



Presentation

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OS13-1 - HEALTH-RELATED QUALITY OF LIFE IN SYSTEMIC SCLEROSIS BEFORE AND AFTER AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANT - A SYSTEMATIC REVIEW

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Background: Autologous hematopoietic stem cell transplantation (AHSCT) for severe rapidly progressive Systemic Sclerosis (SSc) allows significant regression in skin and lung fibrosis and improvements in overall and event free survival up to 7 years after transplant. We undertook this study to synthesize the evidence on changes in health-related quality of life (HRQoL) associated with AHSCT for SSc.

Methods: Autologous hematopoietic stem cell transplantation (AHSCT) for severe rapidly progressive Systemic Sclerosis (SSc) allows significant regression in skin and lung fibrosis and improvements in overall and event free survival up to 7 years after transplant. We undertook this study to synthesize the evidence on changes in health-related quality of life (HRQoL) associated with AHSCT for SSc.

Results: The search returned 656 articles. Eight were selected: 3 uncontrolled phase I or II trials, 2 cohort studies and 3 RCT (ASSIST, ASTIS, SCOT). HRQoL data from 289 SSc patients treated with AHSCT and 125 with intravenous cyclophosphamide (CYC) as a comparator were extracted. HRQoL was assessed using the Health Assessment Questionnaire-Disability Index (HAQ-DI) (n=275 patients), the Short-Form Health Survey (SF-36) (n=249 patients) and the EuroQol 5 Dimensions (EQ-5D) (n=138 patients). HRQoL was analyzed as a secondary outcome in all studies. Quality of the data was assessed as high.

HAQ-DI improved significantly more with AHSCT compared to CYC (-0.58 vs -0.19, p=0.02 at 2 years in ASTIS; 53% vs 16% improved at 4.8 years in SCOT). Scores also improved pre-post AHSCT in the uncontrolled studies (ranging from -0.6 to -1.7 points at one year (all p<0.05), and up to -1.5 points at 7.5 years (p< 0.001)). SF-36 Physical Component Summary scores were significantly better in subjects treated with AHSCT compared to CYC (between

group differences ranging from 26 points at one year in ASSIST and 6.1 points at 2 years in ASTIS ($p=0.01$); 56% vs 15% improved at 4.8 years in SCOT ($p=0.02$). Similar differences pre-post treatment scores were also reported in an uncontrolled study (increase of 20 points, $p< 0.0001$). In ASSIST, there was a trend for the SF-36 Mental Component Summary score to improve in the AHSCT arm (46 vs 58, $p=0.07$) and worsen in the CYC arm (56 vs 42, $p=0.04$) at one year. There were no significant differences between the AHSCT and CYC arms in ASTIS and SCOT with 2.0 and 4.8 years of follow-up, respectively. Post-treatment scores improved significantly compared to pre-treatment in an uncontrolled study (from 51 to 64 points, $p=0.005$). ASTIS showed a significant difference in the index-based utility score of the EQ-5D (0.29, $p< 0.001$) and a non-significant difference in the general health visual analogue scale (6.7, $p=0.36$) at two years, in favour of AHSCT compared to CYC.

Conclusions: Although there is heterogeneity in the reported data, AHSCT in SSc was consistently associated with marked and sustained improvement in HRQoL. This analysis provides additional compelling data for the role of AHSCT in SSc when assessing patient's point of view.

Clinical Trial Registry: NA

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