

What is the potential of cell and gene therapies?

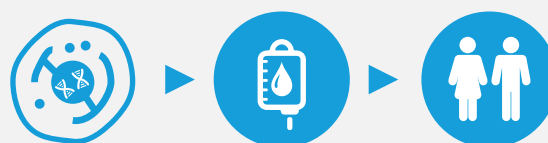
Stem cells already save lives in routine medical practice throughout the world. The use of a patient's own or donated bone marrow to restore their blood and immune system following radiation therapy for blood cancer is a procedure that has been practised since the 1970s.

Building on this understanding of cell biology and thanks, in part, to the scientific and technological advances of the last decade, in which UK research institutions, start-ups and established companies have played a huge role, a new industry has emerged. Cell and gene therapies are a revolution in medicine and their full potential is only just beginning to emerge.

Cell and gene therapies rely on modifying biological activity to restore or install functionality either by introducing healthy cells, modified cells, or new genetic material. There are broadly four categories of therapies in this field:

Cell therapies

Whole cells are introduced into a patient to carry out a therapeutic function.



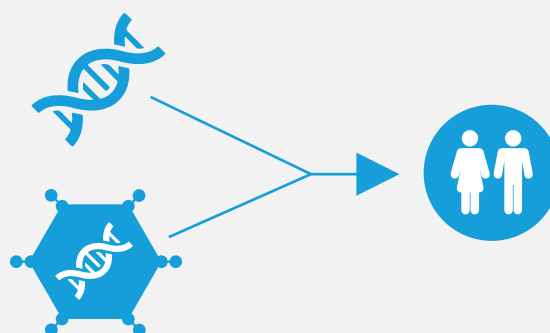
Gene modified cell therapies

Cells from the patient or from another source are modified in the laboratory so that when introduced into the patient they will stimulate a therapeutic effect.



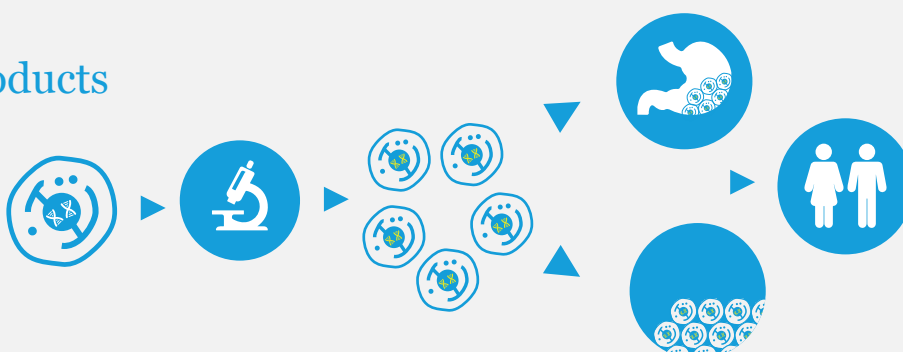
Gene therapies

Genetic material is inserted into the patient by means of a viral vector or another method resulting in a therapeutic effect. The therapeutic effect is gained by the genetic material entering the patient's cells thereby restoring their function or stimulating a therapeutic response.



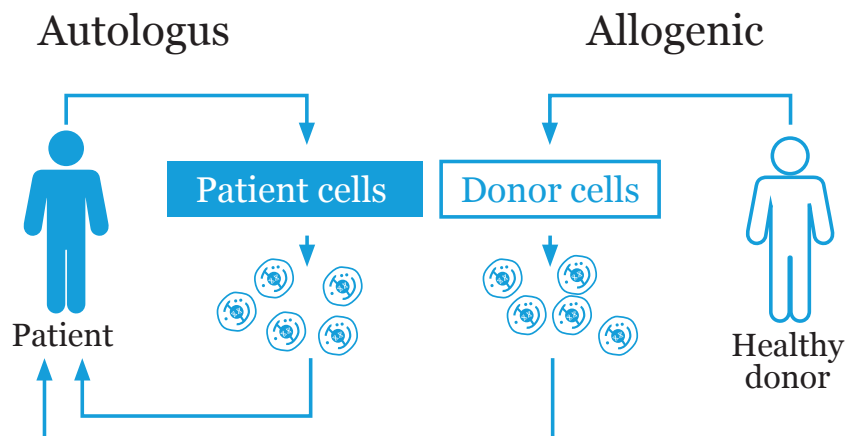
Tissue engineered products

Cells and/or biologically active molecules are engineered to restore, maintain, improve, or replace damaged tissues and organs.



