عنوان مقاله:

Fighting against sickle cell disease by CRISPR/Cas۹

محل انتشار:

کنفرانس بین المللی ژنتیک و ژنومیکس انسانی (سال: 1400)

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خلاصه مقاله:

Sickle cell disease (SCD) is a kind of inherited hemoglobinopathy causedby a point mutation in the HBB gene, which as a result is replaced byglutamic acid in the sixth place on the beta chain with Valine. Thiscondition leads to the formation of a kind of abnormal hemoglobin called"HbS". Here, we evaluate the benefits and challenges of treating peoplewith SCD using CRISPR / Cas۹. Articles from the last six years wereextracted from Google Scholar. Databases such as Nature and PUBMEDwere also reviewed.Ever since CRISPR (clustered regularly interspaced short palindromicRepeats)/Cas۹ became known, a new chapter has been opened in the storyof the treatment of genetic diseases. This system is a compound of smallguide RNAs for recognizing the target DNA sequence and Cas۹ protein asnuclease for cutting of DNA. For the treatment of SCD by CRISPR, the β-globin gene mutated in induced pluripotent stem cells or hematopoietic stemcells derived from patients with this complication can be modified. On theother hand, using CRISPR/Cas۹ can reduce the expression of the fetalhemoglobin inhibitory protein gene (BCL۱۱A). Increased HbF prevents thepolymerization of HbS, and thus we will see a reduction in symptoms.CRISPR/Cas۹ can be used to treat SCD by increasing the expression of HbFor modifying mutations in the HBB gene in iPSCs or HSCs derived .fromthese patients. At the same time, more studies are needed due to thepossibility of off-target activity in CRISPR/Cas۹

کلمات کلیدی:

Sickle cell disease, CRISPR/Cas۹, Gene editing,Hematological disorders

لینک ثابت مقاله در پایگاه سیویلیکا: