#RARE DISEASE DAY

# Rare Disease Day 2025 Conference

## April 29 - 30, 2025
## Calgary, Alberta

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CORD Canadian Organization for Rare Disorders

RARE DISEASE DAY®

# Reimagining Rare: Optimizing the Patient Journey from Diagnosis to Treatment

In these tumultuous times of political, economic and social uncertainty, what IS certain is that the Canadian rare disease community has an ADDITIONAL $1.5 billion to improve access to drugs. The bulk of the funds, $1.4 B, is being allocated by provinces and territories through bilateral agreements. However, there is limited guidance on how the monies should be dispensed to optimally benefit the rare disease community and, by extension, return value to the whole of society. Toward those ends, the rare patient community (led by the Canadian Organization for Rare Disorders), the healthcare sector (under the umbrella of the Canadian Rare Disease Network), and pharmaceutical companies (collectively through RAREi, the Canadian Forum for Rare Disease Innovators) as well as academics and policy makers, have come together to develop and help implement strategies and initiatives that will assure investment of the $1.5 B drug strategy funds will generate optimal return for persons living with rare disease, for health systems, for manufacturers, and society and justify the continued investment.

This Rare Disease Day Conference 2025 builds on the progress we are making together.

## Rare Disease Day Conference 2025

### Reimagining Rare: Optimizing the Patient Journey from Diagnosis to Treatment

## Agenda

### Tuesday, April 29, 2025

**8:30 am – 9:00 am**
Registration and Breakfast
Calgary Westin Downtown – Mayfair Ballroom

**9:00 am – 9:15 am**

- Welcome – Durhane Wong-Rieger, CORD
- Land Acknowledgement – Cathy Evanochko, CORD/TSC
- Political Greetings – Tom Kmeic, MP Calgary Shepard (TBC)
- Seizing the Opportunity to Re-Imagine Rare – Durhane Wong-Rieger; Bill Dempster, 3Sixty Public Affairs

**9:15 am – 9:45 am**

A. Inside Change: How Patient Advocates Provoked and Leveraged Change in Cancer and Blood Systems – Aslam Bhatti, University of Alberta and Durhane Wong-Rieger

**9:45 am – 10:00 am**

B. Perspective: How Industry is Enhancing its Role in Rare – Karen Heim, Alexion

**10:00 am – 11:00 am**

C. Presentation & Discussion: Integrated Comprehensive Care Models
Moderator: Jida El Hajjar

- International Rare Disease Centres of Excellence – Kim McBride, Alberta Children's Hospital
- Navi-Nat: Province-Wide Navigation for Timely Diagnosis of Rare Diseases – Anne-Marie Laberge, CHU Sainte-Justine and Université de Montréal
- Rare and Medically/Socially Complex Care Centres – Tessa Diaczun, BC Children's Hospital (virtual)
- Reflections: Darlene Schopman (virtual), Lisa McCoy, Carla Chabot, CORD

**11:00 am – 11:15 am**
**Break**

**11:15 am – 12:00 pm**

D. Presentation & Panel: Has National Strategy for Drugs for Rare Diseases Improved Patient Access?
Moderator: Durhane Wong-Rieger

Presentation: (20 min)

- Yes, We CAN Monitor Patient Outcomes: Example of Welireg for Von Hippel-Lindau disease – Maryam Soleimani, BC Cancer (virtual)

Panel Discussion:

- Panelists: Susi Vander Wyk, CureSMA Canada; Karen Munro, Canadian FOP Network; Jennifer Adams, Physician & PH1 Mom; – Mina Rajan, Answering TTP; Lori Brown, BDSRA; Monty Keast, Ultragenyx; Lindsay Williamson, CORD; Julie Schneiderman, Theratechnologies
- Therapies Hitting Roadblocks and Speedbumps: Risperdal/Spinraza for Adult SMA; Sohonos for FOP; Evkeeza for HoFH; Brineura for CLN2; Voxzogo for achondroplasia; Oxlumo for PH1

**12:00 pm – 1:00 pm**
**Lunch**

**1:00 pm – 2:10 pm**

E. Presentation & Panel: (How) Can We Achieve Fair, Rational, and Equitable Access to Rare Disease Drugs

   Moderator: Dev Menon, University of Alberta

   - Tania Stafinski, University of Alberta
   - Julie Schneiderman, Theratechnologies
   - Peter Kannu, University of Alberta, Department of Medical Genetics
   - Durhane Wong-Rieger, CORD
   - Kevin Wilson, Former Public Payer, Saskatchewan

