

Allogeneic Hematopoietic Stem Cell Transplantation in Myelofibrosis: Risk Stratification Using MTSS and EBMT Score - A Single-Center Experience

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Background. Myelofibrosis (MF) is a clonal myeloproliferative neoplasm marked by progressive marrow fibrosis, cytopenias, splenomegaly, and constitutional symptoms. Allogeneic hematopoietic stem cell transplantation (allo-HSCT) remains the only potentially curative treatment, but transplant-related mortality demands careful patient selection. The myelofibrosis transplant scoring system (MTSS) and EBMT score are validated tools for risk stratification. We aimed to evaluate transplant outcomes and prognostic factors in a real-world single-center MF cohort, focusing on the discriminatory value of MTSS and EBMT scoring.

Methods. We retrospectively reviewed all adults with WHO-2016–confirmed MF (primary, post-PV, and post-ET) who consecutively underwent allo-HSCT at our center between January 2008 and March 2024. Patients were stratified by DIPSS-plus, EBMT score, and a partial MTSS. Survival was estimated by Kaplan–Meier and compared by log-rank test. Given the limited events (n=15, EPV=3.0), a ridge-penalized Cox model was used for multivariate analysis.

Results. Among 35 patients (median age 57.7 years, 74% male), most had intermediate-2/high-risk disease (83% by DIPSS-plus); 60% received pre-transplant ruxolitinib. Notably, no patient underwent splenectomy—even with spleen size ≥ 15 cm. At a median follow-up of 14.2 months, median OS was 88 months, with 1-/2-/3-year OS of 66%/62%/58%. On univariate analysis, HLA mismatch (HR 4.66, $p=0.005$) and haploidentical transplantation (HR 17.83, $p=0.001$) were the strongest adverse factors. Chronic GVHD was associated with markedly longer survival (114 vs 9 months, $p=0.004$), supporting a graft-versus-myelofibrosis effect. On ridge-penalized multivariate Cox analysis, haploidentical donor was the only independent predictor of inferior OS (aHR 2.17, 95% CI 1.13–4.15, $p=0.019$).

Conclusions. OS did not differ across MTSS groups, likely reflecting limited power. With a median OS of 88 months and 3-year OS of 58%, our results align with international registries. Donor selection was the most actionable determinant of outcome—haploidentical transplantation predicted inferior survival (median 6 months). Optimizing donor matching remains key to improving MF transplant outcomes.