Advancing Cell & Gene Therapy

A Life-Changing Promise; A Nation-Building Moment

What We Heard at the Toronto Symposium on Access to Cell and Gene Therapies

December 2025

A Life-Changing Promise;
A Nation-Building Moment

Executive summary

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Advancing Cell and Gene Therapy (ACGT) is a group of innovative pharmaceutical companies working to foster health system dialogue about the promise of cell and gene therapies (CGTs) and what Canada needs to do to ensure fast and equitable access to them. Building on a successful Discussion Workshop in Montreal in early 2025, ACGT partnered with Life Sciences Ontario (LSO) to convene a half-day event in Toronto on October 30, 2025. This Symposium on Access to Cell and Gene Therapies gathered key stakeholders from Canada's health system, including patient advocates, clinicians, researchers, industry representatives and government officials.

The event included remarks from health system leaders including Garrett Tone (Invest Ontario), Cate Murray (Stem Cell Network), Dr. Alison Symington (Life Sciences Ontario), Dr. Rob Annan (Genome Canada), Dr. Stéphanie Michaud (BioCanRx), and Kim Hanson (Breakthrough T1D). Following this, more than 80 attendees participated in a workshop focused on the promise of CGTs and how to be ready for where science can take us. Calls to action stemming from this workshop included:

1. **Reforming Health Technology Assessment (HTA) and payer models:** Early stakeholder engagement; developing CGT-specific pathways; creating tailored evidence requirements; and implementing outcomes-based agreements.

2. **Ensuring patients can equitably access therapies:** Adopting a national approach to accessing CGTs; targeted clinician education; and supporting patient education.

3. **Strengthening domestic research, manufacturing and data-sharing**: Investing to support research and commercialization; streamlining regulatory processes for facilities; and investing in better data infrastructure.

In the What We Heard report that follows, these themes will be further explored, alongside a detailed summary of speakers' remarks and participants' discussion. Insights, including the calls to action, are drawn from multiple conversations and do not necessarily reflect the diverse viewpoints of all event attendees or ACGT members.

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Introduction

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Cell and gene therapies hold enormous promise for thousands of Canadians with chronic, debilitating and fatal disease and disorders, illnesses and life-threatening injuries, as well as the people who love and care for them. These therapies have the potential to prevent, treat and even cure many diseases and disorders, including ones that currently lack effective treatment options.

Innovation is advancing quickly, and Canadian patients, clinicians and innovators have an opportunity to be at the forefront – if Canada can be ready.

Advancing Cell and Gene Therapy (ACGT) is a group of innovative pharmaceutical companies, including **Alexion, Ferring Pharmaceuticals, Johnson & Johnson Innovative Medicines, Vertex Pharmaceuticals, Pfizer, Novo Nordisk,** and **Ultragenyx**, who are working together to support more equitable access to these therapies in Canada.

To help lay the groundwork, ACGT, in partnership with Life Sciences Ontario and with support from sponsors **Merck**, **AstraZeneca**, **Gilead and Innovative Medicines Canada**, organized the Symposium on Access to Cell and Gene Therapies at MaRS Discovery District in Toronto on October 30, 2025. Recognizing that successful delivery of CGTs demands cooperation across the health continuum, the event convened more than 80 clinicians and researchers, patient advocates, industry representatives, innovators, and government officials from as far away as Vancouver, Montreal, and points in between. Together, they engaged in generative discussion outlining opportunities, examining obstacles and identifying solutions.

This What We Heard report summarizes major themes and advice emerging from that discussion. As noted above, these ideas and recommendations are distilled from multiple conversations involving people and organizations with diverse viewpoints, and do not necessarily reflect the position of individual participants or ACGT members.

About Advancing Cell and Gene Therapy

Advancing Cell and Gene Therapy (ACGT), established in 2023, 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.

