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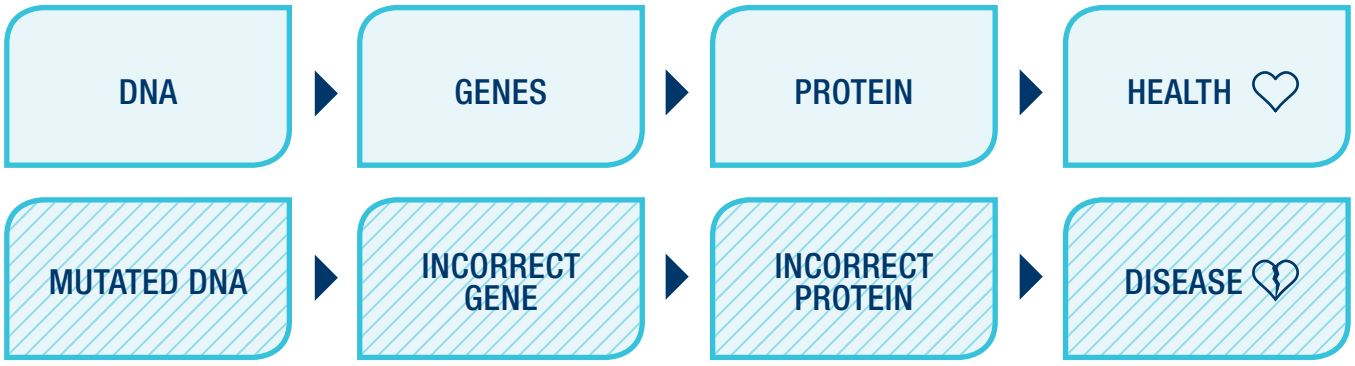
GENE THERAPY

Genetic diseases are caused by errors (or mutations) in one or more **genes. Genes** are strands of **DNA**, which provide the cell instructions for making **proteins.** Proteins are required for the body to function in a healthy manner. Errors in DNA can prevent a gene from making correct proteins, and therefore lead to disease.



Humans have approximately **20,000 genes**, and there are more than **6,000 genetically based diseases.** As our understanding of genetics advances, this number will continue to grow.

TREATING GENETIC DISEASES WITH GENE THERAPY:

Gene therapy is a type of medicine designed to treat a genetic disease by adding the functioning gene or genes into a specific cell (e.g., liver cells, bone marrow cells), which allows the patient's body to return to good health. Gene therapy can also be used to reduce the activity of a harmful gene.

GENE THERAPY APPLICATIONS:

Currently there are many gene therapies being developed to treat multiple diseases, including **hemophilia, inherited retinal diseases, myeloma, phenylketonuria (PKU),** and **Huntington's disease.**

These gene therapies are used on **somatic (non-heritable)** cells. Using gene therapy on **non-heritable** cells means the therapy does not change the genes that a person passes on to their children.

For additional information on other types of technologies used to treat disease, such as somatic gene editing, please visit BIO.org and BIO.org/GenomeEditing, where you can also find BIO's position on human germline editing

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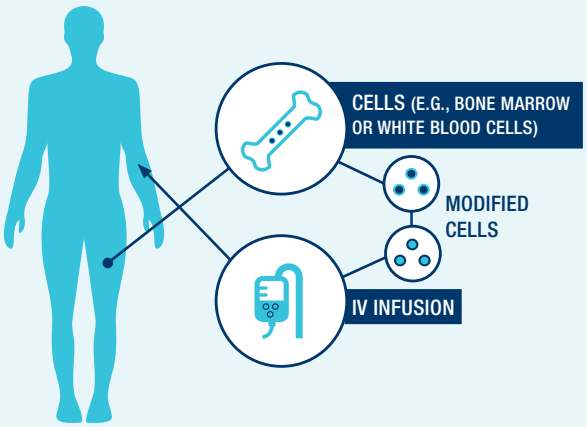
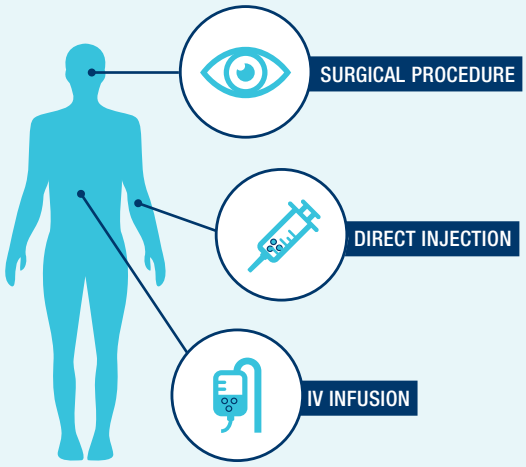
HOW GENE THERAPY WORKS

TREATING PATIENTS WITH GENE THERAPIES

Gene therapy can happen ex vivo (outside the body) or in vivo (inside the body). Each delivery method has benefits and limitations, and preference of method depends on the disease being treated.



EX VIVO GENE THERAPY (OUTSIDE THE BODY)



IN VIVO GENE THERAPY (INSIDE THE BODY)

THERE ARE DIFFERENT TYPES OF GENE THERAPY PRODUCTS, INCLUDING:

- **Plasmid DNA:**
  Circular DNA molecules designed to carry therapeutic genes into human cells
- **Viral or bacterial vectors:**
  Delivery systems used to insert the new genes directly into cells and specific tissues in the body
- **Genetically-modified patient-derived cells:**
  Cells are removed from the patient (i.e., autologous). They are genetically modified, often using a viral vector, and then returned to the patient.
- **Genetically-modified donor-derived cells:**
  Cells are collected from a donor (i.e., allogeneic source). They are genetically modified, often using a viral vector, and then given to the patient

CURRENT BIOTECH ECOSYSTEM

3 approved gene therapy products in the U.S., which include AAV gene therapy (in vivo) and CAR T-cell therapies (ex vivo) to treat a type of congenital blindness or leukemia, respectively

388 drug programs under development

139 companies developing gene therapies

Data collected as of December 2018

For a full list of FDA-approved products visit https://www.fda.gov/biologicsbloodvaccines/cellulargenetherapyproducts/approvedproducts/default.htm