Stem Cell Network | Réseau de Cellules Souches

Workshop Series

Preparing Canada's Health System for Regenerative Medicines and Advanced Therapeutics: Cell Therapies for Type 1 Diabetes

in collaboration with:

Breakthrough T1D™ Formerly JDRF

Shift HEALTH

Additional Partners and Sponsors

IHE INSTITUTE OF HEALTH ECONOMICS ALBERTA CANADA

VERTEX

INNOVATIVE MEDICINES CANADA

June 25-26, 2025 – Ottawa, Canada

Executive Summary (1/2)

Sustained investment in research and discovery has positioned Canada as a leader in regenerative medicine—a broad field focused on repairing, replacing, and regenerating cells, tissues, and organs. Within this field, advanced therapeutics, including cell and gene therapies (CGTs) and tissue-engineered products, are emerging as transformative treatments for a range of diseases. To realize the full health, economic and social benefit of CGTs, Canada must ensure that its regulatory, reimbursement, and implementation pathways support adoption, patient access, and long-term system sustainability.

To address this need, SCN is leading a series of cross-sector workshops aimed at identifying and overcoming key regulatory, reimbursement, and implementation barriers that affect the adoption of advanced therapeutics—particularly CGTs—within Canada's healthcare system. On June 25-26, 2025, SCN partnered with Breakthrough T1D to convene over 30 cross-sector experts in the first workshop of the series, focused on advancing late-stage cell therapies for type 1 diabetes (T1D). Given Canada's history of diabetes research breakthroughs and broader expertise in regenerative medicine, a focus on T1D was a natural first step. Indeed, Canada is at the forefront of developing T1D cell therapies with the potential to deliver transformative treatments that move beyond insulin-based management and alleviate the relentless daily burden of this chronic condition.

The workshop had three objectives: 1) identify key access barriers for T1D cell therapies in Canada, 2) co-develop actionable recommendations to address these barriers, and 3) engage diverse experts to foster new conversations and collaborations. Discussions were organized into five focus areas: translational research; regulatory and HTA; risk-sharing and reimbursement; implementation and adoption; and misinformation and disinformation. *Key takeaways from each focus area are summarized on the next slide.*

Executive Summary (2/2)

Focus Area	Top-Identified Barriers	Potential Solutions *(bold=spotlight solution)*
1 Translational Research	Inadequate funding, limited support for clinical-to-commercial transition, and insufficient expertise in manufacturing standards impede the translation of discovery research to patient treatment.	Strengthen translational research for CGTs in Canada by establishing a concierge support service, building a national manufacturing roadmap with an expert network, and **articulating the full value of CGTs to attract greater funding support**.
2 Regulatory and Health Technology Assessment	Lack of awareness in regulatory and HTA processes, limited CGT value models beyond clinical metrics, and insufficient input on unmet needs from people living with T1D make CGT approval and evaluation challenging.	**Develop educational and navigational concierge for CGT innovators**, partner with T1D community to capture real-world and health-related quality of life (HRQoL) outcomes and engage T1D community to inform trial design and evaluation frameworks.
3 Risk-sharing and Reimbursement	Misaligned understanding of CGT benefits and risks, traditional reimbursement models unsuited for one-time high-cost therapies and limited RWE infrastructure for CGTs hinder value assessment and pricing.	**Engage payers strategically to build shared understanding of CGT impact,** explore novel reimbursement models (e.g. outcome-based agreements), and leverage existing Canadian infrastructure for gathering standardized longitudinal RWE.
4 Implementation and Adoption	Limited CGT delivery sites with advanced capabilities, insufficient training for care teams, and low awareness among T1D community and patient orgs restrict access and effective implementation for upcoming T1D CGTs.	**Establish strategically located CGT centres of excellence,** create educational programs to build healthcare provider expertise, and develop a network of trusted ambassadors to communicate CGT information starting with T1D communities.
5 Health Misinformation and Disinformation	Limited public awareness of reliable CGT information and insufficient understanding of the need and opportunity for improved therapies, including for T1D, hinder informed community engagement.	**Develop a multi-audience awareness campaign on the value of CGTs for T1D** and bridge knowledge gaps through targeted education and existing resources.

Realizing the full potential of these homegrown innovations requires a sustained commitment to strengthening every stage of the CGT innovation pathway. By acting on the solutions identified for top priority barriers, Canada can maximize its ROI in CGTs, expand treatment choices for patients and advance its aspiration to become the world's leading life sciences and research hub.

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Context:

Current Understanding of CGTs and T1D Landscape in Canada

Canada's Leadership in Regenerative Medicine and Advanced Therapeutics

Through substantial and sustained investment in research and discovery, Canada has become a global leader in advanced therapeutics, including cell and gene therapies (CGTs) and regenerative medicine.

Canada is globally recognized for regenerative medicine research

In both 2020 and 2025, Canada was among the **Top 3**[1] in the world in the stem cell and regenerative medicine field[2]

Canada has made significant investments in regenerative medicine

$160M

Has been invested by the **Government of Canada** through SCN into research and clinical trials over the last two decades.

Canada can capture a significant share of the US regenerative medicine market

$14B

In **economic value** to be realized for Canada by 2032[3] if the required ambition and conditions are in place

1. Bibliometric Analysis of Publication Results from Stem Cell Network Supported Research & Survey Results of International Regenerative Medicine Experts; report commissioned by SCN
2. Canada remains most known for "Discovery/Basic Science" and "Pre-translation and Translational" research activities, with recognition growing in "Clinical Trials Activity" and "Knowledge Mobilization"
3. Canadian market size assumes ~10% of US market size as reported by Custom Market Insights (September 2023) and SCN's Strategic Plan: Accelerate 2025-2029

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The Burden and Reality of Type 1 Diabetes (T1D)

There is an urgent unmet need for transformative T1D therapies that go beyond current insulin-based treatments to address the relentless daily burden of this chronic disease for individuals.

The Unmet Need in T1D

~300K

Canada has one of the world's highest rates of **T1D, a relentless, life-altering chronic disease** with enormous health, economic and social impact

Current treatments remain insufficient. Individuals continue to face the daily physical and mental burden of insulin administration, glucose monitoring and constant vigilance to avoid hypo- and hyperglycemia

Key Priorities for Novel T1D Therapies

Surveys and real-world evidence studies have demonstrated the following as key priorities for novel therapies in the T1D community:

- Reducing mental effort
- Simplifying management
- Reducing or eliminating the need for insulin therapy
- Achieving improved glycemic outcomes without added risks
- Reducing or eliminating the risk of hypoglycemia and long-term complications

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A Closer Look: Canada's Leadership in T1D

Canada's legacy of diabetes research breakthroughs has positioned the nation as a global leader in cell therapies that address key priorities and deliver meaningful benefits for the T1D community.

