Canadian Organization for Rare Disorders

Fall Conference 2023

Community – Created:

Canada's Rare Disease Network:

Top-Down, Bottom-Up and Coast-to-Coast

November 29-30, 2023

Delta Calgary Downtown
209 4th Ave SE, Calgary, Alberta

Draft Conference Agenda

We envision Canada's Rare Disease Network as a dynamic collaboration, centred on optimizing patient care, but assuring sustainability by addressing the interests of all stakeholders. Canada's RDN draws upon learnings and best practices from other jurisdictions and other disease areas but is customized for Canada's unique healthcare ecosystem and the unique characteristics of rare diseases. Most importantly, Canada's RDN is uniquely built from the ground up and supported from the top down.

This conference continues CORD's 10+ years of consultations, initially to engage the community to collectively create a rare disease strategy, which had a HUGE role in prompting "top-down" responses, starting with the Ontario Rare Disease Strategy, Quebec's Rare Disease Policy, and the federal government's Rare Disease Drug Strategy. Now, we are at an inflection point where we can move from designing and strategizing to bringing the network to life and assuring sustainability.

Our guiding principles:

- All stakeholders participate as equal partners.
- RD Network evolves with new opportunities and new challenges.
- Outcomes across RD Network are measured to guide investment, demonstrate value and assure sustainability.

Day 1

Wednesday, November 29 (8:30 AM - 5:00 PM)
Implementing Canada's Rare Disease Network and Drug Strategy

8:30 am – 9:00 am
Registration and Continental Breakfast
Delta Calgary Downtown – Glacier Ballroom (2nd Floor)

9:00 am – 9:45 am
Welcome and What We Can Achieve Together Over Two Days

- Welcome: Durhane Wong-Rieger, CORD President & CEO (15 min)
- Opening Address: Welcome: Tom Kmiec, MP, Calgary Shepherd, Alberta (5 min)
- Welcome: Susanne Benseler, Alberta Children's Hospital (10 min)
- Welcome: Gail Ouellette, RQMO (10 min)

9:45 am – 10:30 am
How Patients Are Shaping Canada's Rare Disease Ecosystem
How are "grass-roots" patient organizations led by patients and parents driving advances in rare disease diagnosis, care and treatment? In the context of addressing individual challenges, what do you feel are some ways that you have contributed to improving care in your disease area and beyond?
Moderator: Durhane Wong-Rieger
Panel Discussion (10 mins each):

- Stephen Parrott, Canadian VHL Alliance
- Karen Kelm, Fragile-X Canada
- Maureen Smith, INFORM RARE
- Jida El-Hajjar, ALS Action Canada

10:30 am – 10:45 am
Break

10:45 am – 12:00 pm
Canada's Rare Disease Landscape (Presentation & Discussion)
Moderator: Bill Dempster, 3Sixty Public Affairs

- Caring for Rare: Canada's Health Policy Landscape
    - Peter Cleary, Santis Health (15 min)
- Health Canada: Update on Rare Disease Drug Strategy
    - Jennifer Grandy, Health Canada (10 min)
- Canadian Blood Services: Learnings from national program
    - Graham Sher (10 min)
- Canadian Hemophilia Society: Insights from 70 years of Engagement
    - David Page (10 min)
- Reflections Panel (20 min)
    - Carrie McElroy, Sanofi; Walter Robinson, CORD; Shikha Virdi, Roche

12:00 pm – 1:00 pm
Lunch

1:00 pm – 2:00 pm
Optimizing Rare Patient Journey
What are the building blocks toward optimizing the patient journey and how can we build on those in Canada and elsewhere? What are the tools for measuring the impact of rare diseases and the benefits of diagnosis and treatment? How can we better capture and utilize patient data?

