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(54) Title: GENE THERAPY FOR TREATING BETA-HEMOGLOBINOPATHIES

(57) Abstract: Provided are gene therapy technologies, including specifically designed and tested guide RNA sequences for improved base editors, useful for increasing the expression of the gamma-globin gene. The guide RNA sequences may target the BCL11A erythroid enhancer or the gamma-globin promoter, or both at the same time. The base editors can include nucleobase deaminase inhibitor that inhibits the editing activity of the base editors until they are bound to the target sites. These gene therapy technologies are useful for treating diseases including beta-thalassemia and sickle cell anemia, among others.



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