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Journal of Genetic Syndromes & Gene Therapy

Perspective

Types and Strategies of Gene Therapy

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DESCRIPTION

Based on the usage and administration of various nucleic acids, gene therapy encompasses a variety of strategies and uses the addition of a new protein-coding gene, also known as gene augmentation therapy, which is the simplest and possibly the most basic way of gene-based therapy. The therapeutic gene to be supplied will be the gene's usual wild-type version for monogenic recessive illnesses when the causal mutation is non-functional and anticipated to reverse the illness phenotype by restoring the production of the damaged or absent protein. Gene replacement therapy is another name for this kind of gene augmentation therapy.

The different types of gene therapies include:

Gene augmentation therapy

To treat diseases caused by gene mutations that stops the production of a protein, or another treasure consequence. A functioning copy of the lacking gene is added to the cell's DNA as a result of this therapy the new gene produce functioning protein in high enough quantities to replace the lost protein. This is only effective if the disease's effects are reversible or have not caused permanent harm to the body this can be utilized, for instance, to treat diseases characterized by loss of function, such as cystic fibrosis, by introducing a functional copy of the gene to treat the illness. A hereditary retinal condition known as congenital achromatopsia which is characterized by cone function loss. Patients with the condition are born legally blind and experience significant visual impairment, loss of colour vision, hemeralopia (the inability to see as clearly in stout light as in lower illumination), nystagmus, and excessive photosensitivity. When ACHM is fully developed, only rod vision is observed.

Gene inhibition therapy

Gene inhibition therapy is to introduce a gene whose product either prevents the expression of another gene or obstructs the function of another gene's product the goal of this therapy is to stop a gene from acting in a way that encourages the development of disease-related cells. It is appropriate for the treatment of cancer, genetic disorders brought on by unfavorable gene activity, and infectious diseases. Deactivating or "silencing" the expression of a mutant gene that is not functioning correctly is a therapeutic strategy known as gene inhibition1-3. Gene inhibition involves the introduction of a gene silencer targeting this mutant messenger RNA (mRNA), which stops the production of the disease-causing protein and corrects its toxic effect. Some genetic diseases are brought on by mutated genes that result in the production of mutant proteins or "toxic overexpression" of necessary proteins 1, 5.

Killing of specific cells

The killing of specific cells is to put DNA into a sick cell that will cause it to die. There are two ways to accomplish this: A "suicide" gene in the inserted DNA produces an extremely toxic byproduct that kills the sick cell the sick cells are marked by a protein that is expressed as a result of the inserted DNA, allowing the body's immune system to fight them. With this technique, the inserted DNA must be properly targeted to prevent the killing of healthy cells.

CONCLUSION

In an ovine model of *ACHM* gene-augmentation treatment markedly improves cone function, restores daytime vision, and increases *CNGA3* expression with long-term efficacy and no overt adverse effects. A naturally occurring, cone-rich big animal model of *CNGA3 ACHM* has improved as a result of similar therapy used by our group and others to restore cone-mediated function. Both photopic behavioral maze testing and *ERG* recordings showed restored cone function in the treated sheep, with improvement already perceptible at the initial postoperative assessment, 1-2 months after sub retinal delivery of the *AAV5* vector carrying *CNGA3*. The effect was long-lasting because it is still visible in the first-treated animals more than 3 years after surgery.

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Received: 03-Jun-2022, Manuscript No. JGSGT-22- 18685; **Editor assigned:** 07-Jun-2022, Pre QC No. JGSGT-22- 18685 (PQ); **Reviewed:** 21-Jun-2022, QC No. JGSGT-22-18685; **Revised:** 28-Jun-2022, Manuscript No. JGSGT-22- 18685 (R); **Published:** 05-Jul-2022, DOI: 10.35248/ 2157-7412.22.13.370.

Citation: Miles E (2022) Types and Strategies of Gene Therapy. Genet Syndr Gene Ther.13:370.