

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. Stem cells have been investigated by scientists as potential treatments for various diseases, including heart disease, diabetes and stroke.

There are currently 3 main types of stem cell treatments being investigated as potential therapies for Multiple Sclerosis. They are:

- **Autologous Hematopoietic Stem Cell Transplantation (AH SCT)** – this type of stem cell therapy was featured in a BBC Panorama documentary in January 2016, and was described as a potential 'cure' for Multiple Sclerosis by the media, although clinicians and other expert groups advised caution. 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. An international clinical trial into AH SCT is currently ongoing, with centres in the UK involved. This trial is called the MIST trial
- **Mesenchymal Stem Cell Transplantation (MSCT)** – this 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
- **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?

Recent 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 and disability levels. 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.

There have not yet been any long-term follow-up studies carried out into the effectiveness of AH SCT, therefore we still do not know conclusively if those who have benefitted from AH SCT will continue to do so over time.

The other two types of potential stem cell treatment described above are currently still in very early stages of investigation. Phase 1 clinical trials of MSCT indicate that this might be a useful treatment for people with MS.

What are the potential risks of stem cell treatment?

AHSCT carries a 1.3% mortality rate, which makes it higher risk than other current forms of MS treatment. MSCT does not involve chemotherapy and as such carries a lower risk, but as this treatment is only at phase 1 of clinical trials it is probably too early to conclusively rule out other risks and complications.

Are stem cell treatments currently available in Ireland?

Stem cell treatments for MS are still at the experimental stage and as such, the only safe 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.

MS Ireland's position on stem cell treatment

MS Ireland welcomes progress in stem cell treatment for people with MS. However, a lot more clinical and safety data is required before stem cell treatment can be recommended as a mainstream treatment for people with MS.

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 patient registries.

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. 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, such as the one investigating AHSCT at Sheffield University in the UK, are always very oversubscribed and hard to get access to. 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.

Access to Medicines

On a broader basis, MS Ireland continues to advocate for people with MS in Ireland to be able to access the medicines they need to treat their condition and impact debilitating symptoms. The

'Access to Medicines' campaign handbook, launched in August 2015, provides practical advice for people with MS on their medical rights and options as well as how to engage with the health system.

References and further reading

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

<https://www.clinicaltrials.gov/ct2/show/NCT00273364>

<http://stemcells.nih.gov/info/basics/pages/basics1.aspx>

https://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

<https://www.mssociety.org.uk/ms-research/emerging-areas/stem-cells>

<https://www.mstrust.org.uk/a-z/stem-cell-therapy>

MS Ireland's 'Access to Medicines' campaign handbook can be accessed here: <http://www.ms-society.ie/pages/living-with-ms/information-centre/our-publications>

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