



Autologous haematopoietic stem cell transplant for patients with highly active relapsing remitting multiple sclerosis not responding to high efficacy disease modifying therapies

What is relapsing remitting multiple sclerosis (RRMS)?

Multiple sclerosis is generally diagnosed in young adults and affects the brain and spinal cord causing a wide range of symptoms including fatigue, pain, difficulties with mobility, balance and co-ordination, and vision problems. It can lead to serious disability.

RRMS is the most common form of the disease.

What is autologous haematopoietic stem cell transplant (AHSCT)?

In AHSCT, cells from the patient's bone marrow are collected and frozen. The patient then has chemotherapy to remove the faulty immune system which causes the damage in multiple sclerosis. This is an aggressive procedure which can cause serious side effects.

The cells are then defrosted and returned into the blood stream to rebuild the immune system. The procedure takes about a month in hospital and follow-up visits are required to check on progress.

We were asked to look whether AHSCT would be a good choice for patients whose disease is getting worse even though they are having treatment with the most effective medicines available.

What we did

We looked for studies on how well the procedure works, and how it compares with the best medicines available in terms of its side effects and costs.

We gathered patient views by working with patient organisations.

What we found

We found some evidence that AHCT can reduce symptoms, slow down disability and improve quality of life but that, like the most effective medicines, it is an aggressive treatment with potentially serious side effects. We did not find any studies in patients who had already had the most effective medicines but studies are being done on this.

What is our advice to NHSScotland?

SHTG advised that AHCT should be considered as a treatment option for patients with RRMS which is very active despite treatment with the most effective medicines, as long as they can understand what is involved in the procedure and are willing to accept the risks.

Access to MS treatments should not depend on what part of Scotland you live in.

Future work

Several studies are being done on AHCT for RRMS. The findings of these in the coming years will help us understand how the safety of the procedure compares with that of medicines and also which patients are most likely to benefit from the treatment.

This plain language summary has been produced based on SHTG Advice 07/19 October 2019.