Current State of T1D Cell Therapies

- **T1D is one prominent example of Canada's leadership** in cell therapies[1], a class of advanced therapeutics, and associated technologies
- **CGTs represent a new era of hope for T1D** as pluripotent stem cells can be reliably differentiated into insulin-producing β-like islet cells
- Trials show stem cell–derived islet therapies **restore insulin production and reduce/eliminate the need for injections**
- Several Canadian sites are involved in **Phase 3 trials**, and it is anticipated that market-ready products will be **available within the next three years**
- Cell therapies for T1D are no longer theoretical—they are becoming **tangible solutions poised to transform T1D care**

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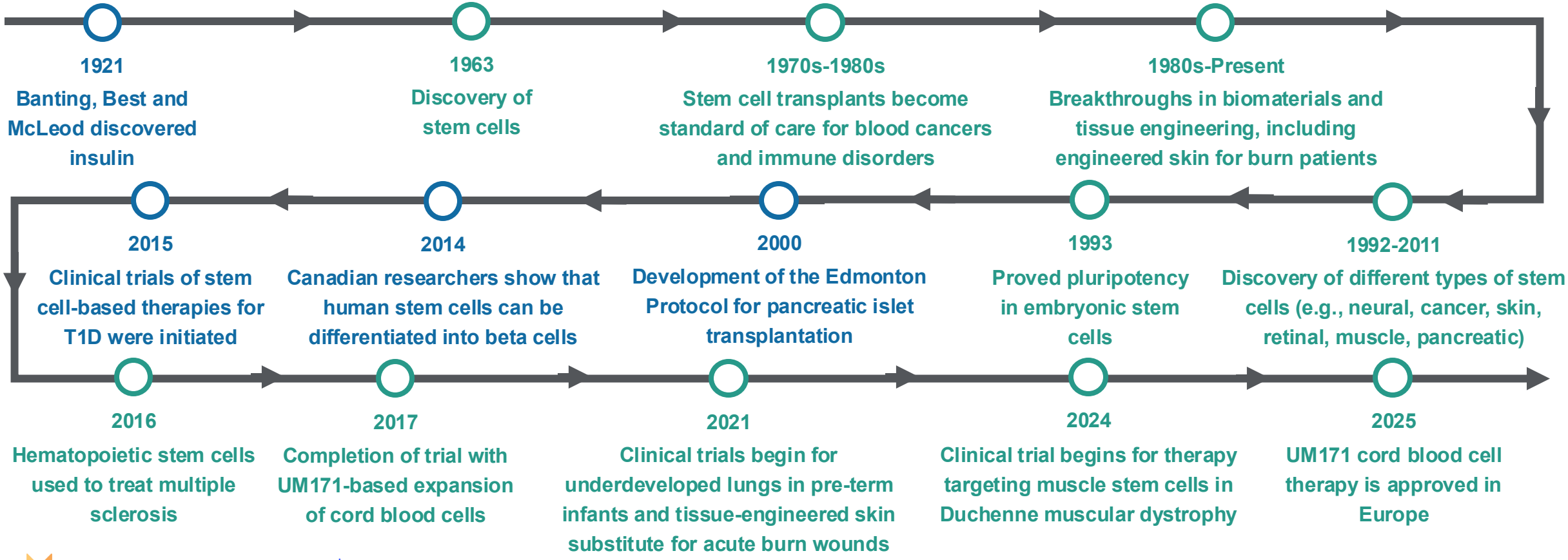
[1] Cell therapies are a notable class of advanced therapeutics with the potential to enable tissue and organ repair or deliver durable treatment for diseases such as T1D, neurodegenerative diseases, rare disorders, and certain cancers.

Canadian Discoveries and Contributions in Regenerative Medicine and T1D

Over the last 100 years, Canadian researchers have made significant contributions to regenerative medicine and T1D research, solidifying Canada's leadership in both fields.



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Discoveries in T1D

Discoveries in regenerative medicine

Seizing Canada's Opportunity in Regenerative Medicine and Advanced Therapeutics

As regenerative medicine and advanced therapeutics move toward clinical use and commercialization, Canada must modernize its regulatory, assessment and adoption frameworks and streamline its innovation pathway to turn domestic research excellence into timely patient access, improved health outcomes, and economic growth.

*"When we invest in science, research and innovation, **we invest in the success of Canadians and in the future of our communities**. Our government is committed to **supporting talented researchers and visionary entrepreneurs** to help drive economic growth."*

Minister of Industry and Ministry Responsible for Canada Economic Development for Quebec Regions, Sept 5, 2025

To achieve this, Canada must:

- **Prepare for next-generation therapies**, offering patients long-awaited treatment choices while advancing Canada's position as a global leader in research, biomanufacturing, and health innovation
- **Identify and prioritize barriers to access**, building an end-to-end innovation pathway that reflects the complexities of advanced therapeutics and maximizes the return on health research investments
- **Co-develop solutions with patients**, to ensure these therapies are implemented in alignment with unmet needs as defined by those they are intended to serve

To this end, SCN is leading a series of cross-sector workshops focused on identifying and addressing key regulatory, reimbursement and implementation barriers so that advanced therapeutics—specifically CGTs—are accessible and adopted by Canada's healthcare system.

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Overview of Workshop Methodology and Approach

On June 25 and 26, 2025, SCN and Breakthrough T1D hosted the first workshop in this series focused on late-stage cell therapies for T1D in Ottawa, Ontario.

Key Objectives for the Workshop

1. Identify key access barriers for T1D cell therapies in Canada
2. Develop actionable recommendations for identified barriers
3. Engage cross-sector experts and foster collaborations

Structure of the Workshop

Pre-Engagement

- Pre-event survey to identify barriers for advancing a hypothetical T1D cell therapy and CGTs
- Pre-read[1] on key enablers and barriers for CGTs developed in the current Canadian landscape

Workshop and Discussion

Level Setting

- Expert presentations to level-set participants for each dedicated focus area

Barrier Validation and Prioritization

- Participant polling to achieve consensus on top barriers to tackle for each focus area

Solution Ideation

- Breakout discussions to explore solutions for each top barrier and a deeper characterization of a selected solution

Report Development

- Post-tabletop report that synthesizes key takeaways on barriers, solutions and their impact for the T1D community and for CGTs more broadly

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Detailed workshop agenda and icebreaker results can be found in the Appendix

1. The pre-read, developed in collaboration with the Institute of Health Economics, provides a snapshot of key barriers and is not intended to be comprehensive

Partners and Contributors

The workshop convened over 30 cross-sector experts—including individuals with lived experience—from SCN's and BT1D's networks across Canada's health, research and innovation ecosystem.

Participating Organizations

- Academia and Research Institutes
- Government and Health Systems
- Industry
- Non-Profit and Patient Organizations

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Five Focus Areas of the Workshop

The workshop explored five focus areas along the innovation pathway for T1D CGTs and their associated barriers that can limit or delay life-transforming therapies from reaching individuals with T1D.

