THE SCIENCE WORLD
A Monthly e Magazine
Popular Article
ISSN:2583-2212
December, 2022; 2(12), 2091-2095

Aspect and Prospect of Gene Therapy in Animals

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https://doi.org/10.5281/zenodo.7424752

Introduction

Gene is the heredity unit or segment of DNA which is transmitted from one generation to the other. Classically a gene was assuming to be a genetic unit by the criteria as a gene is the unit of physiological function that occupies a definite location in the chromosome and is responsible for the expression of the phenotypic character like the wings, color of eyes etc. A gene is a unit of transmission or segregation because it is segregated and exchanged at the time of meiosis. It is a unit of mutation, because by a spontaneous induce change a different phenotypic character is expressed. Because of the defects in the genes many diseases are caused which sometimes have not much cured. Later technique of gene therapy was introduced for correcting defecting genes. Gene therapy is an experimental technique that uses genes to treat or prevent disease. Gene therapy is the type of insertion, alteration or removal of genes within an individual's cells and biological tissues to treat disease.

The following approaches can be made in gene therapy

- Replacing a mutated gene that causes disease with a healthy copy of the gene.
- Inactivating, or "knocking out," a mutated gene that is functioning improperly.
- Introducing a new gene into the body to help fight a disease.

Type

1. On the basis target cell

1. Germline gene therapy: In this, Germ cells, i.e., sperm or eggs are tailored by the introduction of well-designed genes, which are incorporated into their genomes. After the insertion the characters of the gene would be heritable and would be passed on to later generations. This approach is highly effective in fighting genetic disorders and hereditary diseases.

2. Somatic gene therapy: In the case of somatic gene therapy, the remedial genes are shifted into the somatic cells of a patient. Any modifications and effects will be limited to the individual patient only and will not be inherited by the patient's offspring or later generations.

2. On the basis of delivery approaches

1. **Ex vivo technique**

- Isolate cells from the patient with a genetic defect.
- Introduce the therapeutic gene to correct a gene defect.
- Select and grow the genetically corrected cell.
- Transplant the modified cells into the patient.
- In ex vivo the whole process involves genetic modification of cells in culture followed by transplantation.

2. **In vivo technique**

- The direct delivery of a therapeutic gene into target cells in a specific tissue.
- This method could be used on a variety of tissues.
- Examples include the liver, skin, lungs, brain, muscle, and blood cells.
- Gene delivery systems might have two different types likewise in vivo; this process involves direct vector injection into the body.

Method of gene delivery

A gene cannot be directly inserted into a person's cell. It must be transported to the cell using a carrier, or vector. Vector systems can be divided into-

(a) Viral Vectors.

(b) Non-viral Vectors.

Some Example of viral vectors like Adeno virus vector, Adeno-Associated virus vectors. Retro virus vectors and Herpes -simplex virus vectors. The example of non-viral vector is Injection of naked DNA. Physical methods to enhance delivery are Electroporation method, Gene gun method, Sonoporation, Magnetofection and Hydrodynamic delivery. Chemical. method to enhance delivery: Oligonucleotides, Lipoplexes and polymersomes.

