limited to skin, and limited chronic oral GVHD. Neither patient developed EBV-PTLPD. $CD3^+CD4^+ > 400/mm^3$ was achieved by days +174 and +307, and each patient exhibited normal mitogen responses to phytohemagglutinin and pokeweed by days +275 and +384. Vaccine responses to diphtheria and tetanus were noted in the patient in whom titers were studied. There were no unexpected toxicities related to the HSCT procedure, and both patients remain alive greater than one year after HSCT with Lansky scores of 100. Both are 100% donor chimerism by VNTR analysis, are transfusion independent, had resolution of eczema, and have experienced complete immune reconstitution. With these patients, we have demonstrated that rapid hematopoietic engraftment and complete immune reconstitution can be achieved with limited toxicity in patients with WAS who receive a reduced intensity conditioning followed by a T- and B-cell depleted haploidentical graft with specific T-cell add back.

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SUCCESSFUL OUTCOME OF ALLOGENEIC STEM CELL TRANSPLANTATION FOR HIGH-RISK PAEDIATRIC ACUTE MYELOID LEUKAEMIA USING CHEMOTHERAPY-ONLY CONDITIONING AND POST TRANSPLANT IMMUNOTHERAPY

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Objectives: The role of allogeneic stem cell transplantation (SCT) in paediatric acute myeloid leukaemia (AML) remains controversial. SCT has generally been advocated for poor risk disease in first complete remission (CR), the majority of patients following relapse, and as a last resort in refractory disease. **Methods:** Twelve consecutive patients (median age 2.2 years) underwent SCT for high risk AML between 2000 and 2006. Patient and graft characteristics are shown in the table.

Conditioning regimen consisted of busulphan (Bu) 16 mg/Kg, cyclophosphamide (Cy) 120 mg/Kg, melphalan (Mel) 140 mg/m^2; alemtuzumab 1 mg/Kg was added in the unrelated donor setting.

Immunotheraputic strategies were employed to maximise a graft-versus-leukaemia (GVL) response escalating through a reduced dose of alemtuzumab, early taper of cyclosporine A (CSA), donor lymphocyte infusion (DLI) and treatment with alpha-interferon (α-IFN). **Results:** All patients engrafted. Three out 5 patients undergoing matched unrelated donor SCT, who were not in remission, received a 50% reduced dose of alemtuzumab. In 8 children CSA was stopped early < 2 months (n = 4) and < 3 months (n = 4). Six of 12 patients developed acute graft versus host disease (aGVHD), 3 grade 2, 3 grade 1. Five patients have developed limited chronic GVHD (cGVHD). In one patient who developed recipient T-cell chimerism 2 months after SCT despite reduced dose alemtuzumab and early taper of CSA, a DLI of 10^6/Kg was administered followed by α-IFN. He developed grade 1 aGVHD and the donor T cell chimerism increased to 80% but he never achieved full donor haemopoiesis and relapsed 17 months after SCT. In another patient, who developed no GVHD despite cessation of CSA at day 32, α-IFN was administered: he developed grade 1 aGVHD evolving to limited cGVHD and remains in remission at 9 months.

Ten of 12 (83%) patients are alive in CR at a median of 33 months (range 9–80) from SCT. There was no transplant related mortality, but two patients relapsed 3 and 17 months after SCT and subsequently died. **Conclusion:** This early promising data suggests that the combination of Bu Cy Mel should be studied further as a conditioning regimen in children with high risk AML. Given the substantial risk of relapse, and in the absence of GVHD, this approach should be used as a platform for escalating immunotherapeutic strategies with the endpoint of sustaining 100% donor chimerism or achieving non severe GVHD to maximize the potential for a GVL effect.

Patient	Age at SCT	FAB type	Antecedent	Cytogenetics	Disease phase	Donor type	GVHD prophylaxis
1	2.0	M6		3q del, trisomy 21	CR1	MMUD	alemtuzumab CSA MTX
2	2.2	M7		complex	CR1	MUD	alemtuzumab CSA MTX
3	1.7	M6		complex	resistant disease	MUD	alemtuzumab CSA MTX
4	2.7	M5		normal	partial remission after relapse	MMUD	alemtuzumab CSA MTX
5	1.6	M5		t(1;7)(q23;p22) t(7;9;11)	CR2	MMUD	alemtuzumab CSA MTX
6	2.1	M0		t(7;12)	CR2	MMUD	alemtuzumab CSA MTX
7	3.6	M7		normal	partial remission after relapse	MSD	CSA
8	1.4	M0		normal	partial remission after relapse	MUD	alemtuzumab CSA MTX
9	5.7	secondary AML		monosomy 7 trisomy 8	CR1	MMUD	alemtuzumab CSA MTX
10	2.4	M7	MDS	monosomy 7	transformed to AML	MSD	CSA MTX
11	5.4	M2	MDS	monosomy 7	transformed to AML	MUD	alemtuzumab CSA MTX
12	2.2	M5	JMML	del 9q	transformed to AML	MUD	alemtuzumab CSA MTX

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A NOVEL NON-MYELOABLATIVE CONDITIONING REGIMEN FOLLOWED BY ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR PEDIATRIC PATIENTS WITH NON-MALIGNANT HEMATOLOGICAL DISORDERS

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Purpose: To evaluate the efficacy of using a novel non-myeloablative conditioning regimen followed by allogenic hematopoietic stem cell transplantation (HSCT) for pediatric patients with non-malignant hematological disorders. **Paptients and Methods:** Eight patients less than 18 years of age with benign hematological diseases and high risk features were transplanted from HLA identical related donors between October, 2004 and August, 2007. Six patients had sever thalassemia major, one patient had sickle cell anemia and one had pure red cell aplasia. Seven were males and one female. Median age was 12 years (5–16). All patients had high serum ferritin (1500–6000 ng/mL). All patients had significant palpable hepatomegaly (4–8 cm). Four patients had hepatitis C infection, 6 patients had grade II-III hepatic fibrosis on liver biopsy and one patient had uncontrolled Nephrotic syndrome.

All patients received non-ablative conditioning regimen consisted of: Busulphan (2 mg/kg twice daily for two days), Fludarabine (35 mg/m^2/day for 5 days), Horse ATG (30 mg/kg/day for 5 days) and total lymphoid irradiation (500 cGy on Day zero). Cyclosporine and oral Mycophenolate Mofetil were used as Graft versus Host Disease (GvHD) prophylaxis. Six patients received mobilized peripheral stem cells while two patients received G-CSF primed bone marrow cells. **Results:** After a median follow up of 13 months (2–36), all patients are alive and doing well with no blood transfusion requirement. Mean CD34 graft dose was 4.4 × 106/kg. All patients showed primary neutrophil engraftment at a median of 19 days (12–24). Four patients have sustained full donor chimerim, while 3 patients have stable mixed donor chimerism (52–94%), only one patient had graft failure 10 months after HSCT and he is now doing well with full donor chimerism 20 months following second non-ablative transplantation.

No patient required ICU admission post transplantation. Four patients developed acute veno-oclusive disorder within first 30 days after HSCT; all were salvaged with supportive treatment. Three patients had Grade II-III acute GvHD. Two patients had Grade I-II chronic GvHD. Two patients developed posterior