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.

**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.

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.

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.
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](https://www.fda.gov/biologicsbloodvaccines/cellulargenetherapyproducts/approvedproducts/default.htm)