Rare Disease Day 2026 Conference

From Foundations to Impact: Sustaining Momentum in Canada's Rare Disease Strategy

Access. Evidence. Readiness. Alignment.

April 29–30, 2026
Hyatt Regency Toronto

CORD Canadian Organization for Rare Disorders

Join leaders, advocates, researchers, healthcare professionals, and the rare disease community for two days of collaboration, innovation, and meaningful discussions.

Day 1: Theme: Measuring Progress — Sustaining Gains in Access

Wednesday, April 29, 2026

8:00 am – 9:00 am	Registration & Breakfast (King Ballroom)
9:00 am – 9:15 am	**Welcome Address** Durhane Wong-Rieger, CORD
9:15 am – 10:00 am	**Opening Plenary: Federal & Provincial Update: What Has Improved in Timely and Equitable Access?** **Panel: Why Access Matters** • Jennifer Adams (PH1) • Stephen Parrott (VHL) • Zobaida Al-Baldawi, (HoFH) **Presenters:** • Daniel MacDonald, Health Canada, Drugs for Rare Diseases Secretariat **Focus of Presentation:** • 12–18-month update of RD Drug Strategy progress and potential next steps • Improved Timelines for listing drugs on the common drug list • Variability reduction across provinces • RWE Follow-up pilots **Takeaway:** The Strategy has produced measurable improvements. The question now is how to sustain and extend them.

	Open Discussion: • Based on the interim findings, what is the RD Drug Strategy doing toward addressing the four key objectives: support patient outcomes and sustainability, improve national consistency, collect and use evidence, and invest in innovation? • What else could be done for remainder of the Strategy to better meet these objectives or other outcomes
10:00 am – 10:30 pm	**Session 2: Rare Disease Drugs: Innovations and Opportunities** **Presentation:** Bill Dempster/Durhane Wong-Rieger • What are next-generation, innovative, breakthrough therapies for rare and ultra-rare diseases and, increasing for targeted sub-groups among common conditions? • How are these re-defining requirements for diagnosis and treating patients and engaging with them in real-world follow-up?
10:30 am – 10:45 am	**Break**
10:45 am – 12:00 pm	**Session 3: Accelerating Access to Rare Disease Therapies — Canadian Health Ecosystem, pt 1** **Moderators:** Bill Dempster, Durhane Wong-Rieger **Presentations: (15 mins each)** • PMPRB: New guidelines for new medicines balancing affordability and access: Anie Perrault, PMPRB • Health Canada: Modernization of regulatory environment for clinical trials, drug approvals, and special access: Kelly Robinson, Health Canada • CDA: Initiatives (within and without Rare Disease Drug Strategy) and implications for rare disease drugs through the lifecycle: Peter Dyrda, Canada's Drug Agency • pCPA: Independent agency with more formal, transparent, and faster negotiations plus stakeholder engagement: what it means for rare: Wayne Critchley, Global Public Affairs

	• CIHI: Modernizing drug data, ICD-11 codes and registry linkage and impact on rare diseases: Lacey Langlois, Canadian Institute for Health Information (CIHI) • Provincial Updates: Drug access mechanisms relevant to rare, providing faster, financially supportive, and exceptional patient inclusion, including FAST: Bill Dempster, 3Sixty Public Affairs • Private Drug Plans: Update on access to rare disease therapies: Suzanne Lepage, Private Health Plan Strategist **Questions to mull over lunch:** • Why shifts along the rare disease pathway are needed, now. • What are important opportunities that could improve rare/targeted drug access in Canada? • What are outstanding challenges that need to be addressed to assure Canada remains a "tier 1" country for access to innovative therapies?
12:00 pm – 1:00 pm	**Lunch**
1:00 pm – 2:30 pm	**Session 3: Accelerating Access to Rare Disease Therapies — Canadian Health Ecosystem, pt 2** **Moderator:** Bill/Durhane **Presentations: (15 mins each)** • Based on CORD survey findings, what are the social and economic "costs" to rare disease families and to society due to delays in diagnosis and delays in treatment? What are lessons we can learn from the respondents on how to reduce personal and societal impacts? Lesley Soril, Institute of Health Economics (IHE) • Accelerated access pathway for Health Canada priority review drugs: Impact for rare disease therapies. Allison Wills, 20 Sense • What have been changes to timelines for patient access to drugs for rare diseases from regulatory approval to provincial access? Sherry O'Quinn, Morse Consulting

