Advancing Cell & Gene Therapy

The Value of Cell and Gene Therapies

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Cell and gene therapies (CGTs) bring great value to patients, their families, and the health system overall. These innovative treatments hold great promise, often offering significant benefits, changing the course of disease, and improving health outcomes and quality of life for many patients in a number of different disease areas. The following outlines some specific examples of CGTs and their value to individuals as well as our society more broadly.

A gene therapy whose development started over 30 years ago has become a transformational treatment for people with a specific form of inherited retinal disease which causes severe vision loss.

In 2020, voretigene neparvovec-rzyl (Luxturna®) was approved for the treatment of patients with vision loss due to inherited retinal dystrophy caused by RPE65 mutations. Access to this treatment saw patients who were once unable to see clearly **have their vision restored**, often very quickly.[1] There are both individual as well as societal benefits associated with treating and/or avoiding blindness, in terms of the ability of patient to work and contribute to their families and society more broadly.

Chimeric antigen receptor (CAR) T-cell therapies are an example of cell therapies that offer a potentially curative and life-saving treatment option to patients with specific types of blood cancer, who previously would only have had access to salvage chemotherapy[2] or palliative care. As noted in a recent Canadian report,[3] clinicians see CAR T-cell therapies as having the ability to **prolong life** and giving the patient a **chance at cure or complete response**, which is not offered by the current regimens being used for many of these diseases.

Recently approved gene therapies have the potential to cure patients with hemophilia A or B following transfer of a functional gene that produces factor VIII or factor IX to a person's liver cells to replace the patient's own defective gene.[4] When treatment is successful, the patient is able to produce their own clotting factors thus decreasing and/or eliminating the need for frequent IV infusions of factor replacement therapy. This has a significant impact on patient **quality of life**, as well as **health system costs**.[5]

The innovative and highly technical nature of CGTs means that health systems may need to adjust methods of care and delivery in order to take advantage of these new treatments. Given the breadth of the CGT pipeline, it is critical for Canadian health care leaders to be proactive in addressing and/or mitigating implementation challenges to ensure timely and sustainable access to CGTs for appropriate patients.

System readiness is key to achieving the value and promise of CGTs. Health decision-makers need to ensure that Canada has the funding and regulatory mechanisms, health system capacity, and clinical expertise in place to provide timely access to this new generation of therapies.

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[1] Daley J. Four Success Stories in Gene Therapy. Nature (October 26, 2021). https://www.nature.com/articles/d41586-021-02737-7
[2] Treatment given after the cancer has not responded to standard chemotherapy regimens and may include experimental medications.
[3] *Longwoods*. Understanding the Feasibility of Implementing CAR T-Cell Therapies from a Canadian Perspective. Healthcare Policy. February 2021: https://www.longwoods.com/content/26430/healthcare-policy/understanding-the-feasibility-of-implementing-car-t-cell-therapies-from-a-canadian-perspective.
[4] Amit C. Nathwani; Gene therapy for hemophilia. Hematology Am Soc Hematol Educ Program 2019; 2019 (1): 1–8. doi: https://doi.org/10.1182/hematology.2019000007
[5] Canadian Hemophilia Society. All About Hemophilia Gene Therapy – A guide for patients and caregivers. (September 2024). https://www.hemophilia.ca/wp-content/uploads/2024/09/All-About-Hemophilia-Gene-Therapy-2.pdf