials.gov).

See Chapter 7 Umbilical cord blood banking in Australia for more information.

Figure 3: List of accepted diseases for which bone marrow and umbilical cord blood (haematopoietic) stem cell transplantations are performed.

Accepted indications for bone marrow and umbilical cord blood (haematopoietic) stem cell transplantation*

Cancers

- Leukaemia
- Lymphoma
- Multiple Myeloma
- Sarcoma
- Germ Cell Tumour
- Desmosplastic Small Cell Tumour
- Neuroblastoma
- Choriocarcinoma

Non-cancer diseases

- Congenital Immunodeficiencies
- Chronic Neutropaenia
- Congenital Metabolic Disorders
- Congenital Anaemias
- Haemoglobinopathies
- Sickle Cell Anameia
- Thalassaemia
- Amyloidosis
- Leukodystrophies
- Osteopetrosis
- Haemophagocytic Lymphohistiocytosis (HLH)
- Myelodysplastic Syndromes
- Myeloproliferative Diseases
- Autoimmune Disorders
- Aplastic Anaemia
- Paroxysmal Nocturnal Haemoglobinuria

*This is not an exhaustive list

What is the difference between haematopoietic stem cells and mesenchymal stem cells?

Mesenchymal stem cells are another cell type found in bone marrow but are also found in several other sites in the body such as fat and the placenta. Mesenchymal stem cells are particularly interesting to researchers because in addition to their capacity to differentiate into bone, cartilage, fat and connective tissue, they also have anti-inflammatory and immune-suppressing properties.

While there are currently no proven and readily available treatments using mesenchymal stem cells, there are many clinical trials underway. For example within Australia there are clinical trials investigating the use of mesenchymal stem cells to treat osteoarthritis and ligament damage in the knee.

What about other clinical trials using tissue specific or adult stem cells?

There are many clinical trials ongoing both in Australia and overseas using different types of tissue specific (adult) stem cells for many different diseases and injuries. For example, researchers at the University of New South Wales are currently conducting a clinical trial using stem cells cultured on a contact lens to restore sight to sufferers of blinding corneal disease (read more about this clinical trial here). A high profile overseas clinical trial that has recently commenced in Scotland is testing neural stem cells of foetal origin (a type of tissue specific stem cell) in patients who have been left disabled following a stroke. The trial which is being run by UK based company ReNeuron will test the therapy in only 12 patients to see if it is safe and if there are some neurological benefits, with participants monitored for up to eight years (find out more about the trial on the US government clinical trials registry www.clinicaltrials.gov).

Are embryonic stem cells ready to be used in stem cell treatments?

Embryonic stem cells may have great potential for cell based therapies, but as they were only discovered in 1998 (a relatively short time ago in the field of medical research), there is much still to learn about these cells. Researchers are progressing carefully towards therapies as there are safety concerns associated with human ES cells. Pluripotency, the very feature that makes these cells so attractive to researchers, means they are capable of forming any type of cell in the body, including cancer cells. Cancer occurs when a cell loses control of its normal growth mechanisms. To overcome this problem, ES cells for therapeutic use would be differentiated to the point where they can only form the desired cell type, and should no longer have the capacity to form the wrong cell type or cancer cells. Researchers are also working to overcome another possible hurdle which is that human ES cells, like any donor cell or tissue, may trigger rejection by the patient's immune system.

The first clinical trial has now commenced using cells made from human ES cells to treat spinal cord injury. Californian based biotechnology company Geron, commenced a clinical trial in October 2010 with the treatment of its first patient with primitive neural cells made from human ES cells injected into the region surrounding a recent spinal cord injury. Geron's early stage trial will only treat 10 patients and is primarily testing to see if the cells are safe.

Before Geron could commence its clinical trial, there were significant and appropriate actions the company had to clear with USA regulator, the Food and Drug Administration (FDA). As this is the first ever trial involving human ES cell derived cells, the FDA were understandably cautious and in the interest of patient safety Geron's final approved submission included no less than 28,000 pages of details of Geron's experiments in animals, their processes and follow up procedures (this study can also be found on www.clinicaltrials.gov).

What about products and treatments that claim to stimulate my own stem cells?

There is a growing trend towards marketing products that providers claim will stimulate a patient's own stem cells. A current example is bovine colostrum, but there are many others including a growing number of anti-ageing and beauty products. These products do not contain any cells, but are still marketed as 'stem cell treatments'. This is misleading, and there is usually no peer reviewed scientific evidence to back the claims of those who report that applying, ingesting or even injecting these products will do anything to your stem cells.

We advise individuals to be very wary of buying products that make these sorts of claims. They are in general very expensive, and are not backed by any safety data or validated data for their effectiveness.

5 How does research become medicine? How do I understand what is proven and what is unproven or experimental?

What are stem cell treatments?

For the purposes of this Handbook, stem cell treatments are either injections or transplantations of cells termed 'stem cells'. Stem cell treatments can be divided into three broad categories:

1. **Standard practice stem cell treatments**. These are currently available, widely accepted, clinically proven, regulated, non-experimental treatments.