	Translational Research	Regulatory and Health Technology Assessment	Risk-sharing and Reimbursement	Implementation and Adoption	Health Misinformation and Disinformation
Definition	Moves scientific discoveries into clinical applications to deliver benefits to patients (Bench-to-Bedside)	Ensures new products are safe, effective and deliver meaningful benefits to patients and health systems	Addresses financial risk for therapies with uncertain outcomes and ensures equitable patient coverage	Supports patient uptake of approved therapies through the health system in the most equitable manner	Challenges the trust in science, fuels stigma and ultimately delays access and uptake of new therapies
Challenge	Limited research support and resources hinder the progression of therapies from discovery to clinic	Low regulatory and HTA awareness, limited CGT value models, and minimal patient input hinder timely evaluation and approval	Current mechanisms cannot absorb one-time, high-cost therapies, threatening sustainability	Gaps in infrastructure, capabilities and care pathways may hinder effective implementation and adoption of CGTs	Misinformation and/or disinformation may weaken public confidence in CGTs, complicating adoption and access efforts

Addressing these challenges will unleash Canadian innovation, strengthen global competitiveness, and create more equitable access to CGTs—ultimately enabling individuals with T1D to benefit from novel therapies that may deliver transformative health outcomes and relieve the individual and health system burden of this chronic disease.

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Click on one of the focus areas to jump to that section

Summary of Key Takeaways:
Top Barriers and Solutions Identified Across Focus Areas

Focus Area 1: Translational Research

What We Know

Translational research is the **bridge that moves CGTs from lab discoveries to patient treatments** (e.g. preclinical development, manufacturing and process development, translational biomarker and assay development, clinical development). **Without a robust translational pipeline, patients are limited to existing options and promising innovations may never reach the market.**

What Needs to Change	Potential High Impact Solutions
1 **Insufficient support** for transitioning CGTs from clinical to commercial stages	**Create a non-profit concierge service** to support and guide Canadian entrepreneurs through the systems underpinning research translation
2 **Lack of clarity around manufacturing standards** for CGTs in Canada	Engage with a network of manufacturing experts to **develop a Canadian manufacturing roadmap** (e.g., accepted manufacturing standards, education) specific to CGTs
3 **Inadequate funding to support** translation of research into products that can be offered in the clinic or to commercial markets	**Articulate a comprehensive value proposition of CGTs—** capturing their potential to deliver durable treatment and cure, offsetting upfront costs—to increase research funding

See Solution Spotlight

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GMP: Good manufacturing standards
Additional barriers and solutions for translational research in the Appendix

Translational Research – Solution Spotlight

Solution & Aim

A comprehensive and compelling value proposition of a first-in-class CGT for disease such at T1D in Canada is required to drive greater funding support for translational research including clinical trials and research infrastructure.

Key Activities & Enablers

1. **Engage with partner organizations** (e.g. CDA-AMC to convene Health Canada, ISED, patient partners, health economic experts, relevant non-profits and/or funders, and government relations specialists) **to align on objectives, data inputs, outputs and overall narrative** of the value proposition.
2. **Design a value proposition framework capturing T1D CGTs' clinical, socio-economic, HRQoL impacts and indirect impacts on the Canadian health system to guide investment and uptake decisions.** Elements of the framework to include: unmet need and market opportunity, scientific and clinical rationale, CGTs' unique value within the T1D therapeutic landscape, translational gaps, near-term health, social and economic impacts, and the therapy's broader value across diverse patient populations and other disease indications.

Anticipated Outcomes & Impact

A clear and compelling value proposition for CGTs, starting with T1Ds, will provide researchers and developers with the evidence needed to **strengthen public and political support, secure infrastructure funding to translate current and future therapies into the clinic and inform downstream reimbursement and pricing decisions** that enable equitable patient access.

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CDA-AMC: Canada Drug Agency
ISED: Innovation, Science and Economic Development Canada

Focus Area 2: Regulatory and HTA Assessment

What We Know

Regulatory and health technology assessment (HTA) processes are critical steps for **ensuring new therapies are safe, effective, and deliver meaningful value** to patients and health systems. When frameworks **fail to adapt to the unique characteristics** of advanced therapeutics and CGTs, **access to appropriate patients may be compromised.**

What Needs to Change

1. **Lack of awareness and support around the regulatory and HTA process** make CGT approval processes unnecessarily complex and difficult to navigate
2. **Lack of economic models** that accurately capture impact of CGTs beyond clinical metrics for people with T1D and other diseases that can be addressed through CGTs
3. Evaluation of **unmet needs of eligible populations** for new treatments like CGTs are frequently **defined without adequate input** from people with the relevant diseases

Potential High Impact Solutions

Create a non-profit concierge service to support and guide Canadian entrepreneurs through the systems underpinning research translation, regulatory and HTA assessment

See Solution Spotlight

Patient partners, non-profits and advocacy organizations should partner with sponsors, regulators, and others, to **capture the transformative real-world value of CGTs** for diverse patient populations

Engage patients to develop relevant and realistic CGT trial designs and evaluation frameworks, starting with individuals living with T1D

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Additional barriers and solutions for translational research in the Appendix

Regulatory and HTA – Solution Spotlight

Solution & Aim

A concierge service and educational program for patients, clinicians, researchers and manufacturers to support navigation—and build understanding—of Canada's regulatory and HTA processes for CGTs across therapeutic areas including T1D.

Key Activities & Enablers

1. **Collaborate with funders** to secure sustainable financial resources to launch a non-profit concierge service for key players navigating regulatory and HTA processes, develop information/resources and provide engagement sessions for CGT innovation experts.
2. **Partner with educational specialists, innovators** (e.g., researchers, manufacturers) **and regulatory experts** (i.e., Health Canada, CDA-AMC, INESSS) to develop and deliver comprehensive, accessible educational resources that support the navigation of existing regulatory and HTA pathways (e.g., Advanced Therapeutics Products) for CGTs.
3. **Facilitate learning sessions for CGT innovation experts** (e.g., researchers, manufacturers, clinicians, patients) to understand the clinical, scientific, and patient considerations (e.g., efficacy and safety data, manufacturing standards, quality of life impacts, and impact of patient, caregivers, and the health system) that drive regulatory and HTA decision-making.

Anticipated Outcomes & Impact

Comprehensive educational resources and supports delivered by a system concierge will **improve understanding of Canada's CGT regulatory and HTA processes, enabling more efficient approvals, faster patient access and better health outcomes**.

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Focus Area 3: Risk-Sharing and Reimbursement

What We Know

Effective risk-sharing and reimbursement mechanisms **for high-cost, high-impact therapies (e.g. T1D CGTs) help de-risk innovation and enable patient access**. Without these mechanisms, the full financial burden falls on payers (e.g. public insurers, private insurers, or patients) and severely restricts access for individuals living with illnesses, such as T1D, that can be addressed through regenerative medicine.

What Needs to Change	Potential High Impact Solutions
1 **Lack of shared understanding of benefits and risks** of CGTs among payers and manufacturers results in misalignment on value assessment and pricing decisions	**Engage payers at the right level and time** so that they can plan for budgetary cycles, be ready to support adoption and have a strong understanding of full value of CGTs
2 **Traditional reimbursement** models are not designed to handle one-time therapies (e.g. CGTs) with high upfront cost	**Explore novel reimbursement models**, such as an outcomes-based agreements or social-impact bond model for CGTs
3 **Limited capacity and infrastructure** to track and generate real-world evidence on long-term outcomes of CGTs after reimbursement	**Leverage existing Canadian infrastructure** (e.g. CIHI, CNODES, ICES) to support standardized longitudinal RWE generation for approved CGTs to build trust among payers

See Solution Spotlight

Additional barriers and solutions for translational research in the Appendix
CIHI: Canadian Institute for Health Information; CNODES: Canadian Network for Observational Drug Effect Studies; ICES: Institute for Clinical Evaluative Sciences

Risk-Sharing and Reimbursement – Solution Spotlight

Solution & Aim

A strategic payer engagement plan that sets clear engagement timelines[1], integrates patient insights, and seeks alignment on T1D needs and value of CGTs, will build shared understanding between payers and manufacturers, support budget planning and reduce reimbursement uncertainty.

