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HIGH DOSE CHEMOTHERAPY FOLLOWED BY STEM CELL RESCUE IN ADOLESCENTS WITH EWING'S SARCOMA

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Ewing Sarcoma (ES) is the second most common bone tumor in children and young adults. About two thirds of patients with localized disease will have long term survival. However, patients with metastatic disease at diagnosis and patients with progressive or recurrent disease have dismal chance of survival. High dose chemotherapy (HDC) followed by autologous stem cell rescue (ASCR) has not been shown to be unequivocally helpful in a randomized clinical trial. However, several smaller studies have shown clear benefit for HDC in a subset of patients. In this case series we report three consecutive patients with recurrent ES. Each subject or guardian signed an IRB-approved consent to participate in this study. Two patients had localized recurrence of their disease while one patient had bone marrow involvement at recurrence. Patients received carboplatin, etoposide (VP), cyclophosphamide (CPM) and topotecan for salvage chemotherapy. Once a complete remission (CR) was achieved, each patient went directly to transplant using a conditioning regimen of either CPM/VP/Thiotepa (TT) (UPN1) or Busulfan/Melphalan/TT (UPN2 and UPN3). The patient with positive BM involvement was ES negative by PCR prior to transplant. These regimens were well tolerated and none of the patients experienced serious adverse events in the peri or post transplant periods. Median graft size was 5.46 and range 3.0–10.0 $\times$ 10^6 CD34+/Kg All patients had myeloid engraftment on a timely basis (range 11–13 days) and had a range in length of stay of 37–44 days. Median survival was 19 months, with range of 10–24 months. All patients are alive and disease free to date. Following transplant, Lansky/Karnofsky scores were 80–90 at 2 months and all scores were $\geq$ 90 at 6 months. Conclusions that can be drawn from this small series include: 1) HDC followed by ASCR can be well tolerated; 2) durable remission can be achieved following this therapy; and 3) transplanted patients can be expected to have good quality of life post transplant.

Patient data

Patient ID	Age at ASCR	Primary site of disease	Site of recurrence	Time to relapse (mo)	Time from dx to ASCR (mo)	Initial therapy	Length of CR post ASCR (mo)
UPN1	15	spine	spine	47	54	Adria/VP/Ifos	24
UPN2	14	nasal fossa	spine	32	37	Vin/Adria/CPM	19
UPN3	19	chest	chest, BM	41	47	Vin/Adria/CPM	10

Adria: Adriamycin; Vin: Vincristine; Ifos: Ifosfamide.

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OUTCOME OF ALLOGENEIC STEM CELL TRANSPLANTATION FOR 26 CHILDREN WITH MYELOID LEUKEMIA IN SINGLE CENTER

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Background & Objective: Children with acute myeloid leukemia (AML) have poor prognosis. Treated with chemotherapy alone, they can hardly survive if the disease is refractory or relapse. Although the onset of chronic myeloid leukemia (CML) is relatively slow and treatment with Gleevec will further prolong the survival time, it still can not be cured by the targeting treatment. Allogeneic hematopoietic stem cell transplantation will be the only way to cure these diseases. The purpose of this paper was to clarify the role of hematopoietic stem cell transplantation treating patients with myeloid leukemia. **Methods:** A total of 26 consecutive patients with AML and CML in a single institution between May 2001 and September 2006 were included. Among them, 8 were chronic myeloid leukemia (CP = 5, AP = 2, BP = 1) and 18 were AML (CR1 = 9, CR2 = 7, Non CR = 2).5 out of 9 AML got CR1 after at least 2 courses of chemotherapy. The average age was 9.4 years old(range 2 years~17 years)and the average body weight 32.8 kg (range 11.5 kg~79 kg), Patients underwent allogeneic peripheral blood stem cell transplantation (allo-PBSCT) from HLA-identical siblings (n = 2), mismatched family donors (n = 4), and matched unrelated donors(n = 20). All patients received myeloablative regimens with 16~20 mg/kg busulfan and 200 mg/kg cyclophosphomide. For aGVHD prophylaxis patients with HLA-identical sibling donors received cyclosporine (CSA) and methetraxate, while patients with matched unrelated donors received CSA, methotrexate and 15 mg/kg rabbit ATG(Fresenius). After Jan.2004 mycophenolate mofetil were used to enhance GVHD prophylaxis for CML patients. **Results:** After a average follow-up of 20.5 months(9~55 months), 2 (7.6%)patients graft rejected, 7 (27%) patients developed grade 3~4 aGVHD (all with CML), 5 patients having extensive cGVHD. At present, 9 patients have died of relapse (4/26) and TRM (GVHD 4/26 and infection 1/26) while 17 (65%) patients are still alive with disease-free survival. **Conclusion:** Our evidences is convincing that allogeneic stem cell transplantation is conducive to improve the survival rate for children with acute myeloid leukemia and the GVHD associated with unrelated donor transplants can be controlled after take active prevention measures. As to whether the GVHD happened was more severe in children with CML than that of children with AML, It still remain to have more patients and more institutions collaboration to confirm such a conclusion.

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SUCCESSFUL COMBINED UNRELATED UMBILICAL CORD BLOOD HAPLOIDENTICAL TRANSPLANT IN NON MALIGNANT DISEASE

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Over the past decade, UCB transplantation has become a viable alternative donor stem cell source for HSCT in patients with catastrophic diseases treatable with transplantation therapy. UCB cell dose is the best predictor of outcomes after UCB transplantation. In patients receiving lower cell doses, there are significant delays and sometimes failures in myeloid and platelet engraftment. Combined unit transplantation is currently under study to overcome some of these barriers. We took an alternative approach to facilitate early myeloid engraftment after myeloablative preparative conditioning for a teenager with HLH and previous Aspergillus pneumonia. Our hypothesis was that as the immunocompetent UCB cells engrafted, the subject would reject the immunologically incompetent haplo-identical HSCT graft and the patient would convert to full UCB donor. We predicted faster myeloid engraftment in this setting. A 15 year old boy with common variable immune deficiency and HLH refractory to medical therapy underwent myeloablative conditioning with standard TBI/CY/ATGAM and received a composite graft consisting of a 1) 5/6 UCB which delivered 2.5 $\times$ 10^7 TNC/KG and 2) 2 $\times$ 10^6/KG CD34 selected PSSC collected from the father. The subject engrafted on day 9. Initial chimerism was predominantly (>80%) the haplo donor. This was followed by an increase in UCB chimerism (>90% on D + 17). The subject was fully reconstituted with UCB by D + 32. The subject later developed Grade IV GVHD of liver and intestine at the time of full UCB engraftment that was treated with MSCs and liver transplantation. This combined UCB/Haplo approach has the advantage of faster myeloid engraftment than double UCB transplant in subjects at high risk of infectious complications and or graft failure.

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UNRELATED CORD BLOOD TRANSPLANTATION (UCBT) IN PEDIATRIC PATIENTS: A SINGLE CENTER EXPERIENCE IN CHILE

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Umbilical cord blood (CB) from unrelated donors is being used increasingly in pediatric stem cell transplantation and it represents