

patients were re-started on a tyrosine kinase inhibitor (Imatinib -5, Dasatinib -1). One patient had delayed recovery of blood counts and was not restarted on a TKI. One patient relapsed 4 months post PBSCT and died due to refractory disease. Remaining patients remain alive at median 26 months (range 12-86) in complete molecular remission. Of note the patient that never received post-transplant TKI remains in complete molecular remission 86 months following PBSCT.

**Conclusion:** Autologous stem cell transplantation in combination with tyrosine kinase inhibition can provide long term durable remissions in patients with Ph+ ALL who are unable to undergo allogeneic stem cell transplantation.

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### COMPARISON OF AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION WITH AND WITHOUT METAIODOBENZYLGUANIDINE (MIBG) IN PATIENTS WITH HIGH RISK NEUROBLASTOMA

Hamidieh, A.A.<sup>1</sup>, Beigi, D.<sup>2</sup>, Fallabi, B.<sup>2</sup>, Behfar, M.<sup>1</sup>, Jalili, M.<sup>1</sup>, Hamdi, A.<sup>1</sup>, Hosseini, A.<sup>1</sup>, Ghavamzadeh, A.<sup>1</sup> <sup>1</sup>Tehran University of Medical Sciences, Tebran, Islamic Republic of Iran; <sup>2</sup>Tehran University of Medical Sciences, Tebran, Islamic Republic of Iran

**Background:** Autologous Hematopoietic Stem Cell transplantation (auto-HSCT) has been considered for treatment of patients with high risk neuroblastoma. In this study we compared two main strategies of auto-HSCT for patients with high risk neuroblastoma: auto-HSCT alone in patient with negative diagnostic MIBG and auto-HSCT with therapeutic MIBG before HSCT in patient with positive diagnostic MIBG.

**Methods:** The results of 14 patients with high risk neuroblastoma who underwent auto-HSCT at our center from 2008 to 2011, were analyzed. Median age at transplantation was 4.5 years (range = 2-7, 50% male). N-Myc amplification was positive in 9 patients (64.3%). Diagnostic MIBG was asked at first visit from all patients who were referred to our center for HSCT. Patients were divided into two groups according to the result of diagnostic MIBG: MIBG-avid (n = 6) and non MIBG-avid (n = 8). MIBG-avid patients received <sup>131</sup>I-MIBG (12 mci/kg) on day 21 before transplantation. The conditioning regimen used in all patients consisted of etoposide (1200mg/m<sup>2</sup> total dose divided for 5 days), carboplatin (1500mg/m<sup>2</sup> total dose divided for 5 days), and melphalan (210mg/m<sup>2</sup> total dose divided for 3 days). The source of stem cells was peripheral blood in patients. The median numbers of MNC and CD34 injected were 10.71x10<sup>8</sup>/kg /kg, 1.7x10<sup>6</sup>/kg, respectively. All patients received 13-cis-retinoic acid (120-160 mg/m<sup>2</sup> 2 weeks per month) from day +60 to one year after transplantation.

**Results:** Engraftment occurred in all patients. The median time to neutrophil and platelet engraftment were 13 (10-18 days) and 17 days (13-21 days), respectively that was not significantly different between two groups. No severe side effects (like neutropenia, thrombocytopenia and mucositis) were observed in any patients in MIBG-avid group. With a median follow-up time of 13 months (2-35 months), 2 patients (33.3%) relapsed in MIBG-avid group (both died) and 5 patients (62.5%) relapsed in non MIBG-avid group (4 of them died).

**Conclusion:** Despite nonsignificant difference between two groups, MIBG-avid patients seem to have better survival and lower relapse rate. Therefore, much greater number of cases is needed to clarify the role of MIBG therapy in pre-transplant conditioning regimen for autologous hematopoietic stem cell transplantation in high risk neuroblastoma patients.

