

Long-Term Outcomes of Hematopoietic Stem Cell Transplantation for Transfusion-Dependent Thalassemia: A Single-Center Experience from Resource limited setting

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Background: Transfusion-dependent thalassemia (TDT) is a inherited hemoglobin disorder associated with significant morbidity and mortality if managed with conventional transfusion and iron chelation alone. Hematopoietic stem cell transplantation (HSCT) remains the only established curative therapy. Although outcomes have improved substantially over the last two decades, transplant-related complications such as graft-versus-host disease (GVHD) and infections continue to influence survival. This study reports long-term outcomes and prognostic factors following HSCT for TDT in a pediatric cohort from a single center in India.

Methods: We conducted a retrospective observational study of 63 children with TDT who underwent HSCT. Patient demographics, donor and graft characteristics, conditioning regimens, GVHD incidence, and outcomes were analyzed. Overall survival (OS) was estimated using Kaplan–Meier methods. Prognostic factors for OS were evaluated using univariable and multivariable Cox proportional hazards regression.

Results: HLA-identical sibling donors were the most common donor type (n=44, 69.8%). Peripheral blood stem cells were the most common graft source (82.5%). Conditioning regimens included busulfan–cyclophosphamide (BuCy) with anti-thymocyte globulin (ATG) (66.7%), Fludarabine-BuCy-ATG (15.9%), and Fludarabine-Treosulfan-Thiotepa-ATG regimen (17.5%). Acute GVHD occurred in 22.2% and chronic GVHD in 14.3%. At a median follow-up extending up to 9 years, 61.9% (n=39) of patients were alive. OS (SE) at 5 years was 59.6% (48.1% - 73.8%). Age >7 years, gender, donor type, stem cell source, conditioning regimen and GvHD did not influence the outcome on univariate analysis.

Conclusions: HSCT offers durable long-term survival in children with TDT in real-world settings. Age, donor type, stem cell source, conditioning regimen and GvHD did not predict poor outcome in our study. Larger study in a prospective setting with long term follow up would be the way to understand prognostic factors affecting outcomes in the LMIC setting.