Autologus vs. allogenic therapies

Of these therapies, two main types exist. Autologus therapies, using the patients own cells, and allogenic therapies, where cell are derived from a donor and then used to provide treatments for one or multiple patients.



Current uses

Recently, some of the biggest strides in the cell and gene therapy industry have been in oncology, where some therapies have already received approval in the US and Europe. CAR-T therapies are one of the most advanced, and they involve extracting and re-programming T-cells (a type of immune cell), to equip them to more effectively detect and kill cancer cells. This type of therapy has shown astonishing results in patients with otherwise untreatable blood cancers. For example, a one-time treatment for B-cell acute lymphoblastic leukemia (ALL), has shown as 83% remission rate after three months.

However, cancer isn't the only disease where researchers are making progress. For example, other areas in which cell and gene therapies show a great deal of promise include ophthalmology, neurology and haemophilia. Ophthalmic treatments have restored patients sight following macular degeneration by using donor cells to build a new layer of retinal cells and surgically implanting it into the eye. Haemophilia patients have seen their quality of life improve through gene therapy whereby a defective gene was replaced with a functioning one. Patients were then able to produce the factor IX protein, responsible for blood clotting, which was previously lacking. Clinical trial patients treated with the gene therapy saw a 90% reduction in bleeding episodes. Also, stem cells have been shown to stimulate natural repair mechanisms in organs affected by conditions such as stroke.

The industry in the UK

Cell and gene therapy research is underpinned by a range of technologies and support services, which are both driving and supporting the evolution of the industry. The UK is working to build a complete and integrated ecosystem for the development of cell and gene therapies, and this ecosystem will be able to operate at the large scale required to deliver these therapies to a substantial number of patients across the world. Crucial elements of this ecosystem include interaction between the UK Government, the regulators, the National Health Service and the wide range of innovative companies including therapy developers, contract development and manufacturing organisations (CDMOs), and the supply chain.

There are more than 60 cell and gene therapy developers in the UK, more than any other European country and this number is growing. There are over 80 active clinical trials ongoing in the UK and the number of GMP manufacturing facilities is increasing year on year.

The challenges

Cell and gene therapies are novel, creating new manufacturing and supply chain hurdles which have never been encountered before. Challenges include facility design as some of these products are manufactured for use in only one person, rather than batch production for distribution to multiple patients. The handling of the product is also different because cells are the end product, rather than one part of the manufacturing process.

How these therapies are delivered to patients is also new. They don't follow the traditional rules of the pharmacy. New tracking systems to follow the journey of therapies are needed to ensure traceability from the moment cells are taken from a patient until they are re-infused into the same patient. There can be logistical challenges due to the short shelf life of living therapies. Healthcare providers will also require additional training, to administer these therapies, run follow-up monitoring and identify different adverse events. For instance, immune reactions, whereby patients may experience very high fever, dangerously low blood pressure, severe chills or difficulty breathing. While health care professionals are learning how to manage these side effects, there is a lot more work that needs to be done.

Currently cell and gene therapies remain highly specialised treatments that are either experimental, or available to only a small number of patients. Healthcare providers are not used to paying upfront for treatments with the potential to deliver long-term benefits, therefore new payment models are needed to ensure accessibility of these therapies to patients who would benefit.

The future

The outlook is positive. Cell and gene therapies have reached patients in record time, they are here and they're here to stay. The milestones reached in the last few years have given the industry a big boost. With the work of our excellent scientists, the innovative companies in the UK and the surrounding ecosystem, we can be confident that over the next few years these therapies will be making their way into routine delivery and improving health outcomes around the world.

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