Trends and Indications of Hematopoietic Stem Cell Transplantation in Pediatric Population below Two Year Old: Insight from Nationwide Inpatient Sample

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Background: Hematopoietic Stem Cell Transplant (HSCT) is a well-established therapeutic modality for hematological and non-hematological diseases among different age groups. However, the characteristics, causes and trends of HSCT in pediatric population below 2 years are not well identified. We aimed to evaluate the most important features of HSCT in this age group.

Methods: We used the Nationwide Inpatient Sample (NIS) 1997-2014 to identify patients with HSCT by using the International Classification of Diseases, Ninth Revision ICD-9 codes. We excluded patients >2 years. Through using ICD-9 codes, patients were grouped to autologous bone marrow transplant, allogenic bone marrow transplant, and cord blood transplant. In-patient all-cause mortality and trends over years were identified.

Results: A total of 958 Hematopoietic Stem Cell Transplant HSCT patients were identified through Nationwide Inpatient Sampling NIS database from 1997 – 2014. Among HSCT recipients 60.6% were male, 39.4% were female, and 58.6% were white race. Allogenic transplant was the most common 515/958 (53.7%), autologous transplant 289/958 (30.2%), and cord blood transplant 154/958 (16.1%). The most common indications for transplant were Combined Immunodeficiency (n = 180), Acute myeloid leukemia (n = 106), Brain neoplasm (n = 116), Acute lymphoblastic leukemia (n = 82), Neuroblastoma (n = 92), Mucopolysaccharidosis MPS (n = 52), Wiskott Aldrich Syndrome WAS (n = 32) and Myelodysplastic syndrome MDS (n = 6), figure 1. Sixty-three HSCT recipients had in-patient graft versus host disease GVHD post-transplant (6.6%); 18 cases were detected in cord blood transplant, 44 cases in allogenic transplant and one case was detected with autologous transplant in acute myeloid leukemia patient. Overall hospital mortality of the whole cohort was 8.9% (85/958), of which 9.4% (8/85) occurred among GVHD patients. Trends over years are shown in figure 2.

Conclusion: Leukemia, combined Immunodeficiency, brain tumors, neuroblastoma, and MPS are the top five indications for Hematopoietic Stem Cell Transplant in the first two years of life. Inpatient hospital mortality burden in this group was 8.9% and it is trending down over the years. Graft versus host disease wasn’t associated with high percentage of in-hospital mortality and it is interesting that no cases of in-patient Graft versus host disease post-transplant were identified before 2008.
HSCT FOR AUTOIMMUNE DISORDERS

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Autologous Hematopoietic Stem Cell Transplantation in Refractory Crohn’s Disease. Experience of Tertiary Medical Center in Brazil

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Background: Crohn’s disease (CD) is a chronic inflammatory and relapsing disease that can affect any segment of the gastrointestinal tract. A significant percentage of these patients suffer an aggressive disease course, refractory to conventional clinical therapy. Surgery may be required, however the disease tends to recur after the resection.

Aim: Describe the potential clinical benefit of autologous hematopoietic stem cell with T-lymphocyte-depletion as a treatment for aggressive and refractory CD patients. Evaluate its efficacy, safety, toxicity and adverse events and primary and secondary clinical outcomes of follow up after 1 year and 3 years.

Methods: We evaluated 6 patients submitted to an autologous HSCT. All patients were mobilized with cyclophosphamide (Cy) and G-CSF at the dose of 10mcg/kg/dia. The collection of HSC was initiated when the CD34+ count in the peripheral blood was greater than 15cells/mm3. The harvested product was enriched with CD34 + cells through a Clinimacs (Miltenyi Biotec) with a target CD34 + cells above 10E6/kg and less than 1 × 10E4Tcells/kg. The conditioning regimen consisted of cyclophosphamide 50mg/kg for 4 consecutive days, thymoglobulin 1.5mg/kg and methylprednisolone 1g/day from d-5 to d-2.

Results: Five patients refractory CD, between 43y and 19y (median age 38y), were included and follow the autologous HSCT. Corporal index mass 20 Kg/m2, median course of illness 14y, all negative for X-linked inhibitor apoptosis protein (XIAP). Four patients had penetrating disease and two patients had nonstricturing nonpenetrating phenotype. Five patients had 2 to 3 previous intestinal surgeries. All patients used amisulodine and methylprednisolone 1g/day from d-5 to d-2.

After 1y HSCT, all patients achieved the 1st remission (CDAI <150). All patients achieved endoscopic remission (fig) and were steroid and immunosuppressive free remission (CDAI <150). All patients are alive without any malignancy complications, the longest period is 3y, and one restarted ADA as monotherapy (D+722).

Conclusion: HSCT is a complex treatment strategy for severe CD associated with mortality and serious adverse events.