Canadian Organization for Rare Disorders

NEXT GENERATION
RARE CANADA 2026

Building the Next Phase of Rare Disease Drug Access, Readiness and Partnership

DATE	LOCATION	EVENT
November 12-13, 2026	**Delta Halifax Downtown 1990 Barrington St Halifax, B3J 1P2**	**CORD Fall Conference 2026**

DRAFT PROGRAM FOR SPONSORSHIP AND PARTNER ENGAGEMENT

This agenda shows the direction, working method and expected outputs of the November conference. It is designed to be specific enough for sponsorship and speaker outreach while remaining flexible as RDDS evaluation findings, RareCare learning, funding clarity, webinar learning cases and Readiness Advisor work become available.

Core proposition

The next phase of Canada's Rare Disease Drug Strategy should preserve dedicated access to rare-disease therapies while also asking what disease-drug pathway capabilities are necessary for those therapies to reach the right patients, deliver meaningful benefit, and leave the system more prepared for the next innovation.

This is not a wish list for parallel infrastructure. It is a practical planning exercise: using Canadian experience to identify which capabilities are necessary for RDDS investments to translate into patient benefit, system learning and Canada's attractiveness as a place for rare and precision therapies.

Conference method

From provisional learning cases to Phase 2 RDDS recommendations

CORD's fall webinar series and Readiness Advisor process will not attempt to produce definitive disease histories. Instead, they will use provisional Canadian disease-drug pathway profiles as learning cases to identify how readiness develops and what future investments should anticipate.

Step	What it contributes
1. Provisional learning cases	Six Canadian disease-drug profiles are used to examine how therapeutic innovation, clinical capability, patient agency, funding, policy and evidence influenced one another over time.
2. Working archetypes	The cases generate archetypes such as accumulated reusable capability, burden/life-course gaps, specialist-centre limits, precision therapy entering a mature pathway, therapy forcing rapid system change, and treatment arriving before surrounding readiness.

Step	What it contributes
3. Prospective stress-testing	Roundtables apply the archetypes to current and incoming rare or precision pathways, especially RDDS Common List and near-term therapy cases.
4. Phase 2 implications	Findings are converted into practical recommendations for what future RDDS investments should assess, support, require, report and learn.

Why the roundtables matter

The roundtables are the evidence-producing engine of the conference. They are not generic breakout discussions. Each table will test whether a learning-case archetype helps explain a new disease-drug pathway, identify which capabilities are missing, and translate the finding into a concrete Phase 2 implication.

Patient agency + partnership

A central principle runs through the entire agenda: patients and patient organizations are not only consulted after access processes have been designed. Across Canadian rare diseases, they have identified unmet need, created registries, built research capacity, improved diagnosis, defined treatment value, advocated for access, shaped delivery and monitored outcomes. Phase 2 of the RDDS should formalize this role in priority setting, pathway design, implementation and review.

Outputs by design

- A set of learning archetypes drawn from six provisional Canadian pathway profiles.
- Roundtable-generated readiness findings from additional RDDS, rare and precision-therapy cases.
- A focused Phase 2 RDDS recommendation framework grounded in tested pathway evidence rather than assumptions.
- A clearer accountability proposition: public investment should show not only what was funded, but what changed for patients and what capability remained for the next therapy.

NOVEMBER 12, 2026 (DAY ONE)

From Experience to Readiness

What have Canadian rare-disease pathways taught us, and do those lessons travel?

Time	Session	Purpose / focus
8:00-9:00	**Registration & networking breakfast**	Arrival, networking and informal connection across patient, clinical, policy, research and industry participants.
9:00-9:15	**Opening: From Phase 1 learning to Phase 2 readiness**	Welcome and frame the conference task: use Phase 1 experience, external evaluation evidence and Canadian pathway learning to shape practical recommendations for Phase 2 of the RDDS.
9:15-9:45	**Keynote - Closing the delivery gap: lessons from RareCare: Evaluation fo England's Rare Diseases Action Plan**	Josie Godfrey, Co-Lead Investigator & Policy, RareCare / Realise Advocacy, presents findings from the evaluation of England's Rare Diseases Action Plans. Focus: the gap between system activity, service delivery and lived experience; care coordination; equity; measurement; and designing policy for demonstrable impact.

