

The cover features a white background with a purple border. A central white rectangle with a purple border contains the title. The bottom right corner contains the UKSCF logo and tagline. The background is decorated with numerous circles of varying sizes and colors, including purple, black, and white, some with gradients.

STEM CELL THERAPIES HANDBOOK

UKSCF | UK STEM CELL
FOUNDATION

TOGETHER, WE CAN BE THE CHANGE

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INTRODUCTION

ARE YOU CONSIDERING TRAVELLING ABROAD FOR A TREATMENT OR CURE?

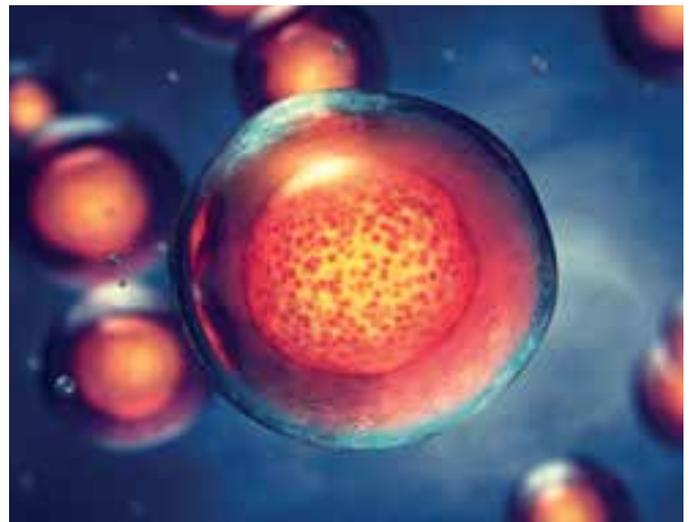
Stem cell therapy offers enormous potential in improving medical treatments for a wide range of debilitating diseases and conditions, including some that have no other hope of cure. However, in many cases, there is still a great deal of work to be done - research, testing and meeting regulatory standards - before this great promise is turned into the reality of safe and effective treatments that are available to all that need them.

Here at the UK Stem Cell Foundation (UKSCF) we are working hard to fund stem cell research and technology, selecting projects that we believe have the best chance of progressing towards real treatments and therapies for patients. Unlike most other funders, we only fund work that is close to being in the clinic and we do not fund more speculative 'blue sky' research. Funding clinical trials is costly and often takes years to complete. A new drug takes on average 12 years to reach the clinic and cell-based therapies are developed in just the same way and take as long.

While this important work is happening, we are receiving a very large number of communications from people who are turning to the internet in search of cures for untreatable diseases and conditions; cures that are not available in the UK, nor offered by the NHS.

We are aware that there are a number of overseas organisations that are offering treatments and, what they term 'cures' using stem cells, often making claims that these are 100% safe and effective. By creating this booklet, we are aiming to help those searching for a cure to make informed decisions about the

treatments being offered, and to highlight any associated dangers/risks. We are genuinely concerned that these treatments are often costly, offer false hope and could worsen a condition or endanger life.



“THIS IS A MAJOR CONCERN FOR US. PEOPLE ARE GAMBLING THEIR HEALTH, LIVES AND LIFE-SAVINGS ON STEM CELL TREATMENTS IN OVERSEAS COUNTRIES THAT DO NOT HAVE THE SAME ROBUST REGULATORY, LICENSING AND SAFETY CONTROLS AS THE UK.”

