# Regenerative Medicine: A Primer on Translating Discoveries in Canada

June 2025

## Background

This descriptive report was developed through a collaboration between the Institute of Health Economics (IHE) and the Stem Cell Network (SCN) to support a two-day workshop held in Ottawa on June 25 and 26, 2025. It draws on a review of current literature and insights gathered through interviews with key informants across the regenerative medicine ecosystem.

The purpose of this document is to provide a shared foundation for discussion. It outlines key policy issues related to the translation and integration of regenerative medicine in Canada. The content reflects the perspectives captured through the research process but does not necessarily represent the formal policy positions of either the Stem Cell Network or the Institute of Health Economics.


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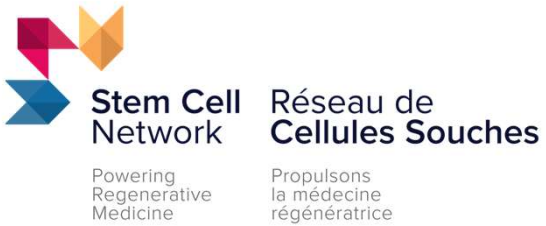

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# Introduction

**Innovations in regenerative medicine, particularly cell and gene therapies (CGTs), tissue engineering, and human cell and tissue products, hold transformative potential to not only improve patient outcomes and provider experiences but to deliver cures for previously untreatable or poorly managed diseases.**

CGTs stand apart from traditional therapies due to their capacity for durable, sometimes one-time interventions that can replace lifelong management of chronic or genetic conditions. Their potential lies in changing the course of diseases—such as hematologic malignancies, rare genetic disorders, and degenerative diseases—rather than simply managing symptoms. They also open new avenues for treatment where no effective options exist and can drastically reduce lifetime care costs and improve quality of life.

However, these therapies challenge traditional paradigms in research, regulation, and care delivery due to their complexity, cost, heterogeneity, and individualized nature. While some CGTs are personalized (e.g., autologous CAR-T cells), others are standardized, off-the-shelf products. Both forms present unique challenges at each stage of the innovation lifecycle.

Canada has made substantial investments in regenerative medicine research, including long-standing funding support from the Stem Cell Network, infrastructure investments such as the Centre for Commercialization of Regenerative Medicine (CCRM),[1] and major research grants through Canadian Institutes of Health Research (CIHR) and Genome Canada. The federal government's investment of $93.5 million in SCN between 2019 and 2029 is just one example. These investments have positioned Canada as a global leader in scientific discovery, with one of the highest rates of regenerative medicine patent filings per capita.

1 - https://www.ccrm.ca

**Yet, without coordinated strategies for translation and commercialization, there is a risk that these innovations will fail to reach patients in Canada—undermining health outcomes and return on public investment and potentially allowing Canadian-developed therapies to be adopted internationally before becoming available at home.**

This primer outlines key enablers and barriers encountered by CGTs as they move through today's translation and access pathways, with emphasis on the Canadian health ecosystem. It aims to establish a shared understanding of the current landscape—particularly the challenges CGTs face in moving from discovery to delivery—so that future conversations can be informed by this context and contribute to improvements in the system.

The Stem Cell Network supports this effort to accelerate CGT development and adoption in Canada through translational research funding, support for commercialization and infrastructure development, convening networks of innovators and end-users, and facilitating policy dialogue.

# “Bench to Bedside” Pathway for bringing new treatments to patients in Canada


The path for bringing CGTs to patients typically involves independent agents that span academic, finance, healthcare, and regulatory sectors.[2] Phases of innovation include phases: discovery and innovation, development and evidence generation, appraisal and regulation, and access and integration.

CGTs transiting these phases of technology innovation create new challenges for innovation and health care policy makers and decision makers, including those involved in regulating intellectual property and marketed products as well as public insurance and healthcare administrators. Some of these challenges are being addressed as new mechanisms and policies have been developed to enable innovation.

Because of their novelty, CGTs present policy challenges not only for regulators and HTA bodies but also for institutional actors such as universities, hospital systems, and public payers. The complex, cross-sectoral nature of these therapies also means that silos in policy, funding, and delivery become significant barriers to scale and access. As new mechanisms emerge to enable innovation—including novel IP policies, dedicated funding programs, and early regulatory engagement—it is essential that these are adapted to the unique needs of CGTs.