Time	Session	Purpose / focus
9:45-10:15	**Canadian case studies: When policy and treatment meet the pathway**	Short Canadian cases from CORD's learning profiles illustrate where strong policy, specialist expertise or therapy access does - and does not - translate into end-to-end patient benefit. Cases will focus on diagnosis, specialist-to-local care, treatment delivery, integrated management and data/accountability.
10:15-10:45	**Panel discussion: What could Canada apply to Phase 2?**	RareCare and Canadian contributors test potential applications for Canada: designing with delivery in mind, linking drug funding to pathway capability, measuring impact at system/service/patient levels, making equity visible, and embedding patient/community partnership from the outset.
10:45-11:15	**Networking break**	Time for sponsor, speaker and participant engagement.
11:15-12:00	**What six pathways taught us**	Synthesize the fall webinars: hemophilia, thalassemia, SCD, CF, SMA and Pompe. Focus on the recurring readiness archetypes and how the Canadian cases reinforce, qualify or extend the morning's RareCare lessons.
12:00-12:40	**Beyond the drug: patient communities build readiness**	LDSF Canada and CDKL5 illustrate readiness built through patient-led research, registries, case-finding, data, trial networks and global connection even before or beyond a reimbursed therapy.
12:40-1:40	**Networking lunch**	Informal continuation of the morning's discussion.
1:40-3:15	**Working roundtables: Apply the archetypes**	Central Day One exercise. Participants stress-test the archetypes and the morning's delivery-gap lessons against new disease-drug pathways and produce practical readiness findings.
3:15-3:45	**Networking break**	Facilitators consolidate table findings.
3:45-4:35	**Roundtable harvest: What transfers, what breaks, what did we miss?**	Identify transferable capabilities, boundary conditions, framework gaps and initial RDDS implications from the challenge cases.
4:35-5:05	**Patient voices: Moments that matter**	Short patient and caregiver perspectives test whether the emerging framework captures what actually matters: diagnosis, expertise, treatment burden, equity, trust, support and outcomes.
5:05-5:30	**Day One synthesis**	Translate RareCare learning, Canadian cases and roundtable findings into the working readiness framework carried into Day Two.
5:30-7:30	**Networking reception**	End-of-day reception for sponsors, speakers and participants.

Opening plenary: From delivery gap to Phase 2 readiness

RareCare provides an external evaluation lens for the Canadian discussion. Its evaluation of England's Rare Diseases Action Plans found that policy visibility and infrastructure can improve without equivalent change in frontline delivery or lived experience. The conference will use that distinction - system activity, service delivery and lived experience - as a practical test for Phase 2 RDDS design.

RareCare keynote (30 minutes): Josie Godfrey will highlight the findings most relevant to future policy: persistent diagnostic and coordination gaps, inequalities across the pathway, limits of activity-based measurement, the need to define expected impact from the outset, and the value of lived-experience evidence in evaluation.

Canadian case studies (30 minutes): Selected CORD pathway cases will show how similar implementation gaps appear in Canada - for example, when a therapy is funded but patients are difficult to identify, when specialist expertise does not connect reliably to local care, or when treatment access is not matched by integrated management, navigation or longitudinal learning.

Application panel (30 minutes): Panelists will ask which RareCare lessons are transferable to Canada and what they imply for Phase 2: what should be designed, funded, measured and held accountable so that RDDS investment produces meaningful patient and system benefit.

The six learning-case archetypes

Learning case	Working archetype
Hemophilia	Accumulated capability becomes reusable infrastructure - comprehensive care, patient-clinician partnership, home management, national product systems, governance and data.
Thalassemia	Clinical sophistication can coexist with treatment burden, life-course transition gaps, population change and fragmented infrastructure.
Sickle cell disease	Readiness must work beyond the specialist centre; acute-care interfaces, trust, equity and distributed system reliability are part of the pathway.
Cystic fibrosis	Readiness largely precedes transformative precision therapy, but success then forces redesign, residual-population attention and treatment-choice capability.
Spinal muscular atrophy	Innovation rapidly creates and reconfigures readiness; screening, diagnosis, eligibility, delivery, patient choice and evidence must move at the speed of irreversible loss.
Pompe disease	Therapy can arrive before surrounding readiness; diagnosis, NBS, population intelligence, advocacy capacity and harmonized access may lag behind treatment availability.

Conference stance on evidence

Profiles are provisional and will be updated as evidence becomes available. The conference does not depend on perfect disease histories; it depends on testing whether the patterns they reveal help Canada anticipate what future rare and precision therapies will require.

Day One roundtables

Apply the archetypes to current and incoming pathways

The afternoon roundtables are designed to move the conference from retrospective learning to prospective readiness. Each table will begin with one challenge pathway, a short brief and one or more archetypes to test. Readiness Advisors will help facilitate and bring forward findings from their pre-conference assessments.

Potential challenge cases

Challenge pathway	What it tests
PH1 / Oxlumo	Find-the-patient pathway: diagnosis, genetic confirmation, nephrology/metabolic expertise, referral, RWE and monitoring.
VHL / Welireg	Hereditary diagnosis, family identification, surveillance, multidisciplinary management and treatment sequencing.
FOP / Sohonos	Ultra-rare recognition, avoidance of harm, concentration of expertise and first disease-specific therapy implementation.

