Advancing Cell & Gene Therapy

Calls to Action: Access to Cell and Gene Therapies in Quebec

Summary of the Montreal Discussion Workshop organized by Advancing Cell and Gene Therapy

June 2025

Advancing Cell & Gene Therapy

Groupe d'action sur le déploiement des thérapies cellulaires et géniques

Calls to Action: Access to Cell and Gene Therapies in Quebec

Executive Summary and Introduction

Executive Summary

Advancing Cell and Gene Therapy ("ACGT") organized an inaugural event in Montreal on February 6, 2025 in partnership with BIOQuébec. The initiative, entitled "Panel d'experts et réflexion sur les thérapies cellulaires et géniques", brought together close to 50 leaders in Quebec's health ecosystem, including researchers, clinicians, industry representatives, government decision-makers and patient advocates, to discuss the challenges and opportunities associated with the deployment of cell and gene therapies (CGTs) in Quebec.

Dr. Stéphane Bergeron, Assistant Deputy Minister of Physical and Pharmaceutical Health at the Ministère de la Santé et des Services Sociaux (MSSS), opened the event with a presentation on MSSS' vision for CGT. The members of an expert panel – including Ghislain Bérard, Dr. Jean-Sébastien Delisle, Jessy Ranger, and Dr. Denis Claude Roy – then shared their views on the issues and potential solutions.

The event continued with a discussion workshop open to all participants. Three main interdependent calls to action emerged from the discussions, forming a continuum for optimizing access to CGTs in Quebec. The calls to action, which are mutually reinforcing, are as follows:

Call to Action 1: Strengthen the research ecosystem
- Diversify funding sources
- Accelerate and simplify approval processes
- Foster collaboration among different actors in the research ecosystem

Call to Action 2: Prepare the health system to deliver CGTs
- Invest in infrastructure dedicated to CGTs
- Strengthen the skills of healthcare personnel
- Improve the geographic accessibility of treatments

Call to Action 3: Increase access to CGTs throughout the health system
- Demonstrate the value of CGTs
- Improve access to data related to its use
- Introduce tailored reimbursement mechanisms
- Increase public and healthcare professionals' awareness of the benefits of CGTs

Participants identified the establishment of a cross-sectoral forum as an essential prerequisite to the Call to Actions' success. This should bring together all parties involved in the healthcare ecosystem along with the Ministère de l'Économie, de l'Innovation et de l'Énergie (MEIE).

Introduction

Despite the revolutionary potential of CGTs for the treatment of certain conditions, numerous challenges remain that prevent their rapid and large-scale deployment in Quebec, limiting patient access to promising innovations. It is in this context that ACGT brought together Quebec health system leaders to identify common courses of action that could accelerate CGTs' implementation.

Consultative Approach

In partnership with BIOQuébec, ACGT organized an inaugural event in Quebec on February 6, 2025 at Centre Phi de Montréal. This consultation and collaboration initiative brought together stakeholders to discuss opportunities and obstacles, and to identify calls to action for facilitating and accelerating access to CGTs.

The event drew close to 50 participants from different sectors, including officials from the Ministère de la Santé et des Services Sociaux (MSSS), the Ministère de l'Économie, de l'Innovation et de l'Énergie (MEIE), and the Institut National d'Excellence en Santé et Services Sociaux (INESSS), healthcare professionals, patient, industry and scientific associations, and Quebec ACGT members.

Considering the depth of the conversations, this report is limited to summarizing the main courses of action identified. It is not a comprehensive account of all the suggestions discussed.

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The Event

The Event in Brief

The three-hour event opened with a presentation by Dr. Stéphane Bergeron, Assistant Deputy Minister of Physical and Pharmaceutical Health at the Ministère de la Santé et des Services Sociaux (MSSS), on the MSSS' vision regarding CGT. This was followed by a panel, which presented four complementary perspectives:

Ghislain Bérard, Pharmacist and Coordinator of the Programme de Gestion Thérapeutique des Medicaments (PGTM) at CIUSSS de l'Estrie – CHUS

Dr. Jean-Sébastien Delisle, Scientific Director of Réseau de Thérapie Cellulaire et Tissulaire (ThéCell) and Professor of Medicine at Université de Montréal

Jessy Ranger, Director of Patient Programs, Health Policy & Advocacy at Myeloma Canada

Dr. Denis Claude Roy, Chief Executive Officer of CellCAN and Professor of Medicine at Université de Montréal

The participants then held discussions during an hour-long workshop on the future of CGTs around the following question:

What concrete measures would enable Quebec to fully benefit from the potential of cell and gene therapies?

