Building a
healthier future

Cell & Gene Therapy

Gene Therapy

Genes: The blueprint for the human body

Genes instruct the body's cells how to produce the necessary building blocks of life in order to function properly. Our DNA, which is made up of our genes, is comparable to a book with words containing just four basic letters: A, C, G and T. Arranged correctly, they provide the building instructions for proteins. Using the body's own language, written in genes, gene therapy delivers specific instructions for the cells to start producing treatments in the place in the body where they are needed.

What is gene therapy and how does it work?

In short, gene therapy is the use of genetic material as a treatment. This can mean the introduction (example shown below), removal, or change of genetic material in the cells of a patient to treat an inherited or acquired disease. Successful gene therapy requires three main components: an optimized vector, a promoter, and a disease specific therapeutic transgene.

Vector

Delivers the transgene to target cells

Think of a vector like a shuttle, transporting the desired gene into the body's cells to restore the function of the defective gene. Currently, viral vectors are the most common vehicle used in FDA-approved gene therapies. These viruses are modified so that they don't cause disease.

Promoter

Drives transgene expression in target tissue

A Promoter is a piece of DNA sitting in front of the therapeutic transgene and activates its transcription, which is the initiating process yielding a protein. Promoters can be rationally designed to act like a switch, regulating the activity of a gene.

Transgene

Holds all the genetic information for the cells to deliver the therapeutic response

The third component is a functional version of the therapeutic gene called a transgene. Once packed into the vector, it travels to the target cell and delivers the correct instructions.

Beyond viral vectors, non-viral techniques are actively being studied for their safety and efficacy. This includes, for example, lipid nanoparticles.

Did you know there are around 5,600 rare genetic diseases[1], 95% of which currently have no treatment[2]?

Making gene therapy a reality for patients in need

Health begins with understanding, and in recent decades, science has come to understand a lot about the human body. Particularly, our genetics. This has unlocked promising options for doctors and patients to treat some of the most debilitating diseases.

With hundreds of ongoing clinical trials and multiple regulatory approvals in recent years[3], gene therapy is already a reality today. What makes gene therapy so powerful is that it addresses disease at its source, the gene, opening the possibility to transform the standard of care for patients who have limited or no treatment options today.

Bayer's commitment to advancing gene therapy

In 2020 we ventured into gene therapy, acquiring Asklepios BioPharmaceutical (AskBio), who are one of the pioneers in gene therapy technologies. The expertise and portfolio of AskBio support us in developing highly innovative treatment options for patients and strengthens our presence in the gene therapy space.

AskBio

Did you know that several gene therapies have been approved for the treatment of various conditions, such as some types of ocular diseases, spinal muscular atrophy, or beta thalassemia, a blood disorder?[3]

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1 Ehrhart F. et al., Scientific Data 8, 124 (2021).
2 Editorial: Spotlight on rare diseases, The Lancet Diabetes & Endocrinology 2019; 7(2): 75
3 Gene, Cell, & RNA Therapy Landscape, Q2 2022 Quarterly Data Report, ASCGT