**UKSCF CHIEF EXECUTIVE,
LIL SHORTLAND**

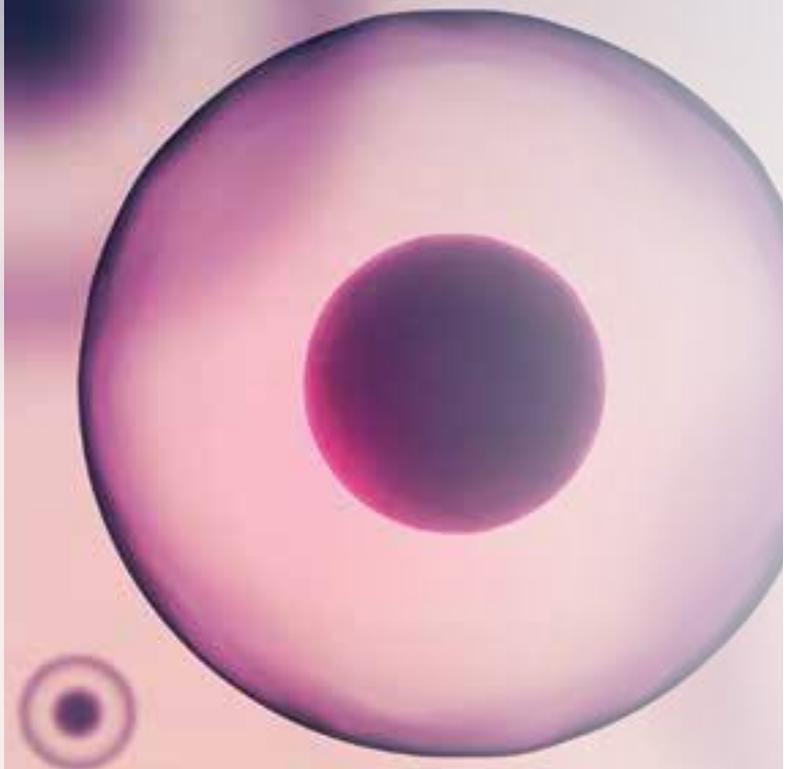


WHAT ARE STEM CELLS?

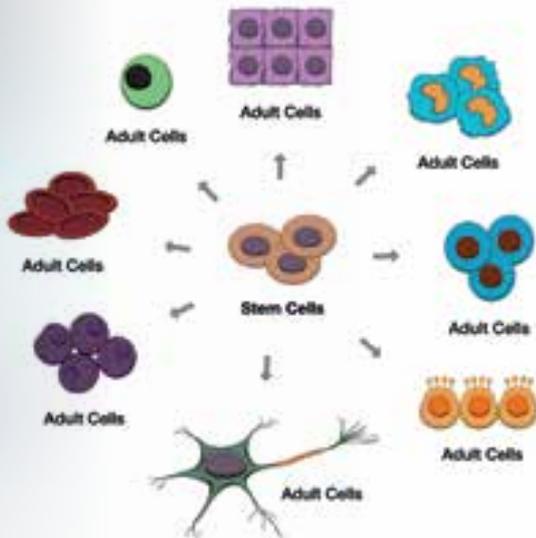
Stem cells are not 'one thing'. There exist different types of stem cell with different capabilities and different levels of difficulty associated with their collection. This means that there is no one way to use them. However, they do have some common characteristics. Stem cells are the human body's natural repair mechanism; they can 'self-renew' (producing more stem cells) and they can 'differentiate' (turn into specific types of cell). As a result, they offer huge therapeutic potential for treating a wide range of conditions where the body's own cells are damaged, missing or malfunctioning.

However, a lot of work is needed to turn them into safe and effective treatments and the amount of work will depend on the type of stem cell used and the tissue in the body being repaired. In the UK, there are many stages of assessment and testing required before the regulatory bodies allow their use in patients. It is important that they provide safe and reliable therapeutic benefit without causing severe side-effects.

Different regulations apply in other countries and, despite the need for further research, stem cell therapies are already being prematurely offered around the world. These treatments are not necessarily safe or effective. For this reason patients need to fully understand any potential risks before undergoing a stem cell therapy.



TYPES OF STEM CELLS

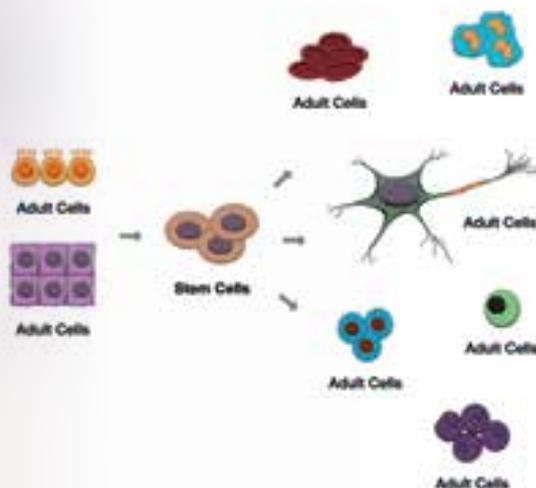
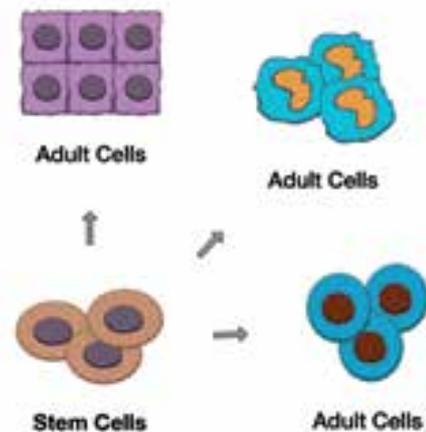


EMBRYONIC STEM CELLS

Are found in embryos at early stages of development (and can become any cell type in the body).

