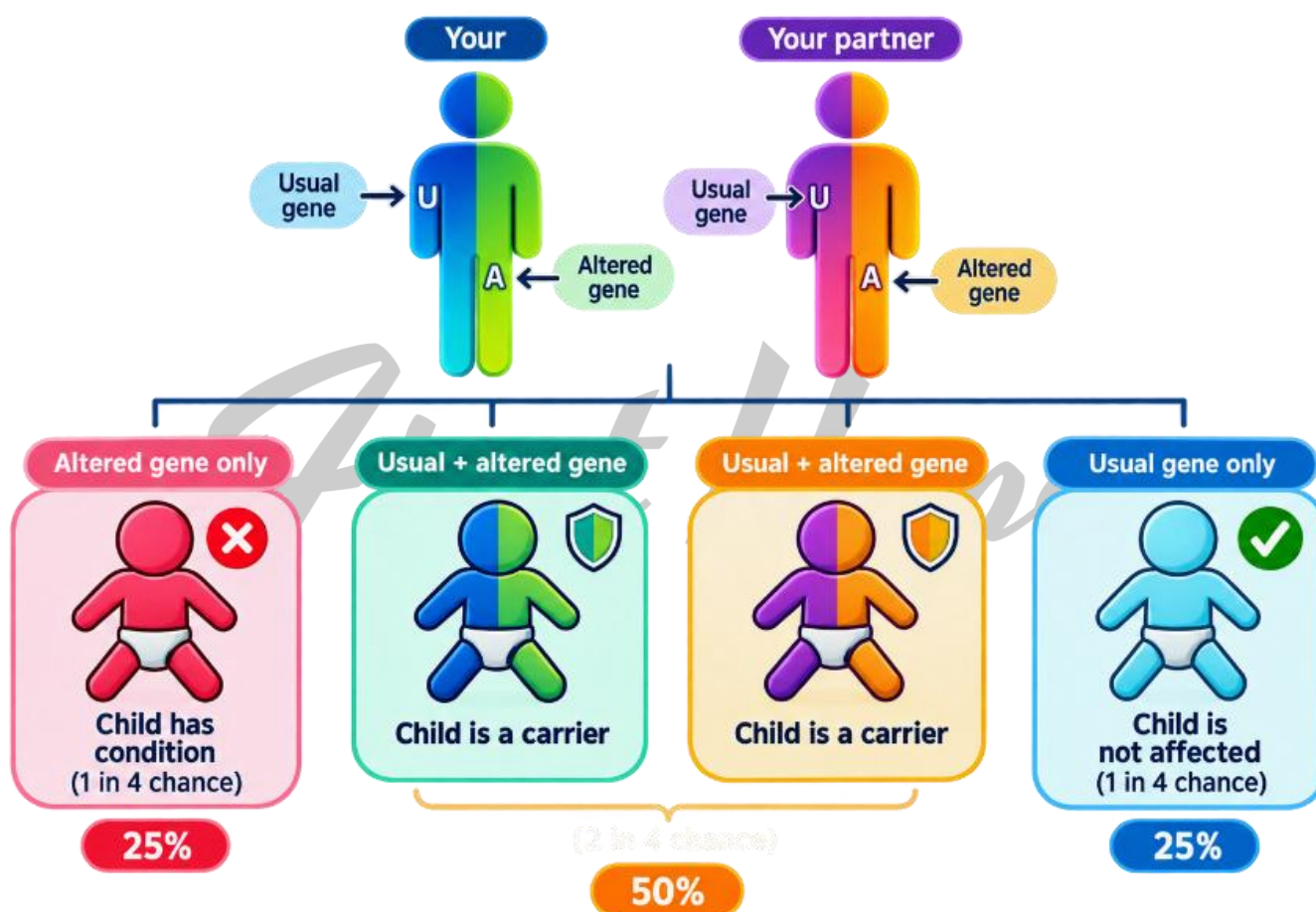


Biologic Medicines in Development for Various Diseases – By Therapeutic Category

Genetic Disorders

What Are Genetic Disorders?

- **Genetic disorders** are diseases caused by changes or mistakes in a person's genes or chromosomes.
- Genes are instructions inside the body that tell cells how to grow, function, and repair themselves.



- When a gene is faulty, the body may make harmful protein, make too little of an important protein, or make none at all.
- This can lead to inherited diseases, metabolic disorders, muscle diseases, blood disorders, and many other conditions.

What Are Biologic Medicines?

