

JUST THE FACTS

6

DISEASES CURRENTLY TREATED
WITH CELL OR GENE THERAPY¹

MORE THAN

100

DISEASES BEING EXPLORED FOR
POTENTIAL TREATMENT WITH CELL
AND GENE THERAPIES

362²

CELL AND GENE THERAPIES
IN DEVELOPMENT

132

A THIRD OF CELL AND GENE
THERAPIES ARE IN DEVELOPMENT
FOR RARE DISEASES

CELL AND GENE THERAPY

Nearly **400** Cell and Gene Therapies in Development Target a Broad Range of Diseases

The idea of altering a gene to cure or treat a disease is fairly new, but with the first U.S. approvals of cell and gene therapies in 2017, that concept is now a reality. And the pace of research and development in cell and gene therapy is increasing. A 2018 PhRMA report on the cell and gene therapy pipeline found **289** therapies in clinical development by biopharmaceutical companies in the United States. Today that number is **362**.

Cell and gene therapy represent overlapping fields of biomedical research with similar therapeutic goals, which target DNA or RNA inside or outside the body. Both approaches seek to modify genetic material to improve functioning or fight disease. Specifically, gene therapy uses genetic material, or DNA, to manipulate a patient's cells for the treatment of an inherited or acquired disease. While cell therapy is the infusion or transplantation of whole cells into a patient for the treatment of an inherited or acquired disease.

The **362** novel cell and gene therapies range from early to late stages of clinical development and are focused on a variety of diseases and conditions from cancer to genetic disorders to neurologic conditions. These therapies represent the translation of basic scientific understandings into innovative new treatment options for patients.

While there are hundreds of potential cell and gene therapies in the pipeline, a few of these innovative medicines have already been approved by the U.S. Food and Drug Administration (FDA) and are helping patients today. As of February 2020, there are **nine** cell or gene therapy products approved in the U.S. – treating cancer, eye diseases and rare hereditary diseases.

Innovative Technologies

Biopharmaceutical researchers are using these new technologies and pursuing innovative treatments in clinical trials today.

Cell Therapy

Cell therapy is the introduction of new cells into a patient’s body to grow, replace or repair damaged tissue in order to treat a disease. A variety of different types of cells can be used in cell therapy, including stem cells, lymphocytes, dendritic cells and pancreatic islet cells. In some cases, such as CAR-T, cells are genetically modified before being (re)introduced into the patient. This is the intersection between gene and cell therapy.

