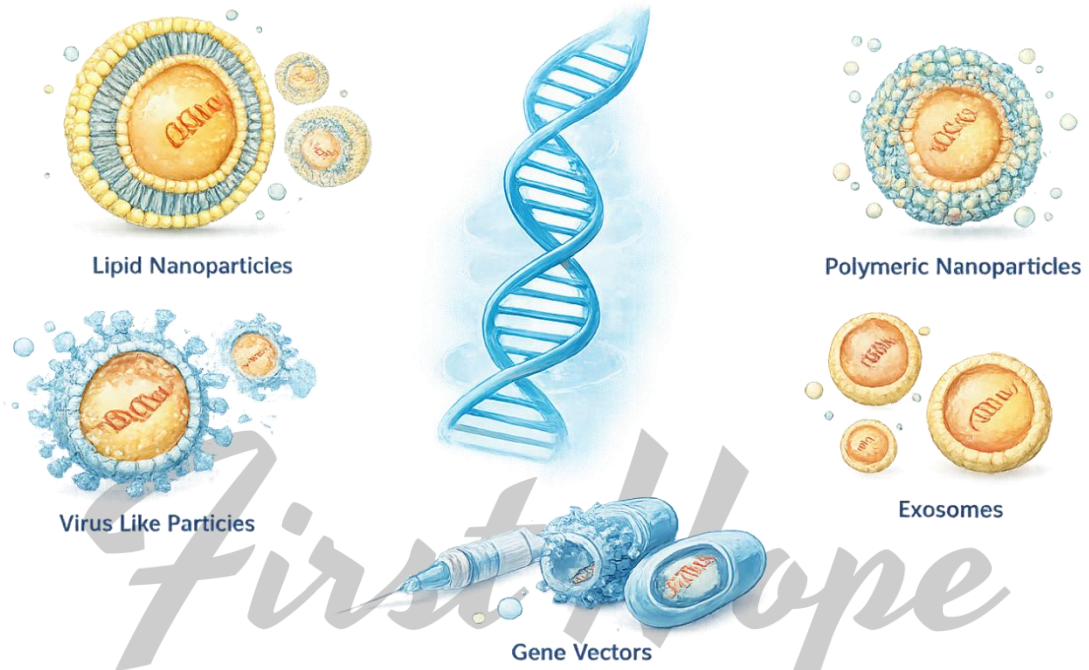
Nucleic Acid Based Therapeutic Delivery System

Nucleic Acid–Based Therapeutic Delivery Systems

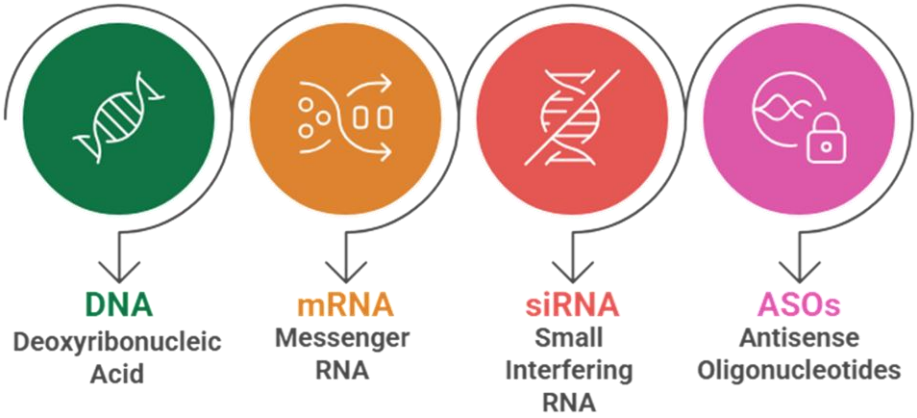
What this means

- 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



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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)

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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.

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- 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

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- 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

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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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