

The Cell and Gene Therapy Journey

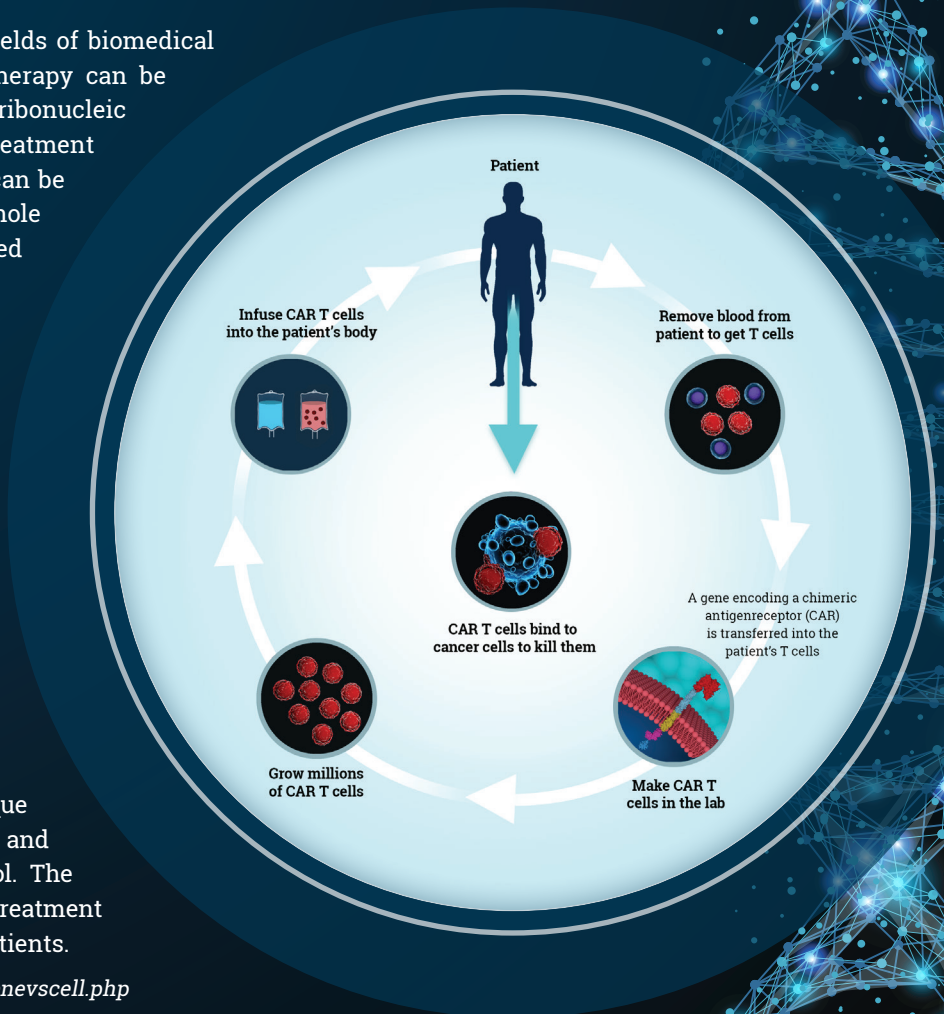
Gene Therapy and Cell Therapy are overlapping fields of biomedical research with similar therapeutic goals. Gene Therapy can be defined as the use of genetic material (usually deoxyribonucleic acid - DNA) to manipulate a patient's cells for the treatment of an inherited or acquired disease. Cell Therapy can be defined as the infusion or transplantation of whole cells into a patient for the treatment of an inherited or acquired disease.¹

One of the most advanced cell therapies is known as Chimeric Antigen Receptor (CAR) T cell therapy. CAR T cell therapy involves the infusion of T cells that have been genetically engineered to express a chimeric antigen receptor (CAR) to reprogram the T cells. The CAR combines the specificity of a monoclonal antibody with the cytotoxic and memory functions of T cells.

These treatments have captured the attention of researchers and the public alike because of the remarkable responses they have produced in some patients—both children and adults—for whom all other treatments had stopped working.

CAR-T Cell Therapy products require unique production and release strategies ensuring speed and efficiency while maintaining strict quality control. The graphic below illustrates how this next generation treatment is manufactured, tested, released, and shipped to patients.

¹ https://annualmeeting.asgct.org/about_gene_therapy/genevscell.php



Approved Cellular and Gene Therapy Products

To date the FDA's Office of Tissues and Advanced Therapies (OTAT) has licensed the following products:

- ALLOCORD (HPC, Cord Blood)
SSM Cardinal Glennon Children's Medical Center
- CLEVECORD (HPC Cord Blood)
Cleveland Cord Blood Center
- Ducord, HPC Cord Blood
Duke University School of Medicine
- GINTUIT (Allogeneic Cultured Keratinocytes and Fibroblasts in Bovine Collagen)
Organogenesis Incorporated
- HEMACORD (HPC, cord blood)
New York Blood Center
- HPC, Cord Blood
Clinimmune Labs, University of Colorado Cord Blood Bank
- HPC, Cord Blood - MD Anderson Cord Blood Bank
MD Anderson Cord Blood Bank
- HPC, Cord Blood - LifeSouth
LifeSouth Community Blood Centers, Inc.
- HPC, Cord Blood - Bloodworks
Bloodworks
- IMLYGIC (talimogene laherparepvec)
BioVex, Inc., a subsidiary of Amgen Inc.
- KYMRIAH (tisagenlecleucel)
Novartis Pharmaceuticals Corporation
- LAVIV (Axicel-T)
Fibrocell Technologies
- LUXTURN A
Spark Therapeutics, Inc.
- MACI (Autologous Cultured Chondrocytes on a Porcine Collagen Membrane)
Vericel Corp.
- PROVENGE (sipuleucel-T)
Dendreon Corp.
- TECARTUS (brexucabtagene autoleucel)
Kite Pharma, Inc.
- YESCARTA (axicabtagene ciloleucel)
Kite Pharma, Incorporated
- ZOLGENSMA (onasemnogene abeparvovec-xioi)
AveXis, Inc.

Source: <https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products>