2. **Investigational treatments**. Where the benefit is not yet proven. These comprise either treatment as part of a clinical trial, or one-off or limited-access treatments termed 'medical innovations' performed under the supervision of a recognised institution.

3. **Unproven treatments.** Where the benefit is not proven and where the treatment is not part of a clinical trial or recognised medical innovation.

A treatment or therapy is 'proven' when it has been approved by appropriate government regulatory bodies. In Australia this would be the Therapeutic Goods Administration (TGA). Approval is given when extensive testing has demonstrated that the treatment is safe or has an acceptable risk to benefit ratio.

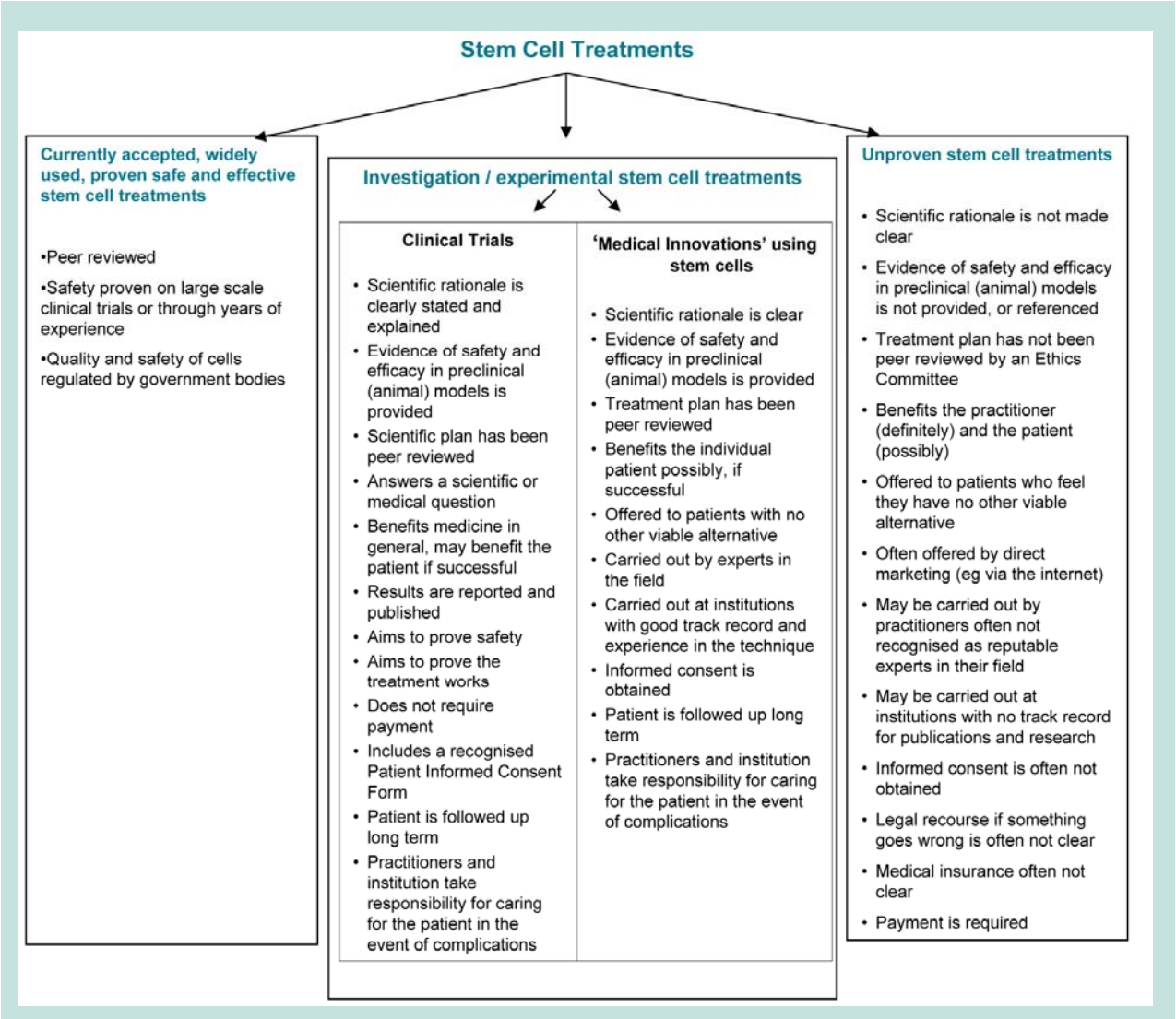
Testimonials from patients who have undergone a particular treatment are not medical proof that a treatment works.

How do I recognise if a treatment I'm considering is standard practice, investigational or unproven?

The only area of medicine in which stem cell therapy is standard and accepted practice is in the area of haematopoietic stem cell transplantation usually via bone marrow transplantation. Bone marrow transplantation has been practised for over fifty years, and is crucial to the treatment of a number of disorders (see Figure 3 for a list of disorders).

This Handbook will assist you to tell the difference between the three treatment categories when assessing a potential stem cell therapy. Figure 4 provides an overview of the categories.

Figure 4: Overview of Stem Cell Treatments



What is involved in an investigational stem cell treatment?

