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Autologous beta-A(T87Q)-globin Gene-transduced CD34-positive Cells

National Cancer Institute

Source

National Cancer Institute. *Autologous beta-A(T87Q)-globin Gene-transduced CD34-positive Cells*. NCI Thesaurus. Code C123881.

A preparation of autologous, CD34-positive hematopoietic stem cells (HSCs) transduced ex vivo with the BB305 recombinant replication-defective, self-inactivating lentiviral vector encoding for an engineered form of human beta-globin (hemoglobin-beta, HBB) gene, beta-A-T87Q (b-A-T87Q) where the threonine at position 87 has been substituted with glutamine, with potential to restore beta-globin expression and function. Autologous CD34-positive stem cells are isolated from the patient's own bone marrow and the cells are transduced with the lentiviral vector. Upon re-infusion of the b-A-T87Q-globin gene transduced CD34-positive cells back into the patient, these cells express b-A-T87Q-globin, thereby allowing the body to make normal hemoglobin and thus normal, healthy red blood cells. Beta-globin, the beta-chain of the most common form of hemoglobin, is encoded by the HBB gene; mutations in this gene prevent normal beta-globin production and are associated with beta-thalassemia and sickle cell anemia. The b-A-T87Q form of beta-globin has increased antisickling activity compared to the wild type protein.