Building a healthier future

Cell Therapy & 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 generally considered to have no concerns for causing disease.

Promoter

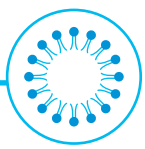
Drives transgene expression in target tissue

A promoter is a piece of DNA sitting before the therapeutic transgene and activates its transcription, which initiates the process ultimately 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 delivery techniques are actively being studied for their safety and efficacy. This includes, for example, lipid nanoparticles.

Gene therapy success stories

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]

There are thousands of known genetic diseases. They affect children disproportionately, and 95% of them have no treatment.[1,2] Many of these are genetic diseases, often caused by a single gene, and perfect candidates to be addressed with gene therapy.[4]

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

Bayer's commitment to advancing gene therapy

Bayer's efforts in the field of gene therapy are driven with our subsidiary AskBio, a gene therapy leader holding more than 750 patents in the area. Our scientists are currently working on the development of multiple potential treatments that are intended to help patients suffering from some of the most debilitating diseases across several therapeutic areas. These include cardiovascular, neurological and rare diseases. We also collaborate with other gene therapy specialists to expand the range of future application of our gene therapy platform.

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, gene therapy is already a reality today. What makes gene therapy so powerful is that it can address many diseases at their source, often a defective gene, opening the possibility to transform the standard of care for patients who have limited or no treatment options today.

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1 Global Data Access for Solving Rare Disease: A Health Economic Value Framework". World Economic Forum and all references cited within, February 2020. www.weforum.org/docs/WEF_Global_Data_Access_for_Solving_Rare_Disease_Report_2020.pdf

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

4. https://www3.weforum.org/docs/WEF_Global_Data_Access_for_Solving_Rare_Disease_Report_2020.pdf