The event closed with a recap of the discussions at each worktable.

Discussion Summary

Participants all agreed on the significant potential of CGTs for patients. Many of them had observed, in their practices, the direct impact of those therapies on the course of the disease in the people treated.

(Note that quotations below have been translated from French.)

Overview of CGTs in Quebec

Dr. Stéphane Bergeron, Assistant Deputy Minister of Physical and Pharmaceutical Health at the Ministère de la Santé et des Services Sociaux

Dr. Bergeron opened the event with a presentation on the role of the MSSS in ensuring access to CGTs.

"The MSSS has already stepped up and is hard at work defining care options to avoid diagnostic errors and ensure effective patient care."

He mentioned several commitments made by the province to address issues faced by people living with rare diseases, for example the introduction of the Politique québécoise pour les maladies rares (rare disease policy)[1] in 2022 and the action plan[2] that followed in 2023.

Dr. Bergeron noted Quebec's past successes with CGTs, the adaptation of the healthcare system, and the need to identify treatment centres and increase their capacity and screening. He stressed the importance of reviewing the real benefits of CGTs in view of their costs, and of demonstrating their long-term benefits.

Dr. Bergeron closed by reiterating that the MSSS wants to be part of the solution in ensuring access to CGTs, which he saw as part of the future.

1. Pour Une Meilleure Reconnaissance Et Prise En Charge Des Personnes Atteintes De Maladies Rares. Government of Quebec.
2. Plan d'action québécois sur les maladies rares 2023-2027. Government of Quebec.

Discussion Summary

Expert Panel

Ghislain Bérard, Pharmacist and Coordinator of the Programme de Gestion Thérapeutique des Medicaments (PGTM) at CIUSSS de l'Estrie – CHUS

Mr. Bérard indicated that CHU's pharmacy departments had asked the PGTM[3] to undertake a review of what would be required to offer gene therapy in hospital pharmacies. He noted that the management of gene therapy medication is still in its early days. The aim of those treatments is to change the disease's natural trajectory, up to healing. This new development offers the opportunity to develop a framework that effectively supports the deployment of CGTs.

As a pharmacist, Mr. Bérard has paid special attention to the need for permanent facilities where CGTs can be safely prepared and administered to patients – both for the sake of the patients and their families, as well as the staff of the healthcare facilities – and to then conduct follow-up. He points out that the financial outlays necessitated by hospital infrastructure will have to be managed responsibly, given that the precautions to be taken with gene therapy vary and depend on the biological risk associated with the vector (viable or non-viable) used, as well as the type and nature of the vector (type of virus, e.g. adenovirus, retrovirus, lentivirus, etc.).

The literature covers four levels of security associated with the four levels of biological risk of the vectors that can be employed. Most centres will be able to prepare and administer level 1 or 2 medications (on condition that they meet the requirements for those types of risk), but for level 3 and 4 medications the protocols are more involved and they should only be dispensed in selected places, limited to one or two centres in Quebec.

Mr. Bérard cited the following obstacles to access: cost, prioritizing patients, and evaluating short- and long-term toxicity. He indicated that it would be important for the Ordre des Pharmaciens du Québec to develop standards and to have infrastructure needs met.

3. LA THÉRAPIE GÉNIQUE dans les pharmacies d'établissement du Québec – Comment s'y préparer état des connaissances. Programme de Gestion Thérapeutique des médicaments.

Discussion Summary

Expert Panel

Dr. Jean-Sébastien Delisle, Scientific Director of Réseau de thérapie cellulaire et tissulaire (ThéCell) and Professor of Medicine at Université de Montréal

Dr. Delisle pointed out that Quebec could play a historic role in CGT given the province's population and university expertise. The objective of the ThéCell network is to develop the first clinical proofs. Because CGTs are a rapidly emerging technology, it is important to review the impact of treatment, from the laboratory to the bedside, so that we can keep improving.

