O-020.

XX CONGRESSO DA SOCIEDADE BRASILEIRA DE TRANSPLANTE DE MEDULA ÓSSEA.

PRÊMIO: ALÍRIO PFIFFER - para o melhor trabalho em falência medular.

Premiação: Certificado.

Haploidentical transplantation with post-transplantation Cyclophosphamide(PT-CY) for the treatment of Bone Marrow Failures(BMF): Analysis of 39 children and adolescents transplanted in Curitiba,Brazil.

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Hematologia – Doenças Benignas.

The availability of matched unrelated donors as well as time to find a donor and the costs related to the acquisition of international grafts are limited in countries with ethnical minorities and fewer resources. Herein we describe the experience of 39 pts with BMF submitted to a haploidentical BMT with PT-CY between 2008 and 2015. The median age was 9 ys (range: 1-16), 74% were male and 95% were CMV positive. Diagnosis: Fanconi Anemia (FA, N=30), severe aplastic anemia (SAA, N= 5), dyskeratosis congenita (DC, N=2) or other inherited marrow failure (N=2). Thirty-five pts were transplanted upfront while 4 pts with FA received this treatment after a primary or secondary graft failure. All pts had failed prior therapies and most were transfused before transplantation. Bone marrow was the only graft source used and all pts received GVHD prophylaxis that included PT-CY on D+3 and D+4, followed by cyclosporine, Mycophenolate mofetil and G-CSF. FA pts received PT-CY at 25mg/kg/day (total dose: 50mg/kg) and pts with other BMF received PT-CY at 50mg/kg/day (total dose: 100mg/kg). Pts with FA received fludarabine(FLU) 150mg/m2 + TBI 200-300cGy +/- CY 10mg/kg without (N=14) or with rabbit ATG 4-5mg/kg (N=16). Pts with SAA, DC or other congenital BMF received CY 30 – 50mg/kg, FLU 150mg/m2 and TBI 200-400cGy. Results: FA: In the subgroup of pts who did not receive r-ATG (N=14), all pts engrafted, despite the presence of donor specific antibodies(DSA) in 2 pts. Three pts had AML and 2 are in remission 1 and 3ys after transplant. The incidence of acute and chronic GVHD was very high. Six pts died due to GVHD (N=4); toxoplasmosis/CMV pneumonia (N=1) and relapse (N=1). Eight pts are alive with a median follow-up of 44 months after transplant including 2 pts rescued after graft failures. In the subgroup of pts receiving r-ATG (N=16), 2 pts presented graft failure and both died despite a 2nd transplant with different donors. One pt had MDS and is in remission one year after transplant. The incidence of acute and chronic GVHD decreased in severity but was still observed. 3 pts died, 2 from graft failure and one due to late acute GVHD. 13 pts are alive with a median follow up of 22 months after transplant, including the other 2pts rescued after graft failure. All patients transplanted with a diagnosis of SAA, DC or other BMF are alive and engrafted. In this group, no pt developed acute or chronic GVHD. Graft failure was observed in one pt with DC who had DSA. This patient is alive and well after a 2nd BMT from a different haploidentical donor. Altogether CMV reactivation occurred in 65% of pts at risk, independent of the primary diagnosis. Conclusion: Haploidentical BMT using PT-CY may be an option for patients with acquired or inherited BMF who need an immediate transplant but lack a matched related or unrelated donor. New approaches to GVHD prophylaxis and treatment are needed in order to improve quality of life and overall survival of FA pts. Haploidentical, Post-transplantation Cyclophosphamide, Bone Marrow Failures