(HIE), cerebral palsy (CP) or congenital hydrocephalus. Most of the CB units (CBU) were privately banked through parental initiatives at the time of the baby's birth. Variable testing was performed by the banks at the time of CB cryopreservation and storage. From 2004 to 2010, the program was approached by thousands of families seeking treatment for their children. Their calls, emails and letters were catalogued in a database and characterized.

CBUs were shipped to Duke from private cord blood banks (CBB) for administration after prequalification of CBB records. To qualify, the number of total nucleated cells had to deliver 1X10e7 cells/kg of recipient body weight, have negative donor screening tests and negative sterility cultures. Patient and CB samples were tested with HLA low-resolution typing to confirm patient/unit match. A CB reference sample was also shipped to Duke for potency testing including enumeration of colony forming units, viability and CD34 counts. Upon confirmation of eligibility, shipment was arranged by Duke's Stem Cell Laboratory and the private CBB.

From March 2004 to December 2010, the PBMT program received approximately 2,667 inquiries about CB stem cell treatment for children with brain injuries. Of those, 745 parents had privately banked their child's CB. When asked to provide the CB report from the CBB, 139 did not send a report and had no further contact with the program. Some children (351) were ineligible: 264 due to patient reasons including diagnosis (165), incomplete records (81), and parents declining (10); and 91 due to CB reasons including low cell count (57), positive sterility cultures (24), and bank issues (10). A total of 255 children were successfully infused with their own CB without any serious adverse events. Units were shipped from 57 different CBBs.

In summary, 27.9% of contacts had privately stored their baby's CB. Of these, 34.2% were eligible for infusion. Of ineligible patients, 25.9% were due to problems with CBU quality. The infusions were well tolerated (Sun et al, Transfusion 2010). Studies to determine efficacy in babies and children with HIE, CP and congenital hydrocephalus are ongoing.

129

OUTPATIENT STEM CELL TRANSPLANTATION USING A REDUCED-INTENSITY CONDITIONING IN TYPE 1 DIABETES MELLITUS

Cantú-Rodríguez, O.G., Treviño-Montemayor, O.R., Lavalle-González, F., Zapata-Garrido, A., Gutiérrez-Aguirre, C.H., González-Llano, O., Gómez-Almaguer, D., Villarreal-Pérez, J.Z. Facultad de Medicina, Hospital Universitario, UANL, Monterrey, Nuevo León, Mexico

Introduction: In recent years a theory of a possible reconstitution of the immune system using autologous hematopoietic stem cell transplantation (A-HSCT) has emerged, in this basis and because of the pathogenesis, patients with newly diagnosis of type 1 diabetes mellitus (DM-1) may benefit with this therapy.

Objective: To quantify the decrease in insulin requirements in patients with DM-1 who underwent A-HSCT using reduced-intensity conditioning (RIC).

Material and Methods: Prospective and experimental study, in which have been included 8 patients with newly diagnosed DM-1, confirmed by measurement of seric anti-GAD antibodies and pancreatic reserve measured from C-peptide levels, in whom was performed A-HCT using RIC on an outpatient basis. A two-day ciclofosfamide (CFM) scheme as well as G-CSF was used for mobilization. The conditioning regimen was based on a four-day CFM and fludarabine.

Results: Of the 8 cases, 6 patients had received the therapy, and 2 are in process; 5 male and 3 female patients, median of age was found in 12.5 (4-18), and the median time from the diagnosis of DM-1 is 3 months (0-9). During the 2-16 months follow-up (median 12) of patients who underwent the procedure, 2 are currently free from insulin use and the remaining have decreased their use by 80%, 50%, 35% and 0% respectively; the mean amount of CD34 + cells infused is in $10.41x10^6$ ($3.78\text{-}19.3 \times 10^6$). In 5 of the 6 patients only one recollection procedure were needed, the other patient required a second.

Conclusion: After 12 months follow-up, 5/6 patients had showed a decrease in the insulin requirements and none patient had developed any complication or needed hospitalization.

Trial Registration. ClinicalTrials.gov Identifier: NCT01121029.

