Abstract: P788

Title: OUTCOME OF SEVERE APLASTIC ANEMIA TREATED WITH ALLOGENIC STEM CELL TRANSPLANTATION COMPARED WITH IMMUNOSUPPRESSIVE THERAPY AS FIRST LINE.

Abstract Type: Poster Presentation

Session Title: Bone marrow failure syndromes incl. PNH - Clinical

Background:

Aplastic anemia (AA) is a form of bone marrow failure as a result of immune-mediated destruction of the hematopoietic stem cells; it is defined as pancytopenia with a hypocellular bone marrow [<25 % (or 25 to 50 % if <30 % of residual cells are hematopoietic)] in the absence of marrow fibrosis or abnormal infiltrates. The treatment of AA depends on the severity of the disease and the age of the patient. Allogenic Hematopoietic stem cell transplantation is the standard of care for young patients with severe AA.

Aims:

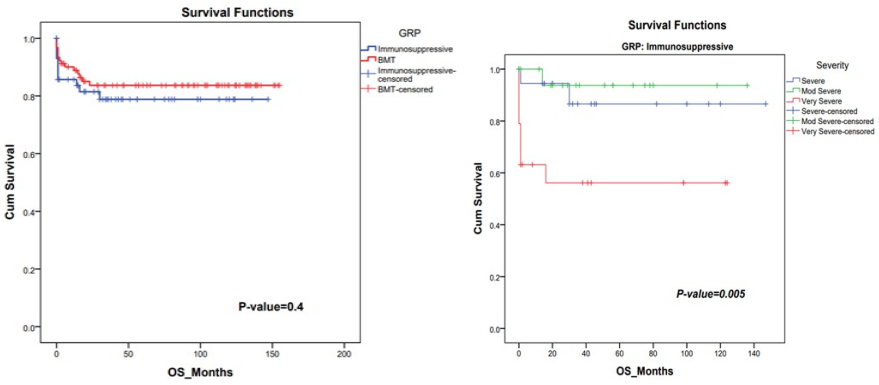
Survival following allogenic Hematopoietic stem cell transplantation (allo-SCT) or immunosuppressive therapy (IST) were compared in aplastic anemia and the prognostic factors related with survival identified.

Methods:

Retrospective analysis of 156 patients, 92 (58.9%) were treated with SCT and 64 (42.0%) with IST. Patient's characteristics were summarized using frequencies with percentages for categorical variables and medians with interquartile ranges for continuous variables. Categorical variables were compared using Chi-square test while continuous variables were compared using Mann–Whitney U test. Probabilities of OS and EFS were summarized using Kaplan-Meier estimator with variance calculated using Greenwood formula. Survival curves were compared using log-rank test. P-value< 0.05 was considered significant. Analysis was conducted using RStudio 2022.07.2 Build 576 ©□ 2009-2022 RStudio, PBC.

Results:

The median age was 28 (17-35 years).Overall survival was 78.8 % in patients given front-line immunosuppressive and 83.7 % in the allogenic hematopoietic stem cell transplantation group(P=0.4).In the IST cohort, under multivariate analysis, Overall survival for moderately severe, severe and very severe aplastic anemia was 93.8%, 86.6% and 56.1 % respectively (P=0.005). While, in allo-SCT group OS for moderately severe, severe and very severe aplastic anemia was 66.0%, 81.4% 86.3 % respectively (P=0.5). Age of 20 years or under positively affected overall survival in allogenic hematopoietic stem cell transplantation group, whereas age more than 20 years negatively affected overall survival in this group. The factors influencing the overall survival were an age under 20-years- and use of allo-SCT.



Summary/Conclusion:

Aplastic anemia in adolescents has a very good outcome. If a matched family donor is available, hematopoietic stem cell transplantation using bone marrow cells is the first choice treatment. If such a donor is not available, immunosuppressive treatment may still be an acceptable second choice also because, in case of failure, hematopoietic stem cell transplantation is a very good rescue option. Therefore, younger age SAA patients, with HLA-matched donors, may benefit more from allo-SCT.

Keywords: Immunosuppressive therapy, Aplastic anemia, Allogeneic stem cell transplant