Convegno Sclerodermia Un percorso tra corpo e anima

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ABSTRACT INTERVENTO

IL TRAPIANTO DI MIDOLLO NELLA SCLERODERMIA: QUALI PROSPETTIVE?

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Background
Since 1996 over 1000 patients suffering from severe autoimmune diseases have received an autologous haemopoietic stem cell transplantation (HSCT) resulting in sustained regression of skin thickening as well as stabilization of major organ involvement.
Objective
We report the effects on survival, skin and lung involvement of HSCT in patients affected by severe diffuse systemic sclerosis (SSc).
Patients and methods
A total of six patients with diffuse SSc were treated by autologous heamatopoietic stem cell transplantation from july 2000 to may 2008. The mean age was 43 years (29-57) and the mean disease duration from the first non-Raynaud symptom was 18 months (12-24).
All the patients included in this study had a modified Rodnam skin score (mRSS) >20 and Scl-70 was detected in 50% cases (n=3/6). The disease duration was ≤ 2 years with a rapidly progressive course, characterized by a worsening mRSS (>20% in 6 months) and/or evolutive lung involvement defined as a forced vital capacity (FVC) or DLCO below 70% predicted (<20% in 6 months) plus evidence of interstitial lung disease evidenced by high-resolution CT scan +/- bronchoalveolar lavage. No significant cardiac or renal abnormalities were detected before HSCT. Exclusion criteria were: mean pulmonary arterial pressure >50 mmHg and ejection fraction <45% (by cardiac echo), creatinine clearance <20ml/min, uncontrolled rhytm disturbances and hypertension.
Peripheral blood stem cells were collected using cyclophosfamide (3 g/m2)except in one case (4 g/m2) and rHu G-CSF (5 to 10 mcg/Kg/day) and were reinfused after positive CD34+ selection. A clinical assessment consisting of skin evaluation (mRSS), pulmonary function tests and echocardiography was conducted before the treatment and every 6 months. Pulmonary HRCT was annually repeated after base-line.
Results
One patient died within 6 months after HSCT for an infective complication considered as treatment-related (Cytomegalovirus disease), despite of specific treatment. At the time of analysis a mean follow-up of 4.6 years (0.6-8.4) is available. Five patients have a follow-up ≥6 months. The mean mRSS at base-line was 32 (20-39). A decrease >25% in mRSS was achieved in 100% of the patients and after six months (n = 5/5) and one year (n = 4/4) , in 75% (n =3/4) after 2 years and in 66 % (n = 2/3) after 5 years. We detected a lung involvement in all the patients before HSCT, which

didn’t get significant variations during the follow-up but in one case (function test and HRCT worsened). Also cardiac and renal function remained stable.

Conclusion

Our data confirm that HSCT may risult in sustained improvement of skin thickening and stabilisation of pulmonary involvement. Cytomegalovirus infection is a common complication of the early post-engraftment phase.

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