2 - https://www.oecd.org/en/publications/the-oecd-innovation-strategy_9789264083479-en.html

Section 2

# Innovation & Discovery

*Commercialization support through SCN since 2001 has catalyzed* ***28 regenerative biotech companies****, led to* ***237 issued patents****, and* ***126 licensing agreements****.*[5]

# Innovation & Discovery

This phase includes early research, preclinical development, and intellectual property (IP) management.[3] CGTs often emerge from academic institutions, where Technology Transfer Offices (TTOs) support patent filings and commercialization strategies. While Canada ranks high in regenerative medicine patent activity,[4] but many innovations fail to translate into commercial successes.

A lack of commercialization capacity and venture capital in Canada can potentially lead companies to relocating, partnering, or being acquired by U.S.-based firms.

3 - https://ised-isde.canada.ca/site/canadian-intellectual-property-office/en/patents-learn-basics-inventing-next-big-thing-learn-why-patents-matter
4 - https://ihe.ca/files/stem_cell_regenerative_medicine_in_canada_current_state_and_future_prospects.pdf
5 – SCN Annual Report 2024-2025

## Intellectual Property

The patent landscape for CGTs is more complex than for traditional pharmaceuticals due to their reliance on platform technologies, delivery mechanisms, and specialized equipment. Canada's Patent Term Adjustment policy supports innovators by extending patents where processing delays have slowed down granting of patents.[6] Entities like Intellectual Property Ontario support innovators with development of IP strategies.[7] Despite such policies and supports, challenges to navigating the complexity and making the most of IP remain.

While generally supportive, TTOs themselves can also be a barrier when institutional goals may prioritize revenue over translational potential. Better alignment between innovators and institutional supports is necessary to enable commercialization, particularly for **high-risk CGTs**.

**Key Enablers**

**Patent legislation** – The role of patent legislation is to provide legal protections, foster investment, and create clear pathways for innovation.

**Key Barriers**

**Complexity** – The inherent complexity surrounding these innovations complicates the drafting of patents that meet requirements.[8] Public institutions often hold a significant share of patents but lack mechanisms for effective commercialization.[9] Overlapping patents on foundational technologies, such as stem cell lines or growth factors, can complicate access.[10]

6 - https://www.dlapiper.com/es-pr/insights/publications/2023/10/canada-to-introduce-new-patent-term-adjustment-regime-to-comply-with-cusma
7 - https://mbd.utoronto.ca/news/uoft-uhn-eir-ipon/
8 - https://www.signalsblog.ca/the-role-and-impact-of-ip-rights-on-innovation-and-investment-in-rm/;
9 - https://patentpc.com/blog/patenting-challenges-in-regenerative-medicine
10 - https://www.signalsblog.ca/the-role-and-impact-of-ip-rights-on-innovation-and-investment-in-rm/

## Potential CGT Risk Factors

**First-in-Class Mechanism or Technology**
Therapies using entirely new platforms or mechanisms (e.g., novel gene editing methods like CRISPR, or next-gen delivery vectors) often lack precedents, making regulatory pathways less defined.

**Limited Clinical Data**
Small or early-stage trials, particularly for rare diseases, may not offer sufficient evidence for safety and efficacy at the time of evaluation.

**Complex or Personalized Manufacturing**
Autologous therapies (using a patient's own cells) require individualized processing and face scalability challenges.

**Durability and Safety Unknowns**
Some CGTs offer curative potential, but with unknown long-term effects—raising payer concerns about cost versus uncertain outcomes.

**Ethical, Societal, or Operational Barriers**
Some may target pediatric populations, involve germline modifications, or raise issues around equitable access and health system capacity.

# Financing Development & Commercialization[11]

**Translating any innovation into the use or sale of a product for use in the healthcare system requires scientific investigation, infrastructure development, and regulatory and market access — each of which demands investment at specific stages.**

These typically include:

- Early-stage research and development (R&D),
- Preclinical and clinical trials (Phases I–III),
- Manufacturing and scale-up, and
- Commercialization and market access.

Each phase has distinct financial requirements and risk profiles, often requiring different types of funding and support mechanisms. The risk of failure increases if funding gaps occur between stages — a particular challenge in regenerative medicine due to long timelines, complex manufacturing, and clinical uncertainty

11 - https://www.nserc-crsng.gc.ca/NSERC-CRSNG/NSERC2030-CRSNG2030/report-rapport/index_eng.asp
12 - https://stemcellnetwork.ca/stem-cell-network-and-capital-bioventures-announce-a-strategic-partnership-to-drive-canadian-regenerative-innovations-forward/

# Financing Development & Commercialization – Key Enablers

**Diversity of funding sources** – A mix of public, private, and philanthropic funding can help mitigate financial risk, especially across the lengthy and capital-intensive development timeline of CGTs. This includes non-dilutive funding (e.g., government grants, research council funding), equity investments (e.g., venture capital, angel investors), and strategic partnerships (e.g., pharma or industry alliances). Innovative models such as milestone-based financing, venture philanthropy, and blended finance approaches can provide flexible support tailored to different stages of development. Ensuring access to these funding streams is especially important for early-stage academic innovators, small biotech firms, and organizations outside major commercial hubs, who may lack the capital or networks to compete for traditional funding.

