

abstract

Haplo-Identical Hematopoietic Stem Cell Transplantation for Pediatric Leukemia using Post-Transplant Cyclophosphamide (PT-Cy) approach, Single-Center Study

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Haplo-Identical Hematopoietic Stem Cell Transplantation for Pediatric Leukemia using Post-Transplant Cyclophosphamide (PT-Cy) approach, Single-Center Study

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Background: Haplo-identical hematopoietic stem cell transplantation (Haplo-HSCT) is an important treatment option in patients who lack matched donors in a timely manner.

Methods: We are reporting all pediatric patients < age of 14 with Leukemia who underwent Haplo-HSCT using PT-Cy approach from 2015-2024 at our institution. Conditioning regimens were myeloablative (TBI-based in ALL and Busulfan based in AML/JMML). GVHD prophylaxis included cyclosporine and MMF starting at Day + 5. Calcineurin inhibitor weaning starts around Day+90 post HSCT. Bone marrow was the source of stem cells in all patients except 2. Supportive therapy was homogenous per institutional guidelines.

Results: 27 patients were included with a median age of 7.2 (1.3-14) year, and (59/41) male/female ratio. HSCT Indications were ALL, AML, JMML in 19(70%), 7(26%), 1(4%) respectively. Pre-HSCT Disease status was CR1 in 8(30%) and CR2 in 19 (70%). The median infused TNC dose was 5.1 (1.1-17.5)X10⁸ /kg. The median time to neutrophil and platelet engraftment was 21 (12-38) and 28(15-120) days, respectively. Grade III-IV aGVHD and cGVHD was seen in 33% and 11%, respectively. The rate of hemorrhagic cystitis, sinusoidal obstruction syndrome and CMV reactivation was 18%, 7%, and 44%, respectively.

The two-year overall survival (OS) and event-free-survival (EFS) were 72% and 55% respectively. Relapse was seen in 8 patients (30%), 4 of them were salvaged with CART therapy or second HSCT. 3 patients developed transplant related mortality from multi-organ failure secondary to severe GVHD, uncontrolled viral infection or capillary leak syndrome. The median follow-up was 5 (0.3-9.5) years, Univariate analysis for gender, age, leukemia type, CR status for OS, EFS was not significant.

Conclusions: Our center experience shows that haplo-HSCT for leukemia is comparable to Haplo-HSCT in the literature; however, additional analysis and improvement is warranted to improve GVHD rates to compare it with matched donor HSCT.