HEMATOPOIETIC STEM CELL TRANSPLANTATION IN MYASTHENIA GRAVIS

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Background. Myasthenia gravis (MG) is a rare neurological disease, autoimmune-mediated, which targets the neuromuscular junction, involving the acetylcholine receptors on the motor endplate. We aimed to evaluate the safety, efficacy of autologous hematopoietic cell transplantation (HCT) in patients with severe, refractory myasthenia gravis who are resistant to conventional therapies.

Material and Methods. Have been selected and analyzed 16 articles from PubMed, NCBI, Google Scholar, as well as medical books, scientific journals published in the 2018-2024 period. Results. Some patients with myasthenia gravis do not respond to standard treatments and experience severe or life-threatening symptoms. Autologous hematopoietic stem cell transplant has shown promise in treating other serious autoimmune neurological disorders and may offer similar benefits for MG. The procedure of autologous HCT includes intensive conditioning chemotherapy regimens to destroy the autoreactive immune system followed by graft reinfusion for blood and immune reconstitution. A retrospective cohort study at The Ottawa Hospital, reports the results of 7 cases of severe MG treated with autologous HCT. Five patients (71%) were diagnosed with concomitant autoimmune or lymphoproliferative diseases related to immune dysregulation. All patients achieved complete stable remission with no residual MG symptoms and freedom from any ongoing MG therapy. Three patients (43%) experienced transient viral reactivations and 1 (14%) developed a secondary autoimmune disease after autologous HCT, all of which resolved or stabilized with treatment. Improvement was first noted within days after autologous HCT and the patients status progressively improved in subsequent months. Abnormal decremental response on repetitive nerve stimulation testing resolved 30 days after transplant. There were no treatment- or MG-related deaths.

Conclusion. Autologous hematopoietic stem cell transplantation shows potential as an effective treatment for patients with MG, it may bring significant improvements in disease control and may reduce the need for long-term immunosuppressive medications.

Keywords: myasthenia gravis, autoimmune neurological disorders, stem cell transplant.