Moderator: Durhane Wong-Rieger

- Documenting Socioeconomic and Quality of Life Impact of Neuromuscular Disorders
    - Homira Osman, Muscular Dystrophy Canada (15 min)
- Patient Data Platforms (aka Patient Registries) to Capture Patient Input
    - Jason Colquitt and Andrea Rogers, Access Healthcare (15 min)
- Framework for Coordinated Lifecycle Clinical Trial Design: From Discovery to Real-World Monitoring to Continuous Improvement
    - Susan Marlin, Clinical Trials Ontario (15 min)
- Reflections Panel (15 min)
    - Paul de Zara, Takeda; Cathy Evanochko, TSC Canada, Alison Drinkwater, Innomar Strategies

2:00 pm – 3:00 pm
Canada's Rare Disease Network of Expertise (Presentation & Discussion)

- No Family Left Behind: Canadian Rare Disease Network
- RD Networks of Expertise: Susanne Benseler, François Bernier & team
    - Comprehensive multi-disease centers, Specialty disease clusters, Hub and Spoke Model assuring continuous expert-managed care by specialist associated with centralized "hubs" and community-based, general health practitioners and allied healthcare professions identified with remote "spokes" (15 min)
    - Value of Genomic Sequencing: Taila Hartley, CHEO, Care for Rare Canada (10 min)
    - Driving toward Consensus on Optimizing Patient Care Pathway: Kim McBride, Alberta Children's Hospital (10 min)
    - Unique Challenges of Diagnosis and Care for Adults with Rare: Nowell Fine, University of Calgary (10 min)
    - IRare: Supporting the whole RD community: Gail Ouellette, RQMO (5 min)
- Reflections Panel (10 min)
    - Alexandra Chambers, Bayer Canada, Alice Williams, Wilson Disease Association; Christine White, National Gaucher Foundation of Canada

3:00 pm – 3:15 pm
Break

3:15 pm – 4:55 pm
Canada's Rare Disease Landscape Creating Knowledge and Cures
Moderator: Bill Dempster

- Determining Socioeconomic Impact of Rare Disease
    - Gillian Currie, University of Calgary (15 min)
- Artificial Intelligence to accelerate diagnosis and disease knowledge)
    - François Bolduc (University of Alberta) (15 min)
- What makes some patients extraordinary responders to rare disease drugs and others experience serious drug-induced harm? New genomic research aims to understand the underlying biology of these outcomes to improve the benefits of these drugs to more patients.
    - Bruce Carleton, University of British Columbia (15 min)
- Cell Therapies
    - Jan-Willem Henning, University of Calgary (15 min)
- Gene therapies
    - Jim Dowling, Hospital for Sick Children, Toronto (15 min)
    - Nicola Wright, Alberta Children's Hospital (15 min)
- Reflections Panel (10 min)
    - Fred Little, Pfizer Canada; Sara Ethier, Cassie and Friends; Rebecca Yu, McKesson

4:55 – 5:00 pm
Wrap Up Day 1 and Anticipating Day 2

5:00 pm – 7:00 pm
Networking Reception – Glacier Foyer (2nd Floor)
All conference participants are invited to attend.

Day 2

Thursday, November 30 (8:30 AM - 3:00 PM)
Implementing Canada's Rare Disease Network and Drug Strategy

8:30 am – 9:00 am
Registration and Continental Breakfast
Delta Calgary Downtown – Glacier Ballroom (2nd Floor)

9:00 am – 9:35 am
Welcome: What We Can Achieve in Two Days
- Welcome and Day 1 Highlights: Durhane Wong-Rieger (5 min)
- Vision for Rare Care in Quebec: Michèle de Guise, INESSS (15 min)
- What We Mean When We say "Uncertainty" and What to Do About It: Kate Harback, Institute of Health Economics (15 min)

9:35 am – 11:00 am
Canada's Rare Disease Drug Landscape (Presentation & Discussion)
Moderator: Bill Dempster

