

The BODY and GENETIC DISEASE

01 CELLS

Every living thing is made up of one or many building blocks called *cells*. The human body has trillions of cells. Each cell contains many parts, each with its own purpose.¹

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CHROMOSOMES

Chromosomes are found in the nucleus (the control center) of each cell. They are made up of proteins and DNA (deoxyribonucleic acid).¹ Except for sperm and egg cells, every cell in a human body normally has 23 pairs of chromosomes (for a total of 46). One set is inherited from your mother and the other from your father.²

DNA

DNA is housed within chromosomes and is like an instruction manual for the body. It is made of 4 chemical building blocks: adenine (A), cytosine (C), guanine (G), and thymine (T). These chemical building blocks pair together to form base pairs. The order or sequence of these base pairs helps determine our physical characteristics, such as skin coloring and height.¹

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GENES

All of a person's DNA is called a *genome*. Within the genome are many smaller sections of DNA called *genes*.³ Each gene is also made up of a unique sequence of bases (A, C, G, T).⁴ Some genes provide instructions that tell each cell how to make proteins.¹

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PROTEINS

Proteins are the workers of the cell. They perform different tasks in cells, such as making eye color, powering muscles, and attacking invading bacteria. They are required for the structure, function, and regulation of the body's tissues and organs.⁵

GENE MUTATIONS & GENETIC DISEASES

A gene mutation is a change in DNA that may alter a protein's function or production. This change can prevent a gene from working correctly. Gene mutations can be passed down from parents (hereditary), but they can also occur randomly or be due to environmental factors (acquired). Some gene mutations are harmless, but others can result in genetic diseases that affect health.⁶

GENE THERAPY

Gene therapy is a type of treatment that aims to add, delete, or correct a gene that is not working properly. Its goal is to treat a disease at the genetic level (the source). Gene therapy is currently being investigated for the treatment of multiple diseases.⁷

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References: 1. National Institutes of Health. Genetics Home Reference. Cells and DNA. Accessed July 27, 2020. <https://ghr.nlm.nih.gov/primer#basics>. 2. Genetic Alliance; The New York–Mid-Atlantic Consortium for Genetic and Newborn Screening Services. *Understanding Genetics: A New York, Mid-Atlantic Guide for Patients and Health Professionals*. Genetic Alliance Monographs and Guides; 2009. https://www.ncbi.nlm.nih.gov/books/NBK115563/pdf/Bookshelf_NBK115563.pdf. 3. National Institutes of Health. Genetics Home Reference. What is a genome? Accessed July 27, 2020. <https://ghr.nlm.nih.gov/primer/hgp/genome>. 4. National Institutes of Health. ACGT. Accessed August 6, 2020. <https://www.genome.gov/genetics-glossary/acgt>. 5. National Institutes of Health. Genetics Home Reference. How genes work. Accessed July 27, 2020. <https://ghr.nlm.nih.gov/primer/howgeneswork>. 6. National Institutes of Health. Genetics Home Reference. Mutations and health. Accessed July 27, 2020. <https://ghr.nlm.nih.gov/primer/mutationsanddisorders>. 7. National Institutes of Health. Genetics Home Reference. Gene therapy. Accessed July 30, 2020. <https://ghr.nlm.nih.gov/primer>.