



What are Cell and Gene Therapies?

Cell and gene therapies are highly innovative therapies, different from traditional medicines. What makes them different, and how do they work? Why are they so promising?

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Cell and gene therapies (CGTs) represent highly innovative therapies that differ from traditional medications used to treat various ailments. They usually target the underlying causes of genetic and/or acquired illnesses with the goal of treating, preventing, and/or potentially curing the disease.

Cell therapy involves the transfer of a specific cell type (or types) with the relevant function (from the patient themselves or from a donor) into the patient in an effort to prevent, manage, or cure their disease.¹ Cancers of the blood and bone marrow, cancers of the lymphatic system, plasma cell disorders, and other conditions that affect the body's ability to make healthy cells are sometimes treated with cell therapies.

Gene therapy involves the transfer of genetic material to treat or prevent disease.^{2,3,4} The transferred genetic material is incorporated into specific cells in order to fix or replace faulty genes with healthy ones to treat, cure, or prevent a disease or medical condition. Gene therapies are currently available in Canada to treat several rare diseases.

CGTs are creating new opportunities for improved health outcomes and quality of life for patients in a wide range of disease areas. A report published in 2024⁵ noted that there are more than 4,000 gene, cell and RNA therapies⁶ in development (see Figure 1). These treatments hold great promise, often offering life-changing benefits, changing the course of the disease being treated and improving the quality of life for many patients.

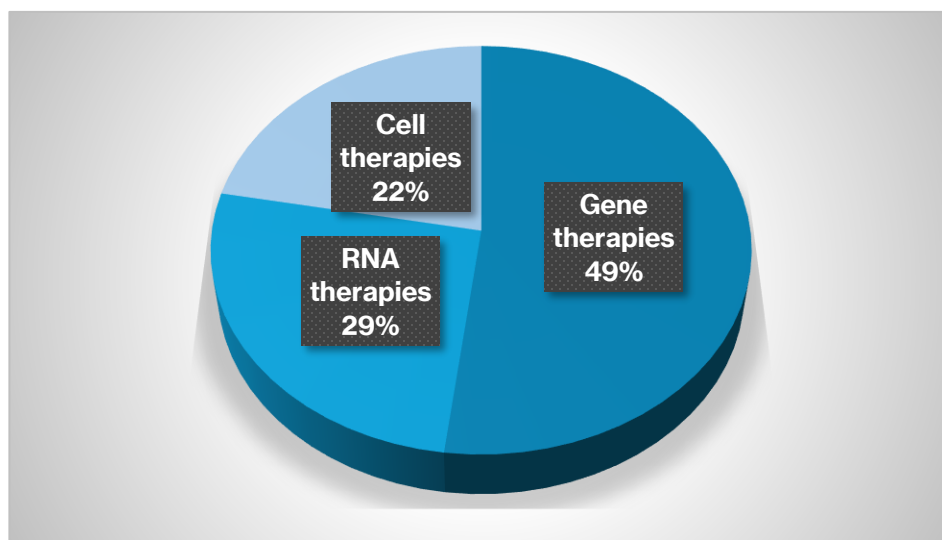


Figure 1. Pipeline of cell, gene and RNA therapies, as of Q3 2024 (adapted from reference 5).

Examples of specific diseases where CGTs are in development include:

- **Cell therapies:** osteoarthritis, type 1 diabetes, Parkinson's disease, acute respiratory distress syndrome, graft-versus-host disease, and spinal cord injury.
- **Gene therapies:** myeloma, acute myelogenous leukemia, non-Hodgkin's lymphoma, B-cell lymphoma, and various solid tumors (e.g., ovarian, liver, pancreatic, and stomach cancer).

¹ American Society of Gene and Cell Therapy. Cell Therapy Basics. <https://patienteducation.asgct.org/gene-therapy-101/cell-therapy-basics>.

² American Society of Gene and Cell Therapy. Gene Therapy Basics. <https://patienteducation.asgct.org/gene-therapy-101/gene-therapy-basics>.

³ NIH - National Heart, Lung, and Blood Institute. What are Genetic Therapies? <https://www.nhlbi.nih.gov/health/genetic-therapies>

⁴ US Food and Drug Administration. How Gene Therapy Can Cure or Treat Diseases. <https://www.fda.gov/consumers/consumer-updates/how-gene-therapy-can-cure-or-treat-diseases>

⁵ American Society of Gene and Cell Therapy. Gene, Cell, and RNA Therapy Landscape Report – Q3 2024 Quarterly Data Report. <https://www.asgct.org/global/documents/asgct-citeline-q3-2024-report.aspx>

⁶ A separate Fact Sheet for RNA therapies will be developed and posted in the near future.