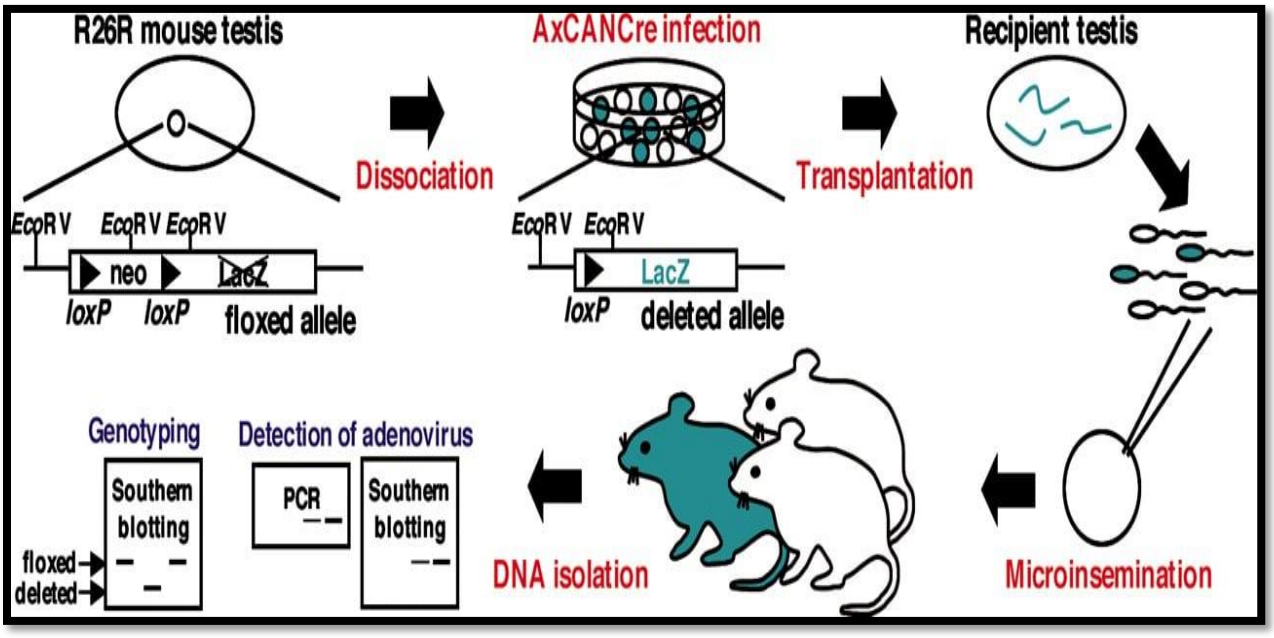

Fig: Diagram of the experimental procedure. Testis cells from donor R26R mice were dissociated by trypsin digestion and infected in vitro by AxCANCre adenovirus. Cre-mediated recombination removed the neo cassette, and LacZ gene expression was initiated under the ROSA26 ubiquitous promoter. The infected cells were transplanted into infertile recipient testes. At 20 weeks after transplantation, recipient testes were mechanically dissociated, and spermatogenic cells were microinjected into oocytes to produce offspring. DNA from the offspring was analyzed by Southern blotting and PCR for integration of adenovirus.

Advantages of Gene Therapy

- Gene therapy is a technique that can be used to modify the genome (germ-line cells, such as eggs or sperm) or somatic cells of specific organs (in vitro or in vivo) of a developing or already developed organism. Gene therapy changes to somatic cells are not inherited by the organism's descendants.
- A vector (usually a disabled virus) is used in gene therapy to 'infect' target cells with the desired gene. In marmoset monkeys, genetic engineering has successfully produced germ-line changes.
- When gene therapy is performed on germ-line cells, the modifications are passed down to the organism's descendants. Hereditary diseases could be cured and eliminated from the germ line using such techniques, and the disease could potentially be eradicated from a species.
- Germline cell therapy cures the disease by altering the gene's DNA sequence at the reproductive level.
- It has the ability to replace defective cells.

- It can help eradicate diseases.
- Gene therapy has the potential to eliminate and prevent hereditary diseases such as cystic fibrosis and is a possible cure for heart disease and cancer.
- Many inherited diseases can be cured by gene therapy.
- Diabetes cured in dog using gene therapy.

Disadvantages of Gene Therapy

- Short lived nature of gene therapy which makes the patients to undergo multiple rounds of gene therapy.
- It is fact that wherever any foreign object in the form of pathogens, vectors or plasmids enter the body, the immune system of the body so response to it. It happens sometimes that immune response does not accept that foreign object and attacks it.
- Problems with viral vectors - Viruses, which is used as vehicles in most gene therapy, present a variety of potential problems to the patient-toxicity, immune and inflammatory responses, and gene control and targeting issues. In addition, there is always the fear that the viral vector, once inside the patient, may recover its ability to cause disease.
- In case of multi gene disorders, such as heart disease, high blood pressure, arthritis are caused by the combined effects of variations in many genes, such type of diseases would be especially difficult to treat effectively using gene therapy.
- It is not always probable that the vector will find the mutated cells, and if it does, it is not certain that the DNA sequence will be expressed. Therefore, better vectors must be developed that can successfully find the faulty cells and insert the DNA sequence accurately.
- Many diseases are polygenic, which means they are caused by several genes. In order for the treatment to be effective, the precise involvement of each gene and the proteins or enzymes for which they code must be determined.

Conclusion

Gene therapy is an emerging field of research and development that seeks new solution to pressing health and environmental problems by combining physical science and engineering with life sciences and medicine. Gene therapy represents the future of medicine and health but its growth is slow in the field application. The real challenge in transplantation is prevention of chronic rejection. The recent development of vectors capable of expressing a gene for extended periods of time has provided new tools to achieve this goal. Newer non-viral vehicles represent a valuable alternative, as they are nearly as efficient as, and potentially safer than viral vectors. Desirable features of future vectors include regulation of gene

expression levels to match clinical needs tissue specific gene expression and multi-cistronic vectors controlled by oral intake of doxycycline or other agents have been tested successfully in vivo but still need to be optimized for clinical applications. Ensure that the genes that have been transplanted are precisely controlled by the body's normal physiology signals.