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CRISPR-Cas9: Revolutionizing Gene Therapy for Genetic Disorders

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Nihat Dilsiz[1]

[1]University of Health Sciences, Experimental Medicine Practice and Research Center (EMPRC) Validebag Research Park, Istanbul, Turkey

nihat.dilsiz@sbu.edu.tr

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Faulty or mutated genes cause multiple human disorders such as cancer, neurogenerative disorders, and cardiovascular diseases. These mutations in genes are usually inherited. Many treatments have been established to cure such diseases, but gene therapy is the most promising strategy. It's an application of biotechnology that is based on correcting and replacing the mutated gene with the healthy gene, providing the genes for the proper expression of desired proteins required for curing the disease. In clinical settings, gene therapy has proved itself as a very promising treatment but has some drawbacks. Traditionally, these methods involved viral vectors that are used to deliver correct genes and replace them with the mutated genes, in patients. These approaches have somehow potential to cure diseases but often off-target effects, limitations in the editing process, and immune responses caused by patients' immune systems are the main challenges that are the main shortcomings of this method. However, the discovery of the clustered regularly interspaced short palindromic repeats (CRISPR) and CRISPR-associated nuclease protein 9 (Cas9) genome editing system (CRISPR/Cas9) in 2012 and its development have increased the value of gene therapy in the therapeutic world [1]. CRISPR/Cas9 is a revolutionizing tool that has been in gene therapy for knocking in and out gene to correct the mutation associated with many genetic diseases. This system has evolved greatly and has many isomers using different strategies to improve both applied, basic research and its clinical application.

The ability of CRISPR-Cas9 to target various genetic diseases is one of its greatest advantages. Mutation in single gene results in monogenic diseases such as cystic fibrosis, muscular dystrophy, and sickle cell anemia, and this system has shown the potential to edit or fix the defective genes, causing these diseases. Even in the case of complex disorders caused by the mutation in multiple genes such as several types of cancer, and cardiovascular diseases, CRISPR has shown very positive results.

The application of CRISPR-Cas9 in gene therapy is very promising and has tremendous potential to give cures for many complex disorders. The ethical issue regarding the editing of human DNA and the inheritance of modified DNA into the next generation is still under discussion and causing hurdles in uncovering the full potential of this system. Researchers are working to increase its efficiency and specificity to reduce the off-target sequences so that only targeted modification can be achieved. Moreover, they are trying to give possible results to reduce ethical concerns.

CRISPR has evolved greatly and has many isomers using different strategies to improve both applied, basic research and its clinical application in the future.

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