were "alive and engrafted" mixed-chimerism (>5% recipient) at latest follow up time point (median 57mths: 6-140) was found in 21%. The highest rate of full donor chimerism was found in uCB (93%), compared to 73% in MSD, 64% in UD and 75% in TCDud. Normal enzyme levels at latest follow up time point were found in all (100%) of the uCB, in 53% of MSD, in 74% of UD and in 75% of TCDud recipients.

Outcomes following SCT is HS are encouraging. Younger age at SCT is a predictor for higher OS. Regarding OS, MSD do better compared to UD and uCB, but no difference was found for EFS. TCDud is a predictor for lower OS/EFS. uCB leads to higher donor-chimerism levels and enzymes which is suggested to have a positive impact on the long term outcomes.

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MYELOABLATIVE (MAC) AUTOLOGOUS STEM CELL TRANSPLANTATION (AUTOSCT) FOLLOWED BY REDUCED INTENSITY (RIC) ALLOGENEIC STEM CELL TRANSPLANTATION (ALLOSCT) IN CHILDREN, ADOLESCENTS AND YOUNG ADULTS (CAYA) WITH POOR RISK HODGKIN'S LYMPHOMA (HL): INDUCTION OF LONG TERM GVHL EFFECT

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Background: Long-term EFS in CAYA with poor risk HL who are induction failures or who relapse or progress following initial therapy is poor (≤30% 10 year EFS) due to relapse and secondary MDS or malignancy (Bradley/Cairo et al., BMT, 2008). Recent data in poor risk adult HL suggests a strong GVHL effect following RIC AlloSCT (Peggs et al., Lancet, 2005 and Sureda et al., JCO, 2008). A recent review by the EBMT reports encouraging results in 51 children and adolescents with chemosensitive HL who received RIC AlloSCT (Claviez et al., Blood, 2009). However, 63% of these patients had already failed MAC AutoSCT, reducing the number of pts who were chemosensitive prior to RIC AlloSCT.

Objective: We prospectively evaluated the safety and efficacy of MAC AutoSCT followed by RIC AlloSCT in CAYA with poor-risk HL.

Methods: Poor-risk HL pts achieving CR, PR, or SD after re-induction were eligible to receive CBV conditioning (Harris/Cairo, ASH, 2004) followed by RIC with busulfan (6.4 mg/kg), fludarabine (180 mg/m^2) ± R-ATG (for unrelated SCT). GVHD prophylaxis consisted of MMF and tacrolimus as we have previously described (Osunkwo/Cairo et al., BBMT, 2004), with discontinuation of MMF on day 30/60 (related/unrelated AlloSCT) and a 4-6 week tacrolimus taper.

Results: Ten pts, median age 18.4 (range 12.3-21.8), M/F 6/4 and disease status at MAC AutoSCT CR (5), PR (3), SD (2). Two refused RIC AlloSCT; of the 8 allografts, 1 related/7 unrelated, 5 UCBT/3 PBSC, HLA matching 6/6 (1), 5/6 (1), 4/6 (5), 8/10 (1), median TNC dose 0.35×10^8/kg (0.09-6.13) and CD34 0.59×10^6/kg (0.04-5.30). All pts (n = 8) engrafted neutrophils following RIC AlloSCT at median day 19 (15-45). Six of 7 evaluable pts engrafted platelets at median day 46 (11-170). The probability of ≥grade II acute GVHD was 37.5% (95% CI 0-63%) and of chronic GVHD 30% (0-58%). Patients achieved median percent donor chimerism of 100%, 100% and 98%, on days 100, 180 and 365, respectively. Probabilities of 1-year and 2-year OS (all, n = 10) were 90% (73-100%) and 80% (59-100%) respectively. Three pts died on days 338, 370 and 746 of varicella, progressive HL and chronic GVHD/aspergillosis, respectively. Seven pts are currently living at a median follow-up of 2191 days.

Conclusions MAC AutoSCT followed by RIC AlloSCT for CAYA with poor-risk HL is feasible and results in excellent early OS. Long-term follow-up and a larger cohort are needed to determine whether this approach improves 10-year EFS compared to MAC AutoSCT alone.