Investigational stem cell treatments are always offered either as part of **clinical trial** or as a **medical innovation**.

Clinical trials are undertaken in order to answer a medical and scientific question. The idea is usually to determine whether a new treatment is safe and effective, and then to publish the results in a peer reviewed journal so that the broader scientific community and patient community can benefit from this knowledge. Peer review is when a therapy or treatment is independently assessed by expert professionals in the field to validate its safety and efficacy measures.

Clinical trials must be evaluated and approved from a scientific perspective by a clinical research committee made up of scientific peers, and by an Ethics Committee made up of a range of people including scientific peers, general public and sometimes clergy. Recently, there has been a requirement that all clinical trials must be listed on a recognised registry so that the international community is aware of trials being run at other sites.

Detailed information on clinical trials and registries can be found in Chapter 6

Medical innovations using stem cells are stem cell treatments recognised by the following features:

- these treatments are offered as one-off or special access treatments in a facility with extensive experience in the technique and the scientific background
- the treatment is experimental but there is good scientific evidence that it may benefit the individual patient.

Medical innovations are an important way in which novel treatments can be attempted but there are strict requirements, including:

- there must be stringent peer review by recognised experts in the field who do not have a vested interest in the treatment
- the practitioner must have submitted a written plan to a peer review committee, such as an Innovations Committee, including the scientific rationale and preclinical evidence that the proposed treatment will be safe and effective
- the practitioner must provide a full description of the type of cells used, how they will be collected, processed and stored, and how they will be administered to the patient
- there must be a description of how the patient will be followed up after their treatment, and what contingencies are in place if anything should go wrong
- the research plan should include a plan for publication of results in a peer reviewed journal
- the patient or guardian must provide full informed consent to the satisfaction of the review committee.

A theoretical case study illustrating this type of treatment is outlined in Figure 5.

Figure 5: Case Study of a Medical Innovation

A case study of a 'medically innovative' stem cell treatment

- A baby is born very prematurely at 27 weeks instead of the normal 40 weeks. The baby is facing life threatening problems due to her immature lungs. She is in the neonatal intensive care but is not responding to usual treatments, and has a high probability of dying of this disease.
- A team of doctors and researchers at the baby's hospital have been working on a method of treating this disorder using adult stem cells, in an animal model. The stem cell method has been successful in animal experiments but has never been tried in humans. The doctors put a proposal to the hospital's Innovative Treatments Committee and Ethics Committee, outlining the evidence in the animal model, and the plan for how they would perform the same procedure for the baby.
- The Committees' review the evidence, the supporting literature and the track record of the treating team and decide to approve the experimental treatment for this individual baby. The baby's parents are given a detailed informed consent form which explains that this is an experimental treatment, and outlines the risks of death or other complications from the treatment. The parents decide to consent to this treatment as they feel their baby has no other option, and is very likely to die in her current situation.
- The treatment is given, and is successful. The treating team write up the results of the treatment as a case report in a medical journal. The journal editor sends the draft of the publication to a number of internationally recognised and respected experts. The experts review the draft and the supporting evidence, and approve the draft for publication in a well respected medical journal.
- This provides proof-of-principle for a formal clinical trial to be proposed at the same hospital, this time treating a group of babies with the same disorder.

Unproven Stem Cell Treatments

Unproven stem cell treatments are rare within Australia as they are restricted by the regulatory requirements of the TGA. Within Australia, like independent regulatory organisations in other countries, the TGA serves to ensure new drugs, therapies and devices are rigorously investigated and assessed to ensure they are safe and they work. New treatments and products must go through this process before being released in the Australian market. This is to protect patients against exposure to significant risks without good scientific rationale and good medical care. Treatments offered in some overseas clinics may not have been assessed by an equivalent independent regulatory organisation in their country.

What is medical travel?

Medical travel (also known as medical tourism, health tourism or global healthcare) is a not a new phenomenon but it is becoming increasingly commonplace. Medical travel is when a patient chooses to seek treatment in another country, either for cost or availability reasons. Virtually every type of health care including plastic surgery, orthopaedic surgery, reproductive treatments, psychiatry, alternative treatments, convalescent care and dentistry are available. Some medical travel is simply a means of getting access to a widely accepted treatment at a cheaper price, or for unproven treatments generally not offered in a patient's home country. Many patients opting for these treatments do so because they feel they have no other alternative treatments available.

Why does medical travel for unproven stem cell treatments exist?

Unlike many other forms of medical travel, the treatments offered overseas utilising stem cells are generally not proven treatments. The potential of stem cells to radically improve ways to treat diseases, many of which currently have no treatments or cures, has inspired significant investment and interest in this growing field of medical research. The sources of stem cells and the high level of hope that patients and researchers invest in the science to one day find a cure for many currently incurable diseases, means that stem cell science has attracted an unprecedented level of media coverage and attention for what is still early stage research. While stem cell research is advancing, the progress of research is much slower than often implied in the media.

As the profile of stem cell science grows, so does the proliferation of clinics offering stem cell treatments in many countries around the world. Providers of stem cell treatments vary widely in their assertions about the conditions that can be treated, the degree of improvement and the cell types and methodology used.

