Abstract

Provided is a new method for activating transcription of a gamma-globin gene. The method uses a single-stranded oligonucleotide (ssODN) containing GATA or an antisense complementary sequence TATC thereof as guidance information, and performs gene editing in a gamma-globin gene regulatory region to form a GATA-containing enhancer element, which can promote the expression of the gamma-globin gene in mature red blood cells. Hematopoietic stem cells genetically edited by the method have normal functions, can significantly improve the expression of fetal hemoglobin after being differentiated into red blood cells, and therefore can be used in clinical treatment of beta-thalassemia and sickle cell anemia.

Figure 1