

Autologous Haematopoietic Stem Cell Transplantation for Relapsed Waldenström Macroglobulinaemia: A 10-Year EBMT LWP Registry Analysis in the Era of Targeted Therapies

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Introduction

Waldenström macroglobulinaemia (WM) is a rare, indolent lymphoma with major survival improvements following the introduction of Bruton tyrosine kinase (BTK), proteasome (PI), BCL-2 inhibitors. However, WM remains incurable, and progressive, refractory disease require effective salvage treatment options. The role of Autologous haematopoietic stem cell transplantation (ASCT) as potential treatment in the era of targeted therapies is debatable.

Methods:

This retrospective analysis evaluated the transplantation outcomes in patients with WM undergoing ASCT over the last decade (between 2015-2024), reported in the EBMT LWP registry.

Results:

Two hundred and sixty-one patients with WM underwent ASCT 151 between 2015-2019, and 110 between 2019-2024. The median age at ASCT was 59 (53-64) years and 184 (70%) patients were male. The Karnofsky performance status (KFS) was ≥ 90 in 180 (76%) of the patients. 144 (55%) patients were transplanted in >24 months. The haemopoietic stem cell transplant comorbidity index (HSCT-CI) was 0 in 188 (74%), 1-2 in 38 (15%), ≥ 3 in 28 (11%) and was not available in 7 patients. The disease status at ASCT was very good partial response (VGPR) in 63 (25%), partial response (PR) in 160 (65%), stable/progressive disease SD/PD) in 24 (10%) and was missing in 14 patients. The conditioning regimen was BEAM based in 121 (47%), Melphalan in 52 (20%), Thiotepa/BCNU in 22 (9%), BeEAM in 13 (5%), FEAM in 9 (4%), TBC in 5 (2%), other in 34 (13%) and was missing in 5 patients.

66 (25%) received proteasome inhibitor-based treatment prior to ASCT, 32 (12%) BTK and 1 had prior bcl2 inhibitor treatment (90 (34%) were exposed in both PI and BTKi as part of prior to ASCT therapy lines).

The median follow up was 4.3 (2.7-5.6) years. The overall survival rates (OS) at 3 and 5 years were 84.1 and 71.8% accordingly, the progression free survival (PFS) 60.5% and 49.5%, the relapse incidence (RI) 34.6% and 44.3% and non-relapse mortality (NRM) 4.9% and 6.2 respectively. The patients transplanted in >24 months from diagnosis had significantly inferior PFS 54.7% vs 69.6% ($p=0.028$), with trend for higher RI 38.2% vs 29.1% at 3 years. For patients who had previously BTK inhibitors there was trend for shorter post ASCT PFS 43.9% vs 62.8% ($p=0.062$) at 3 years and higher RI 51.6% vs 32.2% ($p=0.061$). SD/PD ds were associated with significantly inferior OS 68.9% vs 92.4% ($p=0.007$) at 2 years and higher NRM 13.2% vs 2.7% ($p=0.015$). 41 patients died, 22(63%) of PD, 3 (9%) of secondary malignancy, 6 (17%) of treatment related, 4(11%) of unspecified and in 6 patients the cause was not available.

Conclusion: This EBMT analysis showed that in the era of new agents, selected patients with relapsed WM could be salvaged with ASCT with PFS 61/ 50% at 3 and 5 years and low NRM. However, outcomes appeared less favourable in patients previously exposed to BTK inhibitors, those transplanted later in the disease course, and those with refractory disease at ASCT.

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