According to Dr. Delisle, the main impediment to accessing CGTs is the current hospital culture, specifically the lack of validation of research and innovations. He suggests investing in designated centres and clinical research infrastructure dedicated to CGTs to ensure the development of a local industry that could then more quickly meet patients' needs. He also expressed the need to have an "orchestra conductor", along the lines of what was done for RNA in Quebec.

Dr. Delisle stressed to the importance of anticipating rather than reacting, and that system capacity and effectiveness need to be increased. He indicated that clinical research is a way to prepare. He also pointed out the importance of being ready for the arrival of biosimilars, of improving logistics and the regulatory and legislative framework, and of increasing Quebec's capacity across the CGT value chain.

Jessy Ranger, Director of Patient Programs, Health Policy & Advocacy at Myeloma Canada

Ms. Ranger noted that CGTs are a priority for her organization, highlighting that CGTs have evolved enormously over the past few years, with a positive impact on blood cancers. The arrival of CAR-T therapy, in particular paved the way for outcomes once thought impossible. Ms. Ranger also stressed that a big difference in treatment options for blood cancers is that CGTs have few side-effects; however, there are still several obstacles to wider deployment of this form of therapy. Those obstacles include difficult access due to the shortage of accredited institutes, their concentration in urban centres, and other personal, cultural and language factors.

Ms. Ranger said it is important to think about ways to meet demand. New molecules are being developed in universities, but the regulatory process is currently too onerous to allow patients quick access. A concerted and rigorous planning effort is essential to overcoming those challenges.

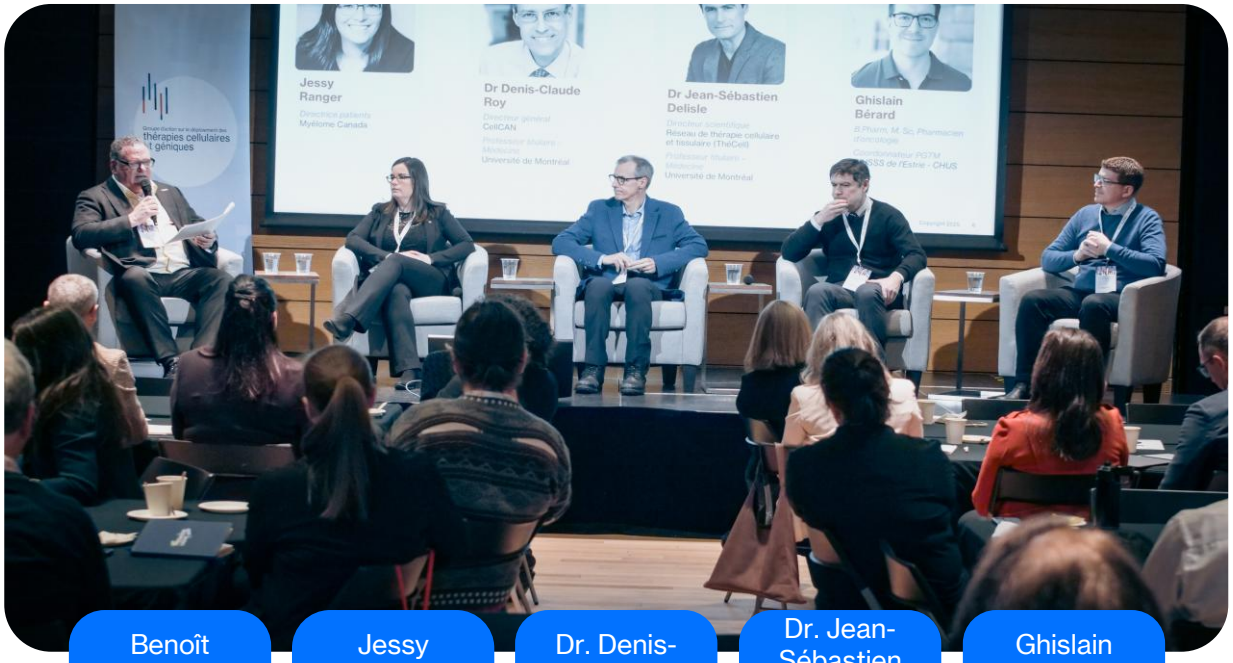
Discussion Summary

Expert Panel

Dr. Denis-Claude Roy, Chief Executive Officer of CellCAN and Professor of Medicine at Université de Montréal

