T5. APLASTIC ANEMIA IN CHILDREN AND ADOLESCENTS –RESULTS OF STEM CELL TRANSPLANTATION, FUNDENI TRANSPLANT CENTER EXPERIENCE

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Background. Aplastic anemia (AA) is a rare disease in children; it's most commonly idiopathic and less often due to a hereditary disorder. However, hereditary bone marrow failure syndromes should be considered in both children and adults before the institution of any therapeutic treatment plan. Hematopoietic stem cell transplantation (HSCT) is the only treatment that definitively restores normal hematopoiesis. HSCT is appropriate: (1) as initial definitive treatment when a HLA-identical sibling donor is available in a patient with Severe aplastic anemia (SAA) or Very severe aplastic anemia (VSAA), (2) after failure of 1 or (traditionally) 2 courses of immunosuppressive therapy (IST) in a patient with SAA or VSAA who has an acceptably matched alternative donor, (3) in patients with constitutional Aplastic anemia who are transfusion dependent and who have a HLA-identical donor related or unrelated.
Aim. We analyzed the transplant indication, complications and results in a cohort of 9 children with AA transplanted or followed in Fundeni Clinical Institute.
Patients & Methods. Nine patients with AA - 5 males, 4 females, age range 4-13 years - were transplanted and monitories in Fundeni Clinical Institute, Pediatric Bone Marrow Transplant Department between 2005-2013. The donor was in 6/9 cases a HLA identical sibling and in 3/9 cases a 10/10 compatible unrelated donor. In 3 cases the transplant procedure has been performed abroad – 2 cases - Israel, 1 case – Italy. The stem cell source was: bone marrow (BM)- 1/9, cord blood (CB)- 1/9, peripheral stem cells (PSC) – 7/9. The conditioning regimen included ciclophosphamide, rabbitt ATG, +/- Fludarabine. GVHD prophylaxis consisted in standard CsA and short MTX with Leucovorin rescue. Chimerism analysis has been performed using STR technique.
Results. Follow-up range was 120-1660 days. Five patients are alive, in good clinical condition. The chimerism analysis shows in 4/5 cases complete donor chimersim, in 1/5 cases stable mixed chimersim. We registered 4/9 deaths: graft failure – 1 case, graft rejection – 1 case, hemophagocitic syndrome-1 case, infection – 1 case. Only 3 patients developed GVHD and received immunosuppression.
Discussions/Conclusion. The distinction between constitutional and acquired aplastic anemia is critical for the choice of the best conditioning regimen: reduced intensity regimen including Fludarabine for constitutional and standard CFA-ATG for acquired aplastic anemia. The number of blood transfusions before transplant seems to be the most important prognostic factor for engraftment.