Canadian Organization for Rare Disorders

NEXT GENERATION
RARE CANADA 2026
MAKING ACCESS REAL

From Faster Drug Pathways to Patient-Ready Rare Disease Care

DATE	LOCATION	PROGRAM
November 12-13, 2026	Delta Halifax Downtown Halifax, Nova Scotia	Rare Canada 2026

DAY ONE - NOVEMBER 12, 2026

HOW PATHWAYS CHANGE

From lived experience and innovation to better patient pathways

8:30 am — 9:00 am	**Registration – Baronet Ballroom (8th Floor)** **Breakfast – Served in Empress Room; Seating in Baronet Ballroom (8th Floor)**
9:00 am — 9:10 am	**Opening - Canada has momentum: now make access real**
	CORD leadership: Durhane Wong-Rieger, President & CEO; Jida El-Hajjar, Board Chair
9:10 am — 9:15 am	**Opening Remarks** ***Pre-recorded video address***
	The Honourable Marjorie Michel, Minister of Health
9:15 am — 9:45 am	**Keynote — From National Vision to Patient Impact: Lessons from England's Rare Disease Strategy and RareCare Evaluation** **Speaker: Josie Godfrey, Co-Lead Investigator & Policy, RareCare / Realise Advocacy**
ABOUT THE SESSION	Learn from one of the world's more developed national approaches to rare disease: a shared national framework, successive action plans, specialist infrastructure and major

	investment in genomic medicine—and examine what the 2026 RareCare evaluation tells us about translating those assets into consistent patient benefit.
WHAT WE'LL EXPLORE	**What England has built and what RareCare learned from evaluating it**. The session will consider: • the value of an integrated national rare-disease vision and sustained action planning; • genomics as a major enabling capability for diagnosis, research and increasingly precision care; • progress in specialist services, research, data and policy infrastructure; • why even substantial national capability does not automatically produce coordinated frontline care; • persistent gaps in diagnosis, local-to-specialist interfaces, care coordination, equity and workforce; • the importance of measuring service delivery and lived experience as well as policy activity and infrastructure.
SESSION FORMAT	30-minute keynote with bridge to Canadian cases.

9:45 am — 10:00 am	**Alberta Spotlight — From Listening to Action: Alberta's Rare Disease Opportunity** **Speaker: Hon. Mike Ellis, Deputy Premier and Minister of Public Safety and Emergency Services, Government of Alberta** **Moderator: Sara Ethier, CORD**
ABOUT THE SESSION	Bring the international RareCare experience into a current Canadian provincial context. Alberta's August 2026 Rare Disease Summit brought patients, caregivers, clinicians, researchers, industry, governments and health agencies together to identify gaps, existing strengths and opportunities for a stronger rare-disease care system. Hear what Alberta learned from that discussion and how continued provincial action could better connect diagnosis, specialist care, treatment access, supports, research and innovation around the patient.
WHAT WE'LL EXPLORE	Minister Ellis will reflect on Alberta's first Rare Disease Summit and the opportunity to move from listening toward action, including: • what government heard about the experience of patients and families; • the importance of earlier diagnosis and more coordinated pathways; • the experience of people with rare or still-undiagnosed conditions who may repeatedly encounter emergency and acute-care services before reaching the right diagnosis or specialist pathway; • Alberta's existing strengths in genomics, data, research, clinical trials and innovation, and the opportunity to connect those capabilities more effectively to patient care; and • how sustained collaboration with patients, providers, researchers, industry and government can inform Alberta's next steps. Those themes are well grounded in the Summit report: participants identified earlier diagnosis, timely and equitable access, care navigation, data/research/registries/clinical trials, and continued collaboration as major priorities.

10:00 am — 10:45 am	**What Six Canadian Disease Pathways Have Taught Us** Patient Leaders: Josie Sirna, Thalassemia Foundation of Canada; Biba Tinga, Sickle Cell Disease Association of Canada; Kim Steele, Cystic Fibrosis Canada
ABOUT THE SESSION	CORD Readiness Webinar learnings: Six related but distinct therapeutic/disease delivery/readiness patterns in Canada and learnings from UK RareCare.
WHAT WE'LL EXPLORE	Hemophilia, thalassemia, sickle cell disease, cystic fibrosis, spinal muscular atrophy and Pompe disease. Focus on archetypes and turning points: accumulated capability, timing, diagnosis, specialist access, local interfaces, treatment burden/choice, transition and learning - not six disease histories.

