Gene Therapy

Fizza Ahsan
1st Year BDS, Islamabad Medical and Dental College, Islamabad Pakistan

Key points

- Introduction
- What is Embryonic Stem Cell
- What is Murine ES cell
- What is Human ES cell

Gene therapy is the modification of gene expression or correction of dysfunctional genes, it offers great potential in treating several diseases. Unlike traditional treatments, gene therapy, through altering of cells, provides possibilities of curing diseases that were once thought to be incurable.1 It's a type of medical treatment that involves modification of DNA. It's an inspiring, yet a complicated treatment option.2

The US Food and Drug Administration (FDA) defines gene therapy as products "that mediate their effects by transcription and/or translation of transferred genetic material and/or by integrating into the host genome and that are administered as nucleic acids, viruses, or genetically engineered microorganisms. The products may be used to modify cells in vivo or transferred to cells ex vivo prior to administration to the recipient".

Gene therapy has three facets:

1. Gene silencing is the inhibition of faulty genes with the use of siRNA, shRNA, and miRNA.
2. Gene replacement is the direct administration of healthy genes in form of plasmids or viral vectors.
3. Gene editing based therapy involves modification of mutations.3

Advantages

Gene therapy has the potential to cure diseases that are thought to be incurable. It works as a one-time dose and treats the persons symptoms for life, also inhibits the transfer of faulty genes to off-springs and helps getting rid of generational diseases.4

A disease named ADA deficiency, which is the resultant of a defective gene in the ADA enzyme, has been given key emphasis in gene therapy at several institutions. It is potent to note that as per a case study, a considerable number of patients who underwent corrective gene treatment were able to recuperate their immune systems and live normal lives inevitably; without special bubbles for isolation which are deemed necessary for the sustenance of a microbe free environment. The stated therapy is proficient in curing an array of complex diseases, namely cancer and coronary diseases. Gene therapy being in its commencement phase is believed to endow effective treatment for innumerable genetic diseases, as it matures.5

Disadvantages

Gene therapy is highly expensive and inaccessible for many people. This method of treatment is still new hence provides no guarantees. It is experimental work so has a lot of uncertainty. It might cause viral infections or alter reproductive cells without detection.6

The most potent challenges are enlisted as the delivery of DNA to the target cells and duration of expression. Genetic therapies, although deemed to be significant in the treatment of innumerable diseases, are still new approaches and hence may hold many risks inevitably, including certain types of cancer, allergic reactions or damage to organs or tissues. Furthermore, The National Institutes of Health neither performs nor funds studies on genome editing that targets sperm, eggs, or embryos in humans. These changes have the potential to be passed on to the patient's children with the perspective of unforeseen adverse effects.

Current trends in gene therapy

As per the contemporary progression of Gene therapy, cancer is the most frequently treated disease. It encompasses an estimate of 60% of all ongoing clinical gene therapy trials in the global arena, followed by monogenetic and cardiovascular diseases.

Presently, an execution of over 1800 approved gene therapy clinical trials worldwide have been carried out or are in an ongoing phase. Adenoviral vectors, retroviral vectors and naked plasmid are classified as the most frequently used gene transfer vectors in clinical trials.

In 2003, China initiated the World's first gene therapy product, namely, Gendicine, described as an oncolytic adenovirus for the treatment of advanced head and neck cancer. Moreover, the commencement of the first CRISPR clinical trial was also in China.

References

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