Challenge pathway	What it tests
NF1 / Koselugo	Distributed lifelong care, specialist eligibility assessment, transition and longitudinal management.
ATTR amyloidosis	Case-finding, subtype identification, cardiology/neurology coordination and expanding treatment choice.
FCS / Redemplo & Tryngolza	Two therapies entering the same ultra-rare pathway, testing genetic confirmation, pancreatitis-risk criteria, treatment choice, sequencing, access and patient-relevant outcomes.
Rare or molecularly targeted cancers	Molecular testing, specialist referral, highly targeted populations, sequencing and delivery capacity.
Additional advisor cases	Cases brought forward by Readiness Advisors or disease communities that confirm, challenge or extend the emerging framework.

Table task

- Identify which learning-case archetype best helps explain the challenge pathway - or whether a new archetype is needed.
- Assess which capability is essential for the next therapy to deliver meaningful patient benefit.
- Identify what is currently known, unknown, variable or fragile in the pathway.
- Specify where patient agency and partnership should be formalized.
- Translate the finding into one Phase 2 RDDS implication.

Table output: Readiness finding card

Finding card field	Question to answer
Archetype tested	Which learning-case pattern is being applied, and does it fit?
Highest-risk gap	Where would the pathway fail first if the therapy were funded tomorrow?
Required capability	What needs to be in place around the drug: testing, referral, expertise, delivery, management, data, navigation, patient information or other support?
Patient/community role	Where should patients or patient organizations help set priorities, design implementation, define outcomes or review results?
Phase 2 implication	What should RDDS assess, support, require, report or learn in this type of case?

Roundtable value for sponsors and partners

Sponsors are supporting a structured national working process, not simply a set of presentations. The roundtables generate usable findings that can inform CORD's Phase 2 recommendations, future advocacy, partnership priorities and implementation conversations with governments and agencies.

NOVEMBER 13, 2026 (DAY TWO)

From Readiness to Design

What should the next phase of the RDDS assess, support, require, report and learn?

Time	Session	Purpose / focus
8:00-9:00	**Networking breakfast**	Informal networking and review of Day One findings.
9:00-10:00	**Signature plenary: Canada's access ecosystem is changing**	Life Sciences & Pharmaceutical Task Force, Health Canada modernization, pCPA ENP, Ontario FAST and CORD's CDA rare-disease HTA review. The question: how can rare and precision therapies use reforms already underway to make Canada better for investment and better for patients?
10:00-10:45	**Treatment choice in an increasingly targeted world**	Molecular eligibility, multiple modalities, sequencing, one-time versus repeatable therapy, patient preference, risk, burden and meaningful benefit.
10:45-11:15	**Networking break**	Time to connect on ecosystem, HTA and targeted-therapy implications.
11:15-12:00	**Accountability + partnership**	What should sustained RDDS investment demonstrate beyond spending and listings? Financial, implementation, patient/community and capability accountability.
12:00-12:45	**Canada without borders**	North American and global rare-disease collaboration: CORD-NORD, research, trials, registries, advanced therapy capacity, shared evidence and patient networks.
12:45-1:45	**Networking lunch**	Transition from plenary framing to design work.
1:45-2:45	**Design studios: What should Phase 2 build?**	Convert Day One findings and Day Two policy context into recommendation components for the next phase of RDDS.
2:45-3:10	**Priority synthesis: From findings to recommendations**	Identify the strongest Phase 2 recommendations, test them against feasibility, patient impact, equity, innovation readiness and accountability.
3:10-3:30	**Closing plenary: Phase 2 RDDS recommendations**	Present the emerging recommendation architecture and next steps for CORD's post-conference policy submission and engagement strategy.

Day Two signature session: Canada's access ecosystem is changing

This 60-minute session anchors the Phase 2 recommendations in real policy movement rather than wishful thinking. It will show that Canada is already experimenting with faster, more concurrent and more adaptive access mechanisms; the question is how rare and precision therapies can use these advantages while also building the pathway readiness required for patient benefit.

Policy element	Talking point for the session
Life Sciences & Pharmaceutical Task Force	Positions patient access, launch attractiveness and innovation investment as linked priorities; supports more predictable, concurrent and competitive processes.
Health Canada modernization	Reliance, international collaboration and accelerated/concurrent pathways can help therapies enter Canada sooner; readiness must move with regulatory speed.

Policy element	Talking point for the session
pCPA ENP + Ontario FAST	Shows that the traditional sequential access pathway can be compressed; the rare-disease question is which therapies should qualify and what patient/pathway evidence must move earlier.
CORD CDA HTA evolution review	The evidence anchor: how rare-disease HTA, uncertainty, patient evidence and patient participation have changed over time - and where assessment still needs to connect more directly to real pathway implementation.