ADULT STEM CELLS

Are found in some tissues in the body and include mesenchymal stem cells (that demonstrate a limited potential for turning into other cell types).



INDUCED PLURIPOTENT STEM CELLS (IPS CELLS)

Are stem cells produced by “reprogramming” normal adult cells from the body so that they become essentially, embryonic type stem cells.

WHAT ARE STEM CELL THERAPIES?

These therapies use stem cells (or, more often, cells derived from stem cells) to repair or replace a patient's damaged cells. New cells can be delivered into the blood or directly to the affected tissue; sometimes stem cells are stimulated from within the patient's own tissue and sometimes they are delivered on a supportive material (scaffold).

Stem cell treatments are well established for only a small number of conditions, including disorders of the blood and immune system, and acquired loss of bone marrow function that can, sometimes, be effectively treated with transplantation of blood stem cells. This technique, which usually uses bone marrow cells, or umbilical cord blood, has been used for over 50 years.



At this time, other stem cell treatments are still experimental; that is, they are not yet fully proven to be safe or effective. Stem cell therapies still require research and investigation to solve issues such as; controlling the quality of cells for transplantation; determining the best type of stem cell to use; deciding on the best method of use for each disease; and ensuring the long-term safety of the treatment. It is worth pointing out that if you are given a drug and don't like your reaction to the drug you can stop taking it. However, when you are given living cells as a treatment, if you don't like your body's response it is more difficult to resolve. Hence, the testing of the stem cell therapies is very important indeed.

WHAT DO I NEED TO KNOW BEFORE UNDERTAKING STEM CELL THERAPY?

HOW DO I KNOW IF AN ADVERTISED CELL THERAPY IS SAFE AND EFFECTIVE?

Any therapy should be supported by good scientific evidence showing it to be safe and effective. For stem cell therapy, look for published and reviewed pre-clinical studies, approval of tests and trials from independent committees such as the Ethics Review Board (ERB) and, most importantly, approval from regulatory agencies such as the Food and Drug Administration (in the USA) or the European Medicines Agency (EMA).

No treatment is guaranteed to be without any side effects, even an approved cell therapy that has been thoroughly tested. However, approved therapies will have been shown to be associated with minimal levels of risk and any such risks associated with the treatment should always be fully explained to you beforehand. Evidence from a good, official, clinical trial should indicate the level of risk / safety associated with a therapy so always start there.

WHAT ARE THE BENEFITS OF AN APPROVED CELL THERAPY?

SHOULD I GET A SECOND OPINION?

It's always a good idea to get the answers to any questions you have. If you're asking for a second opinion, make sure the medical professional is independent of the therapy in question and is indeed a fully qualified medical professional (this can be checked on a 'medical register').

STEM CELL THERAPY CLINICAL TRIALS

Approved clinical treatment or an experimental intervention?

Check carefully that the treatment you are offered has been clinically approved, which means it has passed a 'clinical trial' process and is proven to be safe and effective at treating your disease or condition. Alternatively, you might be offered an experimental intervention (a clinical trial), whose safety and effectiveness has been considered but has not been fully tested in humans. In such a trial, safety is tested and careful monitoring of your response to the therapy is undertaken.

What is a clinical trial?

A clinical trial is a research study that answers a specific question, such as whether a treatment is safe or effective. Sometimes, a new treatment is tested on a small number of patients before it undergoes a larger-scale clinical trial. Clinical trials are categorised in phases as:

Phase I – often recruits only a small number of people to determine; how much therapy is safe to give, how the body copes with the therapy (are there any side effects) and if there are any early indications that the therapy has a positive effect on the clinical condition (but not enough evidence to prove it).

Phase II – often larger (perhaps 100 people) and determines whether the therapy works well enough to test in a much larger group of individuals (Phase III) and looks in more detail at the best dose and any side effects of the therapy.

Phase III – These are much larger trials and they often compare the new treatment to the very best existing treatment/solution that is available in order to show the benefit of the new treatment. Sometimes these trials involve thousands of people in different locations around the world. There can also be Phase IV trials that look in more detail at the treatment after it has already been approved.