**Collaborative partnerships** – Collaborative partnerships can promote leveraging resources, expertise, and innovative funding models. Partnerships can also broaden stakeholder engagement. These could include (non-exhaustive):

+ Cross-sectoral partnerships between private sector innovators and academics (such as the DRIVE-RM consortium);
+ Public -private partnerships and cross-sector incubation programs (such as the such as the Stem Cell Network (SCN)–Capital BioVentures partnership,[12] and Centre for Commercialization of Regenerative Medicine translation centre);[13]

**Supports to enable coordination, infrastructure development and commercialization** – Relevant infrastructure can take a variety of forms including:

+ **Funding mechanisms** – Stable, long-term public investment (e.g., through the Stem Cell Network) ensures continuity from early discovery through to commercialization, helping projects cross critical translational gaps such as preclinical validation or clinical readiness.
+ **"Bio-insurance" platforms and supply chain systems** – These approaches pool financial and operational risk across multiple projects. Bio-insurance platforms can safeguard against failure in early-stage ventures by distributing resources strategically.[14,15,]
+ **Accelerators, incubators, and specialized facilities** – Organizations like JLabs, CapitalBioVentures,[16] CCRM, and OmniaBio offer physical infrastructure, mentorship, regulatory guidance, and access to networks. These supports help early-stage ventures refine their business models, validate technologies, and scale manufacturing in a compliant environment.
+ **Enabling networks and collaborative platforms** – Programs such as Canada's Stem Cell Network and the U.S. Regenerative Medicine Innovation Project convene stakeholders across sectors, fostering knowledge exchange, alignment of priorities, and shared access to tools and expertise.
+ **Bridging capital for clinical transition** – Targeted support during the critical transition from preclinical to clinical research, such as SCN's Accelerator funding, helps innovators collect the data and meet the regulatory requirements necessary to initiate first-in-human trials.
+ **Commercialization-focused programs** – CCRM and initiatives like the National Research Council's Disruptive Technology Solutions for Cell and Gene Therapy Challenge provide expertise, connections, and capital to advance late-stage innovations toward regulatory approval and market entry

13 - French et al., "Global Strategic Partnerships in Regenerative Medicine."
14 - https://www.nce-rce.gc.ca/NetworksCentres-CentresReseaux/PreviouslyFunded-FinancesAnterieurement/CECR-CECR/CCRM_eng.asp
15 - https://thedonnellycentre.utoronto.ca/centre-commercialization-regenerative-medicine-ccrm-0
16 - https://businesshealth.ca/the-jlabs-story-getting-there-from-here/

# Financing Development & Commercialization – Key Barriers

**High costs and financial uncertainty** – Cell and gene therapies face substantial financial barriers throughout development.[18] These stem from the resource-intensive nature of the therapies, the limited availability of early-stage investment, and the uncertainty of long-term outcomes that can affect payer decisions.

+ **Clinical trial costs** – CGTs frequently require small, highly specialized trials involving complex logistics, individualized treatment protocols, and extended follow-up periods. This drives up per-patient costs and creates a higher financial burden compared to conventional therapies.[19,20,]
+ **Limited early-stage risk capital** – Many Canadian innovators face difficulty accessing capital needed to move from discovery to early clinical stages. This is especially true for companies outside major biotech hubs or without established investor networks, leading to stalled development in the "valley of death."[21]
+ **Payer uncertainty and long-term risk** – The high upfront costs and long timelines to realize clinical benefits make CGTs harder to evaluate using traditional health technology assessment frameworks. Public payers may delay or restrict access due to uncertainty around long-term safety, durability, and value for money.[22]

**Uncertainty** – Uncertainty spans regulatory, clinical, manufacturing, and market-related domains. Lack of clarity in the CGT landscape raises perception of risk that may discourage private investment. The uncertainties include short-term clinical study, manufacturing and scaling, and shortage of expertise in clinical translation, commercialization, and manufacturing roles. Addressing these gaps will require coordinated efforts from regulators, researchers, investors, and policymakers to create a more sustainable financial ecosystem for advancing regenerative medicine development.

