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Autologous TNS9.3.55-transduced CD34positive Cells

National Cancer Institute

Source

National Cancer Institute. <u>Autologous TNS9.3.55-transduced CD34-positive Cells</u>. NCI Thesaurus. Code C116734.

A preparation of autologous, CD34-positive hematopoietic progenitor cells (HPCs) ex vivo transduced with TNS9.3.55, a lentiviral vector encoding the human beta-globin (hemoglobin-beta, HBB) gene, with potential to restore beta-globin expression and function. Autologous CD34-positive stem cells are isolated from the patient's own bone marrow, the deficient HBB gene is removed, and the cells are transduced with the lentiviral vector. Upon re-infusion of the TNS9.3.55-transduced CD34-positive cells back into the patient, these cells express beta-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.