

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 three main types of stem cell treatments being investigated as potential therapies for MS. They are:

- **Autologous Hematopoietic Stem Cell Transplantation (AHST)** – this type of stem cell therapy was featured in a BBC Panorama documentary in January 2016 and has generated a large amount of media attention since. It has been described as a potential ‘cure’ for MS 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. Various international trials are currently ongoing in AHST. In the United Kingdom, a small number of centres have begun to provide it on the NHS under specific circumstances to a very small number of people
- **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 AHST 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 also been some recent 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 AHST 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.

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 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/ using the search term “multiple sclerosis AND stem cells”. If you wish to participate in a clinical trial you will need to be referred by your neurologist.

MS Ireland’s position on stem cell treatment

MS Ireland welcomes progress in stem cell treatment for people with MS. However, 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 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.

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 AHST 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

www.clinicaltrials.gov/ct2/show/NCT00273364

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

www.mssociety.org.uk/ms-research/emerging-areas/stem-cells

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