10:45 am — 11:00 am	**Networking Break**

PATIENT AGENCY AND SYSTEM CHANGE

Patients are pathfinders, advocates, contributors and decision makers.

11:00 am — 11:40 am	**Beyond the Drug: When Patient Communities Build Readiness** Speakers: Jida El-Hajjar, CORD/LDSF; Ruchi Ambike, IgA Nephropathy Canada Hub Moderator: Durhane Wong-Rieger
ABOUT THE SESSION	Demonstrate that rare-disease readiness does not have to begin with the arrival or reimbursement of a therapy. Patient organizations can act as system builders—bringing patients, clinicians and researchers together; improving diagnosis and case-finding; creating registries and data; supporting research and trials; and developing the relationships and capabilities that allow future innovation to reach patients. Contrast two communities at different stages of that journey: **Loeys-Dietz Syndrome Foundation Canada**, where substantial patient-led research and clinical infrastructure have been developed, and **IgA Nephropathy Canada Hub**, which is actively building many of the same capabilities around an ultra-rare community.
WHAT WE'LL EXPLORE	**Loeys-Dietz Syndrome Foundation Canada — building readiness deliberately.** Explore how a patient organization moved beyond awareness and support to help build a broader disease ecosystem: clinical and scientific partnerships, patient engagement, research investment and the national CAN-ACT Registry for heritable aortopathies. CAN-ACT was developed through collaboration among LDSF Canada, clinicians and researchers and is intended to strengthen early identification, treatment, research and access to care. **IgA Nephropathy (IgAN) Canada Hub: our goal is to provide information, support, education and resources** to both clinicians and families in Canada affected by IgA Nephropathy. We also strive to advocate for IgAN patients and their families, seeking access to treatment and equity**— building the next layer of capability**.

11:40 am — 12:00 pm	**How Patients Change Systems - CORD 2x2 Model** Patient Leaders: Rachel Brothers, Amyloid Action; Tracy Joshi, NB Amyloidosis Program, Horizon Health Network; Catherine Boivin, CORD (virtual); Kim Howatt, Disability Community Advocate
ABOUT THE SESSION	Demonstrate: Patient agency is a source of pathway innovation and institutional change.
WHAT WE'LL EXPLORE	Introduction to CORD 2x2 Model of patient influence: inside/outside the formal system and individual/collective action. Trace how patients can move from advocating for their own pathway, to organizing communities, to shaping system change, and to serving as formal partners and decision makers. Emphasize that participation inside institutions expands patient influence but complements—rather than replaces—independent advocacy outside them.

12:00 pm — 12:45 pm	**Patients as Pathfinders - From Individual Solutions to System Learning** Speakers: Natasha Hepburn, Parent Advocate; Stephanie McCabe, Parent Advocate (virtual); Lindsay Williamson, CORD; Carla Chabot, CORD
ABOUT THE SESSION	Examine how patients and families identify needs and viable pathways before formal systems have anticipated them, and how successful cases can generate learning for the next patient.
WHAT WE'LL EXPLORE	Learning-case anchors: Naglazyme/MPS VI; SMA access and newborn screening; current Sanfilippo case; Nathan Hepburn successful out-of-country treatment. Common questions: What did the family identify? What pathway did they create? Who helped make it possible? What should change - or remain flexible - for the next patient? Map cases onto the 2x2 quadrants to show model in action.
SESSION FORMAT	Brief case presentations, impact

12:45 pm — 1:45 pm	**Networking Lunch – Served in Empress Room; Seating in Baronet Ballroom (8th Floor)**

DAY ONE - AFTERNOON

FROM LEARNING TO PATHWAY CHALLENGES

Where do pathways still break - and what already helps?

1:45 pm — 3:15 pm	**Interactive Pathway Challenge Workshops**

ABOUT THE SESSION	Participants will identify barriers and facilitators across the patient pathway, creating a shared set of challenges to carry into Day 2.
WHAT WE'LL EXPLORE	Four mixed-stakeholder workshops examine the same questions at different pathway stages: What does the patient encounter? What works reliably? Where does the pathway break? What have patients/HCPs already done to solve it? What could be better connected? What unresolved question should be taken to Day 2?
SESSION FORMAT	Conference participants will work in mixed-stakeholder groups across four pathway stages, supported by workshop facilitators.