- Health Canada Regulatory Innovation: Implications for Rare Disease Therapies: Sophie Sommerer, Health Canada (15 min)
- CADTH: Initiatives to accelerate access to drugs for patients with unmet needs, to manage access in situations with uncertainties, to collect and use RWD/RWE to understand benefits/risks, modify access, and determine value: Trish Caetano, CADTH (15 min)
- Canadian Rare Disease Therapeutic Access and Budget Impact: Lindy Forte, EVERSANA (15 min)
- Pharmaceutical Landscape: initiatives to provide timely access to clinical trials, early and sustainable compassionate access, patient support to manage and report benefits and reports and make appropriate decisions for optimal use
    - RD Therapeutic Pipeline & Access: Bob McLay, Sobi (15 min)
    - Cell and Gene Therapies: Jason Brown, Novartis (15 min)
    - Research and collaboration from clinical trials to funded access to treatments: Gaby Bourbara, Alexion (15 Min)

11:00- 11:15 am
Break

11:15 am – 12:20 pm
Canadian Integrated Lifecycle Drug Development, Access, and Management Model for Rare Disease Populations
Moderator: Bill Dempster

Charting the pathway including: natural history studies, understanding origin and causes of disease, screening, diagnosis, and enrolment in patient registries, drug discovery and development (academic and commercial), (novel) clinical trial design and implementation, early, agile and adaptive regulatory evaluation practices, appropriate access pathways (compassionate, early, criteria-based managed access programs), real-world patient data collection and analysis, reassessment, and valuation (pricing and reimbursement). Framework for Accelerated Sustainable Drug Access

- Framework for Accelerated Sustainable Access to Innovative Rare Disease Therapies: Negotiations with all stakeholders including patients, families, HCPs, payers, and developers to optimize patient access and achieve value for all. (15 min)
- Patient Data Platforms: Platforms to collect real-world patient data that is "owned" by the patient and allows for direct patient use and

integrates across other patient registries, clinician-managed healthcare records, electronic health and hospital records, and other data repositories storing patient information

    - Jason Colquitt/Andrea Rogers, Access Healthcare (15 min)

- Real World Data and Real World Evidence: Contributions to Optimal Drug Usage
    - Tara Cowling, Medlior (15 min)
- Challenges and Success with Win-Win Negotiations
    - Sang-Mi Lee, Morse Consulting (15 min)
- Reflections Panel (10 min)
    - Fred Horne, 3Sixty Public Affairs; Carla Charbot, CORD; Riyad Elbard, CORD; Donna Lawrence, PDCI

12:20 pm – 1:15 pm
Lunch

1:15 pm – 2:45 pm
Negotiations for Accelerated & Sustainable Access
Moderator: Durhane Wong-Rieger & Bill Dempster

Question: In What Ways can Canada accelerate "sustainable" access (effective and cost-effective) to innovative therapies for rare disease patients?

- Five case studies of HTA recommended therapies with initiation and discontinuation criteria for rare diseases with urgent unmet needs (Sohonos, Camyzos, Koselugo, Evzeeka, Welireg)
- Framework for Reasonableness: all-stakeholder negotiation process toward consensus on mutually beneficial (acceptable) outcomes
- Implementation challenges and how to address within pan-Canadian health system with available and acquirable resources
- What does "success" look like?
- Additional cases for "extended" indications (Trikafta for rare CF mutations; Risdiplan for SMA adults >25 years)

Panel:
Tara Cowling, Fred Horne, Michèle de Guise, Trish Caetano, Kate Harback, Sang-Mi Lee, François Bernier, Kim McBride, Tyler Rogers (Ultragenyx), Cory Cowan (Alexion), Yulia Privolnev (Ipsen), Stephen Parrott, Linda Toews (Soft Bones Canada), Alexandra Chambers, Vicky Maldonado, Beth Vanstone (CF advocate), Chris Nicholls (Merck), Tarang Manchanda (Roche), Susi Vander Wyk (Cure SMA Canada), Imran Ali (BMS)

2:45 pm – 3:00 pm
Next Steps

Conference Sponsors

We acknowledge the contribution of all our Corporate Partners to improving the lives of patients and families with rare disorders. The CORD Fall Conference 2023 is made possible by the generous support of the following organizations:

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