Why is the scientific and medical community worried about medical travel for unproven stem cell treatments?

The scientific and medical community is concerned that these treatments are being offered to patients before they have been proven safe and effective. Stem cell treatments, like any treatment, need to be proven safe and effective before they are accepted into practice.

Regulatory bodies in many countries like Australia, New Zealand, the USA and the UK place high expectations on the quality of medical research to prove safety and effectiveness. Scientists are expected to test their proposed treatment rigorously in animal models, and then in properly run clinical trials as discussed previously. In some overseas cases of unproven stem cell treatments, the host country has less regulatory or legal oversight than the patient's home country. Without a formal system for medical negligence claims in many of these countries, there are few legal options for a patient to seek reimbursement or even to have a legal hearing if something was to go wrong during their treatment.

Other concerns centre on the stem cell treatments themselves. These cells need to be stored and handled very carefully so as to avoid contamination and to ensure purity. For example, if

you were to undergo a blood transfusion in Australia, you would know that the blood being used has been screened for blood borne diseases and stored in the appropriately safe manner before the transfusion. The same principles may not apply to stem cell treatments overseas.

6 Clinical trials explained

What is a clinical trial?

Clinical trials are where new treatments, drugs and devices are tested in volunteer patients, to see whether they are safe and effective. Clinical trial research is conducted by experienced medical staff under experimental conditions. All clinical trials must be approved by an independent Ethics Committee that monitors the conduct of the trial and be conducted within the guidelines set out by the TGA. The TGA's website outlines the regulation and processes that must be followed - www.tga.gov.au/ct/index.htm.

All pharmaceutical treatments used in society today, had to be proven effective and safe in clinical trials before they could be made available for widespread use within the community. A high quality clinical trial will be one which the proposed treatment has undergone extensive prior investigation in the laboratory and in animal studies, and will have shown a strong repeatable desired effect.

A clinical trial is generally made up of four levels or phases, which must be passed, before the product or treatment is able to gain regulatory approval.

- **Phase I** – is the first testing of a new drug, treatment or clinical device on a small group of people (20-80) in an attempt to evaluate **safety**. Phase I research studies can include drugs or treatments that have been tested in animals but never in humans. These trials are usually first conducted with 'healthy' volunteers (this is someone with no pre-existing medical condition).
- **Phase II** - generally involves a larger group of people (several hundred) to further evaluate safety and **explore** the **efficacy** of the intervention. This involves one group of patients receiving the experimental drug, while a second 'control' group will receive a standard treatment or placebo (drug containing no active ingredient). Often these studies are double blinded, this is where neither the patients nor the researchers know who is receiving the experimental drug, or who is receiving the placebo. This is so that the study can provide a comparison between the relative safety and effectiveness of the experimental drug.
- **Phase III** – continues to **investigate** the **efficacy** of the intervention in larger groups of people (up to several thousand) by comparing against other similar interventions while monitoring for undesired effects. Once a Phase III study is successfully completed, it can be requested that the drug be approved for availability to the general public.
- **Phase IV** – once the intervention has been marketed, further studies are performed to **monitor** effectiveness and collect information regarding undesired effects. Late Phase III/Phase IV studies often compare an investigational drug with other drugs already available on the market. This is called a 'bio-equivalence study'.

In Phase **III and IV** clinical trials, patients are usually separated into two separate groups; a control group and a trial group. The control group does not receive the new treatment or medicine and act as a means of comparison for the trial group. The trial group is the one chosen to test the effects of the new drug, treatment or clinical device. Neither the patient nor clinicians performing the clinical trial should know which group the patient is in. This is known as a double blind trial and is done to ensure there is no bias or placebo effect.

You can identify a clinical trial using the checklist provided in Figure 6.

Figure 6: Features of a Clinical Trial

Features of a clinical trial

- ❑ Identifies itself as a clinical trial testing an **experimental treatment.**
- ❑ Collects detailed data regarding safety and effectiveness of treatment.
- ❑ Data collection usually includes long term follow up of patients.
- ❑ Expected side effects are listed.
- ❑ Scientific methods and ethical issues have been reviewed by a recognised Ethics Committee.
- ❑ Extensive **Patient Informed Consent Form** which describes in lay terms:
  - scientific rationale of the trial
  - evidence that the treatment is effective in other patients or animal models
  - whether the trial involves treating different groups of patients with different treatments
  - who is running the trial
  - who is funding the trial
  - who stands to benefit from the trial
  - how the results will be published
  - how the trial is insured
  - what plans are in place, if anything goes wrong during the trial
  - how privacy is respected and maintained
  - what the patients' legal rights are if they consent to go on the trial.
- ❑ Patients do not pay to be involved in a clinical trial.

Why are clinical trials so important?

Clinical trials are important for a number of reasons. For the area of stem cell application it is important to know if a treatment is not only safe but also works. To comprehensively understand the workings of a new treatment for a human disease, it is necessary that it is tested on those the new treatment is intended to help.

Once data has been collected, the regulation of clinical trials must be approved by the TGA. The equivalent of the TGA in the United States of America is the Food and Drug Administration and in the United Kingdom, the Medicines and Healthcare Products Regulatory Agency.