**Concentration of spending** – Concentration of funding in specific regions or institutions can also hinder the global diffusion of technologic advancements, creating disparities in access to resources, expertise, and infrastructure.

**Competitive landscape** – While Canada is competitive in biomedical research and development, it faces competition from countries with larger markets and faster regulatory pathways, such as the US and Europe. Canada's federated healthcare system can add to the challenge with different approaches to data privacy and research ethics approval by jurisdiction. This puts pressure on Canadian firms to innovate while navigating complex commercialization processes.[23] The lack of available capital limits the ability of smaller enterprises to scale up or compete with larger multinational corporations.[24,25,]

17 - https://stemcellnetwork.ca/wp-content/uploads/2024/08/4-ACCT-C5R1-Guidelines-EN-FINAL-2024-08-21-1.pdf
18 - https://cca-reports.ca/wp-content/uploads/2018/10/2017-03-08-Regen-Med-Book-ENG-WEB.pdf
19 - https://alliancerm.org/wp-content/uploads/2019/06/Manufacturing-Cures-In-Vivo-PDF.pdf
20 -https://www.nce-rce.gc.ca/NetworksCentres-CentresReseaux/PreviouslyFunded-FinancesAnterieurement/CECR-CECR/CCRM_eng.asp
21 - https://www.nce-rce.gc.ca/NetworksCentres-CentresReseaux/PreviouslyFunded-FinancesAnterieurement/CECR-CECR/CCRM_eng.asp.
22 - https://www.cda-amc.ca/gene-therapy-international-regulatory-and-health-technology-assessment-hta-activities-and
23 - https://www.ccrm.ca/wp-content/uploads/2021/06/Quick-Facts-CCRM-Media-Kit-May-2021.pdf
24 - https://www.globenewswire.com/news-release/2025/01/08/3006224/0/en/Regenerative-Medicine-Market-Size-to-Hit-USD-235-98-Billion-by-2032-Driven-by-Advancements-in-Cell-Therapy-and-Stem-Cells-SNS-Insider.html
25 - https://www.nce-rce.gc.ca/NetworksCentres-CentresReseaux/CECR-CECR/CCRM_eng.asp;

Section 3

# Development & Evidence Generation

# Development and Evidence Generation

Unlike conventional drugs, cell and gene therapies (CGTs) often target small, highly specific patient populations and require customized trial designs. This results in smaller datasets with limited generalizability to broader settings. Many CGTs involve autologous cell manipulation or gene correction, which limits scalability and introduces added complexity into clinical trial design. Given small patient populations and the long, variable progression of many target diseases, CGT trials frequently rely on surrogate endpoints or real-world evidence to demonstrate benefit.

To ensure early-stage innovations can successfully progress to Phase III trials and beyond, Canada must continue to invest in both infrastructure and human capital. This includes expanding the workforce of regulatory scientists, clinical trial coordinators, and advanced manufacturing experts. While infrastructure investments like the National Research Council (NRC)[26] and OmniaBio[27] represent major advancements, they must be matched by supports that enable Canadian biotech companies and academic researchers to access and fully leverage these centres

**Key Enablers**

**Clinical Trial Sites** – Trial sites are essential to advancing cell and gene therapy development in Canada, providing the infrastructure and expertise needed to evaluate these complex treatments. They help build local capacity among healthcare providers and researchers, generating high-quality evidence and improving readiness for clinical adoption. Strengthening clinical trial networks and supporting standardized protocols and data collection, Canada can accelerate the safe, effective integration of new therapies into the healthcare system.

+ An example of a clinical trials network is the Canadian Cancer Trials Group (CCTG).[28] Relevant trials conducted through CCTG have included CAR T-cell therapy.

**Supports for Trials** – CanReview is a national Canadian initiative backed by the Accelerating Clinical Trials (ACT) Consortium that aims to streamline ethics oversight by providing a unified research ethics review process for multi-site clinical trials across the country, all while upholding the highest ethical standards.[29]

**Guidance on Preclinical Evidence** – Regulatory applications for cell therapies face more objections than those for traditional small molecule or biologic drugs, often delaying approval and clinical use. These objections frequently arise from shortcomings in preclinical evidence, such as inappropriate animal models, unclear mechanisms of action, or endpoints that fail to demonstrate therapeutic relevance. More detailed, CGT-specific guidance on preclinical study design is needed to support early-phase trials and reduce preventable regulatory setbacks.[30]

**Key Barriers**

**Risk to Maintaining standard of Care** – Canada also risks falling behind as a trial destination if its standard of care lags other countries, limiting the relevance and competitiveness of domestic trial data. This includes inconsistent access to specialized healthcare, limited access to advanced diagnostics, and concentration of services in urban centers.[31] Given the advanced nature of CGTs, they may be particularly vulnerable.