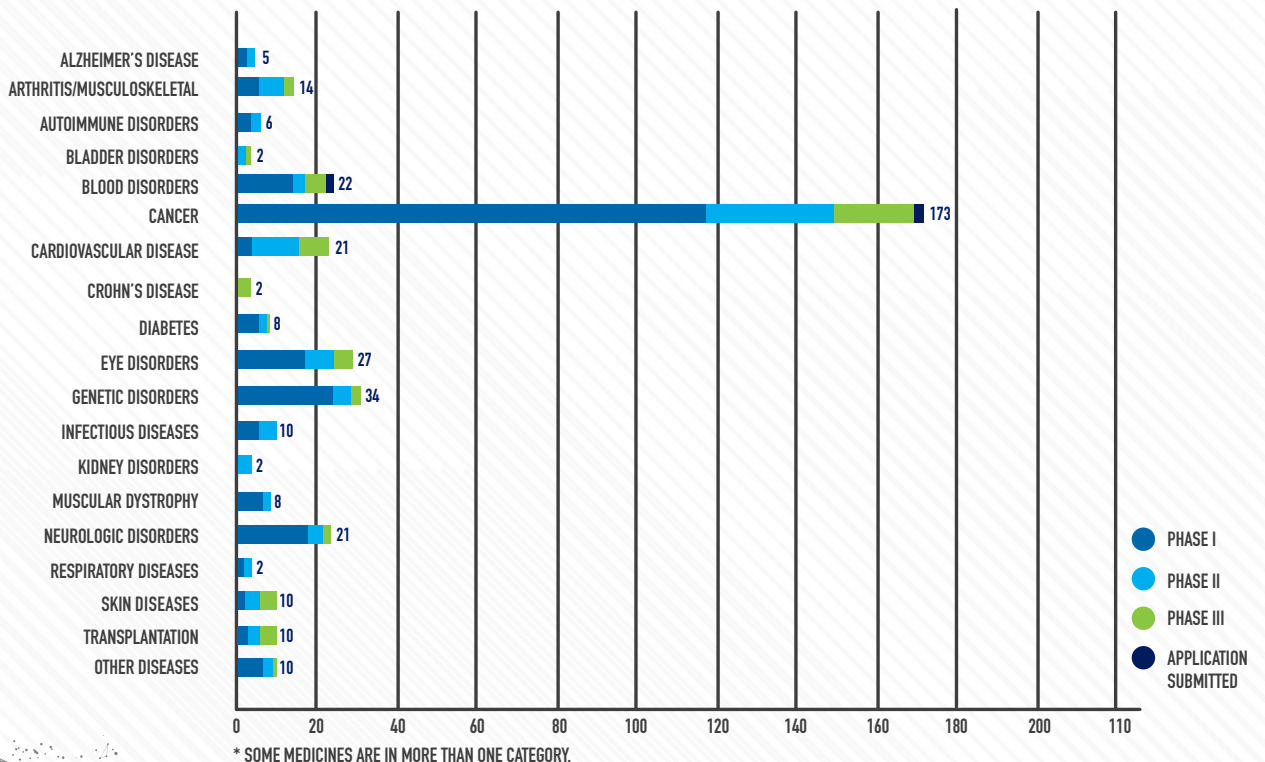
Gene Therapy

Gene therapy seeks to modify or introduce genes into a patient’s body with the goal of treating, preventing or potentially curing a disease. Examples of gene therapy approaches include replacing a mutated gene that causes disease with a healthy copy; or introducing a new or modified gene into the body.

Gene Editing

Gene editing is a technique involving the alteration of genes to correct mutations, introduce new genetic information or remove specific DNA sequences. In gene editing, DNA sections are inserted, replaced, removed or modified at particular locations in the human genome in order to treat a specific disease.

Medicines in Development by Disease and Phase



Cell and Gene Therapies in the Biopharmaceutical Pipeline:

The novel cell and gene therapies in the development pipeline today are the result of years of pioneering research by America's biopharmaceutical research companies. The range of diseases that these therapies can address is broad and covers blood disorders, eye disorders, cancer and infectious diseases, among others. The therapies range from therapies used outpatient to treatments used in hospital procedures. Among the therapies in development are potential treatments for:

- A gene therapy using adeno-associated virus (AAV)-factor VIII is designed to stimulate the production of factor VIII for the treatment of **hemophilia A**. Since relatively low levels of factor VIII are needed to be clinically effective, patients with severe hemophilia A may also benefit from treatment. Compared to current factor VIII replacement therapy, the gene therapy is less invasive and doesn't require multiple intravenous injections per week.
- A gene therapy using AAV vectors is delivering a high-activity Factor IX gene to the liver for the treatment of **hemophilia B**. Hemophilia B is caused by a mutation in Factor IX, which leads to deficient blood coagulation and an increased risk of bleeding or hemorrhaging.
- A second-generation CAR-T cell therapy comprised of genetically-modified T cells, is designed to target B-cell maturation antigen (BCMA) and to redirect the T-cells to recognize and kill malignant myeloma cells. BCMA is a surface protein that is absent in most normal tissues but found in normal plasma cells and the majority of multiple myeloma cells.
- A gene therapy for the treatment of **Stargardt disease** (juvenile-onset macular dystrophy) delivers a corrected version of the ABCR gene directly in the photoreceptors in the retina. Stargardt disease is caused by a mutation of the ABCR gene, which leads to the degeneration of photoreceptors in the retina

RNA Therapeutics Offer Novel Pathway to Treat Disease

RNA interference (RNAi) and antisense RNA are relatively new areas of research and is a pathway that uses the gene's own DNA sequence to turn it off or modify the genes expression. These therapeutics are not cell or gene therapies, but they do offer a new understanding of how genes are regulated in the body's cells. RNA therapeutics can potentially block the mechanism of disease-causing proteins. Today there are more than 60 RNA therapeutics in development (in clinical trials or under review by FDA) by the biopharmaceutical research companies. Since 2016, there have been 6 RNA therapeutics approved by FDA.

Sources:

1. U.S. Food and Drug Administration
2. Number of medicines obtained through public government, and industry sources, and the Adis "R&D Insight" database; current as of February 13, 2020.

