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(54) Title: VECTOR ENCODING HUMAN GLOBLIN GENE AND USE THEREOF IN TREATMENT OF HEMOGLOBINOPATHIES

(57) Abstract: Recombinant lentiviral vectors having a region encoding a functional globin gene; and large portions of the beta-globin locus control regions which include DNase I hypersensitive sites HS2, HS3 and HS4 provides expression of beta-globin when introduced into a mammal, for example a human, in vivo. Optionally, the vector further includes a region encoding a dihydrofolate reductase. The vector may be used in treatment of hemoglobinopathies, including beta-thalessemia and sickle-cell disease. For example, hematopoietic progenitor or stem cells may be transformed ex vivo and then restored to the patient. Selection processes may be used to increase the percentage of transformed cells in the returned population. For example, a selection marker which makes transformed cells more drug resistant than un-transformed cells allows selection by treatment of the cells with the corresponding drug.