limited to skin, and limited chronic oral GVHD. Neither patient developed EBV-PTLPD. CD3+CD4+ > 400/mm3 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 chimera 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 regimen followed by a T- and B-cell depleted haploidentical graft with specific T-cell add backs.

212 SUCCESSFUL OUTCOME OF ALOGENIC 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/m²; alemtuzumab 1 mg/Kg was added in the unrelated donor setting.

Immunotherapeutic strategies were employed to maximise a graft-versus-leukaemia (GVL) response escalating towards 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 107/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 chimera or achieving non severe GVHD to maximise the potential for a GVL effect.

213 A NOVEL NON-MYEOBLATIVE CONDITIONING REGIMEN FOLLOWED BY ALOGENIC HEMATOPOIETIC STEM CELL TRANSPLANTATION FOR PEDIATRIC PATIENTS WITH NON-MALIGNANT HEMATOLOGICAL DISORDERS

Purpose: To evaluate the efficacy of using a novel non-myeloblastic conditioning regimen followed by allogeneic hematopoietic stem cell transplantation (H SCT) for pediatric patients with non-malignant hematological disorders. Patients 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 severe 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/m²/day for 5 days), Horse ATG (30 mg/kg/day for 5 days) and total lymphoid irradiation (500 Gy 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 4.8 × 106/kg. All patients showed primary neutrophil engraftment at a median of 19 days (12–24). Four patients have sustained full donor chimera, while 3 patients have stable mixed donor chimera (52–94%), only one patient had graft failure 10 months after HSCT and he is now doing well with full donor chimera 20 months following second non-ablative transplantation.

No patient required ICU admission post transplantation. Four patients developed acute veno-occlusive 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
reversible encephalopathy syndrome within 50 days post transplant, all of them showed complete clinical and radiological resolution. **Conclusion:** Non-myeloablative HSCT using this conditioning regimen for high risk pediatric patients with benign hematological disorders appears to be promising and worth further study.

### 214 PERICARDIAL EFFUSION (PEF) IS A MAJOR INDEPENDENT RISK FACTOR ASSOCIATED WITH A SIGNIFICANT DECREASE IN SURVIVAL IN PEDIATRIC STEM CELL TRANSPLANTATION (SCT) RECIPIENTS

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**Background:** SCT is the treatment of choice for a variety of pediatric conditions. Potential post SCT morbidities include cardiac toxicity. Rhodes et al (BMT 2005) reported that PEF was seen in 4.4% of pediatric SCT recipients. Risk factors for the development of PEF have been thought to include GVHD, infection and/or underlying disease. There is a paucity of information regarding the etiology, prognosis, treatment and outcome of PEF in a large cohort of pediatric SCT recipients. **Objectives:** To assess the incidence, risk factors, outcome of PEF and the impact of PEF on overall survival (OS) in pediatric SCT recipients. **Methods:** Echocardiograms were performed at baseline prior to 200 SCT (4 were ineligible for review) in 156 patients and when patients had symptoms and/or signs of cardiac or pericardial disease post SCT. Probability of and time to PEF were analyzed by Kaplan-Meier method and risk of PEF and death were determined by multivariate analysis. **Covariates** included age, gender, ethnicity, conditioning regimen, risk status (CIBMTR criteria), conditioning regimen, donor source, CMV status, GVHD, and HLA match. **Results:** The mean age was 8.15 years (+/-0.25 years) with 88 males and 68 females. 102 patients received allogeneic transplants, 34 of them received more than 1 transplant. 116 of 156 recipients had malignant disease. 100 of 156 patients had ablative conditioning. The incidence of PEF was 14.7%. A multivariate analysis shows that older age, poor risk and unrelated cord blood donor recipients are significantly associated with a risk of developing PEF with hazard ratios of 1.125 (1.051–1.205), 3.508 (1.502–8.191), and 5.080 (1.141–22.625), respectively. OS was significantly decreased in patients with PEF versus without PEF (hazard ratio = 4.84, 95% CI, 2.814–8.322, p < 0.0001). Furthermore, in a multivariate analysis, there was a significant decrease in OS associated with PEF, CMV status and poor risk status with hazard ratios of 3.296 (1.73–6.26), 1.89 (1.08–3.30) and 1.91 (1.12–3.31), respectively. **Conclusion:** These results demonstrate an almost 15% incidence of PEF in pediatric SCT recipients. Older, poor risk and UCB donor recipients may be at higher risk of developing PEF. PEF was associated with the most significant impact on overall mortality independent of other risk factors. Improved prevention and therapeutic strategies for development of PEF in post allo SCT recipients may potentially reduce mortality in the future.

### 215 HEMATOPOIETIC STEM CELL TRANSPLANTATION (HSCT) IN WISKOTT ALDRICH SYNDROME (WAS): A SINGLE CENTER EXPERIENCE IN 25 PATIENTS

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WAS is a rare X-linked disease characterized by eczema, microthrombocytopenia, recurrent infections, autoimmune complications and malignancies. The gene responsible for WAS has been identified and termed WASP. Without a successful HSCT, the prognosis of classical WAS with a complete absence of WASP expression is poor. In this report we analyze the outcome of 25 pts transplanted in a single center in a developing country.

### 216 IRON OVERLOAD IN PEDIATRIC HEMATOPOIETIC STEM CELL TRANSPLANT (HSCT) PATIENTS

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**Background:** Iron can be a highly toxic molecule to cells and tissues when it interacts with oxygen and generates free radicals. Free iron, ferritin and transferrin saturation have been shown to increase susceptibility to infections and iron overload following allogeneic HSCT performed between 1992 and 2007. Guidelines exist for iron chelation therapy. We report here on 21 patients aged 0-16 years following HSCT. The prevalence of iron overload has not been defined in this population, and currently, no management guidelines exist. We describe a group of pediatric HSCT patients who were diagnosed with iron overload after developing liver function abnormalities and/or hyper-ferritinemia. **Objectives:** We performed a detailed retrospective review of patients identified as having iron overload following allogeneic HSCT performed between 2001–2007. We included a subset of patients with known...