Dr. Roy provided examples of successes ranging from research discoveries to the creation of Quebec companies. He argued for greater collaboration among the different participants in the health ecosystem, including research institutes, government, the biopharmaceutical industry and other key stakeholders. Such collaboration would help reduce currently excessive wait times before patients get to access innovative therapies. There is a great deal of promising research in Quebec and elsewhere, but, as Dr. Roy pointed out, it is not readily adapted into tangible applications. For Dr. Roy, the future of CGTs depends on de-siloing and collaboration.

Dr. Roy indicated that it would be important for Quebec to work more often – and more collaboratively – with the biopharmaceutical industry to make the province attractive, because CGTs are a new paradigm for care.



Benoît Larose | Jessy Ranger | Dr. Denis-Claude Roy | Dr. Jean-Sébastien Delisle | Ghislain Bérard

What We Heard from the Participants

After the presentations by the Assistant Deputy Minister and expert panelists, participants engaged in discussions to propose tangible calls to action to promote and accelerate access to CGTs in Quebec.

Condition of Success: Establish a cross-sectoral discussion forum

Before addressing the calls to action, participants unanimously identified the establishment of a cross-sectoral discussion forum as an essential precondition for successful access to CGTs in Quebec. The discussions revealed an urgent need to de-silo and foster dynamic collaboration among government, research centres, clinicians, patient groups and the biopharmaceutical industry. This forum would help transform occasional discussions into a permanent collaborative platform through periodic meetings and multisector committees.

But this would be more than a forum for discussion, it would be a catalyst for:

- breaking down silos to foster collaboration and help share expertise and resources;
- validating research and enhancing the clinical research ecosystem; and
- accelerating access to innovations and increasing the number of patients who can benefit from CGTs by anticipating needs more accurately and earlier.

The examples set by the American consortiums Bespoke[4] and BioMaP[5] illustrate this type of cooperation between government, industry and researchers. Those consortia are instances of collaboration between the biopharmaceutical industry and the U.S. government. BioMaP also includes university research institutes.

Proposed Calls to Action

During their discussions, participants highlighted Quebec's very strong research capability. However, they also noted that, although it includes clinical research, it often fails to lead to accessible treatment for patients. Moreover, access to approved treatments also remains a challenge, limited in part by the health system's capacity to provide CGTs.

This section describes the three main Calls to Action identified by participants. Together, these offer solutions for overcoming the obstacles identified by the participants. These interdependent and complementary actions form a continuum: they all converge towards the common objective of optimizing access to CGTs in Quebec.

4. For more information about Bespoke: https://ncats.nih.gov/research/research-activities/gene-targeted-therapies/bgtc-bespoke-gene-therapy-consortium.
5. For more information about BioMaP: https://www.biomap-consortium.org/.

Call to Action 1:

Strengthen the research ecosystem

Given the high cost of CGT research and production, participants considered it necessary to have access to more diversified funding sources for research. Public-private partnerships or co-financing, CGT investment funds or earmarked funds within existing biomedical research funds, and incentives for businesses investing in CGT R&D are a few examples of solutions put forward.

Accelerating and simplifying approval processes is also critical to developing the CGT research ecosystem. The procedures for approving, evaluating and reimbursing, often long and complex, pose a major challenge for the clinical research sector. Yet it is essential to maintain strict safety standards and to rigorously evaluate the effectiveness of treatments.

It is also imperative to foster collaboration among different ecosystem participants in order to share knowledge and resources and maximize the provincial, national and international impact of funded projects.

