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Recent progression in hemophilia gene therapy

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Hemophilia is a bleeding disorder in which coagulation is corrupted. There are different types of Hemophilia, more than 400,000 people around the world living with the disease. Two common types of hemophilia are A and B, defects in coagulation factors 8 and 9 respectively. Hemophilia A is 80-85% of cases. Coagulation factors 8 and 9 located at the long arm of chromosome X and mutations in these genes result in the defective production of coagulation factors. Hemophilia considered an appropriate target for gene therapy, because production of 1% of normal level could adjust the phenotypic problems. Various methods have been developed for hemophilia gene therapy, producing coagulation factors in the patients ultimately. Injection of coagulation factor gene by vector into the stem cells extracted from the patients or vectors containing the transgene insertion into differentiated cells with long survival, such as muscle cells and liver are among the most important of these methods. As well as the most recent methods of gene therapy, gene transfer based on induced pluripotent stem cells is abbreviated iPS. Hepatocytes a very suitable target cells for hemophilia gene therapy because these cells are the main site of synthesis of coagulation factors. Muscle cells also are suitable for the injection of transgenes in gene therapy for hemophilia because of its appropriate secretory power and its availability. The most important and most widely used viral vectors for gene therapy of hemophilia are retroviral vectors and lentiviral and Adeno-associated viruses.

Biography

Reyhane Sadat Saeedi is currently a student in Faculty of Medicine, Tehran University of Medical Sciences, Iran. She is a Member in Students' Scientific Research Center and Student Advisory Committee of Medical School of Tehran University of Medical Sciences. She has published 3 papers in reputed journals.

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