ACGT members include:

ALEXION AstraZeneca Rare Disease · FERRING PHARMACEUTICALS · Johnson & Johnson · VERTEX · Pfizer · novo nordisk · ultragenyx

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Event Summary

The Event in Brief

The half-day event began with expert speakers who set the stage for the workshop discussion. The Symposium opened with video greetings from Ontario's Deputy Premier and Health Minister, **Hon. Sylvia Jones**, followed by introductory remarks by Invest Ontario's Executive Director, Life Sciences, **Garrett Tone**. Participants then heard a keynote presentation by **Cate Murray**, President and CEO of the Stem Cell Network, which outlined Canada's path to leadership in cell and gene therapy innovation and care.

A panel discussion then allowed for a deeper examination of key issues, bringing depth of experience and first-person perspectives to the fore. Moderated by LSO Chair **Dr. Alison Symington**, the panel included:

Dr. Rob Annan – President & CEO, Genome Canada
Dr. Stéphanie Michaud – President & CEO BioCanRx
Kim Hanson – Chief External Relations Officer, Breakthrough T1D

Participants then broke into groups of no more than six for facilitated discussions over the course of 70 minutes, followed by report-backs to the full assembly. The workshop revolved around the two following questions:

What do you find most promising about cell and gene therapies?
What needs to happen to ensure equitable access to CGTs in Canada?

The event closed with a recap of the discussion.

Presentation Summary

Our expert speakers unearthed several key themes to frame the workshop that followed.

The Promise of CGTs: Canada's Moment

Keynote speaker Cate Murray outlined the opportunity and the challenge: "We're at the advent of a new era in medicine, but the choices we make today will determine how, and how fast, we deliver durable health solutions and the cures of tomorrow."

Discussion panelists underscored the transformative potential of CGTs, emphasizing the urgent need for cures given unmet patient needs. Garrett Tone spoke of the work Ontario's government and Invest Ontario are doing to capitalize on the economic benefits of these innovations. Cate Murray tied it into Canada's history of research breakthroughs – insulin, mRNA, GLP-1, stem cells – and challenged us to finish the story we began "with therapies discovered, developed, manufactured, and delivered right here at home."

Opportunities

Research & Innovation: Canada's historic leadership in cell therapy and robust research networks present a strong foundation for continued innovation, while advancements in data collection, analytical capabilities, and precision tools like CRISPR are making for a golden age in biomedical research.

Data-Driven Advancement: The integration of clinical trials into routine care and large-scale population genomic datasets can generate real-world evidence essential for decision-making and therapeutic development.

Better Care: CGTs won't just treat disease, they will restore health – repairing organs, regenerating tissues, and allowing Canadians to age better while reducing strain on, and transforming, our healthcare systems.

Challenges

Access & Equity: CGTs are often concentrated in limited urban centers, creating systemic inequities exacerbated by Canada's vast geography. CGTs are complex, and while some are scalable, others are bespoke with decentralized manufacturing, challenging our system to make them available and to ensure standardization and safety regardless of location.

Assessment & Cost: Therapies are perceived as costly to develop, procure and deliver, and a lack of robust long-term evidence hinders listing decisions. The status quo has been designed for an old paradigm of small molecule drugs sponsored by large manufacturers. Canada's lengthy provincial reimbursement negotiations can significantly delay access, and the cost of working through our system is prohibitive for many Canadian innovators. This contributes to declining investment, inhibiting commercialization.

Data Governance & Sharing: Challenges persist in data governance, secure data sharing, and ensuring diverse, representative datasets for research and clinical application. Data siloing within provinces further restricts access, particularly for smaller jurisdictions and rural areas. Patient and political emphasis on data privacy further complicates data sharing.

Systemic & Jurisdictional Fragmentation: A misalignment of incentives across levels of government, and siloing within and between provinces, hinder coordinated action.

Solutions

Regulatory & System Readiness: Streamlined regulatory pathways that address the complexities of cell and gene therapies, including a prioritized pathway for Canadian CGTs, must go hand-in-hand with health system preparation for the delivery of these therapies – including needed investments in human, clinical and infrastructure capacity.

Reimbursement Reform: Emphasizing the need for outcomes-based reimbursement models informed by real-world evidence, with streamlined processes in parallel to regulatory approvals. Build on initiatives like Ontario's FAST pilot, and foster a better environment for trials, investment and commercialization.

