TREOSULFAN-CONTAINING CONDITIONING REGIMEN PRIOR TO FIRST ALLOGENEIC STEM CELL TRANSPLANTATION IN ACUTE MYELOID LEUKEMIA AND HIGH-RISK MYELODYSPLASTIC SYNDROMES: A SINGLE-CENTER EXPERIENCE AT UMC LJUBLJANA, SLOVENIA

Klara Šlajpah, Irena Auersperger, Mihael Kosi, Nika Kraljić, Polona Novak, Teresa Urdih, Martina Tomažin, Petra Pavlovčič, Matjaž Sever

University Medical Centre Ljubljana, Department of Haematology, Ljubljana; Celje General Hospital, Department of Haematology and Oncology, Celje, Slovenia

Treosulfan, a structural analogue of busulfan, offers potent myeloablative, immunosuppressive, and anti-leukemic activity with reduced toxicity and pro-inflammatory cytokine release. It has been successfully incorporated in conditioning regiments for allogeneic stem cell transplant (allo-HSCT) for various indications in pediatric and adult patient population. We present a single-center experience at UMC Ljubljana, Slovenia using treosulfan-based conditioning regimens in patients undergoing first allo-HSCT for AML or high-risk MDS.

Between May/2023 and May/2025, we treated 16 patients (69% AML, 31% high-risk MDS; median age 62 years, range 50–71) who received conditioning with treosulfan-fludarabine or treosulfan-thiotepa-fludarabine. Treosulfan was administered at 12 g/m^2/day (myeloablative, 44%) or 10 g/m^2/day (reduced intensity, 56%) for three days, with dose adjustments based on age, comorbidities, and performance status. Fludarabine (30 mg/m^2/day × 5) was used in all cases and thiotepa (5 mg/kg/day × 2) in 75%. Stem cell source was peripheral blood in all patients. Donors included matched unrelated (81%), matched sibling (6%), and haploidentical (13%) donors. GvHD prophylaxis varied by donor type, incorporating CSA, MMF, ATG, and PT-Cy as appropriate. All but one patient (who died before engraftment for HLH with cardiovascular complications) achieved neutrophil and platelet engraftment at median of 14 and 25 days, respectively. Acute GvHD (grade II–IV) occurred in 50% (6 grade II, 1 grade III, 1 grade IV); one patient developed moderate chronic GvHD. No sinusoidal obstruction syndrome was observed. Two patients developed transplant-associated thrombotic microangiopathy and one post-transplant lymphoproliferative disorder. Infections occurred in 50% of patients, with one fatal case. At a median follow-up of 158 days, 75% of patients were alive. Causes of death included infection, relapse, PTLD, and HLH (one each).

Treosulfan-based conditioning appears well tolerated, with acceptable toxicity and promising early outcomes, supporting its use in older or comorbid patients undergoing allo-HSCT for AML and high-risk MDS.