Real-World Outcomes of Autologous Haemopoietic Stem Cell Transplantation in Patients with Waldenström's Macroglobulinemia. Report from the Lymphoma Working Party of The European Society for Blood and Marrow Transplantation (LWP EBMT)

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Waldenström's Macroglobulinemia (WM) is classified as a low-grade B- cell non-Hodgkin lymphoma. There are major therapeutic advances for WM that have achieved prolong progression-free (PFS) and overall (OS) survivals. The role of autologous haemopoietic stem cell transplantation (AHSCT) in the era of the new therapies is debatable.

This retrospective analysis is reporting the survival outcomes of AHSCT for patients with WM performed in EBMT centers. Patients with WM, who received a first AHSCT (n=772 with 218 between 2000-2010, 290 in 2010-2015 and 264 in 2015-2021), reported in the EBMT registry between 2000 and 2021, were included in this study. The median age of the 772 identified patients, was 57 years (IQR: 50-62), 72% were male and the median time from diagnosis to AHSCT was 24 months (IQR: 11-58). The number of prior line therapies was, 1 in 24%, 2 in 39% and ≥3 in 37% of the patients. Disease status at AHSCT was ≥VGPR in 36%, PR in 52%, primary or relapse refractory (PRR) in 12%. Karnofsky performance status was <90 in 24%. Conditioning regimen included BEAM in 54%, Melphalan in 17%, EAM in 7%, FEAM in 4%, BuCy in 4%, BeEAM in 3%, ThioBCNU in 3%, TBI based in 4% and other in 4%. Peripheral haemopoietic stem cell source was used in 99% of the cases.

With a median follow-up of 4.6 years (95%CI: 3.9-5.3) the 2, 5 and 10 years the estimated OS rates were 89.4%, 70.4% and 55.3%. The PFS at 2, 5 and 10 years were 68.2%, 46.9% and 31.2%, while the relapse rates (RR) were 28.8%, 48.6% and 61.8%. The Non-Relapse Mortality (NRM) was 3%, 4.5%, 7.1% respectively. The multivariate analysis showed that, the time from diagnosis to AHSCT was the only significant factor impacting on PFS (p= 0.031, HR: 1.39, 95% CI:1.03 – 1.87) and RR (p= 0.039, HR: 1.39, 95% CI:1.02-1.90). Of the 772 patients, 555 (71.89%) were alive and 217 (28.1%) patients died. The cause of death was, progressive disease in 143/772 (18.5%) of the patients, infection in 21 (2.7%), transplant related in 1.16% and secondary malignancy in 1.81%.

This LWP EBMT retrospective analysis, reports the outcomes of the largest cohort to date, of patients with WM treated with AHSCT. It showed that, this fixed duration high dose therapeutic approach, could provide prolong PFS and OS outcomes in selected young SCT eligible patients with acceptable NRM and overall toxicity.