HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR AUTOIMMUNE DISEASES – A 20 YEARS SINGLE CENTRE EXPERIENCE

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Background: Over the past 20 years, hematopoietic stem cell transplantation (HSCT) has been emerging as a promising treatment option for severe cases of autoimmune diseases (ADs). Meanwhile, positive results have been obtained in 3 randomised clinical trials for systemic sclerosis. The goal of this therapy is to induce medication-free remissions by ablating the pathologic autoimmune memory and restoring of self-tolerance.

Objectives: Here, we summarise the clinical outcomes of AD patients receiving HSCT at the Charité – University Medicine.

Methods: In this prospective study, the outcome of 22 patients been analysed after receiving a CD34+-selected autologous HSCT after immunoablation with ATG and cyclophosphamide for different ADs (10 SLE, 4 SSc, 3 vasculitis, 2 mixed connective tissue disease, 1 inflammatory polyneuropathy, 1 autoimmune thyroiditis, 1 autoimmune cerebellar ataxia, 1 polychondritis, 1 inflammatory polyneuropathy, 1 autoimmune thyroiditis, 1 autoimmune cerebellar ataxia, and 1 autoimmune polyneuropathy). Response criteria were defined according to the ACR classification criteria and HCQ dosage <100 mg/ml after a minimum of 2 months of treatment. The matched control was a lupus patient, enlisting within the centre the same week, with a HCQ dosage >800 mg/ml. Each patient answered the BMQ-Specific through a telephone interview. The BMQ-Specific comprises 2-S item factors assessing beliefs about the necessity of prescribed medication (Specific-Necessity) and concerns about the danger of dependence and long-term toxicity of medication (Specific-Concerns). Responses to each statement were scored on a five-point Likert scale (1=strongly disagree and 5=strongly agree). Scores obtained for the individual items within both scales were summed to give total scores for the Specific-Necessity and Concern scales range from 5 to 25. A Necessity-Concerns score was calculated (range from –20 to +20). It expresses the cost-benefit perceived by the patient for taking the medication. Case and control characteristics were compared using usual tests. Diagnosis performance of each BMQ score and necessity-concern differential were studied by the mean of ROC curves.

Results: The BMQ-Specific questionnaire was submitted to 118 patients: 59 cases and 59 matched controls. The concern score was significantly higher in cases (mean 16.8 vs. 12.7, p<0.0001). The necessity score was significantly higher in control (mean 18.4 vs 15, respectively, p<0.0001). ROC curves show a better area under the curve (AUC) with the N-C differential compared to the AUC of separate scores (respectively 0.8 vs 0.7). The sensitivity and specificity are optimal for a N-C differential of 3.

Conclusions: During SLE, nonadherent patients are primarily concerned about the risks inherent to treatment, rather than its effectiveness. To date, we are the only study to show that specific BMQ is an efficient tool for detecting patients at risk of poor therapeutic adherence during certain chronic diseases. Early detection of lupus patients at risk of poor therapeutic adherence could lead to preventive actions.

Disclosure of Interest: None declared.