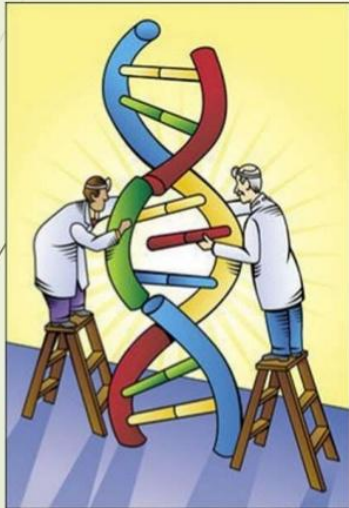


What is Gene Therapy?



- An approach of treating diseases by either modifying the expressions of an individual's genes or correction of abnormal genes.

Cont....

- This can be accomplished by:
- **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.

History



- ▶ **1960's** : The concepts of Gene Therapy was introduced.
- ▶ **1972** : Friedman and Roblin authored a paper in Science titled "Gene therapy for human genetic disease."
- ▶ **1984**: A retrovirus vector system was designed that could efficiently insert foreign genes into mammalian chromosomes.



- ▶ **1990**: The first approved gene therapy in the US took place on 14 September 1990, at the National Institutes of Health (NIH), under the direction of William French Anderson.



- ▶ Four-year-old Ashanti DeSilva received treatment for a genetic defect that left her with **ADA-SCID**, a severe immune system deficiency.