Key Activities & Enablers

1. **Facilitate cross-sector collaborative workshops** bringing together patients, academia, industry, and payers to **identify optimal payer engagement strategies and establish critical evidence requirements** for reimbursement decisions
2. Leverage goals outlined in the engagement plan and a compelling value proposition for T1D CGTs (see slide 14) to **create tailored communication materials targeting payers and decision-makers** that clearly demonstrate T1D unmet needs (e.g., psychosocial and emotional well-being, fear of hypoglycemia, social limitations) beyond clinical metrics and articulate a comprehensive risk-benefit profile of CGTs

Anticipated Outcomes & Impact

The development of a payer engagement plan will equip CGT innovators and manufacturers in Canada with **clear guidance and greater confidence in reimbursement prospects** and foster **a clear understanding of CGTs' benefits, risks, and transformative potential for people with T1D,** allowing organizations like CDA to systematically integrate socio-economic costs and benefits into decision-making.

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1. It is recommended to engage payers approx. 2 years before a decision is needed

Focus Area 4: Implementation and Adoption

What We Know

Implementation and adoption ensure approved CGTs are **effectively integrated into healthcare systems to deliver health and socio-economic value**. Without effective implementation strategies (e.g. infrastructure, training/education, capacity), near-term CGTs, including Canadian treatments for T1D, may fail to reach eligible individuals, undermining investment in Canadian innovation and perpetuating health inequities due to geographic disparities and financial barriers, particularly affecting vulnerable groups.

What Needs to Change	Potential High Impact Solutions
1 **Lack of CGT delivery sites** with advanced capabilities (e.g. cold storage, transplant expertise and post-treatment care), particularly in rural and remote areas	**Establish strategically located centres of excellence,** which may have integrated, pre-approved manufacturing and delivery systems capable of serving multiple provinces[1] *See Solution Spotlight*
2 **Limited training/education** for community care providers/teams supporting patients receiving T1D CGTs	**Create educational programs** (e.g., curricula integration, fellowships and practitioner resources) to build healthcare provider expertise and confidence in T1D CGTs
3 **Insufficient awareness and knowledge** of CGTs among individuals living with T1D and patient organizations providing support (e.g. emotional support, resource-sharing of CGTs)	**Build a network of trained and trusted ambassadors** (e.g., on social media) to translate complex CGT information into appropriate content for different T1D communities

Additional barriers and solutions for translational research in the Appendix

1. Depending on the type of advanced therapy or CGT, manufacturing capabilities may be centralized—outside of the centres of excellence, as in the case of T1D—or decentralized.

Implementation and Adoption – Solution Spotlight

Solution & Aim

A network of centres of excellence specializing in T1D CGTs with trained diabetes care teams should be established. With the goal of revising the current system, a centralized, national patient registry is envisioned that will ensure the network enables pan-provincial access and coordination of pre-/post-transplant care across sites and through satellite referral pathways.

Key Activities & Enablers

1. **Establish a pan-Canadian cross-sector oversight network** to develop and monitor performance standards (e.g. manufacturing standards, care protocols) that ensure centres of excellence meet regulatory and quality requirements.
2. **Strategically select centre of excellence locations** (based on track record of T1D CGTs expertise, transplant experience and existing/available infrastructure) to ensure equitable access across all provinces for individuals living with T1D.
3. **Develop secure data-sharing mechanisms as part of the satellite referral pathways** between centres of excellence and with community care providers to enable seamless patient information exchange, coordinated care planning, and real-world evidence collection for T1D CGT outcomes.

Anticipated Outcomes & Impact

Specialized T1D CGT centres of excellence will **enable improved access to CGTs—including Canadian innovations—across the country, with the ambition of incorporating a centralized patient registry, alongside standardized care protocols and enhanced resource coordination,** to ensure timely treatment delivery, consistent care and contribution to a resilient biotech sector.

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Focus Area 5: Health Misinformation and Disinformation

What We Know

Health misinformation (unintentionally false information) and disinformation (deliberately deceptive content) both **undermine patient access to appropriate treatments by eroding trust in science and healthcare**. In the T1D context, these false narratives misrepresent disease burden, fuel harmful misconceptions like confusion with T2D or patient blame and distort perceptions of the safety and effectiveness of emerging cell therapies (e.g., cure narrative, minimal side-effects or post-treatment sequelae). Misinformation ultimately **discourages timely care-seeking and can reduce patient acceptance of innovative treatments**.

What Needs to Change

1. **Lack of public awareness of reliable information sources** (e.g. SCN, Health Canada, Diabetes Canada) on T1D CGTs

2. **Lack of broad understanding of the need and opportunity** for better and/or intensive therapies for T1D (e.g. risk-benefit profile is not sufficiently compelling for payers)

Potential High Impact Solutions

Develop a multi-pronged, multi-audience awareness campaign about the value of a transformational treatment, such as CGTs for T1D

See Solution Spotlight

Bridge CGT knowledge gaps by leveraging existing resources and providing targeted education on clinical implementation

Additional barriers and solutions for translational research in the Appendix

Misinformation and Disinformation – Solution Spotlight

Solution & Aim

A multi-audience awareness campaign on the transformative value of T1D CGTs that highlights clear comparisons with current standard of care is needed to circulate accurate information and build support for CGT adoption.

Key Activities & Enablers

1. **Establish a cross-sector working group** comprising patient partners, academic institutions, healthcare professionals, industry partners, communications specialists, patient organizations and philanthropists to oversee, design and fund the awareness campaign (e.g. define target audiences, develop impactful and accurate messages, establish campaign metrics, coordinate content delivery).
2. **Develop multi-channel outreach approaches** specifically designed to engage key populations and underserved communities, ensuring equitable access to accurate T1D CGT information through trusted local networks and culturally appropriate materials.

Anticipated Outcomes & Impact

A comprehensive T1D awareness campaign will **increase recognition of the disease burden of T1D and CGTs' transformative potential, drive a paradigm shift from symptom management to potentially curative treatments, build support** for CGT adoption and **accelerate access**.

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Closing

Canada has a strong track record in regenerative medicine research and discoveries and is a global leader in developing CGTs with the potential to transform care for numerous diseases, including T1D. Realizing the full potential of these homegrown innovations will require a sustained commitment to strengthening every stage of the CGT innovation pathway.

By acting on the solutions identified for top priority barriers, Canada can maximize its return on investment in CGTs, expand treatment choices for patients, build a resilient biotech sector and become a world leading life sciences and research hub.