Call to Action 2:

Prepare the health system to deliver CGTs

CGTs requires special infrastructure, which currently restricts access to such treatment to a few hospitals and a limited number of patients. To broaden access, it is essential to prepare the health system to deliver CGTs by investing in dedicated infrastructure. Participants highlighted the need to allocate, as a priority, funds to improve and acquire the infrastructure needed to equip more hospitals to provide CGTs. In that regard, the designation and accreditation of centres by government should be accelerated and simplified. Access to beds and resources for tests and treatment is also an issue, even in hospitals that are already equipped. Thus, it was suggested that beds and resources be dedicated to testing for CGT treatment and follow-up.

Given the relative newness of the area, it would be important to strengthen the skills of healthcare personnel by offering more training and knowledge-sharing to support clinical research teams and those providing commercial therapies. The complexity of care protocols requires specific expertise in patient treatment and follow-up. Helping professionals become more familiar with these therapies will maximize their effectiveness.

Finally, clinical research and the availability of CGTs through the biopharmaceutical industry are concentrated in the major urban areas, which limits access to the therapies for people living in remote areas. In addition to virtual hospitals, another proposed solution was to create satellite centres in regional hospitals connected through a regular communication network with metropolitan centres to provide patient follow-up, thereby improving the geographic accessibility of treatments.

Call to Action 3:

Increase access to CGTs throughout the health system

Demonstrating the value of CGTs is a crucial step in optimizing their benefits in Quebec. Participants all agreed on the significant potential CGTs promise for positive economic impact on the healthcare system and Quebec society. Reducing extended or repeated hospital stays for people afflicted with complex diseases and receiving CGTs could ensure better quality and longer life, even in some cases a cure. That would enable patients' caregivers to contribute more to society, not to mention reducing pressure on the healthcare system. Thus, despite the initial investment, everyone agreed that CGTs are a financially viable solution over the long-term.

To demonstrate the value of CGTs, it is crucial to improve access to data related to their use. Participants suggested several ideas for centralizing that data, including a common database or, as previously mentioned, a cross-sectoral discussion forum for researchers, clinicians and other stakeholders. That data would be used to measure the clinical and economic benefits of CGTs in order to justify the investments required for their deployment and to optimize their use.

In addition, to ensure equitable access to CGTs, it is essential to introduce tailored reimbursement mechanisms. The current reimbursement model, which is the same for all CGTs and other therapies, does not account for the significant impact of the therapy on hospital pharmacy department budgets. A more detailed review of an amortization model that includes the cost of acquiring treatments and the care episode would be warranted. CGTs have potentially significant long-term economic benefits because they reduce demand for care and improve health and quality of life. Amortizing acquisition costs would make it easier to manage the necessary investment for CGTs in the long run.

Finally, it is important to increase the public's and healthcare professionals' awareness of the benefits of CGTs. A better understanding of these therapies and their potential could encourage their adoption and use. Information and public education campaigns could help in that regard.

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Conclusion

Conclusion

Quebec already has what it takes to position itself as a major player in CGTs in Canada. The workshop revealed palpable excitement and shared desire among participants to transform this ambition into reality. This will require concerted action and determination to overcome organizational and operational obstacles.

The workshop highlighted several essential actions, but above all confirmed Quebec's tremendous potential when it comes to CGTs. It will be critical to increase collaboration among stakeholders, de-silo and share resources (particularly research funding and staff), establish CGT-dedicated infrastructure, improve the accessibility of treatments, opt for more regulatory flexibility and increase public awareness about the existence and benefits of CGTs.

All those measures, driven by a spirit of collaboration and innovation, aim to offer patients a wider range of therapeutic options to improve their quality of life and their prognosis. Encouraged by this positive dynamic and the shared conviction of the tremendous potential of CGTs in the treatment of certain serious diseases, the community indicated the time has come to translate evidence into tangible action, and to make Quebec a model of excellence in the field of cell and gene therapy.

About Advancing Cell and Gene Therapy

Advancing Cell and Gene Therapy (ACGT), established in 2024, is a group of leading Canadian innovative pharmaceutical companies working together to improve patient outcomes and ensure Canadians have equitable access to innovative therapies.

Our vision is a Canada where many chronic, debilitating, or even fatal diseases become a thing of the past because of innovative cell and gene therapies. Our mission is to advance a healthcare system in which more Canadians have equitable and timely access to potentially life-changing and life-saving cell and gene therapies.

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