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### COMPARISON OF BUSULFAN + MELPHALAN TO MELPHALAN 200 MG/M2 AS PREPARATIVE REGIMEN FOR AUTOLOGOUS TRANSPLANTATION IN MULTIPLE MYELOMA

Abmed, S., Dinb, Y., Rondon, G., Andersson, B., Jones, R., Bashir, Q., Shab, N., Popat, U., Champlin, R.E., Qazilbasb, M.H., Kebriaei, P. The University of Texas, M.D. Anderson Cancer Center, Houston, TX

**Background:** High-dose chemotherapy with melphalan 200 mg/m<sup>2</sup> (Mel 200) followed by autologous hematopoietic stem cell transplan-

tation (auto-HCT) is the standard treatment for transplant-eligible patients (pts) with multiple myeloma (MM). Most patients eventually relapse after auto-HCT and in efforts to improve the efficacy of the preparative regimen, several groups have evaluated the combination of busulfan (Bu) and melphalan (Mel). We studied the safety and efficacy of a combination of Bu and Mel (Bu-Mel) in patients with advanced lymphoid malignancies, including MM. (Kebriaei P, et al., Biol Blood Marrow Transplant 2011; 17: 412-420). In this study we compared outcomes of patients with MM who received Bu-Mel with a control group of patients who received Mel 200 for auto-HCT for MM.

**Methods:** We identified 30 patients with MM in first remission who received Bu-Mel followed by auto-HCT between 1/2005 and 10/2010. They were compared to a control group (4:1) of 120 patients with MM who received Mel 200 as conditioning regimen for auto-HCT. The groups were matched for year of auto-HCT, age at auto-HCT (+/- 4 yrs), cytogenetic abnormalities, and disease status at auto-HCT. The primary objective was to study impact of conditioning regimens on complete (CR) and overall response rate (ORR), progression-free (PFS) and overall survival (OS).

**Results:** Patient characteristics and major outcomes are summarized in the attached Table. Bu-Mel and Mel 200 groups were similar in median age, renal function and chemosensitivity at auto-HCT, and time from diagnosis to auto-HCT (Table). Median time to neutrophil engraftment in both groups was 10 days (p = 0.8). There was no significant difference in 100-day transplant-related mortality (0% vs. 0.8%, p = 0.2) or grade 2-4 non-hematologic toxicity between Bu-Mel and Mel 200 (80% vs. 66%, p = 0.18) or veno-occlusive disease (none in either group). CR rates in Bu-Mel and Mel 200 were 30% vs. 34% (p = 0.82, Table). Median follow up was 27.3 months. Median PFS for Bu-Mel and Mel 200 were 24.1 and 26.2 months, respectively (p = 0.43, Figure 1). Median OS for Bu-Mel and Mel 200 has not yet been reached (p = 0.24, Figure 2).

**Conclusions:** In this large single center study with long follow up, we demonstrated that a preparative regimen of Bu-Mel is comparable to Mel 200 in safety and efficacy. The two regimens will be compared in a prospective, randomized trial.

**Table. Patient Characteristics/Outcomes**

	Bu-Mel (n = 30)	Mel200 (n = 120)	p
Males	17	68	1.00
Median Age	52.5	52	0.19
Abn Cytogenetics	12	48	1.00
High Risk CG	2	8	1.00
Serum Creat > 1.5 at TP	1	8	0.68
Median Interval Dx to TP	6.8 mo	6.7 mo	0.44
Median CD34	4.63	4.76	0.31
Chemosensitive (>I = atTP)	13%	9%	0.50
Median days to engraftment	10	10	0.81
ANC /I = 500			
CR	9, 30%	41, 34%	0.82
CR + VGPR	20, 66%	72, 60%	0.53
ORR	27, 90%	110, 83%	0.57
100-day TRM	0	1, 0.8%	0.20
Grade 2-4 AE	24, 80%	79, 66%	0.18
Median PFS	24.1 mo	26.2 mo	0.43
Median OS	not reached	not reached	0.24

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### QUALIFICATION OF CORD BLOOD UNITS FOR AN AUTOLOGOUS INFUSION PROGRAM FOR PEDIATRIC PATIENTS WITH ACQUIRED BRAIN INJURIES

Allison-Thacker, J., Fitzgerald, A., Sun, J., McLaughlin, C., Waters-Pick, B., Vinesett, R., Kurtzberg, J. Duke University Medical Center, Durham, NC

Autologous umbilical cord blood (CB) infusion for the treatment of brain injuries in young children has been studied by the Pediatric Blood and Marrow Transplant (PBMT) Program at Duke University Medical Center since 2004. The majority of infusions have been used to treat children with Hypoxic Ischemic Encephalopathy