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Bone Marrow Transplantation in Thalassemia

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In a study from Pesaro, transplanted 26 thalassemic patients revealed persistent mixed chimerism for a long period of time [between 2-11 years]. regardless of the activity of large number of host cells, these thalassemic patients clinically behaved well without transfusion of red cells support. And also all of the 26 patients with mixed chimerism showed stabilized hemoglobin level between 8.3-14.7 g/l.d

In transplanted thalassemic patients, persistent mixed chimerism reveals long-term stabilized transfusion independency which is well enough for a disease-free life.

Dr. Angelucci from Pesaro reported a well documented study: The iron overload in ex-thalassemic patients after transplantation. In this study toxic effects of iron were shown in liver and heart. This perfect study showed that the phlebotomy was the only way to reduce iron load in ex-thalassemic patients. This procedure was carried out at weekly intervals and rapid reduction in serum iron was remarkable. On the other hand, toxic effects of iron in Growth and Development in ex-thalassemic patients were discussed. Management of residual liver damage in the ex-thalassemic patients was reported . Furthermore, the normalization of iron level in ex-thalassemic patients showed normal liver function and normal morphology by liver biopsy.

Application of a successful BMT can not eliminate the iron overload due to pre-transplant transfusions. Long term application of well programmed phlebotomy might be a curative and dependable solution to this life threatening problem.

Dr. Sodani from Pesaro reported a new protocol for class III thalassemic patients. The result of this study was interesting enough to know that class III thalassemic patients benefited from bone marrow transplantation with this new regimen (see Lucarelli, G et al, this issue).

Cord blood cells transplantation in thalassemic patients was presented by Dr. Locatelli from Pavia. The result of this study was compatible with other progenitor cell transplantation. Cord blood is a new source for hematopoetic stem cell transplantation and might be adequate alternative for patients without any matching sibling donor.

Finally, Dr.Aaolu presented the important problems of thalassemic patients in Turkey. Management of thalassemic patients and carrier stage in population and its prevention facilities were opened for discussion. The result of 44 transplanted thalassemic patients showed 62 percent DFS and yearly expectation of thalassemia in Turkey was estimated around 150 cases.

In developing countries like turkey, the results of bmt in thalassemic patients seem to be problematic with 67% of disease-free survival for 5 years rejection rate. But the results of new documented regimens through new approaches and applications are hopeful. In turkey, hemoglobin disorders are major problems and more comprehensive studies need to be done.

With these excellent studies all speakers reported that the thalassemic patients will benefit from bone marrow transplantation. Uniquely, all participants of this session agreed that the bone marrow transplantation in thalassemia must be included in meetings of the EBMT Pedatric Working Party.