Day Two proposition

Phase 2 RDDS should not invent a parallel access system. It should connect dedicated rare-disease investment to reforms already underway and ensure that when Canada becomes faster for rare and precision therapies, it is also ready to find, treat, support and learn from the patients those therapies are meant to serve.

Design studios

Turning roundtable evidence into Phase 2 recommendation components

The Design Studios will not reopen the entire rare-disease agenda. They will start from the Day One readiness findings and Day Two ecosystem context, then ask what a future RDDS can realistically assess, enable and hold accountable.

Studio theme	Core design question
Finding and diagnosing the right patient	Case-finding, screening, genomic/molecular testing, confirmatory diagnosis and referral triggers.
Expertise and comprehensive care	Specialist networks, hub-and-spoke models, integrated care, transition and local-system interfaces.
Timely therapy entry and access	Regulatory/HTA/negotiation alignment, eligibility, interim access, provincial implementation and equity.
Patient information and treatment choice	Shared decision-making, preferences, permanence versus repeatability, risk tolerance, burden, fertility and social supports.
Advanced therapy delivery	Gene/cell therapy capacity, hospital infrastructure, safety, follow-up and cross-provincial referral.
Data, outcomes and learning	Registries, RWE, patient-relevant outcomes, durability, safety, implementation review and public reporting.
Patient organization capability	Where patient groups catalyze readiness and where that work should become shared or publicly supported infrastructure.
National, North American and global collaboration	What Canada must own, what can be shared with the US/NORD and global networks, and where international evidence can support Canadian readiness.

Draft recommendation architecture to be tested

- Continue dedicated RDDS funding for rare-disease therapies and position Canada as a reliable, attractive market for rare and precision innovation.

- Introduce a pathway-readiness assessment for major new rare or targeted therapies, proportionate to the disease, therapy and population.
- Allow RDDS implementation support for enabling capabilities when they are necessary for appropriate use of a funded therapy.
- Embed patient agency and partnership in priority setting, pathway design, treatment-value assessment, implementation and review.
- Require transparent reporting on allocations, listings, implementation, patient access, pathway capability and patient-relevant outcomes.
- Use registries, RWE and longitudinal follow-up to make RDDS a learning strategy, not a one-time reimbursement program.
- Create mechanisms for national, North American and global collaboration where Canada cannot or should not build every capability alone.

Closing plenary: recommendations with a clear line of evidence

The conference should close with a visible chain from evidence to recommendation: RareCare provided an external evaluation lens on the delivery gap; six Canadian learning cases generated archetypes; Advisor and conference roundtables stress-tested them; Day Two policy sessions showed which system reforms can carry the recommendations; Design Studios converted findings into Phase 2 proposals. The closing is therefore not a summary of themes but a preview of CORD's post-conference recommendation package.

Closing test

Can each proposed recommendation answer three questions? What problem did the pathway evidence reveal? What should Phase 2 RDDS do about it? How would patients, governments and partners know whether it worked?

Expected conference outputs

What sponsors and partners are helping CORD produce

Output	Description
Next-generation RDDS framework	What should be preserved, strengthened and added in a future phase of the Rare Disease Drug Strategy.
Rare/precision pathway archetypes	A practical typology of readiness patterns that can be applied to incoming therapies.
Roundtable readiness findings	Structured findings from challenge cases, including RDDS Common List and other emerging rare/targeted pathways.
Patient agency + partnership recommendations	How patient communities should participate in priority setting, implementation design, evidence development and accountability.
Accountability framework	How public investment should report financial, implementation, pathway and patient impact.
Phase 2 recommendation package	A focused set of policy and implementation recommendations for federal, provincial, agency and partner engagement after the conference.

What remains deliberately open

The program is designed to preserve flexibility until the evidence and policy environment are clearer. Specific speakers, exact challenge cases and final recommendations will be refined based on the RDDS evaluation, funding clarity, webinar findings, Readiness Advisor input and sponsor/partner engagement. This is a strength of the process: CORD will arrive in November with a clear method, but not with conclusions that ignore new evidence.

Program development inputs

- Federal RDDS evaluation and any additional public reporting on Phase 1 implementation.
- CORD Rare Disease-Drug Pathway Dialogues and refined provisional profiles.
- Readiness Advisor conversations and written readiness challenges.
- LDSF Canada and CDKL5 readiness cases.
- Developments related to future RDDS funding, Life Sciences/Pharma Task Force response, Health Canada modernization, pCPA ENP and Ontario FAST.
- CORD's CDA rare-disease HTA evolution review.
- Input from patients, clinicians, policymakers, agencies, researchers, industry and partners.

Final positioning

Next Generation Rare Canada is designed to move CORD and the rare-disease community from reaction to readiness: using Canadian experience to shape Phase 2 RDDS before the next phase is finalized, and ensuring patients and patient organizations are recognized as essential partners in access, implementation and accountability.