



Cell therapy: replacing or improving existing cells

RNA, gene and cell therapies are so-called genetic medicines. These innovative therapies treat the cause of diseases caused by:

- mutations found in genes from birth or
- mutations caused by factors such as the sun, air pollution, alcohol, etc.

Traditional medicines can sometimes treat the symptoms of these diseases but are not able to remove the cause. In contrast, genetic medicines can treat the underlying cause. These medications have not only enabled the development of improved therapies but have also produced treatments for conditions for which there is currently no medication.

There are already 100 medicines based on these technologies on the market, treating for example certain forms of ALS and cancer, as well as hemophilia and cardiovascular diseases.

This article provides an overview of cell therapy's benefits and drawbacks as well as some examples of available treatments. It concludes with an overview of future developments.

What is cell therapy?

Cell therapy involves using the patient's own cells or those from a healthy donor to treat or prevent a disease. The cells are 'manipulated' to do something they did not do before. The improvement or replacement of cells is accomplished through techniques such as stem cell therapy or CAR-T.

How does stem cell therapy work?

Stem cell therapy uses stem cells from a donor's blood or bone marrow. The cells are inserted directly into the patient's body, and can be applied, for example, in the treatment of conditions like cancer. The new stem cells can then produce blood cells that can replace cancer cells.

There are already many forms of this therapy on the market. One promising new development is that of our portfolio company Vertex. In October 2023, Vertex reported positive news about one of its cell therapies for type-1 diabetes. All patients who received this cell therapy showed improvement in their glucose regulation. If the therapy proves safe and effective, it will be a major breakthrough in the treatment of diabetes as patients would no longer need to inject insulin daily.

How does CAR-T work?

Chimeric Antigen Receptor T cell (CAR-T cell) technology is used to treat cancer. CAR-T involves extracting T cells, a type of white blood cell that plays an important part in the immune system, from a patient's blood. These cells are genetically modified to specifically recognize and kill cancer cells and then returned to the patient. This is done through an infusion into a vein.

An example is Yescarta for blood cancer (lymphoma), which was approved by the FDA in 2017 and in the EU in 2018.

Advantages and disadvantages of cell therapy

One or more treatments of cell therapy can have long-term or permanent effects. This gives promise for the treatment of diseases for which no effective treatment is yet available.

As with any medical treatment, there are potential risks and side effects associated with cell therapy. For example, immune reactions, unwanted cell division or other unknown long-term effects. Cell therapy technology is still developing, and it is sometimes difficult to predict how the body will react.

The future

Cell therapy appears to have a bright future due to ongoing advancements and breakthroughs. It is not yet suitable for all conditions and patient groups, but as our knowledge of cell biology increases, new applications will be discovered. For example, for neurological disorders and autoimmune diseases.