

# Stem Cell Treatment Briefing Document and Position Paper

## Background

### **What is stem cell treatment for Multiple Sclerosis – what does it involve and how does it work?**

Stem cells are a type of cell that have the potential to transform into many other types of 'specialised' cells that perform specific functions in the body – like muscle, nerve or bone cells. Stem cells have been investigated by scientists as potential treatments for various diseases, including heart disease, diabetes, and stroke.

There are currently three main types of stem cell treatments for MS under investigation. They are:

1. **Autologous Hematopoietic Stem Cell Transplantation (AH SCT)** – this type of stem cell therapy was featured in a BBC Panorama documentary in January 2016 and generated a large amount of media attention at the time. It involves extracting the person's stem cells from their bone marrow or blood, giving them chemotherapy to wipe out their existing immune system and then reintroducing the stem cells to regrow or 'reset' their immune system. There has been an increasing recognition of AH SCT as standard of care for people with highly active MS which is not controlled on currently available disease-modifying therapies (DMT's).
2. **Mesenchymal Stem Cell Transplantation (MSCT)** is an experimental therapy which is currently undergoing clinical trials. It involves a type of stem cell that is usually taken from bone marrow, skin and fat tissue. Some small-scale trials have suggested they can help stimulate re-myelination of nerve cells
3. **Repairing myelin damage** – some scientists are investigating specialist stem cells in the brain that can generate myelin-producing cells

### **How effective has stem cell treatment been found to be for people with MS?**

Results from clinical trials into AH SCT have been promising for some people with MS – the most effective results have been for people with aggressive forms of relapsing-remitting MS, some of whom did experience a considerable improvement in disease activity. So far, the treatment does not appear to be able to repair damage that has already been done, and so it is unlikely to be effective for progressive forms of MS.

**It is important to say that this treatment does not cure MS.**

There have also been some promising long-term follow up studies released which show that effectiveness of stem cell treatment may be maintained over time. In one study, released in February 2017, 69% of people treated with AH SCT continued to show no new relapses, no new lesions and no progression after five years. However, evidence is still needed from controlled,

randomised clinical trials directly comparing AHST against other treatments in both relapsing remitting and progressive MS. Also, longer term follow up is required in studies to see what happens to people a decade or more post treatment. It is known that up to 11% of people with MS will need to restart a disease modifying treatment due to re-emergence of disease activity.

## **What are the potential risks of aHST treatment?**

Studies from 2001 indicated treatment-related deaths at about 1.3% (one or two people in every hundred) for AHST. Looking at transplants carried out since 2005, the mortality rate has decreased to approximately 0.3%. In the 2019 MIST study, there were no deaths or life-changing adverse reactions reported.

There is no standard regime for the use of chemotherapy before aHST takes place. The reason for this is that no clinical trials have compared the different regimes. The result is that the protocol can be different from one site to another.

There are several potential side effects both during and after AHST which include:

Increased risk of infection following the chemotherapy given at the beginning to silence your own bone marrow (conditioning chemotherapy). You would remain immunocompromised until your immune system rebuilds itself following the stem cell transplantation stage. Studies have also shown that there is an increased loss of brain volume after this procedure and is thought to relate to the use of chemotherapy. Close monitoring by your healthcare team would be required as well as the possibility of an isolation room in hospital for several weeks in order to try protect you.

Previous infections such as the viruses that cause shingles, cold sores and herpes – including Epstein Barr virus - can become reactivated. The most common cause of treatment failure is reactivation of Epstein Barr virus.

Other autoimmune conditions such as autoimmune thyroiditis (where the immune system sees the thyroid and the hormones it produces as threats and attaches them) may develop.

You may also experience side effects from chemotherapy, which can include, fatigue, weakness and temporary loss of appetite. It can also increase your risk of bleeding or bruising and your MS symptoms may become worse for some time. Hair loss is common but should begin to grow back within 1 – 6 months. Chemotherapy component are toxic to ovaries and testicles, which can result in high levels of infertility or early menopause.

## **Risks of mesenchymal stem cells**

MSCT does not involve chemotherapy and as such carries a lower risk, but as this treatment is still in the early stages of clinical trials it is probably too early to conclusively rule out other risks and complications.

## **Are stem cell treatments currently available in Ireland?**

**Mesenchymal stem cell treatments for MS** are still largely considered experimental and as such, the safest way to access the treatment is via a licensed clinical trial. There are currently no clinical trials being carried out into stem cell treatment in Ireland, as the country does not have a large enough

population to support a trial. A list of trials taking place internationally can be found at [www.clinicaltrials.gov/](http://www.clinicaltrials.gov/) using the search term “multiple sclerosis AND stem cells”.

