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Autologous CD34-positive BCL11A-disrupted Hematopoietic Progenitor Cells ST-400

National Cancer Institute

Source

National Cancer Institute. *Autologous CD34-positive BCL11A-disrupted Hematopoietic Progenitor Cells ST-400*. NCI Thesaurus. Code C148240.

A population of autologous CD34-positive, B-cell lymphoma/leukemia 11A (BCL11A)-disrupted hematopoietic progenitor cells (HPCs) that may be used for bone marrow autotransplantation. CD34-positive HPCs are isolated from human blood upon apheresis and are genetically modified in vitro using a proprietary zinc finger nuclease (ZFN) technology to specifically disrupt the transcriptional enhancer sequence for the BCL11A gene. Upon infusion into the patient following conditioning chemotherapy, the autologous CD34-positive BCL11A-disrupted HPCs ST-400 can populate the bone marrow and differentiate into a variety of blood cell types including lymphoid cells, myeloid cells and erythroblasts. BCL11A is a suppressor of fetal hemoglobin (HbF) expression. Therefore, disruption of the BCL11A enhancer decreases the expression of BCL11A and stimulates the expression of HbF in erythrocytes that differentiate from ST-400. HbF may compensate for reduced or absent expression of adult hemoglobin in patients, including those with transfusion-dependent beta-thalassemia.