130

MELPHALAN 180mg/m^2 CAN BE SAFELY ADMINISTERED AS CONDITIONING REGIMEN PRIOR TO AN AUTOLOGOUS STEM CELL TRANSPLANTATION IN MULTIPLE MYELOMA PATIENTS WITH CREATININE CLEARANCE ≤60ml/min/1.73 m^2 WITH USE OF PALIFERMIN FOR CYTOPROTECTION: RESULTS OF A PHASE I TRIAL

Abidi, M.H.[1,2], Agarwal, R.[3], Tageja, N.[4], Ayash, L.[1,2], Ventimiglia, M.[2], Deol, A.[1,2], Al-Kadhimi, Z.[1,2], Lum, L.[1,2], Ratanatharathorn, V.[1,2], Abrams, J.[1,2], Uberti, J.P.[1,2] [1] Wayne State University, Detroit, MI; [2] Karmanos Cancer Center, Detroit, MI; [3] Synergy Medical Education Alliance, Central Michigan University, Saginaw, MI; [4] NIH, Bethesda, MD

The majority of multiple myeloma (MM) patients (pts) with abnormal renal function (AbRF) receive attenuated dose of therapy. Melphalan (M) is the most effective conditioning for MM pts receiving autologous stem cells transplant (ASCT). M 140 mg/m^2 is the standard of care in pts with AbRF, as M 200 mg/m^2 resulted in severe oral mucositis (OM). Palifermin (P) as a cytoprotective agent has demonstrated efficacy in reducing the intensity & duration of OM. Due to lack of prospective data on the use of P in MM pts with AbRF, we designed this study to determine the maximum tolerated dose (MTD) of M when used with P, in order to decrease OM.

Methods: Eligibility criteria: creatinine clearance ≤ 60ml/min/1.73 m^2, age ≥18 years, Durie-Salmon stage 2/3, ECOG PS ≤ 2, no dialysis, no active OM & a suitable candidate for ASCT. Level (L) 1 began at M 140 mg/m^2 with P 60 mcg/kg/d, given as I.V bolus on Day -5,-4, -3 and Day +1, +2 & +3 (Stem cells infused on Day 0). M was given on D-2 & dose-escalation proceeded at 20 mg/m^2 increments, up to a maximum dose of 200 mg/m^2 in L4. If no symptomatic G ≥ 3 dose limiting toxicities (DLTs) were noted, an additional cohort of 3 pts was entered at the next dose level. Dose escalations were to stop if ≥ 2 DLTs occurred at a M dose (MTD). Grade (G) 4 OM, G4 diarrhea, ≥ G3 rash, & ≥ G3 cardiac toxicity were considered as DLT; G 3/4 hematological toxicity was acceptable. The G of OM was assessed daily (WHO OM scale-G 0-4).

Results: Nineteen pts were enrolled from 06/2007 to 06/2011. Data on 15 evaluable pts is reported as 4 pts were removed. Median age was 59 years (36-67).

Table.

Patient Characteristics (N = 15)	
Sex – Male- no. (%)	8 (53)
Median Age- yr (Range)	59 (36-67)
Race- Caucasian- no. (%)	12 (80)
Median Creatinine clearance (Range)	42.8 (29-60)
Disease Status at the time of Transplant– no. (%)	
CR	4 (27)
VGPR	3 (20)
PR	3 (20)
PD	5 (33)
Median no. of infused CD34+ cells x 10∧6/Kg (Range)	4.2 (2.6 - 8.0)
Median number of days to neutrophil engraftment (Range)	12 (11-21)
Median number of days to platelet engraftment (Range)	19 (0 - 86)
Median duration of hospitalization-days (Range)	16 (12 -74)

The overall incidence of OM ≥ G3 was 53% (8/15) and a median duration of ≥ G3 OM was 6.5 days (3-42). One patient in L2 (M 160 mg/m^2) developed atrial fibrillation. Two pts in L4 (M 200mg/m^2) developed G4 OM, hence reaching DLT. Three more pts were then enrolled in L3 (M 180mg/m^2). No DLT was observed in 6 pts enrolled in L3. Fourteen pts were evaluable for response at D+100 (4 CRs). One patient died in L4 due to multi-organ failure and infection. The most common adverse events include rash (13 events, no G 3), asymptomatic elevation of amylase (9, 3 pts G 3) and lipase (2, 1 pt G 3) and diarrhea (12, 1 pt G 3 (*C.Difficile +ve*)). Nine pts required narcotics; 4 pts needed TPN/NG feeding. Median duration of hospitalization for ASCT was 16 days (12-74).