عنوان مقاله:

Genetic modification of cystic fibrosis with ΔF508 mutation of CFTR gene using the CRISPR system in peripheral blood mononuclear cells

محل انتشار:

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خلاصه مقاله:

Objective(s): Cystic fibrosis (CF) is an inherited autosomal recessive disease that is caused by mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. The present study aimed to investigate the genetic modification of CF with ΔF508 mutation of the CFTR gene using CRISPR in peripheral blood mononuclear cells (PBMCs). Materials and Methods: Two single guide RNAs were designed to target sequences in the CFTR gene. The transfection efficiency of PBMC cells was examined through evaluation of green fluorescent protein (GFP) expression using fluorescent microscopy. Moreover, a sgRNA-Cas9 plasmid was tested to target the CFTR gene. The ΔF508 gene modification was evaluated and confirmed by PCR and Sanger sequencing methods. Results: Our results indicate the feasibility of site-specific gene targeting with the CRISPR/Cas9 system. 33% of the samples were corrected using CRISPR in mutant locus and confirmed by sequence blast at NCBI databases and primers outside the arm locus. CRISPR/Cas9 approach represents an efficient tool to repair the ΔF508 mutation of the CFTR gene in PBMC Cells. Conclusion: Therefore, the CRISPR system can be highly efficient and specific and provides a powerful approach for genetic engineering of cells and model animals. Generally, the proposed method opens new insights into .the treatment of human diseases

کلمات کلیدی:

CFTR gene, CRISPR, Cystic fibrosis, Peripheral blood mononuclear cells, ΔF508 Mutation