Methods: The surveyed centers accounted for 75% of all pediatric HSCT and were identified by querying the CIBMTR. Two individuals from each of these institutions were survey recipients; one from the Division of Pediatric Critical Care Medicine (PCCM) and the other from the Division of HSCT. The survey was conducted electronically using SurveyMonkey allowing data to remain anonymous. A four-choice Lickert scale was used for clinical questions.

Results: Completed surveys were returned from both the HSCT and PCCM physicians in 22 programs. The percentage of programs that always or commonly perform the following interventions on the HSCT unit are as follows: central venous pressure monitoring (18%), 100% non-rebreather mask (50%), non-invasive ventilation(18%), intermittent hemodialysis (41%), continuous veno-venous hemofiltration (0%), dopamine (36%) with the maximal dose varying by center. In terms of care by PCCM physicians, most all programs use 100% oxygen, non-invasive ventilation and aggressive diuresis prior to intubation; 2/3 of programs attempt renal replacement therapy (RRT). 59% of PCCM physicians always or commonly offer intubation as a time limited trial to HSCT patients; 18% never offer this option. Non-conventional respiratory therapies commonly offered to HSCT patients include high frequency oscillatory ventilation, nitric oxide and RRT. Surfactant use is less common and ECMO use is rare.

Conclusion: There is a notable variation in the level of monitoring and support offered on pediatric HSCT units as well as therapies provided by PCCM physicians to HSCT patients. Not only is this data is important for the design of clinical trials, but studies should be done to determine if these practice variations impact patient outcomes.

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SURVIVAL FOLLOWING ALLOGENEIC STEM CELL TRANSPLANTATION FOR CONGENITAL IMMUNODEFICIENCY AND METABOLIC DISORDERS

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We compared outcomes of allogeneic stem cell transplantation (SCT) for congenital immunodeficiency and metabolic disorders to children of a similar age who received SCT for acute leukemia in first or second complete remission. This analysis was undertaken to address a reluctance among many physicians to refer children with certain congenital immunodeficiency or metabolic disorders for early SCT. The study population includes 343 children (≤5 years) with congenital immunodeficiency (129 with severe combined immunodeficiency [SCID]; 214 with non-SCID) and 354 children with metabolic disorders. This population was compared to 622 age-matched children with acute leukemia (the control population). All transplantations occurred between 1995 and 2005 and the transplant conditioning regimen was myeloablative. In univariate analysis, 5-year overall survival was higher after HLA-matched sibling donor transplants for SCID (80%), non-SCID (84%) and metabolic disorders (78%) compared to the control group (51%, $p<0.001$). Amongst recipients of unrelated donor SCT, 5-year overall survival was higher for non-SCID patients compared to controls (66% vs 50%, $p = 0.05$). The 5-year survival rates were similar after unrelated donor SCT for SCID (41%), metabolic (51%) and good risk acute leukemia, the control group (50%). In multivariate analysis, donor type was the only factor associated with survival; mortality rates were higher after unrelated donor SCT (odds ratio 1.49, 95% CI 1.02–2.16, $p = 0.04$). Further, we observed no differences in survival after unrelated donor bone marrow and cord blood SCT. The data suggest children with congenital immunodeficiency and metabolic disorders who receive an unrelated donor SCT have similar survival rates as children with good risk acute leukemia. The sub-group of children with non-SCID diseases fare the best, with a 5-year overall survival that exceeds that of age-matched patients with acute leukemia. Therefore prompt referral for SCT for these children, before mounting comorbidities render them ineligible, should be encouraged even in the absence of an HLA-matched sibling donor.

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REDUCED TOXICITY MYELOABLATIVE CONDITIONING REGIMEN WITH BUSULFAN, FLUDARABINE AND ALEMTUZUMAB FOR CHILDREN WITH NON-MALIGNANT DISORDERS, MYELODYSPLASTIC SYNDROME (MDS) AND MYELOID MALIGNANCIES

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Major problems with Busulfan/Cyclophosphamide (Bu/Cy) conditioning are acute toxicities and graft failure. In 2000, we replaced Bu/Cy conditioning with targeted i.v. Busulfan/Fludarabine/rabbit ATG (Bu/Flu/rATG) which effectively reduced acute toxicities; however, graft failure rate remained high (21%). In 2004, we introduced targeted i.v. Busulfan/Fludarabine and Alemtuzumab (Bu/Flu/Campath) to reduce graft failure rate. We describe and compare the outcomes of the first 21 Bu/Flu/Campath study patients (median age 8, range 1–16 years) with those of previously published 19 By/Flu/rATG study patients. Diagnoses included MDS or myeloid malignancy (N = 5), hemoglobinopathy (N = 3), non-SCID primary immunodeficiency (N = 6), aplastic anemia (N = 2), autoimmune disorders (N = 2), and metabolic disorders (N = 3). The donors included ≥8/8 HLA-matched (N = 6) and one antigen mismatched (N = 4) unrelated volunteers, and fully matched related donors (N = 11). Stem cell sources were peripheral blood (N = 9), bone marrow (N = 10) or combined sources (N = 2). 19/20 evaluable patients engrafted. One patient with Hurler's disease developed graft rejection 40 days post transplant but was rescued with a 2nd transplant. The median follow-up of living patients is 21 months (range 4.3–51 months). Estimated 2-year post-transplant overall and event-free survival rates were 83 ± 9% and 71 ± 11%, respectively, which is similar to survival in the Bu/Flu/rATG study (89 ± 7% and 74 ± 10%). Three patients died (at 1, 5, and 12 months post transplant) due to intracranial hemorrhage in 1 patient with Evans syndrome, Aspergillus infection and GVHD in 1 patient with adrenal leukodystrophy (ALD), and disease progression and GVHD in 1 patient with ALD. One patient with MDS relapsed 1 year following the transplant, but is alive following the 2nd successful transplant. CMV reactivated in 47% (7/15) patients who were at risk for reactivation and was successfully treated in all. Only 1 patient developed mild VOD (maximum total bilirubin 3.4 mg/dl). The incidence of acute and chronic GVHD was 15% each, respectively. At last follow up 85% of patients remain mixed chimeras. Bu/Flu/Campath conditioning was well tolerated and resulted in durable mixed chimerism in the majority of patients. Compared with Bu/Flu/rATG, Bu/Flu/Campath conditioning resulted in significantly reduced graft rejection rate (2-tailed Fisher's exact $p = 0.03$) in mismatched MUD transplants, but it did not improve overall survival or event free-survival.

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SECOND ALLOGENEIC STEM CELL TRANSPLANTATION (SCT) IN PEDIATRIC PATIENTS

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Graft rejection and/or disease relapse after allogeneic SCT are ominous events that are often associated with dismal outcome; second SCT may be considered as a salvage alternative in these patients (pts) but is generally regarded as a procedure that is associated with high morbidity and mortality. In the pediatric population, the data on the outcome of second SCT are sketchy and scarce. We present here our experience in 51 pediatric pts who underwent a second SCT at our institution (KFSHRC).

Patients and methods: From May 1996 until May 2008, 51 pts with graft failure or disease relapse after SCT (24 females and 27 males) underwent second SCT at KFSHRC; 17 pts had leukemia, 16 pts had non-malignant hematological disorders (NMHD), 16 pts had immune deficiency disorders (IMD), and 2 pts had metabolic disorders. 35 pts were reconditioned with myeloablative regimens (including 19 pts with TBI-regimens), 7 pts with