

Medical (Red) biotechnology

Red or medical biotechnology is the applications of biotechnology in the medical fields and health care.

Gene therapy

Gene therapy is an experimental technique that uses genes to treat or prevent disease. The most common approach for correcting faulty genes is to insert a “normal” gene into the genome to replace an “abnormal” disease-causing gene.

Types of gene therapy

There are 2 types of gene therapy:

1- **Germ line gene therapy**: where germ cells (sperm or egg) are modified by the introduction of functional genes, which are integrated into their genome. Therefore changes due to therapy would be heritable and would be passed on to later generation.

2- **Somatic gene therapy**: where therapeutic genes are transferred into the somatic cells of a patient. Any modifications and effects will be restricted to the individual patient only and will not be inherited by the patient's offspring or any later generation.

Gene delivery

Vectors used in gene therapy are:

1- Viral Vectors

One of the most promising vectors currently being used is harmless viruses. Viruses have evolved a way of encapsulating and delivering their genes to human cells in a pathogenic manner. Some of the different types of viruses used as gene therapy vectors:

Retrovirus, Adenovirus, Adeno-associated viruses [AAVs], Herpes simplex virus [HSV]. Alpha viruses, and Vaccinia or pox viruses.

2- Non-Viral Vectors

Simplest method of non-viral transfection is direct DNA injection. several non-viral methods gene transfer such as: Electroporation, sonoporation, magnetofection and gene guns.

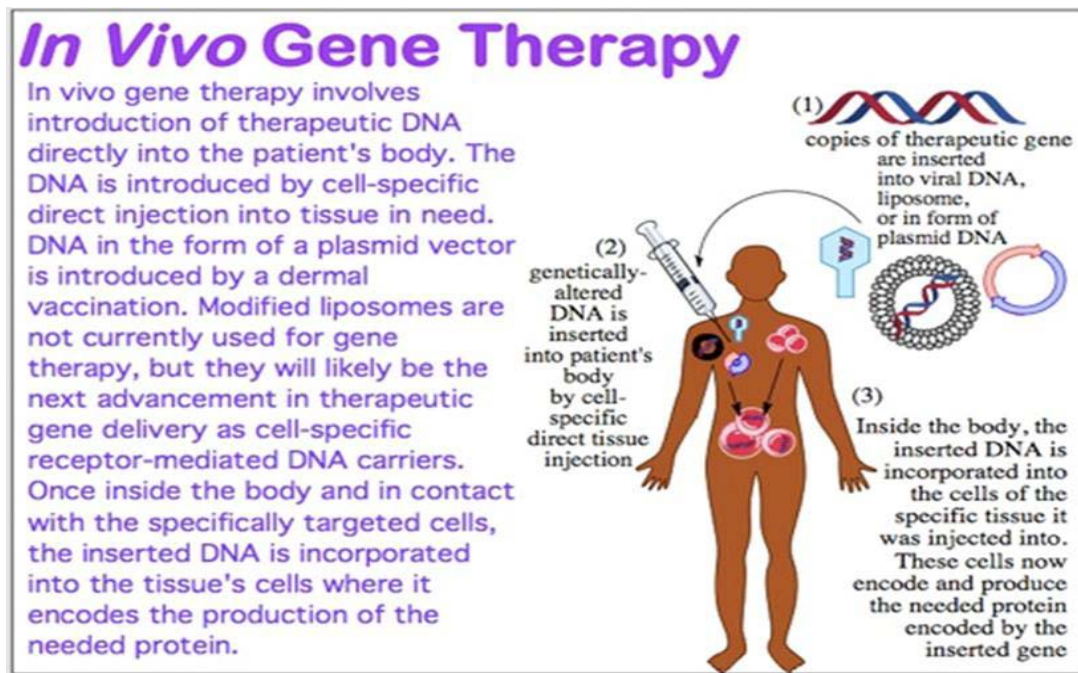
However, these processes are still inefficient, are limited to ex-vivo gene transfer and have undefined cytotoxic effects.

