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Cell-based Gene Therapy

Potential to Transform Medicine

Cell-based gene therapy is one of the most exciting frontiers in medical research

Cell-based gene therapy is a technique that uses genes to treat or prevent disease. Researchers are testing several approaches which may allow doctors to treat a disorder by inserting a gene into a patient's cells instead of using drugs or surgery.

An area of cell and gene therapy of great interest includes the replacement of a missing or defective gene with a functional, healthy copy, which is delivered to target cells with a "vector." Viruses are commonly used as vectors because of their ability to penetrate a cell's DNA. These viral vectors are inactivated so they cannot reproduce and cause disease. Gene transfer therapy can be done outside the body (ex vivo) by extracting bone marrow or blood from the patient and growing the cells in a laboratory. The corrected copy of the gene is introduced and allowed to penetrate the cells' DNA before being injected back into the body.

Cell-based gene therapy has the potential to become a promising treatment option for a number of diseases that range from cancer, transplant rejection, refractory autoimmune disease, inborn errors of metabolism, and Graft versus Host Disease.

Chimeric antigen receptor (CAR) T cell therapy, the latest in cell-based gene therapy

With CAR technology, T cells are drawn from a patient's blood and reprogrammed in the laboratory to create "chimeric" T cells that are genetically coded with new instructions to "hunt" the patient's cancer cells. When these T cells are infused back into the patient, they become a new part of the patient's immune system specifically designed to kill the patient's cancer.

Novartis and the University of Pennsylvania (Penn) are developing CAR immunotherapies for the treatment of cancers

Novartis and Penn have an exclusive global collaboration to research, develop and commercialize targeted CAR immunotherapies for the treatment of cancers. The collaboration was initiated based on CAR research conducted by Dr. Carl June and Penn with CTL019.

CTL019 in B-cell malignancies

As the first investigational CAR therapy identified for development under the Penn-Novartis alliance, CTL019 targets a protein called CD19 that will be investigated in a number of B-cell malignancies such as CLL, B-cell acute lymphocytic leukemia (B-ALL), and diffuse large B-cell lymphoma (DLBCL), among others.

CTL019 clinical trials are underway, and support Breakthrough Therapy designation from the US Food and Drug Administration (FDA)

CTL019 is currently in Phase II trials, which are being conducted in adult and pediatric patients with relapsed and refractory ALL, CLL, and patients with non-Hodgkin's lymphoma (including DLBCL). CTL019 has received FDA Breakthrough Therapy designation for the treatment of pediatric and adult patients with relapsed/refractory ALL.

Novartis Pharmaceuticals Cell & Gene Therapies Unit

Novartis Pharmaceuticals is committed to exploring the potential of this approach with the creation of a dedicated unit to focus on cell-based gene therapies. The C&G TU portfolio aims to transform medical practice by replacing, repopulating or resetting the immune system. In addition to pursuing the potential of chimeric antigen receptor T cell therapy (CART) technology, the Unit is also pursuing a stem cell-based FCRx platform, licensed from Regenerex that is being studied in kidney transplantation with the goal to induce immune tolerance. In addition, the Unit is investigating a compound discovered in house at Novartis known as HSC835, a novel cell therapy approach that may improve stem cell transplantation in patients with limited treatment options.

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