Self-reported changes in the expanded disability status scale score in patients with multiple sclerosis after autologous stem cell transplants: real-world data from a single center


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Summary

In order to reset the immune system to baseline function, autologous hematopoietic stem cell transplantation (HSCT) has been performed in patients with multiple sclerosis (MS). After June 2015, 617 new consecutive patients with MS were autografted in our center with non-frozen peripheral blood stem cells. The autografts were performed on an out-patient basis, after conditioning with cyclophosphamide and rituximab. The aim of the study was the assessment of both safety and efficacy of the method. The study's primary co-end-points were recovery of granulocyte and platelet counts and transplant-related mortality. Secondary end-points were overall survival and clinical response (improvement or stabilization of the self-reported expanded disability status scale score). The protocol was registered in ClinicalTrials.gov identifier NCT02674217.0. We included 401 females and 216 males, with a median age of 46 years. A total of 259 patients had relapsing-remitting MS (RRMS), 228 had secondary progressive (SPMS) and 130 had primary progressive (PPMS) multiple sclerosis. All procedures were initially performed on an out-patient basis and only 32 individuals (5%) required hospitalization. One to three aphereses (median 1) were required to harvest at least 1 × 10^6/kg viable CD34+ cells. The total number of viable CD34+ infused cells ranged between 1 and 37·83 × 10^6/kg (median 5·68). Patients recovered more than 0·5 × 10^9/l absolute granulocytes by day 8 (median, range = 2–14), and platelet values were above 20 × 10^9/l by day 4 (median, range = 0–11). Eleven individuals required red blood cells and six needed platelet transfusions. To date, there have been no deaths attributable to the transplant, yielding a 30-month overall survival of 100%. Patients have been followed for 3–42 months (median = 12). The overall response rate (decrease or stabilization of the self-reported EDSS score) at 12 months was 78% for all patients (83% in RRMS, 78% in PPMS and 73% in SPMS), while the disability progression-free survival was 82% for all patients (86% in RRMS, 78·5% in SPMS and 78% in SPMS). Changes in the self-reported EDSS score in parallel with neurological improvement were observed in people with all types of MS after HSCT, employing the ‘Mexican method’.

Keywords: autoimmunity, multiple sclerosis, stem cells, transplantation