Yang,J Genet Disor Genet Rep 2020,10:1
DOI: 10.37532/jgdgr.2020.10(1).e107

Journal of Genetic Disorders & Genetic Reports

Editorial

A SCITECHNOL JOURNAL

Advancement in Gene Therapy and Genome Editing

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Received Date: January 14, 2020, Accepted Date: January 23, 2020, Published Date: January 28, 2020

Editorial

Genome editing, or genome modification, or gene editing, is a method of genetic engineering in which the genome of a living organism adds, deletes, modifies or replaces DNA. Unlike early genetic engineering techniques that randomly insert genetic material into a host genome, the insertions to particular locations are targeted through genome editing. Gene therapy (also known as human gene transfer) is a medical area that focuses on the use of nucleic acid therapeutic delivery into the cells of a patient as a drug to treat disease. Martin Cline made the first attempt to alter human DNA in 1980, but the first successful transfer of nuclear genes to humans was carried out in May 1989.

The concept of gene therapy is to correct a genetic issue at its source. If, for example, a mutation in a certain gene results in the development of a defective protein in a (usually recessively) inherited disorder, gene therapy may be used to deliver a copy of this gene that does not contain a deleterious mutation and thus produces a functional protein. This technique is called gene replacement therapy and is used for the treatment of inherited retinal diseases. Since the 1970s, genome editing has been around as a means of inserting new genetic elements into organisms. The random nature of which the DNA is incorporated into the host genome has been one downside of this technology, which can impair or change other genes within the organism. Nonetheless, several techniques have been found that target the inserted genes to particular locations within an organism. It has also allowed unique sequences within a genome to be edited as well as decreased off target effects. This could be used for scientific purposes, and in gene therapy, by targeting mutations to particular genes. It may be possible to cure such genetic defects by injecting a functioning gene into an organism and targeting it to replace the faulty one.

The advent of CRISPR genome editing has opened up new doors for its implementation and use in gene therapy, as it allows the correction of a specific genetic mutation instead of pure gene replacement. In the next few years, solutions to medical challenges, such as the eradication of latent reservoirs of human immunodeficiency virus (HIV) and the correction of the mutation that causes sickle cell disease may be available as a therapeutic alternative.

Prosthetic gene therapy aims to allow the body's cells to take over roles that they do not carry out physiologically. One example is gene therapy called vision enhancement, which seeks to restore vision in patients with end-stage retinal diseases.

Photoreceptors, the main light-sensitive cells of the retina, are irreversibly lost in end-stage retinal diseases. Light-sensitive proteins are delivered into the remaining cells of the retina by means of prosthetic gene therapy to make them light-sensitive and thus to signal visual information to the brain. There is still some controversy about which light sensitive protein should be administered to which cells when the first clinical trials are underway.

In addition, there are more questions about the ecological risks of gene drives being introduced into wild populations.