Appendix

Workshop Agenda – Day 1

Time	Topic		Speakers
9:00-9:15	**Icebreaker, Welcome and Introduction**		• Cate Murray, Stem Cell Network • Dr. Sarah Linklater, Breakthrough T1D
9:15-9:45	**Overview of Scoping Paper**		• Dr. Kate Harback, Institute of Health Economics
9:45-11:20	**T1D Cellular Therapy**		• Olivia Kutchkoskie, person with lived experience of T1D • Linxi Mytkolli, Diabetes Action Canada & person with lived experience of T1D • Alex St. John, IRCM & person with lived experience of T1D • Dr. James Shapiro, University of Alberta • Dr. David Thompson, University of British Columbia • Dr. Trevor Reichman, University of Toronto
11:20-12:00	**Lunch and Networking**		
12:00-1:55	**Introduction to Working Sessions 1 and 2**		• Omar Tounekti, Health Canada • Dr. Farah Husein, Canada's Drug Agency • Susan Marlin, Clinical Trials Ontario • Dr. Bruce Verchere, University of British Columbia • Dr. Sowmya Viswanathan, University Health Network
1:55-2:55	**Working Session 1: Translational Research**	**Working Session 2: Regulatory & HTA**	• Shift Health
2:55-3:10	**Break**		
3:10-4:20	**Plenary**		• Shift Health
4:20-4:30	**Summary of Day 1 and Closing**		• Cate Murray, Stem Cell Network • Dr. Sarah Linklater, Breakthrough T1D

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Workshop Agenda – Day 2

Time	Topic		Speakers
8:00-8:15	**Welcome**		Cate Murray, Stem Cell Network Dr. Sarah Linklater, Breakthrough T1D
8:15-9:10	**Introduction to Working Sessions 3 and 4: Risk-Sharing and Reimbursement/Implementation and Adoption**		Graham Statt, District of Summerland Dr. Rebeccah Marsh, Institute of Health Economics
9:10-10:10	**Working Session 3: Risk-Sharing and Reimbursement**	**Working Session 4: Implementation and Adoption**	Shift Health
10:10-10:20	**Break**		
10:20-10:50	**Plenary**		Shift Health
10:50-11:40	**Introduction to Working Session 5: Misinformation and Disinformation**		Dr. Krishana Sankar, Canadian Association of Science Centers Linxi Mytkolli, Diabetes Action Canada & person with lived experience of T1D Alex St. John, IRCM & person with lived experience of T1D
11:40-12:15	**Lunch and Networking**		
12:15-1:35	**Working Session 5: Health Misinformation and Disinformation**		Shift Health
1:35-1:50	**Break**		
1:50-3:15	**Refining Top Solutions**		Shift Health
3:15-3:30	**Closing and Next Steps**		Cate Murray, Stem Cell Network Dr. Sarah Linklater, Breakthrough T1D

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Icebreaker Results: Word Cloud

Figure 1: Word Cloud Generated from the Icebreaker Exercise. 31 participants answered the icebreaker question: In one word, what's your hope for cell and gene therapies in T1D? "Access", "Cure", "Hope" and "Equity" were common words chosen by participants.

Barriers for Translational Research

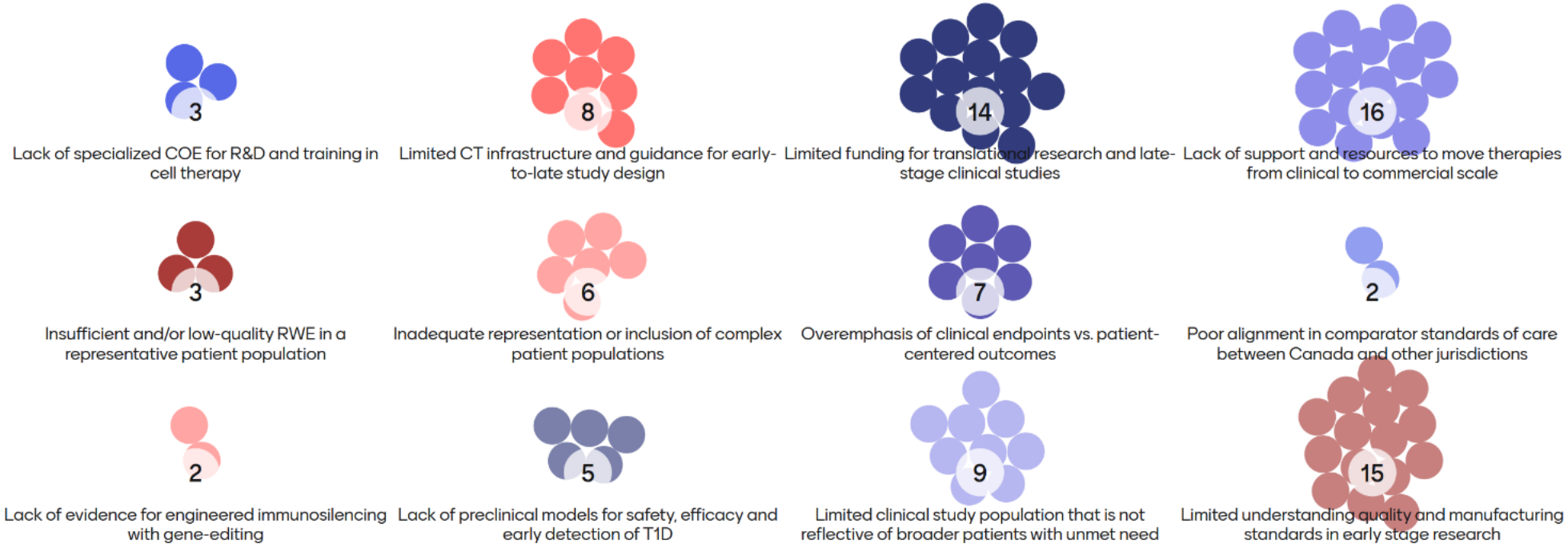

Figure 2: Prioritization results for translational research barriers. Results from a live poll where 33 participants each cast up to three votes to prioritize 12 key barriers to T1D cell therapy translational research. Total votes are shown above each barrier, where one vote is represented by an individual dot.

Recommended Solutions for Translational Research

Barrier 1: Lack of support and resources for T1D-specific therapies transitioning from clinical to commercial scale	**Offer tax incentives to companies using Canadian infrastructure (e.g. manufacturing, CROs etc.) and/or require funding recipients to use Canadian infrastructure**
	Create a non-profit concierge service to support and guide Canadian entrepreneurs through the systems underpinning research translation
	Provide support to GMP facilities for operational activities to maintain readiness, expertise and essential experience
	Exploit current resources, such as automated manufacturing infrastructure to reduce costs for scaled-up production
	Invest in talent development and retention
	Establish a centre of excellence for CGT manufacturing (e.g. Moffat Centre) and embed training as an incentive for growth
	Develop a playbook for researchers on how to protect ideas and form a company and/or secure an attractive licensing deal
Barrier 2: Limited understanding of quality and manufacturing standards in early-stage research	**Tie relevant funding decisions to demonstrated knowledge and application of considerations for quality and manufacturing standards**
	Enhance support to engage an expanded network of experts in manufacturing
	Develop a manufacturing roadmap for researchers and industry, including a consensus on accepted quality and manufacturing standards and timelines for engagement with regulatory agencies
	Provide education and incentives for collaboration with manufacturing experts and regulators at the early stages of product development planning to prevent waste and resource exhaustion; determine a threshold for triggering GMP considerations
	Hire extant talent to bring in expertise and expedite the process
	Convene experts and companies working in this space to achieve consensus on quality and manufacturing standards, and engage regulators to ensure these are aligned with policy
Barrier 3: Limited funding for translational research and late-stage clinical studies	**Articulate a comprehensive value proposition of CGTs—capturing their potential to deliver durable treatment and cure, offsetting upfront costs—to increase research funding**
	Increase resources for funding organizations and agencies
	Academic grants with non-dilutive capital to build portfolio
	Incentivize clinician support for participation in clinical trials; the current billing model disincentivizes clinical service at this stage