The ideal gene delivery vector should be:

- 1- Very specific
- 2- Capable of efficiently delivering one or more genes of the size needed for clinical application
- 3- Unrecognized by the immune system
- 4- Purified in large quantities at high concentration
- 5- Not induce an allergic reaction or inflammation
- 6- Safe for the patient and environment
- 7- Able to express the gene for as long as is required, generally the life of the patient

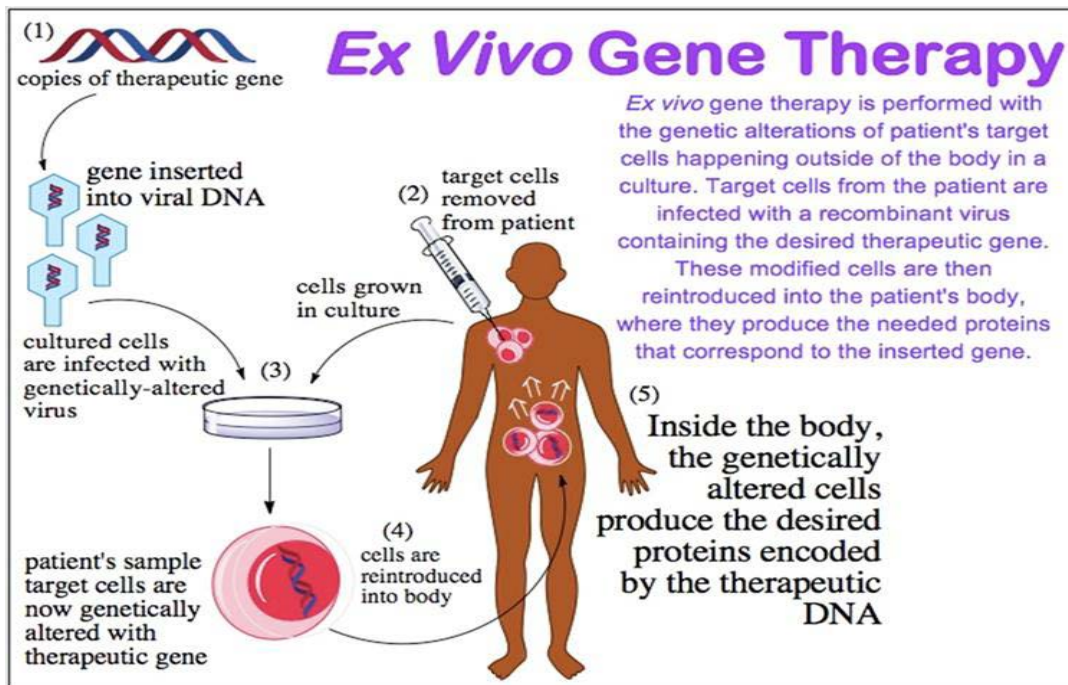
Two techniques have been used to deliver vectors;

1- In vivo gene therapy; the vector can be injected or given intravenously (by IV) directly into a specific tissue in the body, where it is taken up by individual cells.



2- Ex vivo gene therapy; a sample of the patient's cells can be removed and exposed to the vector in a laboratory setting. The cells containing the vector are then returned to the patient.

If the treatment is successful, the new gene delivered by the vector will make a functioning protein.



Stem cell therapy

Stem cells are precursor cells that can divide to produce either more identical stem cells, or many other different cell types in the body. This capability has stimulated enormous interest in the potential of stem cells to replace defective or damaged cells that cause disease.

Two broad categories of stem cells exist:

- 1- **Embryonic stem cells** derived from blastocyst
- 2- **Adult stem cells** which are found in adult tissue

In a developing embryo, stem cells are able to differentiate into all the specialized embryonic tissue. **In adults**, stem cells act as a repair system for the body replacing specialized damaged cells.

Stem cell therapy provides hope for a cure for patients of incurable afflictions such as Parkinson's disease and Alzheimer's disease, and also for people suffering from paralysis resulting from spinal cord injuries. The combination of stem cells with gene therapy might allow rebuilding of new body parts to substitute for old and defective ones. With the use of stem cells to regenerate healthy bone marrow cells, a permanent cure is expected, as healthy cells have the capability to grow and divide continuously.