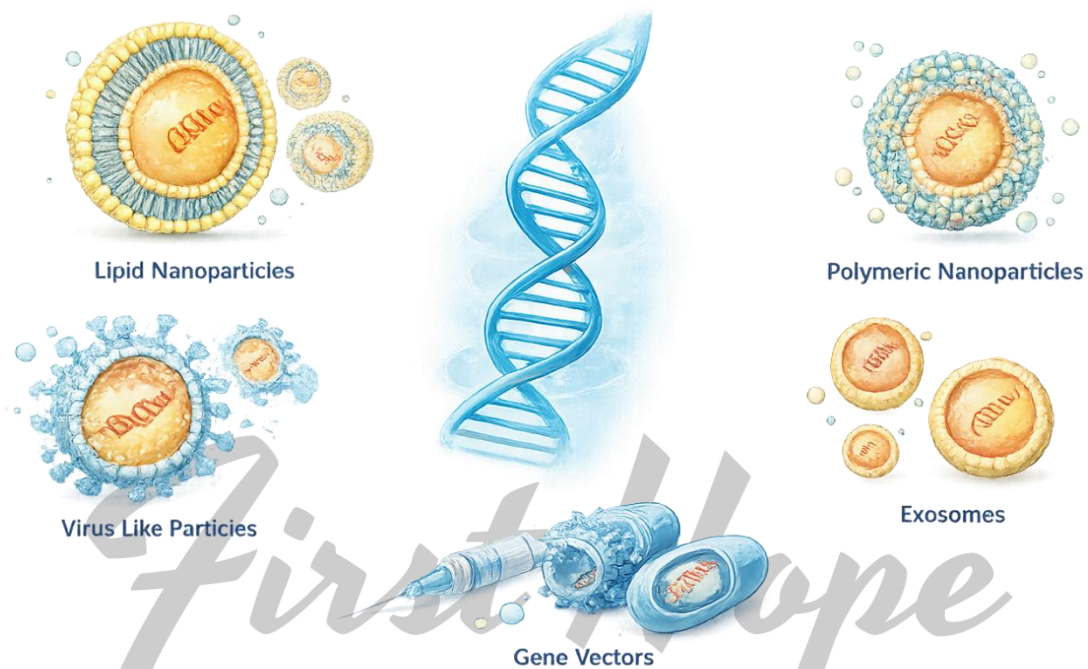


Nucleic Acid Based Therapeutic Delivery System

Nucleic Acid–Based Therapeutic Delivery Systems

What this means

- Nucleic acid–based therapy uses genetic material such as DNA or RNA as medicine.
- Instead of giving a chemical drug, scientists give instructions to the body's cells so they can produce or stop producing certain proteins.



- Every cell in the body contains DNA, which carries instructions for making proteins.
- **Many diseases occur when:**
 - A gene is missing
 - A gene is defective
 - A gene is overactive or underactive
- Nucleic acid–based therapy tries to correct these problems by introducing new genetic material or modifying existing genetic activity.

Types of Nucleic Acids Used



1. DNA (Deoxyribonucleic Acid)

- Used to replace or add a functional gene.
- Example: inserting a healthy gene in place of a defective one.

2. mRNA (Messenger RNA)

- Carries instructions to produce a protein.
- Does not integrate into genome, so effects are temporary.

3. siRNA (Small Interfering RNA)

- Silences specific genes.
- Prevents production of harmful proteins.

4. Antisense Oligonucleotides (ASOs)

- Bind to mRNA and block its function.

Main challenge

- Nucleic acids are fragile and easily destroyed in the body.
- **So, they need a delivery system to:**
 - Protect them from enzymes
 - Help them enter cells
 - Reach the correct tissue
- **Delivery systems**
 - Viral vectors (modified viruses)
 - Non-viral carriers (liposomes, nanoparticles)
 - Physical methods (electroporation, gene gun)

Gene Therapy (Ex-vivo and In-vivo)

- Gene therapy is a technique where genes are added, removed, or modified inside a patient's cells to treat disease.

Ex-vivo Gene Therapy

This method involves modifying cells outside the body.

Steps:

- Cells are taken from the patient (for example, blood cells or stem cells).
- These cells are grown in the laboratory.
- A correct gene is introduced into the cells using a vector.
- Modified cells are checked and multiplied.

- The corrected cells are returned to the patient.

Advantages:

- Better control over gene insertion
- Safer because cells can be tested before reintroduction

Disadvantages:

- More complex procedure
- Expensive and time-consuming

Example:

- Treatment of certain immune deficiencies.

In-vivo Gene Therapy

In this method, the gene is delivered directly into the patient's body.

Steps:

- A vector carrying the gene is prepared.
- It is injected directly into the body (blood, muscle, or organ).
- The vector enters cells and delivers the gene.

Advantages:

- Simpler and less invasive
- Suitable for internal organs

Disadvantages:

- Less control over where the gene goes
- Risk of immune reactions

Example:

- Treatment of retinal diseases.

Potential Target Diseases for Gene Therapy

- Gene therapy is mainly used for diseases caused by genetic defects or abnormal cell growth.

Inherited Disorders

These are diseases caused by mutations in genes.

Examples:

1. Cystic Fibrosis

- Caused by mutation in CFTR gene

- Leads to thick mucus in lungs
- Gene therapy aims to deliver normal CFTR gene

2. Hemophilia

- Blood clotting disorder
- Missing clotting factor gene
- Therapy introduces functional gene

3. Sickle Cell Anemia

- Abnormal hemoglobin structure
- Gene therapy corrects mutation in blood stem cells

4. Duchenne Muscular Dystrophy

- Muscle degeneration due to defective dystrophin gene
- Therapy introduces functional gene or modifies expression

Approach

- Replace defective gene
- Add functional copy of gene

Cancer

Cancer involves uncontrolled cell growth due to genetic changes.

Gene therapy approaches:

1. Tumor Suppressor Gene Replacement

- Restores genes that prevent cancer growth (e.g., p53)

2. Suicide Gene Therapy

- Introduces gene that converts harmless drug into toxic substance inside cancer cells

3. Immunotherapy

- Modifies immune cells to attack cancer (e.g., CAR-T therapy)

4. Oncolytic Viruses

- Viruses engineered to infect and kill cancer cells

Challenges

- Targeting only cancer cells
- Avoiding damage to normal cells

Gene Expression Systems (Viral and Non-viral Gene Transfer)

- These systems are used to deliver genes into cells.

Viral gene transfer

Viruses naturally deliver genetic material into cells, so they are modified for therapeutic use.

Common viral vector Types:

1. Adenovirus

- Does not integrate into genome
- High expression but temporary
- Strong immune response

2. Retrovirus

- Integrates into host DNA
- Long-term expression
- Risk of mutation (insertional mutagenesis)

3. Lentivirus

- Can infect non-dividing cells
- Used widely in gene therapy

4. Adeno-associated virus (AAV)

- Safer, low immune response
- Limited gene size capacity

Advantages

- High efficiency
- Strong gene expression

Disadvantages

- Immune reactions
- Safety concerns

Nonviral gene transfer

These methods use physical or chemical techniques.

Methods:

1. Liposomes

- Lipid vesicles that carry DNA
- Fuse with cell membrane



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