Enhanced Data Sharing: Developing Canada-wide data assets (e.g., Genome Canada's Precision Health Initiative) and integrating research into routine healthcare delivery to generate real-world evidence, while addressing ethical considerations around consent, to diagnose patients and identify new therapies.

Interprovincial Collaboration: Learning from successful models (e.g., COVID-19 response) where barriers were overcome and fostering a culture of coordinated action to dismantle fragmentation and leverage collective strengths, for example through improved data-sharing and better coordination for patients crossing provincial boundaries.

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What We Heard: Workshop Insights

What We Heard from Participants

Following our expert speakers, participants engaged in small-group, solutions-focused dialogue on CGTs in Canada. Insights from this working session are captured below.

The Promise of CGTs

Participants dared to imagine a world in which every disease has a targeted treatment. They saw an opportunity for Canada to take a global leadership role – characterizing this as a nation-building moment. There was a broad consensus that most important is the promise to meaningfully improve thousands of Canadians' lives.

Many noted that these potentially curative therapies can effectively address disease earlier in life – when combined with genetic screening, even before onset. This offers hope for a longer, fuller, better life, avoiding the health system and all the physical, emotional and financial strains lifelong treatment can put on patients and their loved ones. It means less time away from work and school, less reliance on support systems, less disruption to people's lives.

Participants were also seized with the opportunity to establish Canada as a leader in global innovation, capitalizing on our strong research networks and growing manufacturing capacity. Through events like this Symposium, the conversation is coming to the forefront at a moment where Canada is looking to foster home-grown innovation. The CGT space can contribute to economic growth, attracting investment and retaining talent here at home.

Many attendees stressed the opportunity to learn from today's innovation to build tomorrow's healthcare revolution. Each discovery opens new avenues of exploration for therapies, and innovators are working hard to develop off-the-shelf solutions and drive efficiencies that will contribute to decreased costs and wider access.

What Needs to Happen

Plan for delivery early

The delivery of CGTs is complex and highly specialized, meaning the right resources and clinical expertise must be in place. This carries implications for government budgets beyond the cost of reimbursement. Government needs to begin thinking about how to pay for and deliver a therapy well before its health technology assessment (HTA) is complete.

It is important for government officials to engage industry early to receive ~~in~~ pipeline information, and there are other important voices that also need to be at the table. This calls for **early, structured government and regulator engagement with patient groups, academics, clinicians, and industry** when a cell or gene therapy is being readied for the Canadian market. Internally, decision-makers should adopt a whole-of-government approach to better understand resourcing needs and downstream health system savings, which should in turn inform reimbursement decisions (see below). And government could better monitor global developments and innovation, to understand what is in development years before it's brought to Canada.

Moving quickly to assess the full cost and benefits of delivering a therapy – and ensuring adequate health system resources are in place to do so – will expedite decision-making and ensure therapies can be successfully delivered once listed.

Implement a tailored pathway for CGT assessment and reimbursement

CGTs represent long-term, deep investments that carry high risk and high potential; this needs to be considered in assessment and negotiation. Innovators and industry take on considerable risk and investment to develop and trial CGTs.

Many of these treatments, for example bespoke therapies or those targeted at rare disease and oncological conditions, do not support randomized control trials for population size or ethical reasons, and their novelty means there is a lack of long-term data on outcomes. At the same time, existing processes are oncology-focused, even as a growing number of therapies in the pipeline are non-oncological – including some of the most prevalent chronic diseases. While CGTs are resource-intensive interventions, they may result in lower costs over the long term. Governments focused on three- to five-year time horizons, with drug budgets siloed from other health budgets, struggle to understand the long-term costs and benefits of CGTs. This reinforces government's preference for budget certainty over the promise of future savings.

All together, this complexity leads to delays in decision-making and, by extension, access for patients in need. A different kind of approach with different value assessments, supported by real-world evidence, is needed to better evaluate the value of CGTs and get them more quickly to patients and families that need them. This should be paired with innovative approaches to reimbursement that embody multi-party risk sharing.