26 - https://nrc.canada.ca/en
27 - https://omniabio.com/
28 - https://www.ctg.queensu.ca/public/who-we-are
29 - https://canreview.ca
30 - https://bmcmedicine.biomedcentral.com/articles/10.1186/s12916-024-03690-8
31 - https://cca-reports.ca/wp-content/uploads/2019/08/Report-From-Research-to-Reality-EN.pdf

Section 4

# Regulation & Appraisal

# Regulation & Appraisal[32]

Regulatory and health technology assessment (HTA) pathways can be bottlenecks for CGT adoption.

Health Canada performs regulatory review for safety and efficacy. Health Canada evaluates CGTs under the Food and Drugs Act,[33] with no dedicated regulatory framework specific to these products. However, recent amendments allow for greater flexibility under the "advanced therapeutic products" (ATP) pathway.[34]

Health Canada can grant Notices of Compliance with conditions (NOC/c) to expedite access while requiring post-market data. This pathway is critical for CGTs, which often rely on smaller trials and novel endpoints.

Health Technology assessment bodies, including Canada's Drug Agency - l'Agence des Medicaments du Canada (CDA-AMC) and Institut national d'excellence en santé et services sociaux (INESSS), after review, make recommendations on whether a treatment should be reimbursed. CDA-AMC and INESSS are also adapting. The CDA-AMC Complex Review Process covers CGT assessment, including implementation planning.[35] INESSS has a checklist specific to cell and gene therapies.[36]

The Patented Medicine Prices Review Board (PMPRB) sets price ceilings for new products. While intended to protect affordability, this process may potentially discourage investment in CGTs because they are costly to develop and manufacture, requiring higher prices for commercial viability. Final prices paid are subject to reimbursement negotiation.

**Key Enablers**

**Specific Paths and Processes** – Adaptations to existing HC, INESSS, and CDA-AMC processes exist to support review and appraisal of innovative therapeutics like CGTs. Opportunities may exist to continue to improve efficiency and timeliness. While the path may be considered difficult, it is worth noting that some CGTs have successfully reached market under these processes.

**Conditional Approval** – Health Canada may issue a Notice of Compliance with conditions (NOC/c) as a means to provide earlier access to patients for new interventions "intended for the treatment, prevention or diagnosis of serious, life-threatening or severely debilitating diseases or conditions for which a) there is no alternative therapy available on the Canadian market or, b) where the new product represents a significant improvement in the benefit/risk profile over existing products."[37] CDA-AMC can issue a time-limited recommendation which are conditional based on innovator commitments to complete or conduct further clinical studies.[38]

**Dialogue and Transparency** – HC, CDA-AMC, and INESSS have made efforts to engage with manufacturers and promote transparency, with intention to improved stakeholder engagement and alignment of regulatory and payer expectations.

**Key Barriers**

**Burden and Complexity** – Regulatory complexity may undermine investor confidence. Harmonizing global standards and developing clear guidance frameworks could enhance clarity and transparency and reduce ambiguity for developers, lowering development costs and attracting greater investment.

**Lack of dedicated CGT frameworks** – While some adaptations to existing pathways have been brought forward, these are not comprehensive, creating uncertainty and potential confusion.

**Separate processes** – With regulatory and reimbursement decision-making split across Health Canada, CADTH, INESSS, and each potential payer, the Canadian system is fragmented and difficult to navigate, leading to delays, duplication of effort, and inconsistent access to advanced therapies across jurisdictions.39

32 - https://pmc.ncbi.nlm.nih.gov/articles/PMC10162454/
33 - https://laws-lois.justice.gc.ca/eng/acts/f-27/
34 -https://www.canada.ca/en/health-canada/services/drug-health-product-review-approval/advanced-therapeutic-products.html
35 -https://www.cda-amc.ca/cadth-pharmaceutical-reviews-update-issue-26
36 -https://www.inesss.qc.ca/fileadmin/doc/INESSS/Inscription_medicaments/Fiches_inscription/en/Checklist_A_Reevaluation_new_drug_indication_combination.pdf
37 -https://www.canada.ca/en/health-canada/services/drugshealth-products/drug-products/fact-sheets/notice-complianceconditions-therapeutics-products.html
38 -https://www.cda-amc.ca/frequently-asked-questions-time-limited-recommendations
39 -https://cca-reports.ca/wp-content/uploads/2019/08/Report-From-Research-to-Reality-EN.pdf

# Case Study:

# ExCellThera & UM171

***Canadian Innovation, Stalled at Home***

ExCellThera, a Montreal-based biotechnology company, emerged from the collaborative efforts of Dr. Guy Sauvageau and medicinal chemist Anne Marinier at the Université de Montréal's Institute for Research in Immunology and Cancer (IRIC).[a] Their groundbreaking discovery of the molecule UM171 marked a significant advancement in hematopoietic stem cell (HSC) expansion.

