ABSTRACT

The present invention relates to a method for treating hemoglobinopathy in an individual, comprising: (a) an evaluation step: the evaluation step comprises evaluating the ability of a first population of modified CD34-positive hematopoietic stem cells/progenitor cells to produce a desired level of γ-globin or fetal hemoglobin after differentiation, the modified CD34-positive HSPCs of the first population being derived from the individual and being modified to reduce BCL11A function; and (b) a treatment step: the treatment step comprises administering to the individual a second population of modified CD34-positive HSPCs, the modified CD34-positive HSPCs being derived from the individual and being modified to reduce BCL11A function. At the same time, the invention also relates to a method for treating hemoglobinopathy in individuals, a method for selecting individuals suffering from hemoglobinopathy for treatment using the modified CD34-positive HSPCs of the second population, and a method for determining whether an individual suffering from hemoglobinopathy is suitable or unsuitable for treatment using the second population of modified CD34-positive HSPCs derived from the individual and modified to reduce the function of BCL11A.