A successful case of second autologous haematopoietic stem cell transplantation for post-transplant systemic sclerosis relapse

Sirs,

It has been reported that autologous haematopoietic stem cell transplantation (aHSCT) is a very effective therapeutic option for patients with severe systemic sclerosis (SSc) (1). We report a successful case of second aHSCT for post-transplant systemic sclerosis relapse.

In June 2007, the 24-year-old female noted the development of characteristic Raynaud phenomenon, associated with puffy fingers and hands from the onset. We considered the patient an eligible candidate for aHSCT. The patient had a weight of 37.5 kg and a height of 161 cm. The transplant regimen consisted of mobilisation with i.v. cyclophosphamide (100 mg/day) was added in combination with conventional prednisone, however, the skin lesions continued to deteriorate even after 6 months of treatment (cyclophosphamide cumulative dose of 17 g).

In March 2011, her modified Rodnan skin score (mRSS) was 38, and pulmonary function tests showed that her diffusing capacity of carbon monoxide was 52%, predicted FVC 58%. As a refractory diffuse SSc to standard treatments, we carried out a second aHSCT. As we expected, she showed an excellent response to the treatment.

SSc remains a difficult-to-treat disease despite the current conventional therapy. As an intensive immunomodulatory therapy, aHSCT has been suggested for severe treatment-resistant autoimmune diseases. To date, three controlled trials have been done, The American Scleroderma Stem Cell versus Immune Suppression Trial (ASSIST) (2), The Autologous Stem cell Transplantation International Scleroderma trial (ASTIS) (3) and The Scleroderma: Cyclophosphamide Or Tranplantation (SCOT) trial. The available data indicate that aHSCT may result in significant and sustained clinical benefits for carefully selected patients, while, on the other hand, a higher morbidity rate compared with standard treatments. In ASTIS study, the reported treatment-related mortality was 10.4% (3). Rheumatic disease patients seem to be even more at risk to develop secondary autoimmune diseases after they had aHSCT (4, 5). Longer follow-up of transplanted SSc patients is essential.

Disease relapse after aHSCT is a challenge for rheumatologists. In the ASTIS trial, 22.4% patients in the aHSCT group had disease relapse between 12 and 24 months (2). However, no widely-approved guideline is available till now. An on-going United States of America multicentre HSCT trial for systemic sclerosis, referred to as the STAT trial (Scleroderma Treatment with Autologous Stem Cells) vs intravenous pulse cyclophosphamide in diffuse cutaneous systemic sclerosis: a randomized clinical trial. JAMA 2014; 311: 2490-8. and The American Scleroderma Stem Cell transplantation for poor-prognosis systemic sclerosis. Rheumatology (Oxford) 2015; 54: 2126-33.


An on-going United States of America multicentre HSCT trial for systemic sclerosis, referred to as the STAT trial (Scleroderma Treatment with Autologous Transplant) (NCT01413100) has been conducted to find out whether 2 years of post-HSCT mycophenolate mofetil maintenance therapy is able to prevent or delay the recurrence of SSc after aHSCT.

Repeat aHSCT has been used in neoplastic patients. Some research confirmed that a second aHSCT can be applied to patients who had a good response to the first. For example, haematologists have agreed that repeat administration of high-dose melphalan with autologous transplantation in multiple myeloma patients who relapse following a first transplant could be considered (7). In our case, we had been facing a similar situation though diffuse SSc was a non-neoplastic disease. aHSCT treatment in early diffuse SSc fundamentally alters the long-term outcome of patients with poor-prognosis SSc (4). When the disease relapse, the long-term outcomes are poor. And our experience indicated that some SSc patients could benefit from the second aHSCT, who had relapsed disease after the first successful aHSCT. However, the high treatment-related mortality should be always taken into account before carrying out a second aHSCT for SSc patients. And further trial was needed to find out the appropriate indications of its clinical application.

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References