عنوان مقاله:

Potential therapeutic application of CRISPR-Cas9 in Huntington s disease

محل انتشار:

دومین کنگره بین المللی و دهمین همایش ملی نوروژنتیک ایران (سال: 1396)

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خلاصه مقاله:

Backgraound: Huntington s disease caused by a extension of a CAG repeat in huntingtin gene and lead to anelongated polyglutamine tract that is toxic for the brain. So far, the drugs available for this disorder aresymptomatic treatment; unfortunately, they are not successful in curing the disease or slow down the progressionof the disease. But CRISPR/Cas9 recently has been used as an constant treatment. So, the purpose of this studywas to evaluation articles in this field.Methods: Searching for related publications was done using keywords crispr-cas9 and Huntington s disease in PubMed, Scopus and Web of Science databases.Results: The rapid growth of the CRISPR-Cas9-mediated genome editing tool has revolutionized the field ofgene therapy. One of the application this procedure is gene therapy in various diseases like neuromusculardisorders and Huntington s disease. Currently, decrease of HTT mRNA levels with this method is a therapeuticchoice for HD. The major therapeutic profit is expected when the mutant allele is entirely silenced whileexpression from the normal allele is left intact. In addition to therapeutic benefits , this method is also used tomake genetically modified animal models like pig for neurodegenerative disease in order to enhancement insightabout diagnosis, remedy, and prevention of neurodegenerative disorders.Conclusion: Our research provide proof of Therapeutic characteristic of crispr-cas9 in Huntington s diseasetherapy Although CRISPR/Cas9 system has many problems to be solved, such as off-target effects, deliverysystem, efficacy, safety concerns, but is being practical in biotechnology and clinical trial.

کلمات کلیدی:

Crispr-cas9, Huntington s disease, Therapy