

disease (score 3, 4 or 5). Type of donor: Related: 8pts (siblings: 5pts and other related:3pts), Unrelated: 28pts. Source of stem cells: bone marrow (BM): 12pts and umbilical cord blood (UCB):24pts (compatibility 6 / 6: 2pts, 5 / 6: 16pts, 4 / 6: 6pts). Conditioning: Cyclophosphamide 120-200mg/kg + Oral Busulfan 16-20mg/kg +/- rATG. Most pts received cyclosporine and methotrexate as GVHD prophylaxis.

Results: 28pts is alive and well between 6 months and 17 yrs after HSCT (M:4,5ys) with an overall survival(OS) of 80,3% at 5ys. 35pts survived more than 28 days and were evaluable for engraftment. Three pts had no engraftment (all received UCB).One pt died on D+34 with pulmonary aspergillosis and the other two underwent a 2nd UCBT. One is alive and well 7 years after transplant. Nine of 32 evaluable pts developed an acute-GVHD, grades II-IV (grade III-IV: 2pts). Eight of 31 evaluable pts developed C-GVHD (extensive in 3pts). There was no significant difference in OS in the univariate or multivariate analysis in relation to age less than or greater than 5 years (76% vs. 82%), type of donor, related or unrelated (87.5% vs. 75%), source of stem cells, bone marrow or cord blood (91.7% vs. 70,8%), presence or absence of acute-GVHD (78% vs 92%); or Chronic GVHD (75% vs 91%). Eight pts died between 21 to 1832 days post HSCT (M: 137 days), most deaths occurred during the first year of transplant (6pts) and were related to viral infections (4pts), fungal (1pt) and GVHD associated with bacterial infections (3pts). The cumulative incidence of TRM at 100 days was 8% and at 1 year was of 17%. Chemotherapy was well tolerated, but reactivation/acquisition of viral infections (mainly RSV) during the period of neutropenia contributed to the early death in some pts. **Conclusions:** Despite the small number of pts this experience shows an excellent survival for pts transplanted for WAS. Infections complications (viral or fungal) are frequent and must be detected quickly and treated aggressively.

74

EFFECTS OF BODY MASS INDEX (BMI) IN CHILDREN UNDERGOING ALLOGENEIC BONE MARROW TRANSPLANT (BMT) FOR HEMATOLOGIC MALIGNANCIES

Aplenc, R.¹, Pasquini, M.C.², Zhang, M.-J.², Zhu, X.², McCarthy, P.L.³, Ho, V.T.⁴, Cooke, K.R.⁵, Sung, L.⁶, Bunin, N.J.¹ Children's Hospital of Philadelphia; ² CIBMTR, Medical College of Wisconsin; ³ Roswell Park Cancer Institute; ⁴ Dana-Farber Cancer Institute; ⁵ University Hospitals Case Medical Center; ⁶ The Hospital for Sick Children

The rising incidence of pediatric obesity may significantly impact BMT outcomes for malignant diseases, as has been demonstrated in increased mortality in children undergoing BMT for aplastic anemia. Obesity may influence chemotherapy dosing and transplant related mortality (TRM). We analyzed 3,687 children ages 2-18, who received BMT for treatment of hematologic malignancies using either busulfan/cyclophosphamide (BuCy, N = 1,196) or Cy/total body irradiation-based (CyTBI, N = 2,495) conditioning, between 1990 and 2007. Recipients were classified according to age-adjusted BMI percentiles as underweight (<5% [UW], n = 282), at risk of underweight (6-25% [RUW], n = 509), normal (26-75%,[NW] n = 1469), at risk of overweight (76-95% [ROW], n = 987) and obese (>95%, [OB] n = 444). Total doses of chemotherapy administered to patients in the ROW and OB groups were divided by actual and ideal body weight to estimate dose adjustment practices. Median age (10-13 y) and race were similar in all groups; OB group had higher number of patients with acute lymphocytic leukemia (58%), with early disease (52%), unrelated donor recipients (56%) and from a U.S. center (61%). The table below summarizes adjusted probabilities according to BMI groups. Multivariate analysis demonstrated a higher TRM in the OB group (RR 1.32, p = 0.0075) compared to NW. Conversely, patients in the OB group had decreased risk of relapse (RR 0.72, p = 0.0037) compared to NW. There was no significant impact of UW and RUW compared to NW in any outcomes, and no differences in relapse-free and overall survival according to BMI groups. Chemotherapy adjustment assessment demonstrated that among 1,061 patients with available dose information, 171 (16%) had probable dose adjustment for conditioning. Obesity was associated with higher TRM and lower relapse in children with hematologic malignancies, likely related to higher intensity conditioning, as doses were most often calculated based on actual

weight. Additionally, patients with low BMI experienced similar outcomes compared to patients with NW.

Table.

Outcomes @ 3 years	UW (95% CI)	RUW (95% CI)	NW (95% CI)	ROW (95% CI)	OB (95% CI)	p-value
N	282	509	1467	986	443	
TRM	18 (13-22)	19 (16-22)	21 (18-22)	22 (20-25)	28 (24-32)	0.0034
Relapse	33 (28-39)	33 (29-37)	29 (26-31)	25 (23-28)	21 (17-25)	<0.0001
RFS	48 (42-54)	48 (44-53)	50 (48-53)	52 (49-56)	51 (47-56)	0.54
OS	55 (49-61)	57 (52-61)	58 (56-61)	58 (55-61)	56 (52-61)	0.81

Abbreviations: CI, confidence interval; NW, normal weight; OB, obese; OS, Overall Survival; RFS, relapse free survival; ROW, risk of overweight; RUW, risk of underweight; UW, underweight

75

SINGLE CENTER EXPERIENCE OF THIOTEPA, TREOSULPHAN & FLUDARABINE BASED REGIMEN IN THALASSEMIA MAJOR

Choudhary, D.¹, Katewa, S.², Kharya, G.¹, Anjan, M.¹, Setia, R.¹ BLK Super Speciality Hospital, New Delhi, Delhi, India; ² Hospital for Sick Children, Toronto, ON, Canada

The only curative treatment for Thalassemia major (TM) is allogeneic bone marrow transplantation. The most commonly used conditioning regimen is Busulphan, Cyclophosphamide and Anti thymocyte globulin. This has high regimen related toxicities (RRT). To minimize RRT, especially in high risk TM we studied new conditioning regimen in prospective manner. Between February 2010 - September 2011, seventeen children with β -Thalassemia major underwent Allogeneic Bone Marrow Transplant at BLK Super Speciality Hospital. The median age was 12 years. (Range 2-16 yrs.).

There were nine males & eight females, four patients belonged to Pesaro class II and 13 were class III. All patients received conditioning with Thiotepa 8 mg/kg on D -6, Treosulphan 14 gm/m²/day from D-5 to D-3 & Fludarabine 40 mg/m²/day from D-5 to D -2. Sixteen children were transplanted with bone marrow graft from HLA identical siblings (6/6 antigen) and one patient received bone marrow graft from mother (5/6 antigen). Mean cell doses given were: 6.36×10^6 cells /kg BW for CD34+ cells (Range 2.06 - 14.02 $\times 10^6$ /kg) and 7.84×10^8 /kg /BW for mononuclear cells (Range 2.9 - 17.5 $\times 10^8$ /kg). Two patients had ABO major mismatch, 4 had minor mismatch and 1 had bidirectional mismatch. Cyclosporine & Methotrexate was used as GVHD prophylaxis. No patient developed grade III - IV GVHD. None of them required TPN or parental analgesia. Sixteen out of seventeen patients achieved donor engraftment. Median neutrophil engraftment was achieved on D+15 (Range 11 - 17 day) and median platelet engraftment was achieved on D+21 (Range 9 to 34 days). One patient developed grade II gut GVHD. One patient expired on D+6 due to neutropenic enterocolitis, sepsis and intracranial hemorrhage.

Median follow-up period was 12 months (Range 1 -20 mo). Till last follow up, all the patients who achieved engraftment are alive and transfusion independent.

Conclusion: Thiotepa, Treosulphan & Fludarabine based regimen has acceptable toxicities with stable donor engraftment.

76

UNRELATED CORD BLOOD TRANSPLANT (UCBT) IS ASSOCIATED LOW RATES OF LONGTERM, PERSISTENT GRAFT VERSUS HOST DISEASE (GVHD)

Craddock, J.A., Alsultan, A., Quinones, R.R., Keating, A., Hild, E., Benkbali, N., Law, D., Peltz, A., Nuechterlein, B., Drake, K., Smolik, S., Giller, R.H. Childrens Hospital Colorado/University of Colorado School of Medicine, Aurora, CO

Reduced rates of acute and chronic GVHD make unrelated cord blood an attractive stem cell source. Adult marrow and PBSC from unrelated donors are associated with higher rates of GVHD, persistence or progression of GVHD manifestations, need for chronic immunosuppressive therapies, reduced quality of life, infection, organ toxicities, and decreased overall survival (OS). In contrast, recent analyses from our center and others have indicated that GVHD following UCBT does not adversely impact OS (BMT 2011, 46:668-675). To better understand the longterm outcomes of