عنوان مقاله:

Precise Correction of Disease Mutations: an Emerging therapy in Limb Girdle Muscular Dystrophy

محل انتشار:

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خلاصه مقاله:

The limb-girdle muscular dystrophies (LGMDs) with autosomal inheritance are a heterogeneous group of veryrare neuromuscular disorders that usually manifest in the proximal muscles of the hip and shoulder girdles. Theterm ‘limb-girdle muscular dystrophy’ (LGMD) now includes more than 30 genetically defined subtypes ofneuromuscular disorders which are nominated based on a consensus nomenclature. LGMDs divided byinheritance pattern into autosomal dominant (LGMD1) and autosomal recessive (LGMD2) subtypes. Overlaid onthis division a letter is added in order of discovery for discriminating between these subtypes.Therapies for LGMD have focused on disease symptoms include the treat of respiratory and cardiac deficiencies,contracture prophylaxis, and surgery for scoliosis and short tendons so far. However, there are some developingdisease specific therapies such as gene therapy, cell therapy and Transcriptional modification.Interestingly, precise gene correction techniques now exist, consist of both correction of mutations in a specificlocus and also site-specific integration of wild-type cDNA. Genetic information can be corrected byrecombination using single-stranded oligonucleotides (ssODNs). Such ssODNs can be used for precise geneediting via the homology-directed repair (HDR) pathway. However, this method is inefficient processes.Furthermore, designer nucleases are commonly based on zinc finger nucleases (ZFN), meganucleases,transcription activator-like effector nucleases (TALEN) and most recently RNA-guided CRISPR/Cas9 system.The simplicity and versatility of the CRISPR/Cas9 genome-editing system has proven to be remarkably robustmanipulating human genome. In this review our aim is to investigate the efficiency of the CRISPR/Cas9 .systemfor treatment LGMD patients

کلمات کلیدی:

Limb-girdle muscular dystrophies (LGMDs), Neuromuscular disorders, Single-stranded oligonucleotides, Zinc finger nucleases (ZFN), Transcription activator-like effector nucleases (TALEN), CRISPR/Cas9