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POSTER ABSTRACTS

114.SICKLE CELL DISEASE, SICKLE CELL TRAIT AND OTHER HEMOGLOBINOPATHIES, EXCLUDING THALASSEMIAS: CLINICAL AND EPIDEMIOLOGICAL

Comparison of Outcomes of Hematopoietic Cell Transplantation (HCT) for Asymptomatic Patients with Sickle Cell Disease (SCD) and That of Propensity Matched Symptomatic Patients Undergoing HCT

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Introduction
Hematopoietic cell transplantation (HCT), a potential curative option for sickle cell disease (SCD) has typically been offered to patients with severe disease manifestations. With improved outcomes, especially in young children receiving HCT from HLA identical donors, some clinicians are performing HCT for patients without severe manifestations. HCT is associated with risk of substantial short-term morbidity, death, and long-term sequelae. Performance of HCT in patients who have not yet suffered from complications of SCD, raises unique issues of cost-benefit tradeoff, ethics, and healthcare equity. Further, it is unknown whether patients who have not suffered complications of SCD would have better outcomes following HCT. The objective of this study was to compare the outcomes for asymptomatic patients in patients undergoing HCT for SCD and propensity matched symptomatic SCD patients undergoing HCT.
Methods: We queried the HCT for SCD dataset of patients undergoing HCT between 1990-2022 and reported to the Center for International Bone Marrow Research (CIBMTR) registry. Transplant essential data had been submitted for all patients while a subset of patients was assigned to the comprehensive research form (CRF) track. On these patients HCT centers submitted detailed SCD specific baseline data, as well as outcomes relevant to SCD and organ specific complications. The main reason for HCT is one of the variables collected in the CRF. Thus, we selected patients in the CRF track for further study. We describe demographics, baseline characteristics, transplant essential outcomes including overall survival (OS) and event free survival (EFS), graft failure (GF), Acute graft versus host disease (aGVHD) and chronic GVHD (cGVHD), venocclusive disease of the liver (VOD), post-transplant pneumonia/acute chest syndrome or other pulmonary complications(ACS), nephropathy, stroke, avascular necrosis (AVN), vasocclusive crises (VOC). We created a propensity score model, adding baseline variables. For each categorical outcome variable, frequency tables and tests of difference in proportions were derived. Descriptive statistics and tests of difference in means were derived for the two numeric outcome variables. In addition, survival analysis (Cox regression modeling) was conducted to determine the time until an individual experienced graft failure or death and whether event rates were associated with group classification adjusting for age and donor type.
Results: For the entire cohort of 1718 patients undergoing HCT from 1990 to 2022 patients were followed for a median 47.8 months (0.3-312.9), 73.4% of patients with age at HCT <18 years and 74.7% with Karnofsky-Lansky score $\geq$ 90. The 3-year OS was 91.2%, 3-year EFS 75.5%, and GF rate was 17.9%, aGVHD grades II-IV occurred in 18.3%, and cGVHD developed in 22.3%. A total of 763 patients were assigned to the CRF track. The main reason for HCT was reported for these patients, and 49 patients were reported to be asymptomatic at baseline. In the asymptomatic patients majority of the HCT were performed for patients under the age of 10 years (n = 33) in patients with Hemoglobin SS (n = 45) and most occurred between 2013-2018 (n = 24). Average follow up period was 48 months. The most common graft source and conditioning regimens utilized were bone marrow (n = 35) HLA identical sibling donor type (63.2%, n = 31), and reduced intensity conditioning (n = 25) respectively. Other donor types included HLA mismatched relative (n = 9), mismatched unrelated donor and cord blood (n = 5), and matched unrelated was used in 4 patients. There was one death, at 16.5 months with cGvHD. GF occurred in 14.3%, OS was 97.9%, EFS 83.67%, 20.4% developed aGvHD and 28.5% developed cGvHD and none developed secondary malignancies or post-transplant lymphoproliferative disorders. Propensity matched control group (Figure 1) was constructed based on age at HCT, donor type, graft type, HCT comorbidity index, SCD type. There was good variable balance with a small standardized mean difference for matched observations. There were no statistically significant differences in least mean squares for EFS, death, OS, GF, AGVHD, CGVHD, VOD, VOC, AVN, stroke or ACS between the propensity matched groups (Figure 1).

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Discussion: Complications and outcomes of HCT in asymptomatic patients with SCD patients is similar to that in propensity matched symptomatic SCD patients undergoing HCT.

Disclosures No relevant conflicts of interest to declare.

Table 1: Baseline Characteristics Of Patients And Propensity Score Matched Controls

	Group		
	Asymptomatic (N = 48)	Symptomatic (N = 48)	Total (N = 96)
Age			
N	48	48	96
Mean (SD)	8.00 (7.98)	7.81 (7.32)	7.91 (7.62)
Donor type:			
HLA identical sibling	31 (64.58%)	29 (60.42%)	60 (62.50%)
HLA mismatch relative	08 (16.67%)	10 (20.83%)	18 (18.75%)
Matched unrelated donor	4 (8.33%)	05 (10.42%)	9 (9.38%)
Mismatched unrelated donor and cord blood	5 (10.42%)	4 (8.33%)	9 (9.37%)
Sex			
Male	29 (60.42%)	29 (60.42%)	58 (60.42%)
Female	19 (39.58%)	19 (39.58%)	38 (39.58%)
HCT-comorbidity index			
0-2	39 (81.25%)	40 (83.33%)	79 (82.29%)
3+	09 (18.75%)	08 (16.67%)	17 (17.71%)
Disease genotype			
Hemoglobin SS	44 (91.67%)	45 (93.75%)	89 (92.71%)
Hemoglobin Sß-thalassemia	04 (08.33%)	03 (06.25%)	07 (7.29%)
Graft type			
Bone marrow	35 (72.92%)	35 (72.92%)	70 (72.92%)
Peripheral blood	05 (10.42%)	4 (8.33%)	9 (9.38%)
Umbilical cord blood	8 (16.66%)	9 (18.75%)	17 (17.7%)

Figure 1. Survival Analysis of Asymptomatic Patients and Propensity Matched Controls

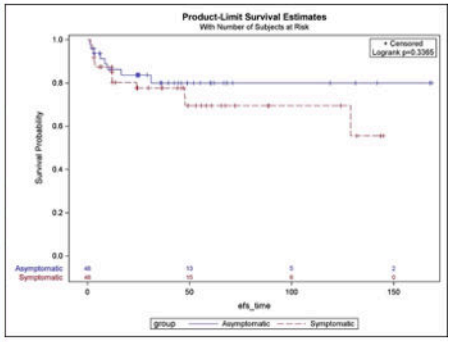

Figure 1

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