While **AHSCT** is not currently provided in Ireland, some people from Ireland have been put forward by their consultant neurologist as a candidate, and have received AHSCT in the UK. This treatment is available in a small number of centres under certain circumstances for a small number of people. To start with this process involves of a person’s case at the Irish Multidisciplinary meeting for AHSCT before a referral can be accepted in the UK. The treatment can be funded by the Irish system.

## **MS Ireland’s position on stem cell treatment**

MS Ireland welcomes progress in stem cell treatment for people with MS. While AHSCT is being increasingly seen as a standard, MS Ireland feel that additional clinical and safety data regarding the various types of stem cell would be beneficial. In addition future treatments that are in a very early stage of development, that include cellular therapy, may potentially provide a benefit beyond the use of stem cells.

MS Ireland believes that research is the most beneficial way to improve understanding of the disease, leading to better treatments, interventions, management and ultimately a cure for MS. MS Ireland would like to see greater investment in Ireland in all types of scientific research into MS and improvement of the infrastructures that allow such research to take place, such as the development of a national register. This will enable us to advocate for service provision at a local level based on the number of people. It would also allow researchers to contact a wider number of people to increase the power of studies that are taking place.

## **Information for those who are thinking of travelling to seek stem cell treatment**

***MS Ireland strongly advises anyone who is considering travelling abroad to seek stem cell treatment to speak to their neurologist.***

### **Safety Considerations**

As outlined above, such treatments can potentially carry a high level of risk and they will not be suitable for everyone with MS. In particular, they are unlikely to benefit patients with progressive forms of MS.

### **Clinical trials**

Clinical trials are always very oversubscribed and hard to get access to.

### **Private clinic access**

Some organisations are offering access to stem cell treatment commercially via the internet, whereby individuals are expected to pay for the therapy. Costs vary from between £30,000 to £85,000. **MS Ireland advises that these centres should be avoided.**

Unless the programme is part of a clinical trial, it may be operating with lower standards of safety than would be required in authorised trials. In addition adequate follow up and monitoring is often not provided by centres that are not running a clinical trial program.

***It is essential that local specialists such as haematologists need to be consulted with and involved before someone goes for a transplant and for a period of monitoring after the transplant. Some people who privately seek stem cell therapy do not have this in place and it is a serious safety concern.***

In addition to speaking to their neurologist beforehand, anyone who does wish to travel abroad for stem cell treatment should give careful consideration to the following when looking for a treatment centre:

- Exactly what kind of treatment is provided – is it AH SCT or something else?
- Whether the clinic is regulated to international standards
- Whether others have benefited from treatment at the clinic
- What follow up is provided
- How safety, side effects and the effectiveness of treatment are monitored over time
- What is, and is not, included in the price quoted and what you will need to pay for in addition to flights and possibly hotel accommodation, for example whether there will be any additional medications that you have to pay for once you're home. The risk of having additional costs may be high if you develop complications after the initial treatment.

**It will also be essential to make sure that there will be proper follow up and support in place upon return to Ireland.**

## **References and further reading**

Information for this document has been taken and adapted from the following websites:

[www.mstrust.org.uk/a-z/stem-cell-therapy](http://www.mstrust.org.uk/a-z/stem-cell-therapy) [www.clinicaltrials.gov/ct2/show/NCT00273364](http://www.clinicaltrials.gov/ct2/show/NCT00273364)  
[stemcells.nih.gov/info/basics/pages/basics1.aspx](http://stemcells.nih.gov/info/basics/pages/basics1.aspx)

[www.mssociety.org.uk/ms-research/research-blog/2016/01/7-things-you-need-know-about-ahsct?utm\\_source=Facebook&utm\\_medium=link&utm\\_campaign=panorama\\_ahsct&utm\\_content=s even\\_things\\_blog](http://www.mssociety.org.uk/ms-research/research-blog/2016/01/7-things-you-need-know-about-ahsct?utm_source=Facebook&utm_medium=link&utm_campaign=panorama_ahsct&utm_content=s%20even%20things%20blog)

[www.mssociety.org.uk/ms-research/emerging-areas/stem-cells](http://www.mssociety.org.uk/ms-research/emerging-areas/stem-cells)

<https://jnnp.bmj.com/content/early/2023/08/14/jnnp-2023-331864.long>

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