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Gene therapy is one of the hottest topics in modern medicine. The use of stem cell and gene therapies is progressively expanding in treatment of a wide variety of inherited metabolic and hemostatic diseases, malignant disorders as well as skin and neurological diseases. Gene therapy also shows promise as a treatment strategy for hereditary bleeding disorders.

Some reports have indicated successful gene therapy in animal models with hemophilia. Recently, gene therapy using adeno-associated viral (AAV) vectors have had some success in patients with hemophilia B. Gene therapy changes the severe disease to a mild or moderate phenotype, increasing the basal levels of factor IX to 5-10% and dramatically ameliorates bleeding in patients with severe hemophilia.

The side effects are related doses and immunologic response to the vector. Advances in vector design are important consideration to lower adverse effects of the therapy. Mesenchymal stem cells (MSCs) are multipotent stem cells, and have immunomodulatory properties. MSCs can increase expression rFIX, and improve expression level to increase the amount of secreted hrFIX.

Gene therapy may be initially successful, but the expression of factor IX after viral injection is unstable, due to immunologic complications. Immune suppression may improve the outcome in patients after gene therapy.

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