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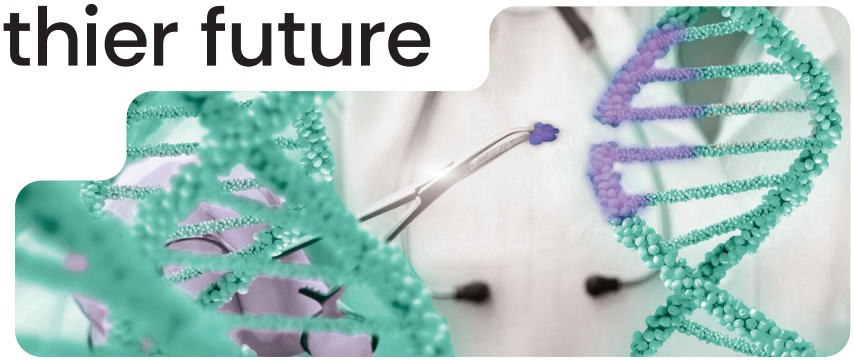
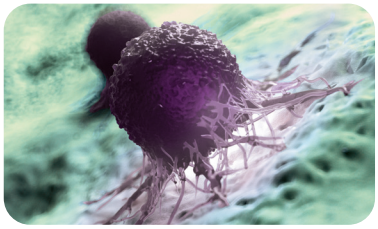
Building a healthier future
Gene Therapy



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.

Delivers the transgene to target cells

VECTOR

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

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

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.

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]

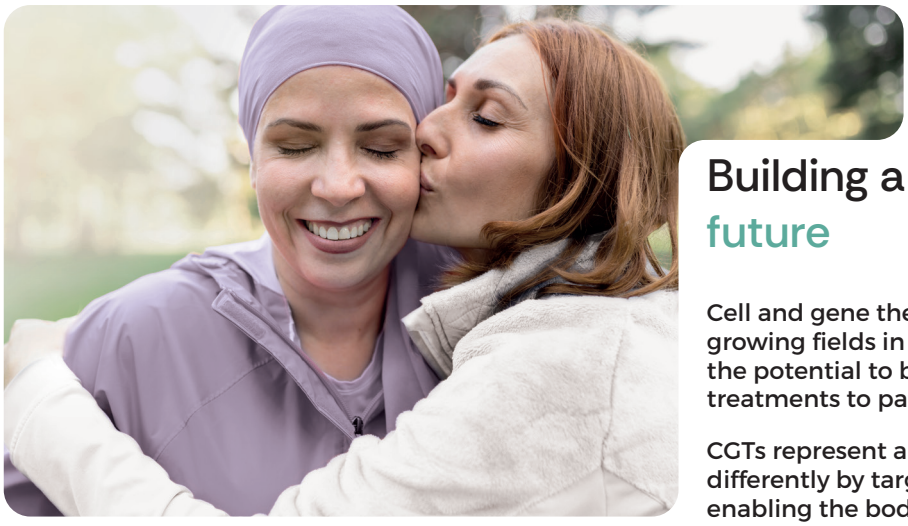
Gene therapy success stories

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]

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.



Building a healthier future

Globally, there are more than **3,800** CGTs in development.

Cell and gene therapies (CGTs) are some of the fastest growing fields in modern healthcare, and they have the potential to bring targeted and personalized treatments to patients.

CGTs represent an important opportunity to treat diseases differently by targeting the underlying cause of disease or enabling the body to restore vital functions.

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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

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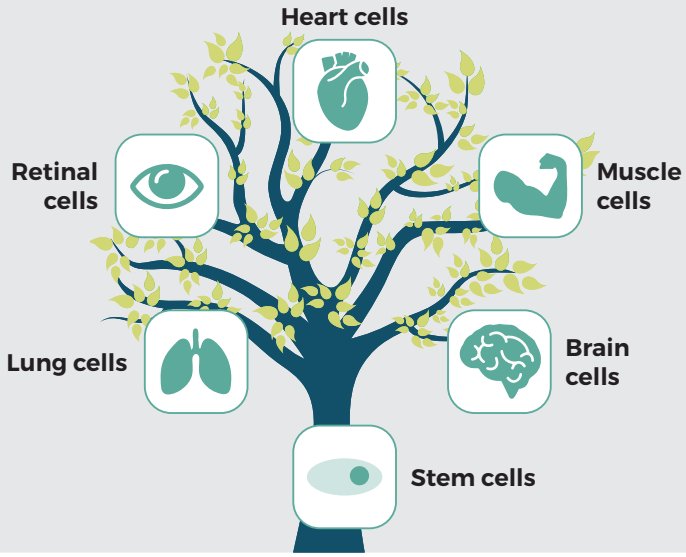
Building a healthier future
Cell Therapy

What is cell therapy?

Many diseases are caused by malfunctioning or damaged cells. What if, instead of treating disease symptoms, doctors could restore function and health by replacing cells that were lost or damaged by disease?

All specialized cells in our body, such as brain or heart cells, begin as tiny, blank canvases full of potential and possibilities. Otherwise known as stem cells, these cells can change and mature to take on their own distinct form and function.