Interactive Pathway Challenge Workshops

Four pathway stages; mixed tables identify what already works, where pathways break, and what Day 2 needs to solve.

CHALLENGE CASES:

PH1 (Oxlumo), VHL (Welireg), FOP (Sohonos), NF1 (Koselugo), ATTR amyloidosis, FCS (Redemplo & Tryngolza), HoFH (Evkeeza), Rare or molecularly targeted cancers

Moderators: Sandra Salama & Bill Dempster

WORKSHOP	CORE QUESTIONS	LEADS
1. Find & diagnose	Recognition, screening/case finding, genomic or other testing, confirmatory diagnosis, referral triggers.	Rachel Brothers & Tracy Joshi
2. Reach expertise	Specialist networks, multidisciplinary care, rural/remote access, transition, local-to-specialist interfaces.	Krysta Davies Foss & Jessica Head
3. Access & deliver	Eligibility, HTA/reimbursement, exception routes, treatment sites, logistics, navigation and timely initiation.	Belinda Yap & Carla Chabot
4. Manage, support & learn	Longitudinal care, patient support, PROMs/PREMs, registries/RWE, caregiver burden and learning back into the pathway.	Sara Ethier & Karen Kelm

3:15 pm — 3:45 pm	**Networking Break**
3:45 pm — 4:15 pm	**Workshop Harvest and Day 1 Challenge Statements** Workshop leads, with patient, health-care professional and industry perspectives. CORD synthesis – Durhane Wong-Rieger

ABOUT THE SESSION	Identify transferable capabilities and practical failure points across the four pathway stages.
WHAT WE'LL EXPLORE	Report findings by pathway need rather than disease: identification/diagnosis; referral/expertise; access/delivery; integrated management/support; data/outcomes; and escalation routes when standard pathways do not fit. Distinguish what Canada already has from what needs new enabling action. Close by agreeing 5-7 challenge statements for Day 2 leaders, e.g. How can patients bring forward pathways the system has not anticipated? When should an exception change the routine pathway? Where can processes become more concurrent? How do we know access improved the patient journey?

4:15 pm — 5:00 pm	**What We Need the System to Help Solve Tomorrow**
ABOUT THE SESSION	Day 1 closes by bringing together the needs and challenges identified across patient pathways and setting the stage for Day 2's focus on system response. Closing remarks: Durhane Wong-Rieger
WHAT WE'LL EXPLORE	Key Day 1 challenge statements will carry forward into Day 2.

5:30 pm — 7:30 pm	**Networking Reception – Bluenose Ballroom (8th Floor)** End-of-day reception for all participants.

DAY TWO - NOVEMBER 13, 2026

HOW THE SYSTEM CAN RESPOND

From changing access processes to patient-ready delivery

8:30 am — 9:00 am	**Registration – Baronet Ballroom (8th Floor)** **Breakfast – Served in Empress Room; Seating in Baronet Ballroom (8th Floor)**
9:00 am — 10:30 am	**Signature Plenary – Canada's Access System Is Changing: Can We Connect It End to End?** Panelists: • Kelly Robinson, Health Canada regulatory/PDD • Daniel MacDonald, RDDS / Health Policy • Suzanne McGurn, Canada's Drug Agency • Mauro Chies, pCPA Moderator: Bill Dempster, 3Sixty Public Affairs
10:30 am — 10:45 am	**Networking Break**

BEYOND THE MEDICINES PATHWAY

Who else makes rare care work, what is coming next, and can the pathway deliver?

10:45 am — 11:30 am	**Panel 1 – Beyond Approval and Reimbursement: Who Makes Rare Care Work?** Panelists: • Kristin Tweel, Genome Atlantic • Francois Bernier, Canadian Rare Disease Network • Thierry Lacaze, RareKids-CAN • Stephen Betschel, CHAEN • Andrew Bakhit, Bayshore Moderator: Durhane Wong-Rieger
ABOUT THE SESSION	Move deliberately from the morning's regulatory/HTA/negotiation discussion into health-services delivery and the wider rare-disease ecosystem. Pathway focus: Deliver, Manage & support.
WHAT WE'LL EXPLORE	The RD Drug Strategy created an important access and funding lever. What other organizations and capabilities must connect around it so that access becomes care? Common question to every panelist: What is the role of your organization, agency or profession in making the rare-disease patient pathway work - and what could Canada do now to make your contribution more effective?
SESSION FORMAT	Multi-stakeholder panel; focus on function in the pathway, not organizational presentations.