	• Why MUST Canada adopt "Access with Evidence Development" models for rare disease therapies? What do we need to set up learning projects? Who needs to be involved? What is the potential return on investment? Rebeccah Marsh, Institute of Health Economics (IHE) **Panel Questions: (30 mins)** • What did you hear that resonated with you (good or bad)? What must Canada do immediately, that is, next 12 months? • What should we propose for Phase 2 of Rare Disease (Drug) Strategy that addresses biggest opportunities and most pressing concerns? • What are consequences if we DON'T make a hard pivot and start doing things differently? **Panelists: Multi-stakeholder** • Sherry O'Quinn, Morse Consulting • Leigh Funston, Alexion • Jennifer Adams, PH1 • Alex Wellstead, Novartis
2:30 pm – 2:45 pm	**Break**
2:45 pm – 4:00 pm	**Session 4: Putting Ideas into Action** **Brief Presentation/Discussions:** • What are implications of MFN for innovative rare therapies? What are risks? What are opportunities? Wayne Critchley, Global Public Affairs • From Advocacy to Action: Reforming the Special Access Program? Katherine Aldred, The Hospital for Sick Children • Model for sustainable access to innovative therapies from discover to patient support; Leszek Lisowski, Gene2Cure Foundation **Small Working Group Discussions** Using the therapy case type assigned to your table, design a pilot access-and-learning pathway that would support rapid, sustainable, and managed access from treatment introduction through real-world monitoring and

assessment. Tables are asked to move beyond “Can the system absorb innovation?” and instead ask, “What must be in place for this therapy type to be introduced, monitored, learned from, and sustained equitably?”

Suggested therapy case types (one per table):

1. High-cost chronic infused or enzyme replacement therapy
2. One-time or limited-course gene therapy / advanced therapy
3. Precision therapy requiring rapid molecular diagnosis
4. Ultra-rare individualized or very small-population therapy
5. Targeted therapy for a rare subgroup within a larger/common condition

Tables will discuss:

- the patient pathway from diagnosis to monitoring
- what system enablers must be in place
- one realistic pilot project that could begin within 12–18 months
- key opportunities, risks, and de-risking strategies
- what success should look like

Questions each table should answer:

1. What is the biggest current barrier to real patient benefit?
2. What is the most important missing or weakest system function?
3. What should the pilot project actually do?
4. Who would need to be involved?
5. What should be measured in the first 12–18 months?
6. What would make this pilot scalable or transferable?
7. What is the biggest implementation challenge, and how could it be mitigated?

Report-back format (4 minutes per table):

- Therapy type

	• Biggest current barrier • Proposed pilot • Key partners needed • What success would look like • One major challenge or risk **Output:** Consensus-based proposal for a practical, de-risked access pathway linking treatment introduction, delivery, monitoring, and learning.
4:00 pm – 4:20 pm	**Closing gaps: Enabling education and independence for youth living with rare diseases** **Summary/overview**: Youth living with rare diseases may face unique and compounding financial barriers to accessing and completing post-secondary education, with long-term implications for wellbeing, independence, and participation in society. Drawing on patient and youth insights, this session will highlight these challenges, explore why education is a critical enabler beyond employment, and introduce Canada's first, pan-rare Scholarship Program – made possible through the generous support of Alexion Canada – designed to directly respond to these patient-identified gaps and support youth in pursuing post-secondary education. Speakers: Svenja Espenhahn, Canadian Rare Disease Network (CRDN), Ian Stedman, Andrea Herscovitch
4:20 pm – 4:30 pm	**Closing Day 1 and Preview Day 2**
5:00 pm – 8:00 pm	**Networking Reception** (Regency Ballroom) All conference participants are invited to attend.