Results from clinical trials can in turn lead to the development of new medicines and treatments for various diseases and conditions.

Without clinical trials it is impossible to fully understand if stem cells can act as an effective treatment for the condition it is being intended. Experimental treatments, even with evident successes, cannot be deemed worthy by the medical community until tested properly and scrutinised under peer review.

Ethics and clinical trials

In order for a clinical trial to be conducted ethically, it is essential that a number of considerations are taken into account. They are as follows:

- there is an obligation for researchers to explain all possible outcomes to patients including the risks involved, this is known as informed consent
- researchers should not receive payment for conducting a trial, likewise a patient should never be asked to pay for their participation in a trial
- trials should be specifically designed to produce useful conclusions
- the potential benefits derived from a trial should not be embellished upon.

What are the potential concerns with clinical trials?

There are a number of potential issues which can arise throughout and after the conclusion of a clinical trial for both the consistency of results and participating patients.

There are two confounding factors which may interfere with the efficacy of a clinical trial. The first is related to recovery whereby a person may recover spontaneously on his/her own accord and not as a result of the new treatment. The second is the placebo effect whereby after treatment the belief that one's state will improve leads the patient to report that it has.

In addition, there are possibilities that:

- there will be side effects from the new treatment or medicine
- the new intervention will not work altogether
- extensive visits will be required as part of the trial
- you will be a part of the control group who will not receive the trial treatment.

Sometimes patients who are part of this control group are offered the treatment after the conclusion of the clinical trial. Or, if you do happen to be a part of a clinical trial control group there is the possibility that you will need to participate in a second clinical trial, where yet again you may be selected into the control group.

My specialist doesn't seem to be aware of any trials available in Australia. How do I find out what is available?

It is nearly a universal requirement now that clinical trials are listed on a publicly accessible registry. These can be searched on the internet by going to the website and typing in the search terms that you are interested in.

Major clinical trials registry websites are:

- www.anzctr.org.au is the Australia and New Zealand Clinical Trials Registry and lists clinical trials in Australia and New Zealand
- The Queensland Clinical Trials Network (www.qctn.com.au) and Nucleus Network (www.nucleusnetwork.com.au) both coordinate Australian clinical trials and have lists on their websites an calls for volunteers for trials
- www.clinicaltrials.gov is maintained by the USA's National Institutes of Health and lists all federally and privately funded clinical trials, including many Australian trials
- www.cancer.gov/clinicaltrials/ is maintained by the USA's National Institutes of Health and lists clinical trials relating to cancer
- www.aidsinfo.nih.gov is also maintained by the USA's National Institutes of Health and lists clinical trials for HIV/AIDS

- https://spinalnetwork.org.au/node/27 by the Australia and New Zealand Spinal Cord Injury Network keeps up to date lists of spinal cord clinical trials in Australia, New Zealand and overseas
- https://trials.jdrf.org/patient/ is a new international site launched by the Juvenile Diabetes Research Foundation that includes a number of type 1 diabetes stem cell clinical trials
- www.controlled-trials.com/ This UK based site allows users to search, register and share information about randomised controlled trials. The website is overseen by an international advisory group.
- https://eudract.ema.europa.eu is the clinical trials database of the European Medicines Agency of the European Union.

There are also many other privately sponsored clinical trials websites run by individual pharmaceutical companies and research institutes but the trend recently has been to preferentially list in one of the more centralised registries listed above, in particular the NIH registry.

It is advisable to discuss any trials that you are considering with your treating specialist. Your specialist can then contact the Principal Investigator of the trial to find out more information and check your eligibility for the trial.

7 Umbilical cord blood banking in Australia

Blood from the umbilical cord is rich in stem cells and there is the option for people to donate umbilical cord blood to 'banks' for the future use of a patient in need.

Public umbilical cord blood banking

There is an extensive public umbilical cord blood banking system in place throughout Australia and the rest of the world. By donating your child's umbilical cord blood it can then be used for a potentially life saving transplant for a patient in need. A patient requiring a stem cell transplant would be treated with stem cells from the sample most closely matching their own tissue type. Along with this strong network, the process undertaken by the public banking system ensures rigorous screening procedures. There are now over 300,000 units registered worldwide for public use, increasing the chances that a suitable unit will be found when needed. There is no cost involved for the donation to a public umbilical cord blood bank.

Contacts for public umbilical cord blood banks in Australia

There are three major public umbilical cord blood banks in Australia which are funded by both the Australian and various state governments. Their contact details are as follows:

Melbourne
BMDI Cord Blood Bank
phone: +61 3 9345 5834
email: cord@cryptic.rch.unimelb.edu.au website: www.mcri.edu.au/pages/cordbloodbank/

Sydney
Sydney Cord Blood Bank Sydney Children's Hospital
phone: +61 2 9382 0371
email: enquiries@scbb.com.au website: www.sch.edu.au/departments/acbb

Brisbane
Queensland Cord Blood Bank
phone: +61 7 3163 8000
email: foundation@mater.org.au website: www.materfoundation.org.au/

A public cord blood bank is also under construction in Western Australia. For more information visit http://www.rotarywacordblood.org.au/

Private umbilical cord blood banking

There is also the option to store umbilical cord blood in private umbilical cord blood banks. Private umbilical cord blood collection and storage services are generally available across Australia for an upfront cost and yearly fees.