A CGT-specific pathway beyond today's complex review is needed to address issues unique to these therapies. This pathway should span regulators, HTA bodies, health system regulators, the pCPA and public drug plan managers with **tailored evidence requirements** (for example, single-arm trials plus real-world evidence) and timelines. As noted above, negotiation tables should be broadened to include stakeholders across government to fully capture delivery costs and downstream savings, informing **outcomes-based agreements**. The recently-announced FAST program in Ontario is a good model to expand and replicate elsewhere.

Ensure the patients who can most benefit from therapies are able to access them

It is in the mutual interest of patients, clinicians, government and industry to ensure the patients most likely to benefit from therapies are able to access them. This requires **a national approach to accessing cell and gene therapies built on better inter-provincial collaboration**. The complexity of delivering CGTs limits the number of centres that can offer them. This ensures a high quality of care and clinical expertise, but complicates access, forcing some patients to relocate or travel long distances to access care, sometimes across provincial boundaries. A hub-and-spoke model, with Centres of Excellence integrated with local follow-up care, must develop.

With limited health system resources, provinces hosting regional centres that deliver CGTs are incentivized to treat their own patients first. For some therapies that disproportionately impact a given community, one or two provinces might incur most of the cost of care. And provincial initiatives like the FAST program benefit some Canadians but not all.

Whether moving within a province's borders or across them, government must **provide adequate supports for patients who need to travel or relocate to receive CGTs**. This should: encompass the full patient journey, including out-of-pocket costs before and after receiving a therapy; consider support for families or caregivers where appropriate; and be easy to access. Care access and clinical education is especially important in rural areas so patients can be identified and connected to the right supports.

Diverse populations should be included in clinical trials and health datasets to better understand genomic diversity and the impact of therapies on different communities. Decision-making about access pathways and what patients can best benefit from therapies should also be sensitive to a patient's socioeconomic status, membership in an equity-deserving population, informal caregiver support, and mental wellness, among other facts that impact how much they will benefit from a therapy.

Finally, participants also discussed the promise of point-of-care manufacturing and off-the-shelf therapies to, in time, bring CGTs closer to patients across Canada, while recognizing the technical and financial challenges that may make this a distant possibility. Others proposed a national Centre of Excellence network with harmonized protocols and interprovincial referral agreements, paired with a centralized navigator with a single entry point and unified policy voice, to ease access and level the playing field nationally.

Support awareness and education

Clinical education is important – doctors need the right training (and Canada needs the right mix of specialists) to successfully connect patients to, and deliver, CGTs. Communities of practice can support knowledge-sharing and the development of best practices. And outreach to clinicians should specifically include rural providers.

We also need stronger efforts to **educate and inform patients and caregivers** and to fight misinformation. This should include education to decision-makers and the public about the value of CGTs. Patient charities have a critical role to play; industry and government can do more to support them, so they can better engage and inform their patients. Special attention should be given to equity-deserving communities, many of which have complicated histories with the health care system. Better resourcing will also help patient organizations involve their communities earlier and more broadly in the HTA process.

Foster made-in-Canada innovation and manufacturing

Canada has a strong research ecosystem but needs to be a better place to invest in emerging health technologies or risk losing talent and innovation to more competitive scientific nations. **Governments at all levels must ambitiously increase investment to support research and commercialization of CGTs** to help scale this growing sector; streamlining assessment and reimbursement processes will also help. This will help drive economic growth while retaining CGT talent within our own borders – and attracting it from across the world.

There is an opportunity to **reduce red tape that limits our ability to build manufacturing and research capacity in Canada**. Permitting and bureaucracy slows down domestic manufacturing scale-up; regulations and requirements should be reviewed and adjusted to avoid unnecessarily stifling growth and innovation. Government should explore other ways of driving down the cost of manufacturing, for example by supporting the development of best practices that can facilitate greater efficiency and reliability.

Finally, as discussed in Cate Murray's keynote address, there are important differences between commercially scalable therapies and those that are small-scale. The latter can be challenging to capitalize and to assess. Canada should develop a **national pathway for bespoke point-of-care CGTs that do not have traditional industry sponsors**.