Day 2: Theme: Designing Sustainable Access and System Readiness

Thursday, April 30, 2026

8:30 am – 9:00 am	**Breakfast** (King Ballroom)
9:00 am – 9:15 am	**Day 1 Recap and Framing for Day 2** Durhane Wong-Rieger, CORD *Day 1 focused on what the Rare Disease Drug Strategy has achieved in access. Day 2 asks the next question: what must be in place in Canada's health systems so that access can be sustained, evidence can be generated, and therapies can produce meaningful benefits for patients and families?*
9:15 am – 10:45 am	**Session 5: From Research to Access- Preparing the Next Wave of Therapies** *To show that Canada's rare disease pipeline is real, but that research success alone is not enough. To reach patients, therapies need diagnostic readiness, clinical pathways, delivery capacity, post-market monitoring, and evidence systems.* **Moderator:** Maryam Oskoui, McGill University **Keynote:** Dr. François Bernier, Alberta Children's Hospital Research Institute Canada has demonstrated capacity in translational research, diagnosis, registries, innovative therapeutic development, and system readiness. The next question is not only what Canada should fund in research, but what must be in place for Canadian-origin and other innovative therapies to reach patients and generate impact. **Research showcase: "Made-in-Canada therapies and need for policy support"** **Presentations: (12 min each)** **Case 1: Pediatric cellular/gene therapy and Canadian manufacturing readiness** **Lead:** Nicola Wright, University of Calgary

Pediatric Cellular Therapy: Canadian manufactured viral specific T cells, CAR-T cells for refractory autoimmune disease, and “Made in Canada” gene editing therapies for inherited blood and immune disorders.

Case 2: Canadian-origin tissue reconstruction therapy

Lead: Danielle Larouche, CHU de Québec–Université Laval

Multiple tissue-engineered therapies have been brought into clinical trials and clinical use firL venous ulcers, severely burned patients, corneal limbal stem-cell deficiency, and recessive dystrophic epidermolysis bullosa

Case 3: Canadian made CAR-T therapy

Lead: Megan Mahoney, BioCanRx

Canada’s Immunotherapy Network: advancing discoveries for treatment, accelerating clinical trials, and building infrastructure to deliver therapies that save lives of those with cancer and beyond.

Case 4: Novel Gene Therapies for Rare/Ultra-Rare

Lead: Leszek Lisowski, Gene2Cure Foundation

NFP dedicated to development and clinical translation of novel gene therapies for rare and ultra rare conditions, bridging gap between scientific discovery and patient access

Discussion (30 min):

- Where does Canadian innovation stall: target discovery, manufacturing, trial execution, regulatory engagement, or commercialization?
- What system conditions are needed for innovative therapies to be diagnosable, accessible, monitored, and evaluated?
- What must be different for therapies intended for 1, 10, or 100 patients?
- What should the remaining Phase 1 period prioritize if Canada wants therapies not just approved, but actually integrated into care?

	Output: Shared list of the top system-readiness barriers between research success and patient impact.
10:45 am – 11:00 am	**Break**
11:00 am – 12:30 pm	**Session 6: From Health System to Patient Impact-What Health Systems Must Be Ready to Do** **Moderator:** Ella Korets-Smith, Gene Therapy Canada **Presentation** • International Research Initiative in Rare Diseases – Towards Social Pharmaceutical? Conor Douglas, York University (pre-recorded) **Panelists:** • Risini Weeratna, National Research Council • Jida El Hajjar, Loeys-Dietz Syndrome Foundation & CORD • Jagdeep Walia, Kingston Health Sciences Centre • Laurene Redding, Gilead **Core Framing:** If Canada wants innovative therapies to reach patients and produce outcomes that matter, health systems must be ready to do more than fund drugs. They must support diagnosis, coordinated care, monitoring, data collection, and learning over time. The question is not only what Canada should invest in, but what provinces and institutions must be prepared to deliver. **Prompt Questions:** • What needs to be in place from diagnosis through post-market monitoring if innovative therapies are to be used well? • What is needed to support real-world evidence collection during the remainder of Phase 1, even if a formal pre-market "access with evidence" model is not yet in place? • What provincial or institutional conditions are most critical: centres/expertise, diagnostics, navigation, data systems, psychosocial supports, workforce, telehealth? Output