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SINGLE CENTRE EXPERIENCE OF HEMATOPOEITIC STEM CELL TRANSPLANTATION FOR PATIENTS WITH COMPLEX AUTOIMMUNE ENTEROPATHIES

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Background: Complex autoimmune enteropathies encompass a wide range of disorders including X-linked immune dysregulation, polyendocrinopathy, enteropathy (IPEX) syndrome with FOXP3 gene mutation, IPEX-like syndrome (similar features as IPEX without gene mutation) and other unclassified autoimmune enteropathies.

Method: A retrospective study of patients with complex autoimmune enteropathies who underwent hematopoeitic stem cell transplantation (HSCT) at Newcastle General Hospital, one of two nationally designated centres for such procedures in the UK, was performed.

Results: 13 patients were identified who fulfilled the inclusion criteria; 2 (15%) IPEX, 8 (62%) IPEX-like and 3 (23%) unclassified autoimmune enteropathy. Median age at the time of HSCT was 2.5 years, ranged between 5 months to 19.5 years old. 9/13 (69%) patients were male. All underwent HSCT with 10/13 (77%) unrelated (URD) including 3 cord transplant, and 3/13 (23%) matched sibling (MSD). The conditioning regimen used was Fludarabine/Melphalan in 6 (46.2%), Busulfan/Cyclophosphamide in 3 (23%), Treosulfan/Cyclophosphamide in 2 (15.4%) and Treosulfan/Fludarabine in 2 (15.4%). 100% donor chimerism was achieved in 10 (77%) patients including one after the unconditioned boost infusion 27 days post HSCT. 3 (23%) patients have mixed donor chimerism with 60% donor T cells. Ten (80%) patients are alive with resolution of enteropathy and have discontinued immunosuppression, one remains an inpatient after HSCT about 100 days ago. 5/13 (38%) patients had no graft versus host disease (GvHD) and 6/13 (46%) had only grade II-III skin GvHD. Two (15%) died from complications related to treatment of grade III skin and gut GvHD.

Conclusion: HSCT can be curative in patients with severe complex autoimmune enteropathy. GvHD is a common significant complication which can be life threatening.

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SUCCESSFUL HALF-DOSE BUSULFAN/FULL-DOSE FLUDARABINE BASED REDUCED INTENSITY CONDITIONING IN HIGH-RISK PEDIATRIC AND ADULT CHRONIC GRANULOMATOUS DISEASE (CGD) PATIENTS

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Objective: To examine the feasibility, safety and efficacy of hematopoietic stem cell transplantation (HSCT) in high-risk pediatric and adult patients with chronic granulomatous disease (CGD) using a low-toxicity conditioning regimen based on half-dosed (50-60%) Busulfan, full-dose Fludarabine and in-vivo T-cell depletion.

Patients and Methods: 11 CGD patients (5, 7, 12, 13, 17, 18, 20, 23, 29, 35 and 39 ys; n = 8 gp91-phox, n = 2 p22-phox and n = 1 p47 phox deficient) are described. All patients were therapy-refractory to conventional treatment suffering from infectious and/or inflammatory complications at HSCT, e.g. colitis, active infection (Aspergillus, Neisseria, Actinomyces, Staph. aureus) or had former lung aspergillosis. Stem cell donors consisted of 5 matched sibling, 4 MUD (10/10) and 2 MMUD (9/10) donors. Conditioning included 180 mg/qm Fludarabine (d -8 to -3), oral/iv. Busulfan (6.4-12 mg/kg; d -4 to -2; in pediatric patients with adjusted Busulfan kinetics) and Antithymocyte-Globulin Fresenius (4 × 10 mg/kg; d -4 to -1). In 4 patients (5, 7, 12 and 17 ys) receiving MUD or MMUD transplants, ATG-Fresenius was replaced by 0.5 mg/kg Alemtuzumab (Campath IH) (d -8 to -6). As stem cell source, bone marrow (2.3 to 6.0 × 10 6 CD34/kg) was used in the majority of cases (n = 10),