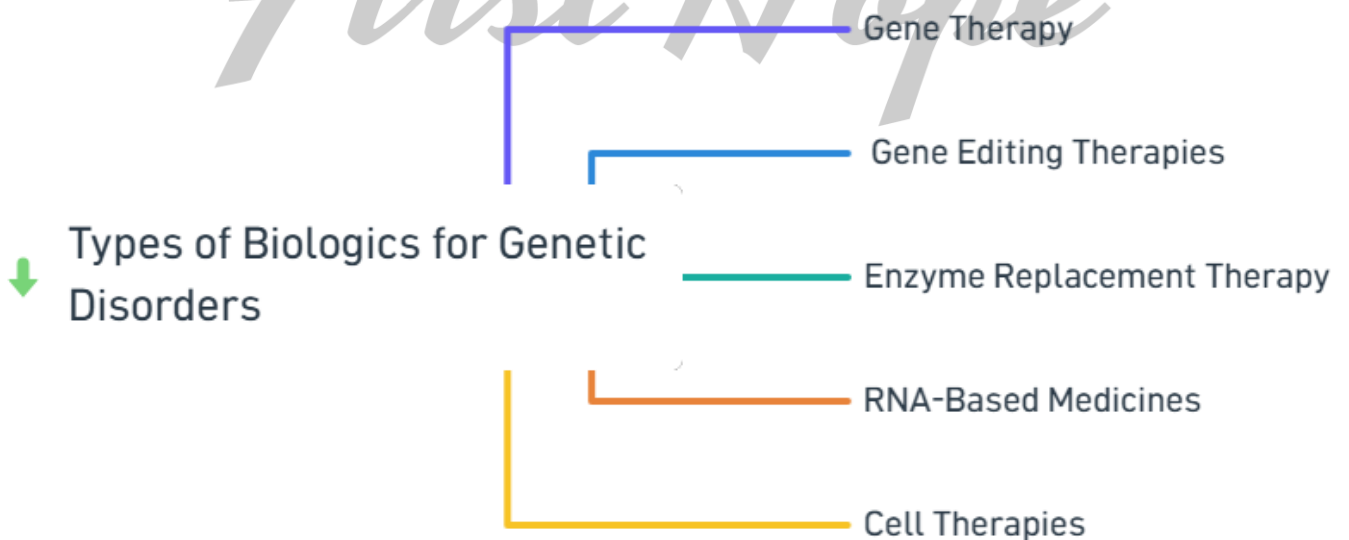
- Biologic medicines are treatments made from living organisms or their components.
- They are often produced using biotechnology.
- Unlike ordinary chemical drugs, biologics are large, complex molecules.
- **They may include:**

- Monoclonal antibodies
- Gene therapies
- Cell therapies
- Protein replacement therapies
- Enzyme replacement therapies
- RNA-based treatments

Why Biologics Are Important for Genetic Disorders

- Many genetic disorders happen because something important is missing or defective.
- **Biologics can:**
 - Replace missing proteins
 - Deliver healthy genes
 - Correct defective genes
 - Modify how cells behave
 - Slow disease progression
 - In some cases, offer long-term correction

Types of Biologics Being Developed for Genetic Disorders



A. Gene Therapy

- Gene therapy tries to treat disease by adding, replacing, or repairing faulty genes.

How it works:

- Scientists use carriers called vectors (often modified viruses) to deliver healthy genes into cells.
- The healthy gene may help the body make a needed protein.

Being developed for:

- Cystic fibrosis
- Hemophilia
- Duchenne muscular dystrophy
- Sickle cell disease
- Spinal muscular atrophy

Goals:

- Treat disease at its cause
- Reduce symptoms
- Possibly provide long-lasting benefit from one treatment

B. Gene Editing Therapies

- These go beyond adding a gene and aim to directly fix mutations.

Examples of technologies:

- CRISPR
- Base editing
- Prime editing

How they help:

- Cut or repair damaged DNA
- Turn harmful genes off
- Restore normal gene function

Research areas:

- Inherited blindness
- Blood disorders
- Rare metabolic diseases

C. Enzyme Replacement Therapy

- Some genetic diseases happen because the body lacks certain enzymes.

Biologic treatment:

- Scientists make the missing enzyme in laboratories.
- Patients receive it by infusion.

Used or being developed for:

- Gaucher disease
- Pompe disease
- Fabry disease

- Lysosomal storage disorders

Purpose:

- Replace what the body cannot produce

D. RNA-Based Medicines

- These biologics act on RNA, which helps cells make proteins.

Types:

- Antisense therapies
- RNA interference therapies

What they do:

- Silence harmful genes
- Correct faulty protein production
- Reduce toxic proteins

Examples in development:

- Huntington's disease
- Muscular disorders
- Rare inherited diseases

E. Cell Therapies

- Some treatments use modified living cells.

Examples:

- Stem cell therapies
- Modified blood-forming cells

Possible uses:

- Replace diseased cells
- Repair damaged tissues
- Restore normal function

Challenges in Developing Biologics for Genetic Disorders

- Many disorders are very rare.
- Small patient populations make research harder.
- Gene delivery can be difficult.
- Long-term safety must be studied.
- Some therapies may be extremely expensive.

Eye-Related Disorders

What Is Eye-Related Disorders?

- These are diseases affecting vision or structures of the eye.
- Some lead to mild vision problems.
- Others may cause blindness.

Common conditions being targeted:

- Macular degeneration
- Diabetic retinopathy
- Glaucoma
- Retinal diseases
- Inherited blindness
- Dry eye disease

Why Biologics Are Used in Eye Disease

- Many eye diseases involve:
 - Abnormal blood vessel growth
 - Inflammation
 - Cell damage
 - Genetic defects
- Biologics can target these specific problems.

Types of Biologics in Development on Eye-Related Disorders



A. Anti-VEGF Biologics

- VEGF means Vascular Endothelial Growth Factor.
- It causes abnormal blood vessels to grow.



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