Invest in modernized, shared data systems

Canada needs **to invest in better data infrastructure while dropping barriers to data sharing**. This needs thoughtful data governance that is mindful of risks and issues including cybersecurity, privacy, and Indigenous data sovereignty.

Stronger and more transparent data is foundational to many of the solutions outlined above. More continuous data will allow for better identification of patients who can benefit CGTs. Better collection of real-world evidence will support Canadian research and innovation while helping inform decision-makers as they assess therapies, and track outcomes as therapies are adopted.

The ideal would be a national data platform, a portal which can reach patients regardless of address and would allow clinicians to diagnose, register, trace, track and follow a patient – helping build and support patient pathways to access CGTs.

One other idea that arose at many tables was the need for **a national forum that brings together all the key stakeholders** – industry, patients, clinicians, researchers, and hospital leaders – to engage government with one voice. Patient experience must be at the centre, and clinicians have an important role to play unpacking the issues and impacts.

Calls to Action

Taken together, these themes suggest the following calls to action:

Call to Action 1

Reforming Health Technology Assessment (HTA) and payer models

Early Stakeholder Engagement: There is a need for early, structured government and regulator engagement with patient groups, academics, clinicians, and industry when a cell or gene therapy is being readied for the Canadian market. Additional lead time will help put adequate health system resources in place to deliver CGTs.

Develop CGT-Specific Pathways: Create distinct, agile pathways within HTA bodies (CADTH/INESSS) and pricing negotiations (pCPA) that are tailored to the unique characteristics of CGTs, rather than forcing them into existing frameworks designed for traditional pharmaceuticals. Take a whole-of-government approach to understanding costs and long-term savings associated with CGTs.

Tailored Evidence Requirements: Accept and integrate diverse forms of evidence, such as single-arm trials, real-world evidence (RWE), and patient-reported outcomes, especially for rare diseases where large randomized controlled trials are often impractical.

Implement Outcomes-Based Agreements: Structure payment models where the full cost or a significant portion of the payment is contingent on the therapy achieving predefined clinical outcomes over a specified period, sharing risk between manufacturers and payers.

Call to Action 2

Ensuring patients can equitably access therapies

A national approach to accessing CGTs: Founded on stronger inter-provincial collaboration, ensure patients can access CGTs regardless of postal code. This should include stronger financial supports for patients and families who need to travel or relocate to access these therapies.

Targeted Clinician Education: Implement specialized training and continuous education programs for healthcare professionals across various disciplines to enhance their understanding of CGT indications, referral pathways, and post-treatment management. Ensure rural providers are included in these initiatives.

Support Patient Education: Develop and disseminate clear, accessible educational materials for the general public, patients, and caregivers to counter misinformation, manage expectations, and build trust in CGTs. Better support patient charities as they help their communities understand and engage.

Call to Action 3

Strengthening domestic research, manufacturing and data-sharing

Invest to Support Research and Commercialization: This should include targeted funding and support for the establishment and expansion of Good Manufacturing Practice (GMP) facilities within academic medical centres and specialized hospitals while creating a national pathway for commercially non-viable therapies.

Streamline Regulatory Processes for Facilities: Reduce bureaucratic hurdles and expedite permitting processes for the establishment and operation of CGT manufacturing and research facilities to accelerate capacity growth.

Invest in Better Data Infrastructure: At the same time, drop barriers to data-sharing and ensure diverse populations are captured in datasets. This will facilitate many of the proposals above, to better assess the value of CGTs, identify people who can benefit from them, and track patient outcomes.

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Conclusion

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Canada has a long history of medical innovation, strong research infrastructure, and the opportunity to become a global leader in the next healthcare revolution. With smart collaboration, investment, and flexibility, we can forge the infrastructure we need to bring CGTs to our country, the health system expertise and capacity to deliver them, and the business and innovation environment to support innovation and manufacturing here at home.

This is a nation-building project. Its success will bring life-changing therapies to thousands of Canadians. The time to act has come – and it's important we get it right.

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