We conclude that despite excellent outcome with low GVHD-incidence and low NRM after HSCT from MD in children with high risk ALL the HSCT from MSD results in faster engraftment and immunoreconstitution and less severe infections and justifies the use of minor sibling donors.

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Allogeneic Hematopoietic Stem Cell Transplantation (HSCT) Outcomes for Juvenile Myelomonocytic Leukemia (JMML), a 15-Year Single Center Experience

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Background: JMML is a rare, highly aggressive clonal myeloproliferative disorder typically diagnosed in infants and young children. Allogeneic HSCT is widely accepted as the standard curative therapy for JMML, but success is limited by engraftment failure or disease relapse. Five-year overall survival ranges from 50-55%.

Objective: Evaluate our single center allogeneic HSCT outcomes for JMML.

Methods: We retrospectively reviewed data on all JMML patients who received allogeneic HSCT at our center from 1998-2013. Data collected were age at diagnosis and HSCT, time to HSCT, sex, race/ethnicity, conditioning regimen, stem cell source, total nucleated cell dose, graft-versus-host disease (GVHD) prophylaxis, time to neutrophil (absolute neutrophil count, ANC) and platelet (PLT) engraftment, presence of acute and/or chronic GVHD, relapse, cause of death, and last time of follow-up. Descriptive statistics were used for data analysis.

Results: Eleven patients were diagnosed with JMML and received allogeneic HSCT over the last 15 years in our center. Median age at diagnosis was 1.8 (range 2.9-42.1) months. Six of 11 (54.5%) were males. Eight of 11 (72.7%) were Hispanic, 2/11 White (18.2%), and 1/11 Black (9.1%). Median age at HSCT was 26.2 months (range 4.1-51.0) with median time to HSCT 6.1 months (range 1.2-17.1). All received myeloablative conditioning; 8/11 (72.7%) received busulfan, cyclophosphamide, and melphalan with 5/11 (45.5%) receiving ATG. GVHD prophylaxis was CsA+steroids (4), CsA+MMF (1), and CsA+MTX (1) and tacrolimus+MTX (5). Stem cell source was unrelated umbilical cord blood (UCB) (7) and bone marrow (BM) (4). UCB HLA-disparities were 5/6 (5), 4/6 (1), and 3/6 (1). Three of 4 received matched related donor (MRD) BM while 1 received matched unrelated donor (MUD) BMT. Median time to ANC > 500/μL was 21 days, to PLT > 20,000/μL was 33 days, and to PLT > 50,000/μL was 44.5 days. One patient (UCB) had engraftment failure and received a 2nd HSCT with UCB after 200 cGy total body irradiation+fludarabine+ATG. Acute GVHD occurred in 6/11 (54.5%), limited chronic GVHD in 2/11 (18.2%) and extensive chronic GVHD in 1/11 (9.1%). With a median follow-up of 4.2 years, 8/11 patients are alive (72.7%). Cause of death was progression to AML (1), sinusoidal obstructive liver syndrome and HHV6 encephalitis (1) and chronic GVHD with bronchiolitis obliterans (1).

Conclusions: The overall survival for JMML in our center (72.7%) over the last 15 years is comparable to published data. Despite historically poor disease-free survival, no patients have experienced recurrent JMML post-HSCT in our cohort. We hypothesize that this observed trend toward superior survival may be due to undefined favorable characteristics in our predominantly Hispanic patient population and younger age at diagnosis. Our experience supports the use of HLA-mismatched UCB for unrelated donor HSCT in JMML.

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Plerixafor: A Magic Drug for Urgent PBSC Mobilization from an Adolescent Donor after Failed Bone Marrow Harvest

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Introduction: Failure to harvest sufficient stem cells from either Bone marrow (BM) or peripheral blood is a rare but known impasse in stem cell transplantation (SCT). If second boost is not given within 96 hours then chances of rejection post second SCT are very high. Plerixafor (Mozobil) is an effective drug for rapid (within 24 hr) stem cell mobilizer as it reduces CD34+ hematopoietic stem cells anchoring to the BM microenvironment through reversible inhibition of the SDF-1α/CXCR4 axis. Case: A 4-year-old girl of thalassemia major underwent allo-SCT from a 6/6 loci HLA-identical 17 year old sister. Pre-transplant work-up showed no discernible pathology except bidirectional major ABO mismatch. Conditioning regimen consisted of intravenous busulphan (12.8 mg/kg), cyclophosphamide (200mg/kg) and ATG (90mg/kg). Pre depletion and after red cell and plasma depletion, harvested CD34+ cell dose was 2 and 0.5 million/kg/recipient respectively. In view of the failed BM harvest, a decision was eventually made, after obtaining the consent of the parents, to attempt donor CD34+ cell mobilization with plerixafor and G-CSF. The donor received G-CSF (5 mcg/kg s.c.) for 3 consecutive days at 1800 hr, while plerixafor (240 mcg/kg s.c.) was given 15 hr later, after last dose of G-CSF. Five hour after plerixafor dosing, the donor’s WBC had reached 64.0 x 10⁹/L. The harvested CD34+ cell dose of 176 x 10⁹/kg/recipient infused. Donor tolerated plerixafor well without any adverse effects. Her neutrophil and platelets engrafted on day +19 and +21 respectively. The patient and donor remain well 2 months after SCT. Donor would be followed every 6 months for next 3 years for any long term adverse effects of plerixafor.

Conclusion: Our case highlights that plerixafor is an effective agent for rapid PBSC mobilization from a normal healthy donor, if initial BM harvest fails. However, donor should be monitored for the long term safety of plerixafor.

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Bone Marrow Transplant for Children with CML

Presenting in Blast Crisis in the Era of TKIs

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Background: Although management of CML has changed dramatically in the era of tyrosine kinase inhibitors (TKIs),...