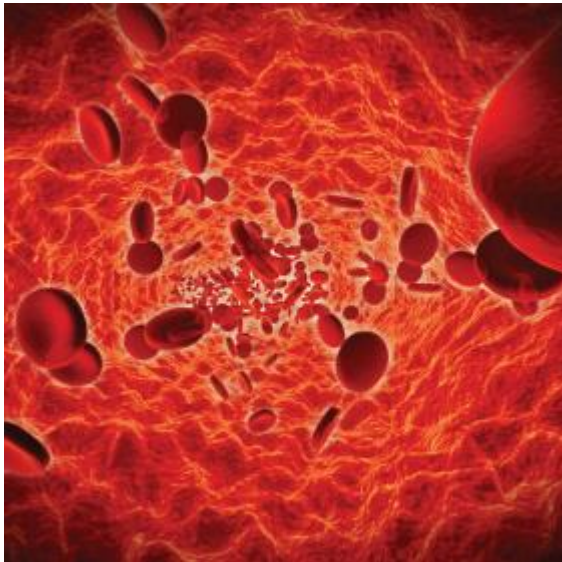


Clinical trials – determining the safety and effectiveness of treatments

11th December, 2016



**There is a lot of interest in the MS community in the potential treatment option for MS known as autologous haematopoietic stem cell transplant (AH SCT). Research into this form of treatment for MS has been going on for some time now, and we are getting a clearer picture of how this intensive form of therapy may fit within the treatment landscape for some forms of MS.**

[AH SCT](#) is a two-step treatment, consisting of chemotherapy (similar to what cancer patients receive for certain blood cancers), to kill off the majority of the immune cells followed by the reinfusion of blood cells to help try and re-build the immune system. More information on this treatment can be found [here](#).

As for any medical treatment, there needs to be rigorous testing to show that it is effective and safe. Especially with diseases that can be as variable as MS, clinicians need to know which type of patient is going to benefit, how to best apply the therapy, and how to avoid any potential complications or side effects. This type of information is typically ascertained in a multistage clinical trial process. The first stage is to assess whether it is safe. Then, using gold-standard randomised controlled trial designs, it is determined if the treatment effectively treats the disease and whether the trial treatment is better or equivalent to the current standard treatment options.

To date, AH SCT has been investigated in a number of international observational studies of groups of patients, and through some small or ongoing randomised trials. However, one of the difficulties in fully understanding the risks and benefits of AH SCT has been due to the variation in the way the studies have been carried out, including participants with different types of MS or levels of disease, and different forms of chemotherapy used. Variations in the way people are treated make proper comparisons difficult, which means it takes longer to reach a valid conclusion from the clinical trial results, and to use the data to achieve a change in hospital or government policy.

Encouragingly, evidence is growing that AH SCT may be a treatment option for a small proportion of people with MS, particularly younger people who are still in the active inflammatory stage of their disease and particularly for those who don't respond to other therapies. The clinical trials that have been conducted so far have also shown that it does not work for everybody, and there are considerable risks. Additionally, it is not yet known whether those patients who respond will have long-term protection against their MS symptoms returning. However, the current trials are giving us a clearer picture of this treatment and are leading to further refinement in its application and optimisation of the outcomes for patients receiving this treatment in the future.