Unlike public umbilical cord blood banking, the umbilical cord blood stored in private banks can only be released for the exclusive use of the donor. Information on private banks can be found by asking your doctor or through an online search.

The **Royal Australian and New Zealand College of Obstetricians and Gynaecologists** (RANZCOG) have a statement regarding umbilical cord blood banking. They have different recommendations depending on your specific risk level for diseases that may require treatment involving umbilical cord blood in the future. This statement is available through the RANZCOG website at www.ranzcog.edu.au/publications/statements/C-obs18.pdf and should be discussed with your physician.

8 The laws governing stem cell research in Australia

Laws which regulate stem cell research differ markedly from country to country. Australia has a highly regulated yet permissive framework that allows research and ultimately clinical application using many different types of stem cells.

All Australian research that uses human tissues of any kind must be conducted under the highest ethical standards. This includes ES cells, tissue specific (adult) stem cells, iPS cells and ES cells created via somatic cell nuclear transfer. While some forms of stem cells are more widely accepted by the community, they all use donated human tissue and should be subject to the highest level of scrutiny. In Australia, all human research is governed by Australian law that establishes rights for participants and ethical oversight of the use of human tissues in research, and imposes general and specific responsibilities on researchers and institutions.

Is embryonic stem cell research legal in Australia?

Since 2002, Australian scientists have been **permitted** to use donated in vitro fertilisation (IVF) embryos in research. Under the Commonwealth legislation - *Research Involving Human Embryos Act 2002* – scientists can apply for a licence from the National Health and Medical Research Council (NHMRC) to use donated human IVF embryos for stem cell research or research to improve infertility treatments and IVF, provided that the embryos are no longer required for infertility treatment. Additional legislation was also introduced in 2002, the *Prohibition of Human Cloning Act 2002*, which made it illegal to create, or even attempt to create, a human using cloning technology. Australia was one of the first countries in the world to introduce laws to govern the use of human embryos in research.

In 2005 the Australian legislation was reviewed by an independent committee resulting in amending legislation - *Prohibition of Human Cloning for Reproduction and the Regulation of Human Embryo Research Amendment Act 2006* - which specifically allowed Australian researchers to apply for a licence to use somatic cell nuclear transfer technology (also known as therapeutic cloning, see ASCC Fact Sheet 4) for stem cell research within a strict set of criteria. The amending legislation also increased the penalties associated with any attempts to abuse this technology to clone humans, with reproductive cloning remaining specifically prohibited.

There is no legislative framework regulating the use of human stem cells (embryonic, iPS or tissue specific/adult) after they have been created. However, the use of human stem cells in

research must comply with relevant NHMRC guidelines and have institutional human research ethics committee approval. For more information, visit www.nhmrc.gov.au. In addition, the use of donated human tissues in research including foetal tissues is regulated by individual state based human tissue legislation.

In 2011 the legislation is again being reviewed. Details of the review can be found at https://legislationreview.nhmrc.gov.au/.

What about the use of stem cells in therapies? Is that legal?

Stem cell therapies such as bone marrow and umbilical cord blood transplants for the treatment of certain diseases such as leukaemia and other cancers have been available in Australia for many decades. Within Australia the Therapeutic Goods Administration, as part of the Australian Government Department of Health and Ageing, has responsibility for regulating any product for which therapeutic claims are made including medicines, medical devices, blood and tissues including cellular therapies. Stem cells are regulated by the TGA under its Biologicals Framework which sets out clear guidelines for the use of these cells in therapies. These guidelines exist to ensure that when medicines and therapies are made available to Australians they are of a high quality and they are safe.

Refer to Chapter 5 for an overview of the process of how research becomes medicine.

The ASCC's Fact Sheet 6: Ethics and Law of Stem Cell Research contains further information.

For information on international stem cell legislation and regulations see Global Regulation of Human Embryonic Stem Cell Research.

9 What to consider should you decide to travel for any medical treatment: some summary points

The following points contained within Figure 7 are for further consideration for those who are considering travelling overseas for experimental stem cell treatments.

Figure 7: What to consider before you decide to travel

- **Beware of patient testimonials and self proclaimed success rates.** These are unqualified and unreliable recommendations. The only sources of evidence that can be relied upon to confirm that a treatment is safe and effective are independent clinical trials approved by government regulators, peer review assessment in international scientific journals and replication of results by other laboratories.

- **Beware of the language barrier.** This is often reported as a problem by patients who have undergone treatment in overseas clinics. It is advisable that you have an interpreter at all times or you are personally fluent in the language, to ensure you understand all of the procedures and risks that you are consenting to and the rights you have, if the treatment does not meet your expectations or harms you in any way.

- **Find out about the risks.** Some of the experimental therapies on offer may pose significant health risks for patients including **infection**, **immune system rejection** and possibly **cancer** later in life. A key feature of ES cells is that they are undifferentiated and pluripotent. They can become any cell in the body including cancerous cells. ES cells introduced to a patient, in an undifferentiated state, may become tumours or the wrong cell type.

