SHORT ARTICLE

Cell Therapy 2020: Genome editing via CRISPR/Cas9, a new therapeutic strategy for brain disease- Zahra Keshavarz- Iran

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ABSTRACT

Neurodegenerative diseases with a known genetic background, including Alzheimer's disease (AD), Huntington's disease (HD), Amyotrophic lateral sclerosis (ALS) and Central Nervous System (CNS) tumors are attracting researchers to utilize more efficient treatment system based on genome editing strategy. Current treatment approaches for CNS disorders are neurosurgery, radiotherapy and chemotherapy; however, none of these are completely considered effective even in combination therapy, so developing new therapeutic strategies is demanded.

Key Words: *Central Nervous System; Therapeutic Strategy.*

INTRODUCTION

Genome editing is a group of technologies that allows scientists to make accurate, targeted changes to the genome in order to prevent, diagnose and treat human diseases. One of the most recent technologies that are based on a bacterial nuclease is known as CRISPR/Cas9 which has generated considerable interest for future biomedical researches. While CRISPR/Cas9 is a simple method, it is faster and more effective than many other available genome editing tools. In the process of understanding neurodegenerative diseases, there are many detected signalling pathways in human genome, potential for genome editing. Recent studies have demonstrated that using CRISPR/Cas9 for regulating these signalling pathways leads to a promising novel therapeutic method. By genomic alterations we will be able to target many aspects of the neurodegenerative diseases such as inflammation, progression, proliferation, survival, invasion, migration, angiogenesis and drug resistance. In this review we summarize recent advances based on targeting signalling pathways by CRISPR/Cas9 tool and compare results with other treatment agents to evaluate its potency for introducing a novel and powerful therapeutic agent.

Faculty of Pharmacy and Pharmaceutical Sciences, Isfahan University of Medical Sciences, Isfahan, Iran.

Correspondence: Zahra Keshavarz, Faculty of Pharmacy and Pharmaceutical Sciences, Isfahan University of Medical Sciences, Isfahan, Iran. e- geneticmedres@esciencejournal.org

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