

Genomic Medicine: Understanding Genome Engineered Cell Therapies



Cell therapy is the use of living cells as a therapy to treat disease. Cell therapy involves the transfer of viable, purified cells into a patient. Once in the patient's body, they grow and replace, or repair damaged tissue or abnormal body function for the treatment of a disease or condition. The most common type of cell therapy is bone marrow transplantation, also known as blood stem cell or hematopoietic cell transplantation. This is a procedure to replace damaged or destroyed bone marrow with healthy bone marrow stem cells and restore the body's ability to create the red blood cells, white blood cells and platelets it needs.

New medical technologies are being developed to enable the use of other cell populations for cell therapy and to explore how cells used for cell therapy can be optimized to enhance or add a new function to improve their therapeutic effects.

Cell therapies rely on obtaining and isolating donor cells from one of two sources, either allogeneic or autologous. Cells can be taken from a healthy donor who is not the patient, then modified in a lab and administered to a patient (allogeneic). Other times, the patient is the donor and, after the cells have been modified, is also the recipient (autologous). Allogeneic or autologous cell therapies each have specific advantages and disadvantages. Both approaches are being explored by scientists for different diseases and different patients.

An example of this type of engineered cell therapy are CAR T cells. CAR T cells harness the body's T cells, a type of white blood cell, which are an essential part of the immune system. T cells protect the body from threats of disease by searching for and destroying foreign substances in the body (antigens). Normal T cells can recognize and eliminate cells that have become damaged, infected by viruses or have turned cancerous.

CAR T cell therapy is an approach that, through genome engineering, adds a specially designed receptor called a chimeric antigen receptor (CAR) recognizing specific antigens, to transform normal T cells into CAR T cells. This process allows the T cells to recognize and, for example, destroy cancer cells.

Once sourced from a donor, changing normal T cells into CAR T cells requires delivering specific genes into the cells. A vehicle called a vector is used to carry the new gene into the cells. Viruses are commonly used as vectors and are made harmless by removing all disease-causing genetic information. This creates a shell to insert the healthy gene and deliver the genetic instructions that allow a T cell to become a CAR T cell.

CAR T cells are genetically modified, then expanded in a laboratory to multiply them in number. Then these cells are returned to a patient's body via infusion, enabling the new CAR T cells to find and eliminate the tumor cells.

CAR T cells are a treatment for some of the most difficult to treat blood cancers. Three CAR T cell therapies have recently been approved to treat certain cancers of the blood and have been demonstrated to be remarkably effective in treating cancers for which other treatments were not available. Scientists continue to study how to apply genetic engineering as an approach to enhancing other types of cells as treatment for disease.

Regulatory T cells (or Tregs) are another type of white blood cell and act as the key regulators of the immune system. They take on the role of peacekeepers, directing other T cells to cease fire, ensuring the immune system does not mistakenly attack healthy organs while still protecting the body from harmful viruses and bacteria.

Like T cells, Tregs can be genetically engineered with a CAR, creating CAR Tregs which are being studied as a cell therapy to precisely target autoimmune and inflammatory disorders.

CAR T cells and CAR Tregs are customized treatments created using an individual patient's own T cells (autologous). An emerging approach is developing allogenic cell therapies. These treatments utilize readily available cells from healthy donors which are genetically engineered and infused into a patient. The aim of this approach is to reduce the cost and time to treatment, bringing a new type of therapy to as many patients as possible.

Engineered cell therapy, like other genomic medicine technologies, has the potential to enable highly personalized therapies to address the underlying mistakes in DNA that lead to some diseases, bringing new hope to patients with certain types of cancer, autoimmune or inflammatory disorders or rare diseases for which there are few treatments.

Learn More

For more information on genomic medicines, including engineered cell therapy, visit the websites below. If you or a family member have a genetic-related condition, your physician or a genetics professional can help you understand if a genomic medicine approach is right for you, or help you find an appropriate clinical trial.

Alliance for Regenerative Medicine | alliancerm.org

American Society of Gene & Cell Therapy | asgct.org

