Gene and cell therapy

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Abstract

Emerging gene-editing technologies are nearing a revolutionary phase in genetic medicine: precisely modifying or repairing causal genetic defects. This may include any number of DNA sequence manipulations, such as knocking out a deleterious gene, introducing a particular mutation, or directly repairing a defective sequence by site-specific recombination. All of these edits can currently be achieved via programmable rare-cutting endonucleases to create targeted DNA breaks that can engage and exploit endogenous DNA repair pathways to impart site-specific genetic changes. Over the past decade, several distinct technologies for introducing site-specific DNA breaks have been developed, yet the different biological origins of these gene-editing technologies bring along inherent differences in parameters that impact clinical implementation. This review aims to provide an accessible overview of the various endonuclease-based gene-editing platforms, highlighting the strengths and weakness of each with respect to therapeutic applications. Gene therapy has historically been defined as the addition of new genes to human cells. However, the recent advent of genome-editing technologies has enabled a new paradigm in which the sequence of the human genome can be precisely manipulated to achieve a therapeutic effect. This includes the correction of mutations that cause disease, the addition of therapeutic genes to specific sites in the genome, and the removal of deleterious genes or genome sequences. This review presents the mechanisms of different genome-editing strategies and describes each of the common nuclease-based platforms, including zinc finger nucleases, transcription activator-like effector nucleases (TALENs), meganucleases, and the CRISPR/Cas9 system. We then summarize the progress made in applying genome editing to various areas of gene and cell therapy, including antiviral strategies, immunotherapies, and the treatment of monogenic hereditary disorders. The current challenges and future prospects for genome editing as a transformative technology for gene and cell therapy are also discussed.

Biography:

Arshad is a student studying in Busthanul Uloom Arabic College,KANNUR and a former student at Former Trikaripur.

Speaker Publications:

1. Osteogenic potential of Punica granatum through matrix mineralization, cell cycle progression and runx2 gene expression in primary rat osteoblasts; November 2014-DARU Journal of Pharmaceutical Sciences 22(1):72
2. Formulation and biochemical evaluation of designer diet enriched with botanicals for bone health; Food Sci Nutr 2020

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