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Potential therapeutic implications of hematopoietic stem cell gene therapy in correction of mutation-causing human beta-thalassemia

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Hematopoietic stem cell gene therapy (HSC-GT) or *in-vivo* gene-based autologous stem cell transplant is one of the most exciting clinical tools to emerge from the gene therapy. By using integrating vectors, therapeutic transgenes can be efficiently transmitted to all HSC-derived mature hematopoietic stem cells via mitosis. This strategy generates an alternative or complementary clinical therapy for bone marrow transplant patients with no related human-leukocyte-antigen-matched or geno-identical donors. Successful HSC-GT clinical trials have been conducted to treat several genetic and immune diseases of the hematopoietic system as X-linked severe combined immunodeficiency (SCID-X1) and β- thalassemia. In this research we will study the effect of transfection of an erythroid promotor vector mediated normal human β-globin gene sequence (hemoglobin subunit beta- HBB) construct to HSCs culture media from β- thalassemia patients.

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