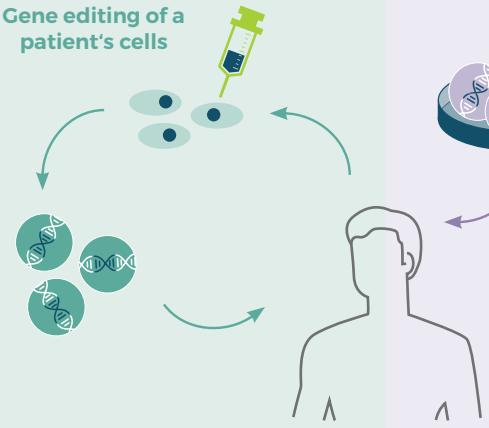
In short, cell therapy is the use of living cells to treat diseases: engineered and cultivated cells are transferred to the patient in order to replace or repair damaged cells or to transfer repurposed cells to enhance their function. This is rather complex and can be performed in two different ways:



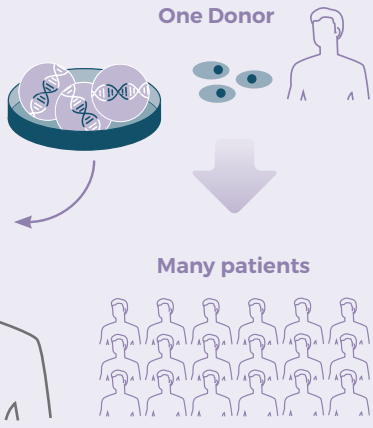
The human body is made up of around **200 different types** of cells, each specialized to carry out a particular role.

Autologous cell therapy

Transplanted cells come from the patients themselves. This therapy limits the concern for transplant rejection, which is a benefit for recipients. However, creating large batches with controlled quality poses a challenge.



Allogeneic cell therapy



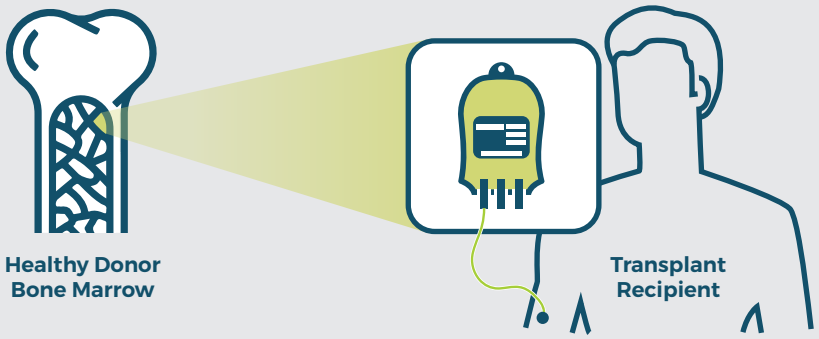
Transplanted cells come from a donor, who is a person other than the recipient of the cells. This is promising because donor-based therapies can form the basis of "off the shelf" products. The downside is that "foreign" cells are more easily rejected by the recipient.

Replace degenerated cells or repurpose cells

Cell therapy success stories

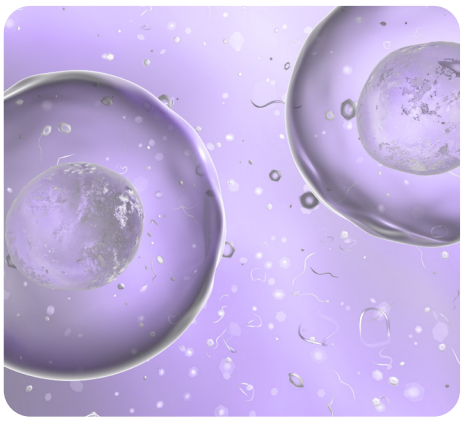
First-generation cell therapies are already positively impacting the lives of patients around the world. Using advanced technologies allows for the development of therapies for blood disorders, including some types of leukemia and lymphoma. In fact, there are already several approved oncological cell therapies that are currently used to treat patients.

Bone Marrow Transplant

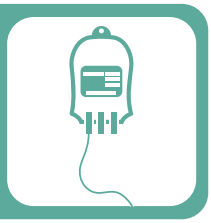


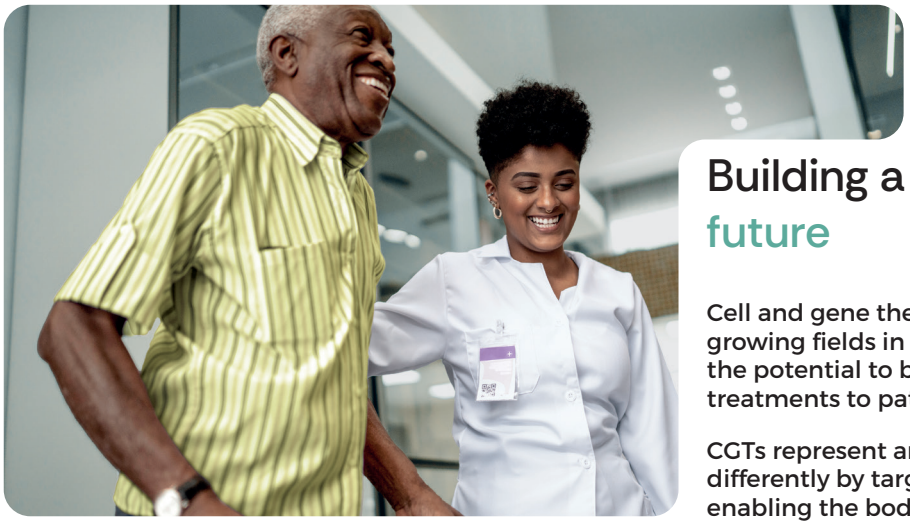
Many patients have by now received stem cell transplants, for example bone marrow transplants.

These stem cells help cancer patients to develop new blood cells after their own stem cells have lost their ability to do so, e.g. after chemotherapy.



Did you know that bone marrow transplants are one of the most established examples of cell therapy used to effectively treat diseases? In fact, more than **50,000** of these transplants take place each year worldwide![1]





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[1] Alijurf M., Weisdorf, D., Alfraih, F. et al., Bone Marrow Transplant 2019; 54: 1179-1188.