Abstract# 5175

T Cell–Depleted Related Haploidentical Peripheral Blood Stem Cell Transplantation in a Patient with Fanconi Anemia. Cagliari Experience.

Maria Adele Sanna MD [1]*, Maria Grazia Orofino MD [1]*, Fausto Cossu MD [1]*, Maria Carmen Addari MD [1]*, Antonio Piroddi MD [1]*, Giuseppa Fabiana Rizzo MD [1]*, Manuela Badiali PH [1]*, Renzo Galanello MD [1] and Francesca Argiolu MD [1]*. [1] *Scienze Biomediche e Biotecnologie, Clinica Pediatrica Centro Trapianti Midollo Osseo Ospedale per le Microcitemie, Cagliari, Italy .*

Stem cell transplantation is presently the best treatment for Fanconi Anaemia (FA) patients developing bone marrow failure. 70% of success is reported in patients with a HLA identical sibling whereas the outcome for HSCT in those transplanted from unrelated donors is in the range of 29–43%, graft rejection, GVHD and regimen related toxicity beeing the main causes of failure. This results limited the ability to perform marrow transplantation other than HLA identical siblings for this disease. Recently a fludarabine based cytoreductive regimen has been successfully used in T cell depleted haploidentical/mismatched transplant of FA patients. We report a case of a 7 year old boy with bone marrow failure since 1999. Androgens treatment was uneffective, no HLA identical family donor was available and the search for a suitable marrow or cord blood unrelated donor was unsuccessful. After 4 years he underwent T–cell depleted haploidentical PBSCT from his father. Conditioning regimen was: fludarabine 30 mg/mq from day –6 to day –3, cytoxan 300 mg/mq from day –6 to day –3, rabbit ATG (3.75 mg/kg) from day –5 to day –3. GvHD prophylaxis consisted of cyclosporine 1 mg/kg from day –1 . The donor received G–CSF 8 ug/kg/dose twice daily for 6 days and underwent leukapheresis on day 5 and 6. Donor stem cells were depleted of T cells by positive selection of CD34+ cells using the Clinimacs device according to the suggested procedures (Milteny Biotec). On day 0, $15.3x10^6$ x kg CD34+ cells were infused with 1.5×10^5 CD3 + cells .The clinical postransplant course was uneventful. Neutrophil engraftment ($>0.5 \times 10^9$) occurred on day 14 platelet count $>100x10^9$ on day 15. He was discharged on day 39 without signs of GVHD. Molecular analysis of DNA–VNTRs at 1, 3, 6, 9, 12 months showed >95% donor chimerism on peripheral blood. At 14 months after transplantation the patient is well, normal blood cell count (WBC 5.4 x 10^9/l, Hb 13.6 gr /dl, platelets 293x 10^9 /l). Count of T–cells are reported in the normal reference range (CD3+ :1865 ug/l, CD8+ :1026ug/l, CD19+ :732ug/l, CD56+: 452). Karnofsky score is 100%.
Conclusion: the case reported shows that the fludarabine based regimen and the infusion of a high number of T–cell depleted CD34+ was successful in absence of peri–transplant complications and can be proposed for the cure of FA patients at high risk of clonal disease and without HLA–matched sibling donor.