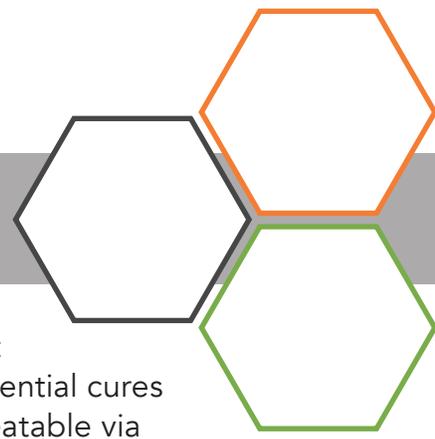


## REGENERATIVE MEDICINE & ADVANCED THERAPIES



The regenerative medicine and advanced therapies sector is truly the next frontier for patients, developing revolutionary, durable treatments and potential cures for some of humankind's most devastating diseases – many currently untreatable via conventional treatments – through the use of transformative scientific discoveries and technologies. These products are intended to augment, repair, replace or regenerate organs, tissues, cells, genes and metabolic processes in the body.

This sector holds the promise of addressing many currently unmet medical needs, and represents a major paradigm shift in medicine – treating the root causes of disease and disorders to stop or even reverse progression.

### **GENE THERAPY AND GENOME EDITING**

Gene therapy seeks to modify, replace, inactivate or introduce genes into a patient's body with the goal of durably treating, preventing or even curing disease.

Gene therapy techniques can also be used to genetically modify a patient's cells outside of the body, which are then re-introduced to deliver modified or corrected cells into the body, an approach known as gene-modified cell therapy.

Gene therapy may be performed *in vivo*, in which a gene is transferred to cells inside the patient's body, or *ex vivo*, in which a gene is delivered to cells outside of the body, and are then transferred back into the body.

Genome editing is a practice in which DNA is inserted, replaced or removed using "molecular scissors" or artificially engineered gene constructs. Types of genome editing technology include CRISPR, TALEN, zinc finger nucleases and more.

### **CELL THERAPY**

Cell therapy is the administration of viable, non-genetically modified cells into a patient's body to grow, replace or repair damaged tissue for the treatment of a disease. Cells can be administered allogeneically, in which the patient receives cells from a donor, or autologously, in which the patient receives cells from his or her own body.

### **TISSUE ENGINEERING AND BIOMATERIALS**

Tissue engineering combines scaffolds, cells and biologically active molecules into functional tissues to restore, maintain or improve damaged tissues. Biomaterials are medical devices designed to interact with living systems, providing physical structures and support for engineered tissues.

### **ALLIANCE FOR REGENERATIVE MEDICINE (ARM)'S ROLE**

ARM's goal is to educate multi-sector stakeholders about the latest scientific progress and delivery challenges, specifically focusing on areas relating to regenerative medicine. We are bridging the gap between industry and patient groups who are working toward the common goal of durable and potentially curative therapies.

## GLOSSARY OF TERMINOLOGY

**Allogeneic cell transplant** – A procedure in which a patient receives cells from a donor.

**Autologous cell transplant** – A procedure in which a patient receives cells from his or her own body.

**DNA (deoxyribonucleic acid)** – A molecule found in the cell's nucleus that contains an organism's genetic information.

**Ex vivo gene therapy** – A practice in which a gene is transferred to cells outside of the body by delivery of desired gene into the cells. The modified cells are then transferred back into the body.

**Gene** – A short section of DNA that forms the basic physical and functional unit of heredity.

**Genome** – An organism's complete set of DNA, comprising all genes. Each genome contains all the information needed to build and maintain that organism.

**Induced pluripotent stem cells** – Adult cells that have been genetically reprogrammed into an embryonic-like pluripotent state that enables the development of an unlimited source of any type of human cells needed for therapeutic purposes.

**In vivo gene therapy** – A practice in which a gene is transferred to cells inside the patient's body.

**Pluripotent stem cell** – A type of "master cell" that is capable of becoming one of many different cell types in an organism.

## ADDITIONAL RESOURCES

American Society of Gene & Cell Therapy | [www.asgct.org](http://www.asgct.org)

California Institute for Regenerative Medicine | [www.cirm.ca.gov](http://www.cirm.ca.gov)

European Society of Gene & Cell Therapy | [www.esgct.eu](http://www.esgct.eu)

International Society for Cellular Therapy | [www.celltherapysociety.org](http://www.celltherapysociety.org)

International Society for Stem Cell Research | [www.isscr.org](http://www.isscr.org)

Tissue Engineering International & Regenerative Medicine Society | [www.termis.org](http://www.termis.org)