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Solutions in light blue boxes were noted as most impactful by workshop participants

Barriers for Regulatory and HTA Processes

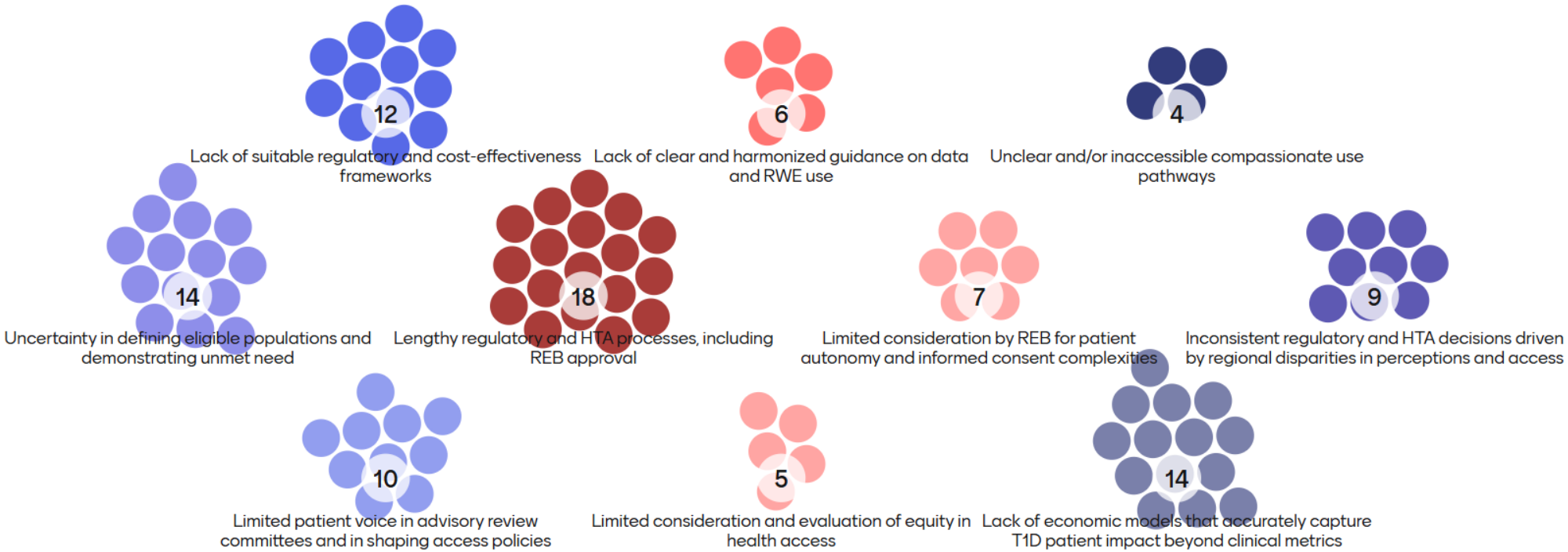

Figure 3: Prioritization results for regulatory and HTA barriers. Results from a live poll where 34 participants each cast up to three votes to prioritize 10 key barriers to regulatory processes and health technology assessment (HTA) for T1D cell therapies. Total votes are shown above each barrier, where one vote is represented by an individual dot.

Recommended Solutions for Regulatory and HTA

Barrier 1: Lengthy regulatory and HTA processes, including Research Ethics Board (REB) approval for cell and gene therapy research and clinical studies	**Educate patients, clinicians, researchers on the regulatory process to drive collective advocacy on improvements to existing regulatory pathways**
	Enhance navigation support for innovators to create a seamless experience across the regulatory ecosystem; consider a formal "screening step" prior to pre-submission meetings with regulatory or HTA bodies to identify potential roadblocks early
	Develop an expert-driven, CGT-specific pathway starting with Health Canada and onwards to facilitate early engagement, reduce downstream delays, and align on data needs to support conditional funding and/or risk-sharing
	Form a consortium focused on enhancing access pathways and facilitating the entry of cell therapies into diverse global markets; leverage international regulatory collaborations to reduce redundancies
	Establish a national concierge or ambassador model to help manufacturers understand the regulatory and HTA ecosystem; clarify process steps and decision makers, provide tailored advice, connect manufacturers to the right agency contacts at the right time
	Promote centralized coordination across federal and provincial government bodies to align timelines and improve transparency—addressing the knowledge gap for innovators outside the system
	Develop and leverage AI to accelerate regulatory processes
	Revise the Notice of Compliance (NoC) framework to make it more efficient, transparent, and attractive to manufacturers seeking market entry
Barrier 2: Lack of economic models that accurately capture T1D patient impact beyond clinical metrics (e.g., QoL, treatment burden, work disruption)	**Engage with patients early-on in industry and HTA economic modelling process to ensure QALYs are tailored to diverse T1D population and accurately reflect the innovation's economic value to patients and systems**
	Although methods exist for capturing multi-dimensional impacts that are patient-centered, greater awareness, communication, and transparency is required to ensure confidence in decision-making
	Support innovation ecosystem through scenario-based budgeting; advocate for innovative reimbursement mechanisms (e.g. milestone payments, outcomes-based agreements) that spread costs and align incentives
	Position cell therapies as part of a broader therapeutic ecosystem, emphasizing their role in supporting personalized care and realistic budgeting rather than serving as a universal solution for T1D
	Consider the adoption of an agreed-upon severity-based modifier to help guide reimbursement thresholds, especially for high-burden chronic diseases like T1D to support greater flexibility in willingness-to-pay thresholds
	Establish a clear and shared definition of value across key players—including payers, clinicians, and patients—to ensure alignment in decision-making and evaluation frameworks

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Solutions in light teal boxes were noted as most impactful by workshop participants

Recommended Solutions for Regulatory and HTA

Barrier 3: Uncertainty in defining eligible populations and demonstrating unmet need due to availability of existing treatments for T1D	**Build clinical, scientific and patient consensus to inform HC/HTA bodies; inputs could include collaborative clinical trial designs (with redefined eligibility criteria that better reflect T1D heterogeneity), patient preference studies and expanded evaluation frameworks that reflect the multidimensional impact of T1D**
	Engage in international collaboration to align definitions of high-risk populations and unmet need; regulatory bodies often look for documented consensus—this can be proactively built and submitted
	Incorporate patient input early in clinical trial design, including during the protocol development phase, to align on real-world definitions of burden and unmet need; ensure patient preferences and values are captured and integrated into pre-submission meetings and health technology assessments
	Revisit the use of data from Special Access Programs (SAPs) in Canada, where such data currently cannot be included in regulatory submissions, and consider advocating for policy changes that would allow observational or SAP-collected data to contribute to evidence packages, as is permitted in other jurisdictions

