COMPOSITIONS AND METHODS FOR TREATING HEMOGLOBINOPATHIES

ABSTRACT

The present invention features compositions and methods for editing deleterious mutations associated with hemoglobinopathies, such as sickle cell disease (SCD). In particular embodiments, the invention provides methods for correcting mutations in a beta globin polynucleotide using modified adenosine base editors termed "ABE8" having unprecedented levels (e.g., >60-70%) of efficiency.

Fig. 1A