11:30 am — 12:00 pm	**Panel 2 – Innovator Response: What Must Be Ready Before the Therapy Arrives?** *Collaborating with innovators to build the pathways, evidence and access models these therapies require* Panelists: • Carrie McElroy, Sanofi • Monty Keast, Ultragenyx • Angela Behboodi, Amgen • Bob McLay, Sobi Moderator: Sandra Salama
ABOUT THE SESSION	The next generation of rare and precision medicines is beginning to deliver benefits that were unimaginable only a few years ago—from therapies that can alter the course of devastating childhood diseases to gene, RNA, cell and highly targeted treatments designed for very small molecularly defined populations. Yet the great majority of people living with rare diseases still have no disease-modifying treatment. The challenge is therefore twofold: **bring more transformative therapies forward and create health systems capable of identifying the right patients and delivering those therapies successfully when they arrive.** Canada has an opportunity to position itself as a country where rare-disease innovation can reach patients early and effectively. This session asks what Canada must have ready—from diagnosis, specialist networks and treatment infrastructure to evidence generation, reimbursement, patient support and long-term follow-up—to become an attractive and reliable environment for the next generation of therapies.
WHAT WE'LL EXPLORE	A fast-moving moderated conversation with innovators bringing different types of rare and highly targeted therapies to patients. Rather than company presentations or pipeline updates, panelists will respond to a common challenge: **what is becoming harder about bringing highly effective medicines to patients, and where could Canada do things differently?** Companies are developing therapies with unprecedented potential while facing an increasingly complex global environment: very small patient populations, high development and manufacturing costs, evolving evidence requirements, specialized delivery infrastructure, uncertain reimbursement pathways, international pricing pressures and difficult decisions about where and when to launch. The discussion will explore: • **What has to be ready before the medicine arrives?** How do companies find patients, establish diagnostic pathways, build clinical expertise and prepare treatment sites? • **Where are the biggest barriers now emerging?** Which are scientific or technical, and which arise from health-system processes, economics or fragmented access pathways? • **What are companies currently having to build or bridge themselves?** Patient identification, testing, navigation, evidence generation, treatment logistics or support? • **Where could Canada lead?** What would make Canada a preferred place for clinical development, early launch and access while also delivering better outcomes for patients?

	• **What should Phase 2 enable?** Which capabilities could make Canada more ready for therapies that do not fit conventional models of assessment, reimbursement or care delivery? Closing common question to all: **If your next transformative therapy were approved tomorrow, what is the one thing Canada would need to have ready to ensure the right patient actually benefits from it?**
SESSION FORMAT	Open format panel discussion with facilitated question/answer.

12:00 pm — 12:50 pm	**Panel 3 – Rare Disease, Precision Medicine and Rare Therapeutic Populations: *How Do We Find the Patient — and Is the System Ready When We Do?*** Panelists: • Kahlil Lawless, Illumina • Michael Mackley, IWK • Kristen Hummel, MPN Network • Marissa Lopez, Sumaira Foundation Moderator: Durhane Wong-Rieger
ABOUT THE SESSION	Explore how genomics and precision medicine are reshaping the meaning of "rare." Increasingly, patients are defined not only by a rare disease diagnosis but by a specific gene, variant, biomarker or molecular subtype that may identify a very small therapeutic population within a more common condition. The session will examine what this means for Canada's ability to identify the right patient, connect them to expertise and translate molecular diagnosis into timely access to appropriate care and treatment.
WHAT WE'LL EXPLORE	The panel will move from **genomic capability to real-world patient identification and system readiness**. **Kahlil Lawless (Illumina):** how genomics and molecular characterization are creating increasingly precise therapeutic populations across rare disease, cancer, cardiovascular disease and other areas, and what national genomics capability can contribute to finding patients. **Dr. Michael Mackley (IWK):** what happens re: clinical recognition, testing, interpretation, confirmation, referral and implementation, and where the pathway can fail even when the relevant genomic information is available. **Kristen Hummel (MPN Network):** patient perspective from myelofibrosis/MPNs, illustrating what it means to navigate a rare, molecularly informed blood-cancer pathway and how diagnosis, specialist access, geography and equity shape the real experience of precision medicine. **Marissa Lopez (The Sumaira Foundation):** patient and advocacy perspective from AQP4-positive NMOSD, illustrating how a patient presenting through a familiar neurological pathway can ultimately belong to a very small biomarker-defined therapeutic population. Question: How much of access depends on getting the diagnosis—and the subtype—right in the first place?