In June 2024, ExCellThera announced that the European Medicines Agency (EMA) had accepted for review its Market Authorisation Application (MAA) for UM171 Cell Therapy (INN-dorocubicel) under the Accelerated Assessment procedure based on promising Phase 1 and Phase 2 clinical data in over 100 blood cancer patients across multiple trials conducted in Canada, the US and Europe.[b] This regulatory pathway is reserved for medicines deemed of major public health interest, aiming to expedite the review process. UM171 Cell Therapy had previously received Orphan Medicinal Product designation and access to the Priority Medicines (PRIME) scheme from the EMA, as well as Regenerative Medicine Advanced Therapy (RMAT) designation from the U.S. Food and Drug Administration (FDA).[c]

**Challenges in the Canadian Landscape**

Despite its Canadian origins and international recognition, UM171 Cell Therapy has faced hurdles in domestic adoption. In a panel at Stem Cell Network's Till and McCullough Meetings in 2024,[d] Dr. Sauvageau highlighted the fragmented nature of Canada's regulatory and funding systems, noting that while Health Canada expressed interest in the therapy, existing regulations required initiation of Phase III trials before considering accelerated approval—a stipulation not aligned with the EMA's more flexible approach, in particular with respect to high unmet need life-threatening orphan indications and related novel medicinal products. Additionally, the lack of coordinated support for commercialization and infrastructure development in Canada has impeded the therapy's local availability.

**Implications**

ExCellThera's journey underscores the challenges Canadian biotech companies may encounter in translating scientific innovations into accessible therapies within their own country. The case highlights the need for streamlined regulatory pathways, cohesive funding mechanisms, and robust support for commercialization to ensure that homegrown medical advancements benefit Canadian patients.

a- https://excellthera.com/about/
b- https://excellthera.com/news/excellthera-announces-emas-acceptance-under-accelerated-assessment-of-market-authorisation-application-maa-for-um171-cell-therapy-for-patients-with-hematological-malignancies-who-lack-a-rea
c- https://excellthera.com/news/excellthera-announces-international-non-proprietary-name-and-new-orphan-drug-designation-for-um171-cell-therapy/
d- https://stemcellnetwork.ca/tmm/till-mcculloch-meetings/

Section 5

# Access & Integration

# Access & Integration

The final step in CGT translation involves reimbursement and health system readiness. CGTs often require large, one-time payments, but deliver long-term or curative benefits. This mismatch between cost and budget cycles may challenge traditional reimbursement decision making and mechanisms.

**Price Negotiation**

The pan-Canadian Pharmaceutical Alliance (pCPA) negotiates pricing after HTA, but provinces ultimately decide whether to fund a product. When a treatment serves a small population or has a particularly long time horizon to fully understand durability of the treatment, the HTA recommendations may reflect the resulting uncertainty. This often takes the form of recommended price reductions. Significant price reduction recommendations may result in drawn out negotiations, slowing access, or result in an industry decision not to pursue reimbursement of the product with the public payers in Canada.

There may be a role for innovative reimbursement agreements that include risk sharing in navigating these circumstances. Risk-sharing agreements—such as outcomes-based or subscription models—offer promise for CGTs but are not yet widely implemented in Canada

.**Health System Integration**

For example, CAR-T, an immunotherapy that engineers a patient's t-cells to fight cancer,[40] delivery also requires the coordination of hospitals, specialists, diagnostic services, and follow-up care. This can be challenging when care delivery and funding often occurs across different silos (***see diagram on the next page***).[41]

Hospitals, which often bear the operational burden of delivering CGTs, require dedicated funding mechanisms and streamlined processes for formulary inclusion. Delivery requires change management and, potentially, investment in facilities not typical across the existing health system like clean rooms.

Programs such as Alberta's Health Innovation Platform Partnerships and the NHS Genomic Medicine Service provide examples of integrated funding approaches centering innovation that could inform Canadian reforms.

Data integration, standardization, and health informatics systems may need to be upgraded to support longitudinal monitoring, which is essential for value-based payment models.

