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Gene Therapy-A hope for Hemophilia Children

Hemophilia A and B are the X-linked genetic disorders, presenting with the low levels in coagulation factor VIII (FVIII) and factor IX (FIX) leading to recurrent bleeds and multiple complications 1. Hemophilia is prevalent throughout world & in seen in all the racial groups. Thousands of children in Middle eastern countries are suffering from these crippling disorders. These children are forced to be away from the routine activities of their age fellows spending most of the time in hospitals 2. Present management protocols for hemophilia concentrate on the intravenous infusion of clotting factor concentrates. However, this approach is far away from permanent cure. Newer approach for curing the disease is the development of gene-based therapies for hemophilia in order to attain sustained high levels of serum clotting factors to correct the bleeding diathesis. Different types of viral and non-viral genetic rearrangements to a range of target cells in the body, i.e. hematopoietic stem cells, hepatocytes 3, skeletal muscle cells, and endothelial cells, have been considered for possible hemophilia gene therapy. So far the most promising vectors are Adeno-associated virus and lentiviruses. The results of gene therapy were very satisfactory in murine and canine laboratory models and these promising studies encouraged clinical trials in human suffering from this debilitating disorder. Consistently high levels of clotting factors VII and IX were observed in initial clinical trials resulting in long term avoidance of infusions of deficient factors in the patients. But some the corner stones are yet to be turned as it has been observed that the gene-modified cells are a potent target for immune exposure to effector T cells, resulting in recurrence of bleeding episodes 4. More effective measures are needed to achieve a full hemostatic correction in our children presenting with either hemophilia A or B. Once fully developed and free of all the hazard, it would be like bright sun shine in darkened lives of Hemophilia patients and their families.

Speaker Biography

Tahir Jameel is working as professor of Hematology in King Abdulaziz University, Jeddah Saudi Arabia. He has a vast teaching experience of Clinical and academics in Hematology. He has published many research articles in national and international medical journals. He is coauthor of three medical books, has written many chapters is multiauthor books. He has vast exposure in Pediatric Hematology. especially in managing Thalassemia, Hemophilia other hemoglobinopathies.

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