عنوان مقاله:
Hemophilia Gene Therapy; Clinical and Molecular Aspects

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خلاصه مقاله:
Hemophilia is a coagulation disorder in which bleeding time is prolonged. There are a number of hemophilia subtypes and morethan ۴,۰۰۰,۰۰۰ individuals are suffered worldwide. The most common types of hemophilia are type A and B in which coagulationfactor VIII and IX are defected respectively. Type A hemophilia is responsible for ۸۰% to ۸۵% of cases. The genes of ۸ and ۹ coagulationfactors located on the long arm of X chromosome and mutation in these genes causes disturbance in coagulation. This diseaseis a very good target for gene therapy because if amount of protein production reaches ۱% that of normal the disease phenotypeis modified. Different methods of hemophilia gene therapy include increased production of coagulation factors via insertion ofattributed genes into patient's stem cells by vectors, or insertion of transgenes into differentiated cells with prolonged survival suchas muscle or liver cells. One of the most recent advances in hemophilia gene therapy is using induced pluripotent stem cells (iPS)for gene transfer. Hepatocytes are very good candidates for hemophilia gene therapy due to their natural capacity for productionof coagulation factors. Myocytes are also suitable for injection of transgene because they are available and have sufficient secretorypower. Most important and useful viral vectors for hemophilia are retroviral, lentiviral, and Adeno-Associated viruses. Amongtheseonly the retroviral vectors target dividing cells.

کلمات کلیدی:
Hemophilia, Gene Therapy, iPS