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Solutions in light teal boxes were noted as most impactful by workshop participants

Barriers for Risk-Sharing and Reimbursement

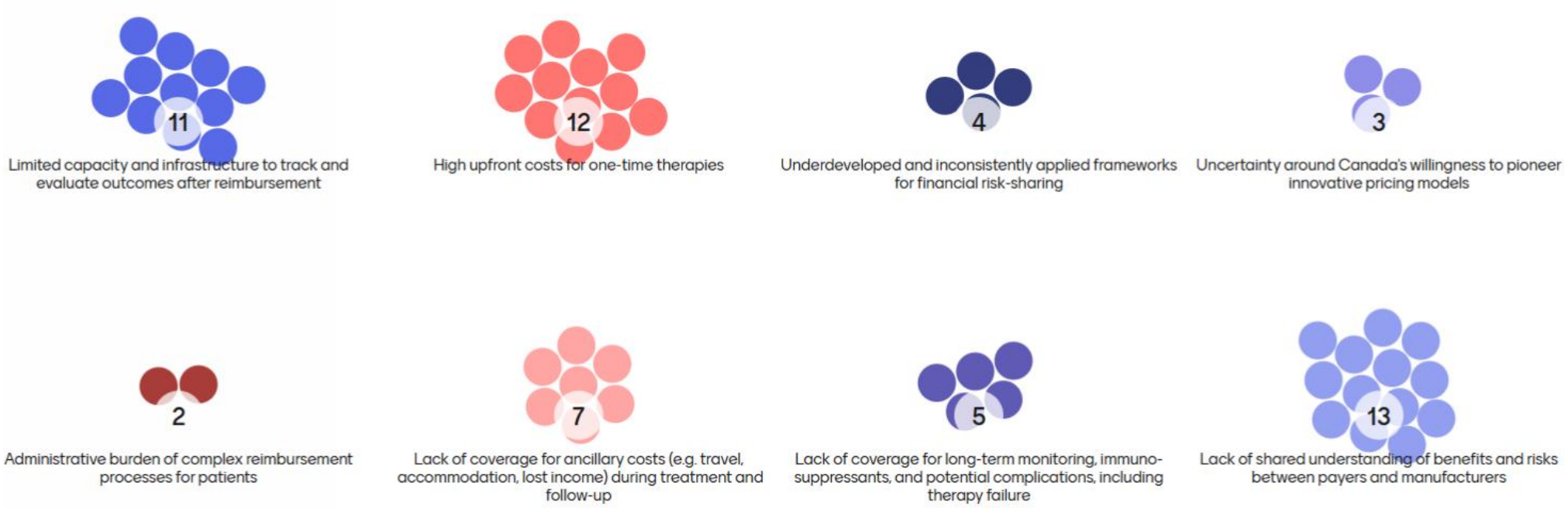

Figure 4: Prioritization results for risk-sharing and reimbursement barriers. Results from a live poll where 29 participants each cast up to three votes to prioritize eight key barriers related to the risk-sharing and reimbursement of T1D cell therapies. Total votes are shown above each barrier, where one vote is represented by an individual dot.

Recommended Solutions for Risk-Sharing and Reimbursement

Barrier 1: Lack of shared understanding of benefits and risks of cell therapies between payers and manufacturers	**Engage decision-makers (payers) at the right time and at the right political and decision-making level; briefing the Deputy Ministers FPT table with high-quality, patient-informed data ~2 years before an expected reimbursement decision would enable better health system budget and access planning and reduce uncertainty for manufacturers**
	Leverage the patient voice early in the development process as a common dialect between manufacturers and payers to help define an aligned benefit/risk narrative
	Convene group meetings with patients, industry, and payers ahead of anticipated drug launches to enable early engagement with key actors; these sessions can serve as a form of scientific advice, fostering dialogue on benefits and risks while providing an opportunity for early feedback and collaboration
	Increase visibility of market landscape and competition for payers; increased competition can help drive costs down
	Develop public education for politicians and payers to address misinformation that T1D is being well-managed currently with insulin and to position cell therapies as an opportunity to transform patient lives
Barrier 2: High upfront costs for one-time therapies clash with traditional reimbursement models	**Explore a social impact bond-like reimbursement mechanism specifically for T1D cell therapy supported by a government-backed payback mechanism**
	Explore payments in installments though this will need buy-in from manufacturers
Barrier 3: Limited capacity and infrastructure to track and evaluate outcomes of CGTs after reimbursement	**In addition to exploring innovative reimbursement schemes (e.g. CBAs, amortization models) to mitigate high upfront costs, Canada has an opportunity to be more strategic about leveraging existing infrastructure, research and innovation capacity, industry partnerships and public procurement mechanisms to develop and scale cost-effective homegrown cell therapy solutions while building our life sciences economy**

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Solutions in light yellow boxes were noted as most impactful by workshop participants