	A shortlist of the minimum health-system readiness functions needed to support sustained access and RWE.
12:30 pm – 1:30 pm	**Lunch**
1:30 pm – 3:00 pm	**Session 7: From Access to Impact — Using the Logic Model to Assess Readiness and Define Provincial Priorities** **Moderator:** Durhane Wong-Rieger/Bill Dempster **Session purpose:** To introduce the logic model as a practical tool for assessing current readiness and identifying realistic next steps for provincial planning, pilot projects, and outcome measurement. **Part A — Brief framing presentations (45 min total)** **1. Introduction to the Logic Model (10–12 min)** **Speaker:** Durhane Wong-Rieger, CORD **Core message:** The logic model links: • **Determinants** — system enablers such as governance, funding, centres of expertise, workforce, diagnostics, data infrastructure • **Delivery** — what actually changes in care, such as referral pathways, multidisciplinary care, navigation, monitoring, psychosocial support • **Outcomes** — whether patients and families are actually better off clinically, psychosocially, economically, and in their care experience This model can be used not only to evaluate programs, but also to assess whether national or provincial rare disease plans are complete, patient-centred, and capable of translating commitments into meaningful results. **2. Model Example 1: Real-world monitoring / evidence generation (12 min)** Speaker: Dr. Roberto Mendoza, The Hospital for Sick Children

Question addressed: What does a system need in place to support meaningful long-term RWE after access?

3. Model Example 2: Care coordination / navigation / integrated support (12 min)

Speaker: Cara Grobbecker & Carlee Stokes, London Health Sciences Centre

Question addressed: What does a system need in place beyond drug funding so patients can move through care coherently and safely?

4. Model Example 3: Caring for Ultra-Rare (12 min)

Speaker: Dr. Andreas Schulze, The Hospital for Sick Children (virtual)

Question addressed: How can persons with ultra-rare conditions be supported in health system?

Part B — Working group exercise (45 min)

Exercise title:

Current State, Priority Gaps, and Feasible Next Steps

Each table works on one pathway:

1. Diagnosis and referral readiness
2. Access to funded therapy and monitoring
3. Care coordination and navigation
4. Psychosocial and family support integration
5. Data / RWE infrastructure

Table template:

Step 1 — What exists now?

Using the logic model:

- **Determinants:** What system enablers currently exist?
- **Delivery:** What actually happens now?
- **Outcomes:** What are patients and families experiencing now?

Step 2 — What is missing?

Identify:

- one major determinant gap
- one major delivery gap

	• one major outcome that is unmeasured or poorly addressed **Step 3 — What is one feasible next step?** Identify one realistic pilot project, planning priority, or system intervention that could begin within 12–18 months. **Step 4 — What would success look like?** Name: • one enabling condition • one likely challenge • one measure of success **Report-back and consolidation** Each table reports: • current state in one sentence • priority gap • feasible next step / pilot • one success measure **Output:** 1. Minimum System Readiness Elements for Sustained Rare Disease Access 2. Initial Provincial Readiness Assessment Framework 3. Priority Pilot Areas for the Next 12–18 Months
3:00 pm – 3:30 pm	**Closing Plenary: Sustaining Momentum — From Drug Access to System Readiness** **Moderator:** Bill Dempster **Session purpose:** To synthesize the conference message and shift the closing frame from "more access" to "durable, measurable, system-supported access." **Final framing:** The Strategy has produced gains in access for selected therapies. The next challenge is structural: how to ensure that newly arriving therapies, non-listed therapies, and future innovations are supported by systems that can diagnose patients, connect them to care, monitor

	outcomes, and generate evidence. Sustained momentum therefore requires not only access pathways, but also provincial and institutional readiness from diagnosis through long-term management and learning. **Suggested closing questions:** • What are the minimum building blocks for sustained rare disease access in the remainder of Phase 1? • What should be assessed now at provincial level? • What pilots are ready to start? • What should the conference recommend as immediate next steps? **Output:** Conference statement on: • minimum readiness elements • priority pilots • need for provincial planning/readiness processes **Panelists:** • Lindsay Williamson, SMA/CORD • Alice Williams, Wilson Disease Association/CORD • Rebeccah Marsh, IHE • Sang-Mi Lee, Morse Consulting • Bob McLay, Sobi
3:30 pm	**Wrap-up and Next Steps**

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