

The Role of Allogeneic Stem Cell Transplantation for Relapsed Waldenström Macroglobulinaemia During the Era of Novel Agents: A 10-Year EBMT Registry Study

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Introduction:

The introduction of immunochemotherapy, novel targeted agents, including Bruton tyrosine kinase (BTK) inhibitors, proteasome inhibitors (PIs), BCL-2 inhibitors, has substantially improved outcomes for patients with Waldenström macroglobulinaemia (WM). Patients with relapsed, refractory, or progressive WM disease continue to require effective salvage strategies. The role of allogeneic haematopoietic stem cell transplantation (alloSCT) in the era of novel agents remains poorly defined. Although, alloSCT offers the potential for long-term disease control and possibly cure, its use is limited by significant non-relapse mortality (NRM).

Methods:

This retrospective registry analysis by the EBMT Lymphoma Working Party (LWP) evaluated the outcomes of patients with WM undergoing alloSCT between 2015 and 2024.

Results:

Ninety patients underwent alloSCT between 2015 and 2024. Fifty-nine (66%) were male, with a median age at alloSCT of 56 years (range, 45–64), and 55 (68%) had a Karnofsky Performance Status $\geq 90\%$. Twenty-five (28%) underwent alloSCT within 24 months of diagnosis. Prior therapies included PIs in 23 (26%) patients, BTK inhibitors in 33 (37%), BCL-2 inhibitors in 10 (11%), and 43 (48%) had received more than one novel agent and 24 (27%) ASCT before alloSCT. At transplantation, disease status was very good partial response in 32 (37%) patients, partial response in 44 (51%), stable/progressive disease in 11 (13%), and unavailable in three. Myeloablative conditioning was used in 26 (30%) patients, reduced-intensity in 61 (68%); most frequently used regimens were BuFlu-based (21%), CyFlu (20%), BuFluThio (15%),

Flu-based (14%), and FluMel (13%). Peripheral blood was the stem cell source in 83 (93%) patients. Donors were related in 44 (54%), unrelated in 39 (46%). After a median follow-up of 3.1 years (IQR, 2.3–4.3), the overall survival (OS) rates at 3 and 5 years were both 59.1%. Progression-free survival (PFS) was 54.6% and 48.5% at 3 and 5 years, respectively. The cumulative incidence of relapse (RI) was 23.3% and 29.4% at 3 and 5 years, while NRM was 16.3%, 22.1%, and 22.1% at 1, 3, and 5 years, respectively. Prior exposure to BTK inhibitors was significantly associated with a lower relapse incidence ($p=0.026$), while patients transplanted more than 24 months after diagnosis had superior OS ($p=0.035$). The cumulative incidence of grade II–IV acute graft-versus-host disease (aGVHD) was 19% (grade III–IV aGVHD 11%). Chronic GVHD occurred in 34% and 45% of patients at 1 and 5 years, respectively (extensive cGVHD was observed in 2% and 4%). Thirty-two patients died, including 10 (31%) from PD and 14 (44%) from NRM; the cause of death was unavailable for eight patients.

Conclusion:

This EBMT registry analysis demonstrates that alloSCT remains a potentially salvage treatment option for carefully selected patients with WM in the era of novel agents, providing durable disease control with 3- and 5-year PFS rates of 55% and 49%, respectively. Prior BTK inhibitor exposure was associated with a lower RI, whereas transplantation performed more than 24 months after diagnosis was associated with improved OS. Despite these favourable long-term outcomes, alloSCT is associated with substantial NRM, underscoring the need for careful patient selection.

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