Mounica M.J Regen Med 2020, 9:2
DOI: 10.37532/jrgm.2020.9(2).163

Journal of Regenerative Medicine

a SciTechnol journal

Editorial Note on Gene therapy

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Abstract: Gene therapy is a Medical Experimental technique uses therapeutic genes to treat or to prevent diseases. Most of the inherited diseases and genetic diseases caused by the mutation deletion or insertion of the base pairs onto the genetic material causing Misfolding or absence of protein synthesis in translation. The missing or incorrect gene is replaced by the New synthetic gene and introduce intothe cell as a Functional substitute. Biological vectors like Viruses have been using to transport or to deliver the nucleic acids or genes into the patient's cells as a drug. AAV (adeno-associated virus), lentivirus is used for performing gene insertions In vivo and Ex vivo.

Keywords: Somatic Cell, Gene Therapy, gRNA and RNA

Two types of cells are used in gene therapy are Somatic Cell Gene Therapy (SCGT) in this therapeutic gene are transferred to any cell other than gametes, germ cell, stem cells. Immunodeficiency like hemophilia, thalassemia, cystic fibrosis these types of disease is treated with this therapy. It's not followed a hereditary so it is widely accepted by the nations. Complete correction of a gene is not yet possible

Germline gene therapy is one the type where therapeutic genes are transferred to the Germ cell like Egg or sperm into their genomes. It's a heritable process where the gene transferred to the offspring too. So, this is mostly not accepted because of Moral Ethics and unpredictability in future generations. To deliver a functional gene into a targeted gene vectors are used. number of viruses are utilized for human cistron medical aid, together with retroviruses, adenoviruses, herpes simplex, vaccinia, and adeno-associated virus. just like the genetic material (DNA or RNA) in viruses, therapeutic. deoxyribonucleic acid may be designed to easily function a short-lived blueprint that's degraded naturally or (at least theoretically) to enter the host's ordering, changing into a permanent a part of the host's deoxyribonucleic acid in infected cells. The introduced vector binds to the semipermeable membrane of the host and therefore the vector is packed in sac to move it to the target cell. and therefore, the sac gets countermined by getting into the cell cathartic vector. Vector then binds with cell introducing it's designed cistron tic material into the host ordering and incorporates into it so creating the proteins victimization new gene within the ordering.

Non-viral vectors have also been used as a substitute agent for biological agents. Like, Injection of Naked DNA through electroporation, gene gun, suboperation magnetoceptions and use of oligonucleotides etc have been used. Several approaches to genome editing have also been developed. CRISPR-Cas9, which is short palindromic sequence is the most effective gene editing technique to date

The CRISPR-Cas9 system consists of two main molecules that introduce a change into the DNA. An enzyme called Cas9, acts as molecular scissors that can cut the two strands of DNA at

a specific location in the genome so that specific strand can be added or deleted. A piece of RNA called guide RNA (gRNA) that consists of a small piece of pre-designed 20 base pair. RNA sequence located within a longer RNA scaffold. The scaffold part binds to DNA and the pre-designed sequence 'guides' Cas9 to the right part of the genome. This makes sure that the Cas9 enzyme cuts at the right point in the genome.

The gRNA has RNA bases that are complementary to those of the target DNA sequence in the genome. This means that, the guide RNA will only bind to the target sequence and no other regions of the genome. The Cas9 follows the guide RNA to the same location in the DNA sequence and makes a cut across both

strands of the DNA. At this stage the cell recognizes that the DNA is damaged and tries to repair it.

Genetic material can be transferred via a vector that is defined as the vehicle that is used to deliver the gene of interest. The ideal vector would transfer a precise amount of genetic material into each target cell, thereby allowing for expression of the gene product without causing toxicity. Gene transfer via the viral vectors is called transduction while transfer via the non-viral vectors is called transfection.

Citation: *Mounica M (2020) Editorial Note on Gene therapy. J Regen Med 9:2.*

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Received: July 10, 2020 **Accepted:** July 20, 2020 **Published:** July 27, 2020

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