	Discussion will centre on four questions: • Can we identify the patient? • Can we confirm the diagnosis and connect them to the right expertise? • What does the patient actually experience along that pathway? • Can the wider health system make this routine rather than exceptional?
SESSION FORMAT	10-12 min modality overview + 10-12 min patient-identification presentation + 25-30 min multi-stakeholder discussion.

12:50 pm — 1:45 pm	**Networking Lunch – Served in Empress Room; Seating in Baronet Ballroom (8th Floor)**

DAY TWO - INTERACTIVE LAB

HOW TO ASSURE CANADA IS READY

Simulating patient pathways for rare and highly targeted therapeutic populations

1:45 pm — 2:45 pm	**Panel 4 / Interactive Lab – How Can we Assure Canada Is Ready? Simulating the Patient Pathway** Moderators: Jida El-Hajjar & Bill Dempster Facilitators/Respondents: Michelle Cooper, Trish Guimond, Michelle Burleigh
ABOUT THE SESSION	Drug funding is one entry into the rare disease system. As a context for Canada's Strategy for Drugs for Rare Diseases, the session will open with brief presentation of historical global archetypes of rare disease frameworks and where Canada fits. Then, using three fictional patient cases, participants will explore the connections to diagnosis, expertise, treatment delivery, continuing care and family support that turn funded access into patient benefit. Each table will choose one practical improvement and identify who needs to act.
WHAT WE'LL EXPLORE	Each table receives one case and starts at the point that matters most for that patient. Use six functions—identify, diagnose, specialist, treat, manage and monitor—to work backwards and forwards, revisiting steps as needed. Support and patient choice apply throughout. Four questions guide the discussion: What needs to happen? Where could it get stuck? What would help? Who needs to act? For these simulations, assume that an appropriate therapy can be funded once diagnosis and clinical eligibility are confirmed. No Day 1 worksheet is needed. Finish with one priority change and the patient benefit expected.
SESSION FORMAT	10-minute introduction and case briefing; 45-minute table simulation; 5 minutes to prepare a short report. Record the table number and case A, B or C, then complete four statements: Our patient needs…; The main sticking point is…; We would change…; We need this actor to…. Report-back follows at 2:45–3:10 pm.

SIMULATED CASES

A. The child who must be found early — A young child has worsening movement and development but no clear diagnosis; testing and treatment expertise are at a distant centre. Objective: reach expert assessment and treatment before avoidable decline. Consider recognition, prompt testing and referral, family support and follow-up.

B. The adult hidden in a common specialty — An adult in routine cardiac care has worsening symptoms and a family history suggesting an inherited condition. Testing, specialist review and local care sit in different services. Objective: recognize the condition and connect diagnosis to treatment and continuing care.

C. The molecularly rare cancer patient — A patient with a common cancer has a molecular result identifying a small treatment group. An individualized cell-based therapy may be appropriate, but assessment and treatment are at another centre. Objective: turn the test result into timely specialist treatment, with coordinated preparation, support and follow-up near home.

2:45 pm — 3:10 pm	**What the Simulations Revealed - What Does Canada Need to Enable?** Moderators: Jida El-Hajjar & Bill Dempster
ABOUT THE SESSION	Tables share one priority improvement and its intended patient benefit, using the four short report statements. Allow about one minute per table. Proposed actions are requests for discussion, not assumed commitments.
WHAT WE'LL EXPLORE	Across the three cases, ask system respondents: What can already be done? What needs a better connection? What needs new capacity or funding? Who should take the next conversation? CORD will carry the practical proposals into the closing discussion and follow-up.

3:10 pm — 3:30 pm	**Closing – From Faster Access to Patient-Ready Pathways** Durhane Wong-Rieger
ABOUT THE SESSION	The conference will close with practical propositions for strengthening patient-ready rare disease pathways in Canada.
WHAT WE'LL EXPLORE	Emerging CORD Phase 2 Patient-Pathway Framework: preserve and accelerate drug access; recognize patient agency; connect diagnosis, expertise, treatment and support; build responsiveness for rare therapeutic populations; enable flexible solutions when routine pathways do not fit; learn from every patient and pathway; and measure whether the patient journey actually improved.

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