- **Beware of a lack of long term follow up.** Many treatments do not have long term studies with appropriate control studies to verify the effects. Little information is available regarding the long term monitoring of patients involved in unproven stem cell treatments. This may be an indication that these treatments have not been given regulatory approval.

- **Beware of the hidden financial costs.** The providers charge a considerable sum of money for these untested treatments. The cost of treatment is not the only expense which should be considered when considering overseas treatment. One should also consider other expenses such as the airfare, travel insurance, food, transportation, medication and physical therapy costs.

- **Beware of a lack of scientific rationale.** Treatments available overseas are highly experimental with little understanding exactly how effects are achieved.

- **Beware of cells from another person.** Stem cells from another person, when introduced to a patient are likely to be rejected if they are not immunologically matched. Patients may be instructed to take immunosuppressant drugs, which suppress the immune system, prior to treatment. Suppression of the immune system makes patients susceptible to disease and infection.

- **Consider your insurance status.** Travel insurance generally may not cover you for a pre-existing condition. You should check your policy thoroughly with your insurer before travelling.

- **Find out where the cells come from.** Sources of cells can be aborted foetal tissues, cord blood, ES cells or adult stem cells. Knowing the source of the stem cells or tissues being used in the treatment is vital. If the cells are purchased, this may be illegal or unethical in that country. The purchase of cells in Australia is certainly illegal and unethical. It is important to know if the cells are purchased or imported from another country. Human tissues have to be handled and declared when being imported. Transportation of these cells and tissues requires special handling and can only be done by qualified couriers. Cells and tissues may need to be quarantined, depending on where they originate from.

- **Find out about viral screening.** It is important to know if the cells used in the therapy have been thoroughly screened for HIV, hepatitis, West Nile Virus and other forms of blood borne diseases, even if the cells are your own (autologous). Viruses can be transmitted during culture or storage in close proximity to cells from other patients.

- **Find out about bovine products.** The handling and storage of cells is very important and you should check that the cells have not been processed using any bovine materials as this can carry a risk of variant Creutzfeldt-Jakob disease (mad cow disease). Cells should be stored and handled in high grade clinical facilities accredited for current clinical grade good manufacturing standards (cGMP). This is an international standard that ensures the cells are not exposed to contaminants.

- **Beware of practitioners who cannot or will not publish their work.** Some claim to be protecting patents, others may claim that journal editors discriminate against them, preventing them publishing. Scientific evidence to support the claims by a number of stem cell clinics in China, India, Central America and some countries in Europe is yet to be published in peer reviewed journals. Many of these practitioners guard their process tightly which is contrary to the mainstream scientific premise of peer review.

10 More information

We hope that by reading the ASCC Patient Handbook and discussing it with your doctor, you will have an informed opinion about a number of key areas involving stem cells. Before making any important decision, the more information you are armed with the better. Here is a list of websites and other resources you may find helpful:

Australian Stem Cell Centre
The ASCC's website contains detailed fact sheets and information on stem cell research in Australia and links to international stem cell groups. For the latest updates in the stem cell field the ASCC also produces a newsletter on international news and breakthroughs.
www.stemcellcentre.edu.au

International Society for Stem Cell Research (ISSCR) - Closer Look at Stem Cell Treatments & Patient Handbook
Closer Look at Stem Cells is a website that aims to assist patients to understand what is currently possible and evaluate the claims made by the providers of these treatments. The website also contains a link to the ISSCR Patient Handbook.
http://www.closerlookatstemcells.org

Spinal Cord Injury Network, Australia New Zealand
The Spinal Cord Injury Network has a detailed website including information on clinical trials and a position statement on Stem Cell Interventions for Spinal Cord Injury.
https://spinalnetwork.org.au/

MS Society UK
The MS Society UK have joined with other patient groups to publish 'I've got nothing to lose by trying it' a booklet which explains how to tell the beneficial from the bogus in the face of miracle cure stories, new wonder drugs and breakthrough therapies that are increasingly promoted in the media and elsewhere.
http://www.mssociety.org.uk/news_events/news/press_releases/sense.html

The MS Society UK has also published Stem Cell Therapies in MS which is available from the MS Society Queensland.
http://www.msqld.org/pdf/stem_cell_therapies_in_MS.pdf

Medicines Australia – Clinical Trials
Provides information on clinical trials, why they are important and how to participate in one.
www.medicinesaustralia.com.au/pages/page39.asp

Stem Cell Pioneers
Stem Cell Pioneers is an independent, moderated blog. It is dedicated to the discussion of all kinds of stem cell treatments for all types of diseases. The information exchanged on the forum is based upon personal experiences and opinions of the users. It is not intended as a substitute for consulting with your own physician or other health care provider.
www.stemcellpioneers.com

Stem Cell Network, Canada
The Canadian Stem Cell Network has a comprehensive *For Patients* section on their website including over ten dedicated fact sheets on stem cell treatment in relation to particular diseases.
http://www.stemcellnetwork.ca

11 Glossary

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

Allogeneic transplantation. Cell, tissue or organ transplants from one individual to a genetically different person.