**2:10 pm – 3:00 pm**

F. Presentation & Panel Canada's Rare Disease Framework

   Moderator: Sara Ethier

   - Canadian Rare Disease Network: Origins, Structure, Strategic Plan – François Bernier, University of Calgary (15 mins)
   - Rare Kids Can: Stimulating and Coordinating Research and Clinical Trials – Thierry Lacaze/Breanne Stewart, RareKids-CAN (15 mins)
   - Newborn Screening – Hilary Vallance, BC Children's Hospital (virtual) (15 mins)
   - Q&A

**3:00 pm – 3:15 pm**
**Break**

**3:15 pm – 4:15 pm**

G. Connecting to Care and Support

   Moderator: Jida El Hajjar

   - Precision Kidney Clinic – Louis Girard, University of Calgary
   - Epilepsy Care for Rare and Not Rare – Felippe Borlot, University of Calgary
   - Huntington Clinics and Family Service Teams – Shelly Redman, Huntington Society of Canada
   - Support Networks for Ultra-Rare – Jida El Hajjar, Loeys-Dietz Syndrome Foundation Canada; Karen Munro, FOP Network; Mina Rajan, Answering TTP; Janelle Kujawa, Debra Canada; Julie Schneiderman, Theratechnologies

**4:15 pm – 4:45 pm**

Reflections: Successes, Learnings, Unanswered Questions, Opportunities, and Challenges
Moderator: Bill Dempster

- Panel: Aslam Bhatti, Tania Stafinski, Angela Behboodi, Amgen Canada; Jida El Hajjar

**5:00 pm – 7:30 pm**

**Networking Cocktail Reception -** Bonavista/West Foyer
All conference attendees are welcome to attend.

## Wednesday, April 30, 2025

**8:30 am – 9:00 am**

Registration and Breakfast
Calgary Westin Downtown – Mayfair Ballroom

**9:00 am – 9:45 am**

A. Day 2: What We Imagine Together, We Can Do
   - Introduction to Day: Durhane Wong-Rieger (5 min)
   - Perspective: Maureen Smith, Canada's Drug Agency (10 min)
   - Rare Moms: Darlene Schopman (virtual), Jennifer Adams, Lori Brown, Christine White, Alice Williams (30 min)

**9:45 am – 10:30 am**

B. Achieving Optimal Outcomes from National Strategy for Drugs for Rare Diseases (NSDRD) Bilateral Agreements: What is IN and What is NOT?
   Moderator: Walter Robinson

   - Timelines to Access: Katherine Scott, Morse Consulting
   - Panelists: Katherine Scott, Morse Consulting; Peter Brenders, TO4 Group Inc; Kevin Wilson, Former SK Public Payer; Bennet Lee, Sanofi; Sara Ethier, CORD

**10:30 am – 10:45 am**

**Break**

**10:45 am – 11:05 am**

C. Smart Solutions for Rare Conditions: AI, Synthetic Data, and the Future of Access
   - Rebecca Marsh, Institute of Health Economics

- Myrna Bittner, RUNWITHIT Synthetics

**11:05 am – 12:30 pm**

D. Hiding in Plain Sight: Persons at Risk for Rare Disease
   Moderator: Durhane Wong-Rieger
   - ThinkRare Diagnosing the Undiagnosed – Kym Boycott, CHEO
   - Acute Care Alberta - Saventic Health project – Kim King, Saventic Health
   - Facial Imaging – Benedik Hallgrimsson, University of Calgary
   - Engaging Dentists in Early Detection of Rare – Daniel Graf, University of British Columbia
   - Integrating Phenotypic and Genomic Data – Orion Buske, PhenoTips

**12:30 pm – 1:30 pm**
**Lunch**

**1:30 pm – 2:15 pm**

A. Integrated Comprehensive Rare Disease Care and Treatment: Making Personalized Therapy Available for All
   Moderator: Kate Harback, Institute of Health Economics
   - Panelists:
     - Ex Payer: Chad Mitchell, Global Public Affairs
     - Industry: Philippe Hebert, CSL Behring
     - Patient Group: Susi Vander Wyk, CureSMA Canada

**2:15 pm – 3:15 pm**

B. All About Data
   Moderator: Durhane Wong-Rieger
   - Real-World Patient Monitoring through Patient Support Programs – Alison Oliver, Cencora; Andrew McElroy, McKesson Specialty Health (15 min)
   - Patient Registries and Real-World Evidence – Trish Caetano, Canada's Drug Agency (virtual) (15 mins)
   - Assessing the Value of Finding and Treating Rare: Social and Economic Impacts – Deborah Marshall, University of Calgary (15 mins)
   - International Learnings on RWD/RWE from Drug Development to Real-World Monitoring – Alicia Granados, Sanofi Global (15 mins)

**3:15 pm – 3:30 pm**

Summary Discussion and Next Steps – Durhane Wong-Rieger

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