Finally, education and engagement strategies targeting both clinicians and patients are needed to build familiarity with CGTs, manage expectations, and support informed decision-making.

## Examples: Integrated Funding Approaches for Innovation

### Alberta Health Innovation Platform Partnerships (HIPP)

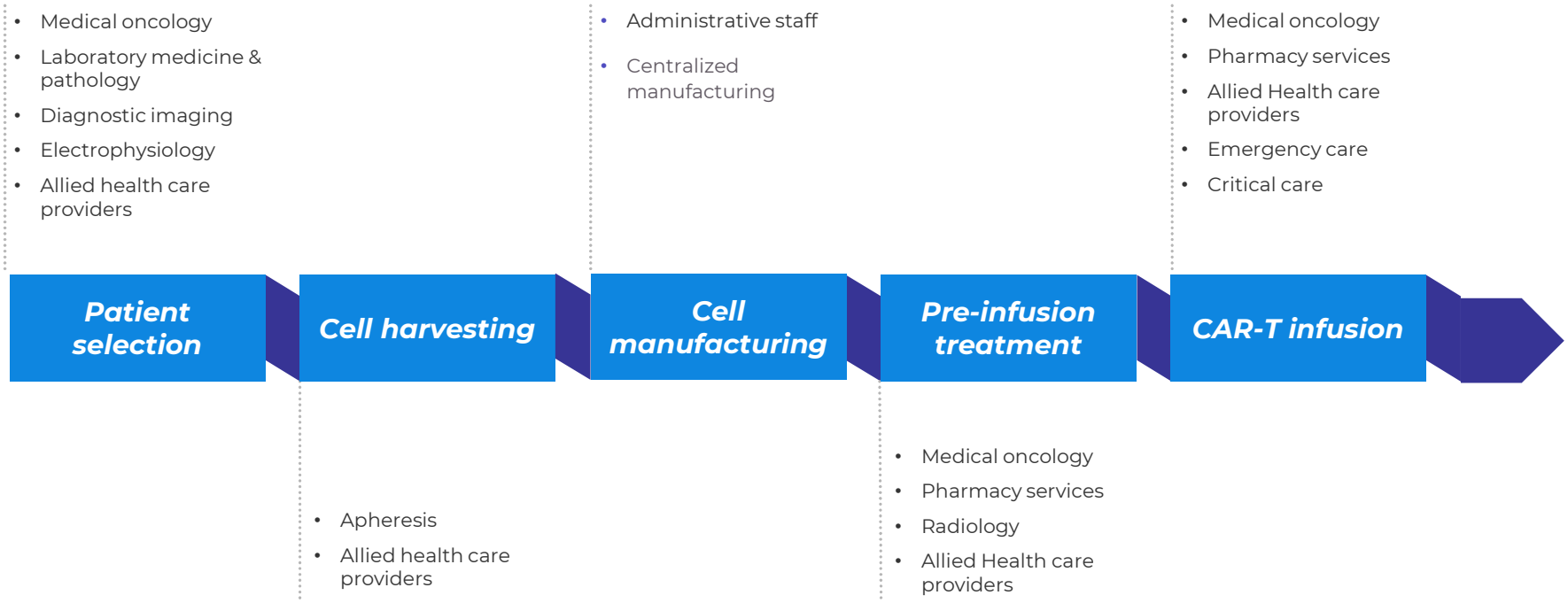
HIPP supports collaboration across academia, health systems, and industry to accelerate the development and integration of health innovations, including precision medicine and emerging therapies. By aligning research funding with health system priorities and providing infrastructure support, the program helps bridge the gap between discovery and implementation within Alberta's healthcare system.[42]

### NHS Genomic Medicine Service (GMS)

The GMS offers a national framework for delivering genomic testing and integrating genomic technologies into routine care across England. It combines centralized infrastructure with dedicated funding pathways, enabling equitable access to advanced diagnostics and supporting the clinical adoption of genomic and cell-based therapies through a unified, system-wide approach.[43]

40- https://www.cancer.gov/about-cancer/treatment/research/car-t-cells
41- https://www.albertahealthservices.ca/assets/info/cca/if-cca-car-t-cell-therapy.pdf
42- https://albertainnovates.ca/funding/health-innovation-platform-partnerships-program/
43- https://www.england.nhs.uk/genomics/nhs-genomic-med-service/

# Example: Care pathway for CAR-T & Associated Healthcare Services[41]




41 - https://www.albertahealthservices.ca/assets/info/cca/if-cca-car-t-cell-therapy.pdf

# Access & Integration – Key Enablers

**Early access programs** – Timelines governing funding decisions may be highly variable and could depend on budget cycles, the complexity and size of the healthcare system and other factors.

