B-Thalassaemia - a Major Health Problem in Pakistan

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Lung cancer is the leading cause of cancer related b-thalassaemia is a major health problem in Pakistan. It is the most prevalent genetically transmitted blood disorder with a carrier rate of 5-8%; around 5000 children are diagnosed each year in the country.1,2 The mainstay of treatment is blood transfusion and iron chelation therapy. Enormous efforts are being made to prevent this disorder by mass education, pre-marital counselling, screening patients' families and discouraging carrier marriages.2 Researchers are also looking for new modalities of treatment to provide better quality of life and increased survival in these patients.

Twenty-five years ago this disorder was thought to be non-existent but with awareness and improved diagnostic facilities, thousands of children are diagnosed each year. Designated thalassaemia centres were created in early 1980's, which improved the quality of life at least during first few years of their life. But overall, almost all patients receive sub-optimal transfusion. Some patients receive iron chelation only at the time of blood transfusion. Only during last 4 years, few centres started managing them with hyper-transfusion and standard iron chelation therapy protocols.

Bone Marrow Transplantation (BMT) has been established as the only curative treatment option for b-Thalassaemia major. Encouraging results produced by the pioneering work of Seattle group and consistently improving results of Pesaro group changed the outlook for thalassaemic patients Lucarelli et al. from Italy recently published 14 years follow up data; which indicated 91% thalassaemia free survival in class-I (n=124) and 84% thalassaemia free survival in class-II (n=297), whereas 58% (n=122) thalassaemia free survival in class-III patients and 62% disease free survival in adult thalassaemia patients (n=109). Overall survival including all groups represents 73% thalassaemia free survival.3

Smaller series from the two British Centres (Westminster and Manchester) and one centre from Turkey in thalassaemic patients showed a 71.0% overall survival.4 The pioneer work published by Farzana et al reported the results of allogeneic PBSC transplantation for b-thalassaemia major from Pakistan.5 These authors reported early results with a 66.6% overall survival in 12 patients. In this issue of JPMA, Khalilullah et al presented their results in a cohort of 19 patients undergoing allogeneic stem cell transplantation using bone marrow as a source. Considering the initial results from a new transplant centre in a developing country, the results are encouraging. Overall disease free survival of 78.9% and 68.4% was commendable.6 Both centres in Pakistan have been through a steep learning curve and gradually reached to international standards.

In a new transplant programme, there are many issues which need to be addressed. Firstly, successful transplant programmes share their data with either international bone marrow transplant registry or European bone marrow transplant registry. There are local and national registries as well. Sooner or later, national transplant registry needs to be established. Secondly, despite large family size, about a third of all patients do not have HLA identical sibling donors in Pakistan. National bone marrow donor database is also mandatory to provide HLA matched unrelated donor bone marrow stem cells. Thirdly, post fellowship training programme should be initiated for doctors interested to pursue carriers in this speciality. Nursing staff need also to be inducted in the transplant programme.

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