Autologous transplantation. Cell, tissue or organ transplants from one individual back into the same individual. Such transplants are often performed with blood products or bone marrow and do not induce an immune response and are not rejected.

Blastocyst. An early stage embryo about five-seven days post fertilisation containing about 150 cells and is the size of a pinhead. A blastocyst consists of two types of cells: the inner cell mass cells, from which embryonic stem cells are derived, gives rise to all the organs and tissues of a future embryo and foetus; and the trophoblast which forms a portion of the placenta.

Cell culture. The growth of cells in a controlled environment and in an artificial solution that provides nutrients and growth factors.

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

Cell based therapies. A treatment that involves stem cells being induced to differentiate, or develop, into specific cell types required to repair or rebuild depleted cell populations or tissues.

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

Efficacy. Efficacy is the capacity to produce an effect.

Embryo. The conceptus(?) developed from the fertilised egg (zygote) until it becomes a foetus, which in the human, is approximately eight weeks later.

Embryonic stem cells. Also known as ES cells, derived from the blastocyst. ES cells are self-renewing (can replicate themselves) and have the potential to differentiate into most cell types in the body.

Foetus. The conceptus(?) that follows the embryo stage and develops till birth and displays the characteristics of the adult species.

HTLV-1 - Human T-Lymphotropic Virus Type I. HTLV-1 is a 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 cell or 'precursor' of mature blood cells and are found in adult bone marrow, umbilical cord blood, peripheral blood and foetal liver.

Induced pluripotent stem cell (iPS cell). Stem cells derived from mature/differentiated cells of the body by reprogramming through genetic manipulation, which resemble the pluripotent embryonic stem cells.

In vitro fertilisation (IVF). IVF, fertilisation achieved outside the body, is an assisted reproduction technique where the egg cell and the sperm cells are brought together in a dish (i.e. in vitro), so that the sperm can fertilise the egg. The fertilised egg, a zygote, will form the embryo which can then be implanted into the womb for establishing pregnancy.

Mesenchymal stem cell. A type of adult stem cell found in several tissues of the body including bone marrow and the placenta which can give rise to a number of tissue types such as bone, cartilage, fat tissue, and connective tissue. Mesenchymal stem cells have shown promise for treatment for a number of diseases

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

Peer review. Peer review is 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 the stem cell to develop into many types of cells in the body. ES and iPS cells are examples of pluripotent stem cells.

Progenitor cells. A progenitor cell is a transitional form of stem cell that can differentiate, but can no longer renew itself. Progenitor cells are restricted to the generation of a few types of specialised cells.

Regenerative medicine. A treatment in which stem cells are induced to differentiate into a specific cell type required to repair damaged tissues or to replenish a depleted cell population.

Somatic Cell Nuclear Transfer (SCNT). SCNT refers to the removal of a nucleus, which contains the genetic material or DNA, from virtually any cell of the body and its transfer by injection into an unfertilised egg (oocyte) from which the nucleus has also been removed. The newly reconstituted egg is then stimulated to start dividing. After five-seven days in culture, embryonic stem cells can then be removed. These embryonic stem cell lines are genetically identical to the cell from which the DNA was originally removed. To date, SCNT has not been achieved successfully in humans to create a human embryonic stem cell line.

Stem cell. An unspecialised/undifferentiated cell with the ability to renew indefinitely and to produce specialised cell types in the body.

Stem cell line. Stem cells that have been established and propagated in culture and maintained consistent characteristics and potential.

Australian Stem Cell Centre

Australian Stem Cell Centre

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Disclaimer

The information and reference materials contained in the Australian Stem Cell Centre Patient Handbook are intended solely for the general information of the reader. The Patient Handbook is not to be used for or relied upon as a basis for treatment purposes, but rather for discussion with the patient's own physician. The information presented here is not intended to take the place of professional medical care and advice. The information contained herein is neither intended to dictate what constitutes reasonable, appropriate or best care for any given health issue, nor is it intended to be used as a substitute for the independent judgement of a physician on any given health issue.

The information in the Patient Handbook is made available as a community service on the understanding that the Australian Stem Cell Centre and its employees and agents shall have no liability (including but not limited to liability by reason of negligence) to the users for any loss, damage, cost or expense incurred or arising by reason of any person using or relying on the information and whether caused by reason of any error, omission or misrepresentation in the information or otherwise. Information posted in the Patient Handbook, including all references and links, are not intended to be and must not be taken as advice. Users of the Patient Handbook will be responsible for making their own assessment of the information and should verify all relevant representations, statements and information with their own professional advisors.

References to any products, services, hypertext link to third parties or other information by trade name, trademark, supplier or otherwise does not constitute or imply endorsement, sponsorship or recommendation by the Australian Stem Cell Centre or its employees or agents.

Links to other sites are provided only as an information resource. These links are provided simply as a service, and it should not be implied that the Australian Stem Cell Centre recommends, endorses or approves of any of the content at the linked site(s), nor is the Australian Stem Cell Centre responsible for their availability accuracy or content.

The Australian Stem Cell Centre is grateful to all of the contributors and to those that have read all or part of the document and helped with specific points.