**Integrated funding for innovation and mainstream care** – Healthcare administrators will often make a distinction between essential or medically necessary services that are paid for as part of a healthcare insurance package, versus investigational procedures that are paid for through privately and publicly funded research programs. Budget holders may be willing to pay for the ancillary services (e.g., diagnostic imaging or critical care) required to support research of new innovations but are often reluctant to pay for the innovations themselves. In some healthcare systems, the recognition for the early introduction of valuable innovation has led to specialized programs that fund innovative products as part of mainstream healthcare delivery. Examples are the Alberta Health Innovation Platform Partnerships Program, and the NHS Genomic Medicine Service.

**Innovative Agreements** – New reimbursement approaches that reduce uncertainty about the costs and effectiveness of care could speed access and enable paying for value. This could involve a number of risk-sharing approaches including volume or outcomes-based risk sharing agreements or novel approaches to financing including subscription models.[44]

**Change Management** – Successful CGT integration requires coordinated efforts to adapt institutional processes, update clinical workflows, train personnel, and align funding mechanisms—ensuring that health systems are equipped to deliver these novel therapies efficiently and equitably.

44- https://pubmed.ncbi.nlm.nih.gov/20226559/

# Access & Integration – Key Barriers

**Fragmented and Siloed Funding Mechanisms** – The introduction of new technologies like CGTs is often slowed by the lack of coordinated funding across multiple budget holders. In systems where hospital budgets, drug programs, and disease-specific funds operate in silos, it can be unclear who is responsible for covering costs related to pretreatment, monitoring, or ancillary services like fertility preservation.

+ The absence of a formal framework for assigning financial responsibility can lead to hesitation among stakeholders and delays in access.
+ Jurisdictional fragmentation across provinces further complicates the picture, as funding models and policies vary widely, with no national consistency.
+ In contrast, centralized systems that can pool or reallocate funds across programs are often better positioned to implement innovative care pathways efficiently.

**Healthcare Provider and Patient Education** – The specialized nature of CGTs requires new knowledge and skills across the care continuum. Without targeted education and training, clinicians may be unprepared to deliver these therapies, and patients may lack the information needed to make informed decisions.

**Health Equity Concerns** – Access to CGTs is likely to be concentrated in large urban centres with advanced infrastructure, raising concerns about equitable access for patients in rural or remote areas.

**Prolonged Reimbursement and Implementation Negotiations** – Lengthy negotiations between manufacturers, payers, and health authorities can delay time-to-access, especially when responsibilities for delivery and funding are unclear or contested.

**Health System Capacity Constraints** – The delivery of CGTs requires specialized infrastructure and highly trained personnel, both of which are in limited supply. Key issues include:

+ Shortages in skilled human resources (e.g., apheresis nurses, lab technologists).
+ Uneven geographic distribution of facilities capable of delivering advanced therapies.

**Infrastructure Misalignment in Practice** – Real-world examples, such as the need for apheresis in certain gene therapies, highlight how existing infrastructure can be inadequate or poorly aligned with the demands of CGT delivery.

Section 6

# Conclusion

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**Canada is home to world-class science and a publicly funded health system well-positioned to support innovation. With targeted reforms and greater cross-sector integration, it can become a global leader not only in regenerative medicine research, but also in its evaluation, implementation, and delivery.**

As regenerative medicine advances from scientific discovery to clinical application, Canada must adapt its systems to ensure these innovative therapies are delivered equitably, efficiently, and sustainably. The current environment—characterized by fragmented coordination, uneven infrastructure, limited early-stage capital, and regulatory uncertainty—risks delaying patient access and limiting the country's ability to lead in this rapidly evolving field.

To address these challenges, progress is needed across multiple dimensions:

**Policy alignment** across regulatory, reimbursement, and service delivery systems to streamline decision-making and reduce uncertainty for innovators.

**Investment in enabling infrastructure** including clinical trial capacity, manufacturing, and implementation supports—especially in decentralized regions—to promote equitable access and reduce delivery bottlenecks.

**Flexible, coordinated funding models** that support translational research and commercialization, and allow shared risk across public and private sectors.

**Capacity-building and engagement** strategies for clinicians, administrators, and patients to ensure the system is ready to adopt and manage complex regenerative interventions.

**National coordination mechanisms** such as trial networks and real-world data platforms, to foster shared learning, reduce duplication, and accelerate scale-up.

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