Be aware that if you enter a clinical trial you might be allocated a place in a control group and not the treatment group. A control group is a group of individuals with the same clinical condition as yourself but who do not receive the treatment. This is to prove that any responses seen in those treated is due to the treatment and not coincidence. Being in a control group is just as good a contribution to the development of a new therapy as being in a treatment group but from a personal point of view you may have an opinion on this.

Also, be aware that even if a new treatment appears to work, the decision to make it available or not depends on several other factors that will result in a recommendation by an independent organisation called NICE (National Institute for Health & Care Excellence).

How can I find out about clinical trials that use stem cells?

UK Medical professionals will know what is available for a particular disease or condition. It is also worth contacting research institutions such as universities and databases such as www.clinicaltrials.gov / www.isrctn.com / www.clinicaltrialsregister.eu / www.ukctg.nihr.ac.uk/trials

What is an 'informed consent form' or 'treatment consent form'?

You may be asked to sign a form before undergoing treatment, particularly if it is part of a clinical trial or a research project. The form must outline the aim of the research, the structure of the trial, the role of the patient and any potential risks. It should also state who will perform the study and how long it will last. Information should be provided in simple, clear language; if there is anything you do not understand you are strongly recommended to find the answers to your questions before signing the form.



SEVEN REASONS TO BE CAUTIOUS ABOUT A STEM CELL THERAPY

1. Testimonials from other patients may be unreliable; desperation for a treatment to be effective sometimes leads to unclear judgement about the recovery, which is incorrectly attributed to the stem cell therapy.
2. A single type of stem cell is unlikely to cure a range of different diseases; any such claims should not be taken seriously.
3. The source of the stem cells and the exact nature of the treatment should always be stated; if not, you should seek clarity on these matters.
4. Any treatment claiming to be 'zero risk' should not be trusted; all therapies involve some risk, and this should be made clear to the patient.
5. When cells are placed into your body it is difficult to remove them if something goes wrong. They must be tested to be safe and effective.
6. These are a new and exciting 'breed' of therapy and most legitimate therapies are still in the research and development stage not the 'ready for use in humans' stage.
7. We believe that a patient should never pay to take part in a clinical trial; be wary of any costs, hidden or otherwise.

STEM CELL THERAPY - CHECKLIST

If you are considering undergoing stem cell therapy, we recommend using this checklist to ensure you understand the issues, procedures and associated risks before you begin.

I know what stem cells are and I understand that there are different types of stem cell with different uses.

I realise that stem cell therapy uses stem cells (or cells derived from stem cells) to replace or repair damaged tissue.

I know whether stem cell therapy is well established for my condition (or whether it is still experimental and unproven).

I understand the procedures involved in my therapy (i.e. the type of stem cell used, the method by which it is delivered, and the aftercare that is recommended).

I know that my approved stem cell therapy has undergone the correct process during its development (i.e. general research, pre-clinical studies (in vitro and in animals), clinical trials, and approval by a national or regional regulatory agency, e.g. FDA or EMEA). Alternatively, that the therapy is part of an approved and regulated, formal clinical trial.

I understand that if the therapy is experimental (non-approved), that the safety and effectiveness is not proven.

I understand that if the therapy is part of a clinical trial, that the safety and effectiveness is not proven and, by taking part, I might experience side-effects.

If I agree to take part in a clinical trial, I know that I need to understand and sign an 'Informed Consent Form' or 'Treatment Consent Form', which includes extensive information about the trial.

I am aware that no medical treatment is 100% safe, and all the risks have been explained to me. These include any immediate risks that could arise, and any possible long-term side effects that can be expected.

I understand what would happen if the procedure gives rise to an adverse reaction. For example, what medical care would be provided, what the cost of this additional care would be, and what costs would be incurred as a result of an extended stay in a foreign country.

I am satisfied that the clinic undertaking the treatment is equipped to deal with medical emergencies (if something should go wrong).

I understand how any follow-on treatment would be given and how would this be provided if the patient was travelling from abroad.

I understand that having an unlicensed/unapproved cell therapy could preclude future treatment in the patient's country of origin. (For example, would the NHS treat me after any experimental intervention received abroad?)

I have investigated what would be covered by travel and health insurance providers when travelling overseas for treatment.

I have investigated and understand my rights while taking part in treatment. For example, whether I am allowed to withdraw from the treatment at any time and, if so, whether this would incur additional costs. I also understand what would happen if I was injured as a result of participating in treatment.

I have answers to all the questions I have about the treatment.

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TOGETHER, WE CAN BE THE CHANGE

Funding the development of
innovative cell-based treatments
for clinical applications