

Autologous Haematopoietic Stem Cell Transplantation in Treatment Naïve Patients with Rapidly Evolving Severe Multiple Sclerosis

Joyutpal Das¹, John Snowden^{2,3}, Sona Mistry⁴, Helen Jessop², Simon Bell⁵, Basil Sharrack^{1,5}

¹Department of Neuroscience, ²Department of Haematology, Royal Hallamshire Hospital, ³Department of Oncology & Metabolism, ⁴Medical School, The University of Sheffield, ⁵Sheffield Institute for Translational Neuroscience

Objective:

To assess the safety and efficacy of autologous haematopoietic stem cell transplantation (AHSCT) in treatment naïve patients with rapidly evolving severe multiple sclerosis (RESMS).

Background:

AHSCT is a very effective treatment in patients with highly active relapsing remitting multiple sclerosis (RRMS) who failed to respond to standard disease modifying therapies (DMTs). International guidelines advocate its use in treatment naïve patients with 'malignant' MS (1).

Design/Methods:

Seven (5 males, 2 females) treatment naïve patients with highly active RRMS received AHSCT in a single academic centre. All patients had RESMS defined as two or more severe relapses associated with one or more gadolinium enhancing magnetic resonance imaging (MRI) lesions during the preceding 12 months. All seven patients had poor long-term clinical and radiological prognostic features.

Results:

The median age at diagnosis was 31 (27-52) years. The median time between symptom onset and treatment was 9 (3-42) months. The median pre-treatment EDSS score was 5.5 (3-7.5) and all patients had rapidly progressive disease course leading to significant disability in multiple domains of the neurological system over a relatively short period. In addition, the pre-treatment MRIs showed multiple gadolinium enhancing cranial and spinal cord lesions on multiple occasions. The median follow up was 18 (3-36) months and the median EDSS at the last follow up was 3 (2.5-6.5). No relapses were observed post-AHSCT. One patient had a single new gadolinium enhancing lesion 6 month after treatment, but no further new or enhancing lesions were observed in any other follow up scans. No CTC grade 3 or 4 toxicities were observed and there was no treatment related mortality.

Conclusions:

AHSCT is safe and effective in treatment naïve patients with RESMS. Further studies are required to compare its efficacy with standard DMTs.

1. Snowden et al. Bone Marrow Transplantation (2011), 1–21

Study Supported by: n/a