



Canadian Organization
for Rare Disorders

NEXT GENERATION RARE CANADA 2026 MAKING ACCESS REAL

From Faster Drug Pathways to Patient-Ready Rare Disease Care

DATE

November 12-13, 2026

LOCATION

Delta Halifax Downtown
Halifax, Nova Scotia

PROGRAM

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 & Breakfast

9:00 am —
9:15 am

Opening - Canada has momentum: now make access real

CORD leadership: Durhane Wong-Rieger, President & CEO; Jida El-Hajjar (Board Chair)

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: David Page, Bleeding Disorders Canada; Josie Sirna, Thalassemia Foundation of Canada; Biba Tinga, Sickle Cell Disease Association of Canada (TBC); Kim Steele, CF Canada (TBC); Susi VanderWyk, Cure SMA Canada; Audrey Belouin, Canadian Association of Pompe (TBC)
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, Executive Director LDSF; Jordan Stead, CDKL5 Canada (TBC) 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 CDKL5 Canada, 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. CDKL5 Canada — building the next layer of capability. Teresa Wong brings the experience of a smaller, caregiver-led community navigating diagnosis, specialized care, clinical research and cross-border trials while working toward a Canadian registry and centre of excellence for CDKL5 Deficiency Disorder.

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; 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 decisionmakers. 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, Minister of Health (TBC); Lindsay Williamson, CORD; Ruchi Ambike, IgA Nephropathy Canada Hub
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	
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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
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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), Rare or molecularly targeted cancers

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.	David Page, BDC (TBC)
3. Access & deliver	Eligibility, HTA/reimbursement, exception routes, treatment sites, logistics, navigation and timely initiation.	Belinda Yap, HEOR (TBC)
4. Manage, support & learn	Longitudinal care, patient support, PROMs/PREMs, registries/RWE, caregiver burden and learning back into the pathway.	Sara Ethier, CORD

**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 the Day 2 breakfast and plenary.

5:30 pm — 7:30 pm	Networking Reception End-of-day reception for all participants.
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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 & Breakfast

9:00 am —
10:30 am

Signature plenary - Canada's access system is changing: can we connect it end to end?

Panelists:

- Pharmaceutical & Life Sciences Sector Task Force - Michelle Boudreau
- Health Canada regulatory/PDD - Kelly Robinson
- RDDS / Health Policy - Daniel MacDonald
- Canada's Drug Agency – Suzanne McGurn
- pCPA - Mauro Chies
- Provincial public drug plan/implementation - Atlantic province representative + Ontario FAST perspective (additional representatives to be announced).

10:30 am —
11:00 am

Networking Break

BEYOND THE MEDICINES PATHWAY

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

11:00 am —
11:45 am

Panel 1 - Beyond approval and reimbursement: who makes rare care work?

Speakers: Canadian Rare Disease Network (TBC); Thierry Lacaze RareKids-CAN; Genome Canada (TBC); Stephen Betschel, CHAEN; Andrew Bakhit, Bayshore; Patti Bryant, Dravet Canada

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.

11:45 am — 12:35 pm	Panel 2 - Rare Disease, Precision Medicine and Rare Therapeutic Populations: <i>How do we find the patient — and is the system ready when we do?</i> Kahlil Lawless, Illumina; Michael Mackley, IWK; Kirsten Hummel, MPN Network; Marissa Lopez, Sumaira Foundation
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.

12:35 pm — 1:30 pm	Networking lunch
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DAY TWO - INTERACTIVE LAB

HOW TO ASSURE CANADA IS READY

Simulating patient pathways for rare and highly targeted therapeutic populations

1:20 pm — 2:40 pm	Panel 3 / Interactive Lab – How can we assure Canada is ready? Simulating the patient pathway Moderator: Jida El-Hajjar; Facilitators (TBC): System responders: Day 2 plenary panelists seated at tables
ABOUT THE SESSION	Participants will stress-test the full patient pathway using fictional composite cases informed by real therapeutic modalities and known access and delivery barriers.
WHAT WE'LL EXPLORE	Each table starts from the Day 1 challenge statements and proposed fixes, then works from from patient identification through diagnosis, eligibility, referral, access, delivery, support and outcomes. Common questions: Who finds the patient? What confirms eligibility? Who owns referral? Where is treatment delivered? What can prevent access even if therapy is funded? What support is needed? What outcomes must be learned? Which barriers can already be solved with Canadian capabilities, and which genuinely require something new?
SESSION FORMAT	10-minute case briefing + ~45-minute table simulation + ~25-minute report-back / response panel.

SIMULATED CASES

A. The child who must be found early — Progressive ultra-rare genetic condition; benefit depends strongly on treatment before irreversible decline. Tests early identification, confirmatory genetics, specialist referral, advanced-therapy delivery, family decision support and long-term follow-up.

B. The adult hidden in a common specialty — Later-onset inherited cardiovascular condition with a targeted/RNA-based therapy. Tests clinical recognition, genetic or molecular confirmation, cascade testing, specialist networks and longitudinal monitoring.

C. The molecularly rare cancer patient — A common cancer becomes a very small therapeutic population through a molecular/biological characteristic. Tests molecular testing, referral, individualized cell-based treatment logistics, treatment-centre capacity and post-treatment follow-up.

2:40 pm — 3:10 pm	What the simulations revealed - what does Canada need to enable? Facilitators: LDSF/CORD Moderator: Jida El-Hajjar
ABOUT THE SESSION	The session will bring together lessons from the simulations to identify recurring barriers, existing facilitators and practical actions that can strengthen patient-ready pathways.
WHAT WE'LL EXPLORE	Synthesize across cases: early identification; testing/diagnosis; specialist connection; access/exception routes; treatment-site capacity; patient support and logistics; cross-provincial pathways; data/RWE; and feedback loops. Separate needs that require new investment from those that primarily require better connection or implementation.

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.