Autologous CD34-positive BCL11A-disrupted Hematopoietic Progenitor Cells BIVV003

National Cancer Institute

Source

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A population of autologous cluster of differentiation 34 (CD34)-positive hematopoietic progenitor cells (HPCs) that are transfected ex vivo with zinc finger nuclease (ZFN) messenger ribonucleic acid (mRNA) targeting the B-cell lymphoma/leukemia 11A (BCL11A) locus, with potential usage for transplantation in patients with sickle cell disease (SCD). CD34-positive HPCs are isolated from human blood upon apheresis and are genetically modified in vitro using ZFN technology to specifically cleave and disrupt the erythroid enhancer of the BCL11A gene. This suppresses the production of sickle hemoglobin. Upon infusion into the patient following conditioning chemotherapy, the autologous CD34-positive BCL11A-disrupted HPCs BIVV003 can populate the bone marrow and differentiate into a variety of blood cell types including lymphoid cells, myeloid cells and erythroblasts. As BCL11A is a suppressor of fetal hemoglobin (HbF) expression, disruption of the BCL11A enhancer decreases the expression of BCL11A and stimulates the expression of HbF in erythrocytes that differentiate from BIVV003. HbF may compensate for reduced or absent expression of adult hemoglobin in patients with SCD.