Barriers for Implementation and Adoption

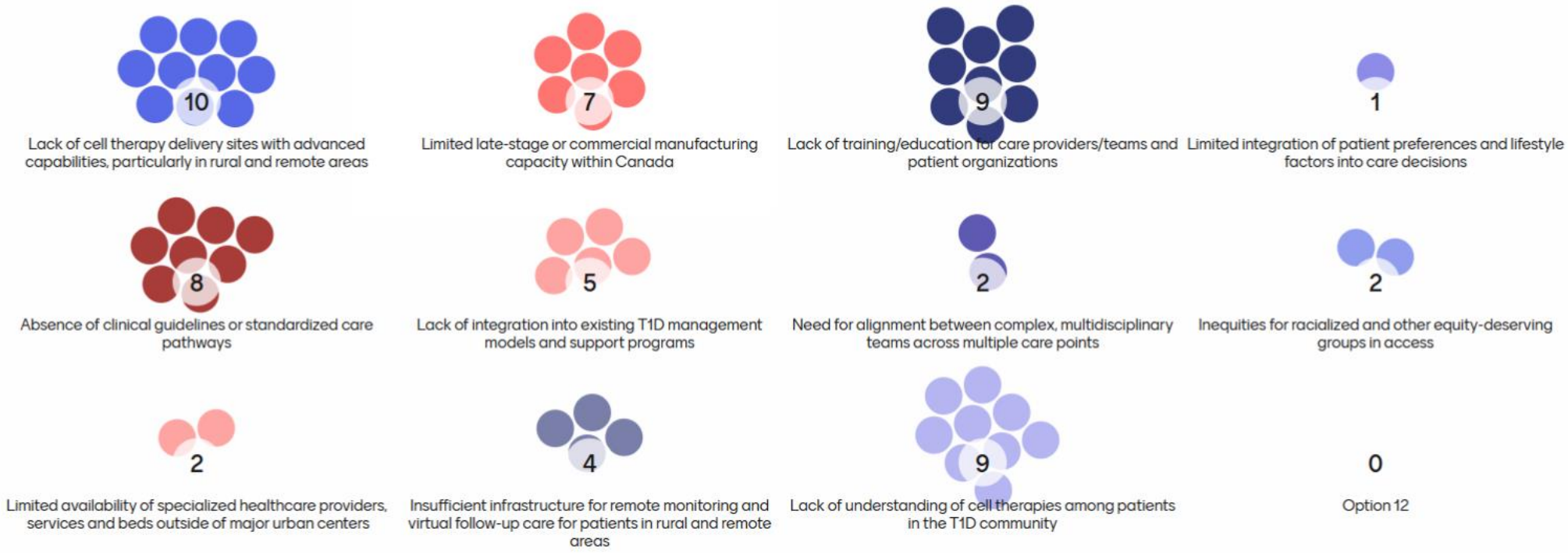

Figure 5: Prioritization results for implementation and adoption barriers. Results from a live poll where 30 participants each cast up to three votes to prioritize 11 key barriers to implementation and adoption of T1D cell therapies. Total votes are shown above each barrier, where one vote is represented by an individual dot.

Recommended Solutions for Implementation and Adoption

Barrier 1: Lack of cell therapy delivery sites with advanced capabilities (e.g., LN2 storage, transplant services), particularly in rural and remote areas	**Advance centralized centre(s) of excellence for manufacturing and delivery in some/most provinces that are colocalized and pre-approved (e.g. by Health Canada) to manufacture a range of cell therapy types/products**
	Expansion of tertiary care transplant programs (e.g. public and/or private like Vertex's patient support programs) for patients to support their ability to travel to tertiary centres for the delivery of T1D cell therapies
	Develop inter-provincial agreements for access to tertiary and specialized care centres for T1D patients, as not every P/T will have a centre that can deliver cell therapy
	Design a care pathway that supports patient access to tertiary care for treatment, with coordinated follow-up provided in community or local healthcare settings; implement telehealth for follow-up appointments (~3 months after transplant) and improve information sharing between tertiary care centres and patients' providers
	Allow for pre-approval of centralized centres as a manufacturer for any type of product to encourage industry use and increase capacity
Barrier 2: Lack of training/education for care providers/teams and patient organizations for T1D cell therapies	**Formal early and continuing education/exposure (e.g. seminars/classes in medical school and nursing school, formalized fellowship for transplant endocrinologists, and clear and simple reference package for GPs) to increase willingness of HCP community to provide specialized care to T1D patients**
	Form a satellite network of specialized teams trained from the centre(s) of excellence
Barrier 3: Lack of understanding of cell therapies among patients in the T1D community	**Strengthen and expand trusted social media sources tailored to audience e.g. popular influencers trained in KM who can deliver the message as a trusted source**

Solutions in light pink boxes were noted as most impactful by workshop participants

Barriers for Misinformation and Disinformation

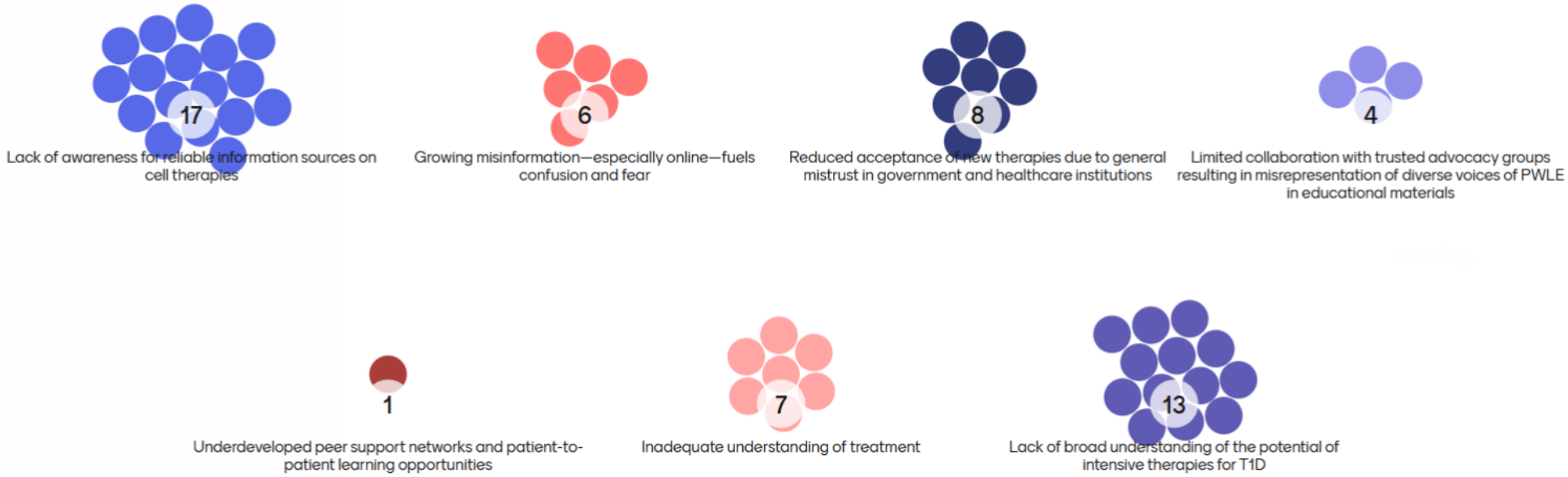

Figure 6: Prioritization results for barriers related to misinformation and disinformation. Results from a live poll where 29 participants each cast up to three votes to prioritize seven key barriers posed by misinformation and disinformation of T1D cell therapies. Total votes are shown above each barrier, where one vote is represented by an individual dot.

Recommended Solutions for Misinformation and Disinformation

Barrier 1: Lack of awareness of reliable information sources on cell therapies (e.g. SCN, ISSCR, Health Canada, Diabetes Canada) for the public	**Develop content for a broad range of HCPs to share with patients regarding cell therapies and building alignment**
	Build (or partner to expanding the Swiss LLM) a trusted chatGPT that specializes in information on cell therapies – however must overcome challenges with accuracy
	Meet people where they are through sources/messengers they already trust: • **Empower patient advocates to toggle between clinical/scientific fact and community** • **Engage with people through existing social media channels and in community settings (e.g. places of worship, libraries)** • **Leverage diabetes supply channels (e.g. Diabetes Express) to deliver info**
	Provide early health literacy education to improve critical thinking and resourcefulness
Barrier 2: Lack of broad understanding of the need and opportunity for better and/or intensive therapies for T1D (e.g. risk-benefit profile is not sufficiently compelling)	**Address payer knowledge gaps by leveraging existing resources such as the all-party diabetes caucus to amplify messaging (e.g. dispel myths, strong narrative around "disease-modifying" and "potential cure") and provide a "heads-up" for budget planning within the next 3-5 years for "soft targets" to be integrated**
	Provide targeted continued education to endocrinologists (and incentivise them to take training) on the need for and importance of T1D cell therapies—potentially creating a sub-speciality on islet cells transplantation and cell therapy

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Solutions in purple boxes were noted as most impactful by workshop participants