

# BIOTECHNOLOGY and BIOPHARMACEUTICS

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-GENE THERAPY



## **WHAT IS GENE THERAPY?**

**Gene therapy modifies a person's genes to treat or cure disease.**

## **EDITING**

**Replacing a disease-causing gene with a healthy copy of gene**

## **SILENCING**

**Inactivating a disease-causing gene that is not functioning properly**

## **ADDITION**

**Introducing a new or modified gene into the body to help treat a disease**

# TYPES OF GENE THERAPY

**Somatic CELL gene therapy**

**Germ line gene therapy**

**Gene Therapy Approaches**  
*ex vivo* and *in vivo*



# **DIFFERENT CARRIER SYSTEMS ARE USED FOR GENE DELIVERY**

**PHYSICAL**

**CHEMICAL**

**BIOLOGICAL**

Typically, viruses are used as vectors because they have evolved to be very good at sneaking into and infecting cells. But in this case, their motive is to **insert the new genes into the cell.**

**September 14, 1990, Ashanti DeSilva**  
**adenoside deaminase (ADA): bubble boy disease**



ANY  
QUESTION?



Thank you

