The Torch
Vol. 3 (11)

Advancement in CRISPR-Cas9-based medicine to treat transthyretin amyloidosis

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Recent progress has shown that CRISPR-Cas9-based drugs can be delivered into the body to target the liver and reduce the expression of genes that cause thyroxine amyloidosis or transthyretin amyloidosis (ATTR). A recent clinical trial has proven the success of gene editing in vivo; the results show that it is possible to safely edit the genome of the cells in our body. ATTR protein is a protein that is involved in the transport of thyroid hormones in the body. Patients with this condition generally experience pain, weakness, and the inability to control basic body functions. Since this is a complicated disease, it is difficult to treat patients as it requires months of administration of traditional drugs that make use of interfering RNAs. Therefore, CRISPR-Cas9 was considered the best course of treatment as it focuses on diseases that are quite challenging to cure. The nano-formulated RNA package contains a guide RNA, that targets the gene transthyretin (TTR) and the mRNA which encodes the Cas9 endonuclease for making the genomic cuts. In the ongoing clinical trials, it was found that the drug had no severe side effects. If the drug continues to succeed in the clinical trials, the treatment for ATTR patients may one day be as simple as a two-hour infusion of the lipid nanoparticles at an outpatient centre.

Keywords: CRISPR-Cas9, Gene therapy, CRISPR medicine, Genetic disorder treatment, Thyroxine amyloidosis

Citation:
Shripushkar G. Advancement in CRISPR-Cas9-based medicine to treat transthyretin amyloidosis. The Torch. 2022. 3(11). Available from:
https://www.styvalley.com/pub/magazines/torch/read/advancement-in-crispr-cas9-based-medicine-to-treat-transthyretin-amyloidosis.