

# Rare Disease Day Conference 2016

## Canada's Rare Disease Strategy: Building on Success

March 9 - 10, 2016  
Sheraton Ottawa Hotel  
150 Albert Street, Ottawa, Ontario

## Agenda

### March 9, 2016

**8:30 am - 10:00 am**

#### Plenary 1: Rare Disease Strategy: Learning from Across Canada

*Discussion Lead: Durhane Wong-Rieger, CORD*

In March 2015, CORD engaged stakeholders to critique Canada's DRAFT Rare Disease Strategy. In May 2015 we took a revised Strategy on regional tour (with a couple more stops to make). Now, in this 1st Plenary Session, a panel of key stakeholders will discuss current and emerging contexts for healthcare and how the Strategy can provide a platform for a comprehensive and collaborative national Rare Disease Program

- A. Welcome: How Rare Disease Strategy Serves All Canadians, Alex Munter, CHEO
- 1. Five (5) Strategic Pillars Integrating Rare Disease Issues, Wayne Critchley, CORD/Global Public Affairs
- 2. Feedback from the Tour: Regional Expertise and Opportunities, Durhane Wong-Rieger, CORD
- 3. Reports on Regional Roundtables:
  - o Regulatory framework and HTA, Barbara Sabourin, *Health Canada*
  - o Access to Drugs for Rare Diseases, George Wyatt, Wyatt Health
  - o Newborn screening and diagnosis, Robin Hayeems, *The Hospital for Sick Children*

**10:00 am - 10:45 am**

#### Plenary 2: Introducing Rare Alliance Canada: Vision, Rationale, Goals, Partners

- B. Keynote: Michael Apkon, *The Hospital for Sick Children*
  - o Industry Sector: Andrew Casey, *BIOTECanada*
  - o International Impact: David King, *Shire (International)*

**10:45 am - 11:00 am Refreshment Break**

**11:00 am – 12:45 pm**

### **Plenary 3: Building on Canada's Rare Expertise**

Canada has a wealth of expertise relevant to rare diseases. In this 3<sup>rd</sup> Plenary Session, a panel of healthcare leaders from across the country will speak to the successes and learning in policy, research, clinical expertise, and supportive care that can serve as the building blocks for a comprehensive and integrated national Rare Disease Program. What is the current status or “best practice” in this area as it relates to rare diseases? How would a Canadian Rare Disease Strategy contribute?

*Chair: Wayne Critchley*

- Genetics and Genomics for Diagnosis and Prevention: Ronald Cohn, *The Hospital for Sick Children [Toronto]*
- Applying Public Health Expertise to Rare Diseases: David Wilkinson and Jay Onysko, *Public Health Agency of Canada*
- Community Support: Brett Thombs, *McGill University*
- Access to therapies: Sandra Anderson, *Innomar Strategies*
- Research: Kym Boycott, *Children's Hospital of Eastern Ontario* & Safina Adata, *Canadian Institute for Health Research*

**12:45 pm – 1:45 pm Lunch**

**1:45 pm – 3:15 pm**

### **Plenary 4: Building on Canada's Rare Expertise**

Experts from various disciplines will discuss opportunities and challenges in implementing rare disease programs and the potential role for the Rare Disease Strategy and Rare Alliance Canada in applying research to improving care, treatment, and support.

*Discussion Lead: Bill Dempster*

- Metabolics Best Practice Guidelines: Clara Van Karnebeek, *BC Children's Hospital*
- Neuromuscular Disorders: Jodi Warman, *Children's Hospital of Eastern Ontario*
- Neurological and Auto-Inflammatory: Susanne Benseler, *Alberta Children's Hospital*
- Cardiovascular Diseases: Gregor Andelfinger, *Sainte Justine University Hospital Center*
- Blood and Cellular Disorders: David Page, *Canadian Hemophilia Society*
- Evidence-Informed Healthcare for Rare Childhood Diseases: Beth Potter & Julian Little, *U of Ottawa School of Epidemiology, Public Health and Preventive Medicine*

**3:15 pm – 3:45 pm**

### **Plenary 5: Patient Feedback**

A panel of patients will reflect on their experience living with a rare disease and their responses to the opportunities within a Rare Disease Strategy.

*Discussion Lead: Cathy Evanochko, Tuberosus Sclerosis Canada*

Panelists: Connie Côté, *Health Charities Coalition of Canada*, Robin Sulley, *Myeloma Canada*, Mark Williams, *Canadian MPN Network*, Mary Sunderland, *Foundation Fighting Blindness*

**3:45 pm - 4:00 pm** Summary and Set Up for Day 2

**6:00 pm - 7:00 pm** Cocktail Reception

**7:00 - 10:00 pm** Rare Disease Day Awards Gala Dinner Celebration

## **March 10, 2016**

**8:30 am - 9:00 am**

Recap of Day 1 and Objectives for Day 2

**9:00 am - 10:15 am**

**Stories of Innovation and Inspiration - Disease Knowledge, Drug Discovery, Development and Access**

- Understanding and Treating Hypophosphatasia: Canadian Contribution: Philippe Crine
- Cures Within Reach/Cures Accelerator: Safina Adatia, *Canadian Institutes of Health Research*
- Homozygous Familial Hypercholesterolemia: Rare Treatment for Rare Disease: LeAnne Bloedon, *Aegerion*

**Implementing and Measuring Success - Canada's Rare Disease Strategy**

**10:15 am - 12:00 pm**

Participants will work in small groups toward developing Rare Canada Alliance, defining “next steps” and identifying “quick wins” in the implementation of Canada's Rare Disease Strategy for each pillar. Discussants will guide participants through an international environmental scan, SWOT analysis of the Canadian landscape, measuring and identifying success to date, focused “brainstorming” of strategic opportunities, identification of strategic “next steps.”

- Screening, Diagnosis and Prevention: LEAD: Kym Boycott, *CHEO*, Chris Trevors, *Life Labs*
- Care and Centres of Expertise: LEAD: Clara van Karnebeek, BC Children's & David Page, *Canadian Hemophilia Society*
- Community Support: LEADS: Cathy Evanochko, *Tuberous Sclerosis Canada*, Isabel Jordan, *Rare Disease Foundation*
- Access to therapies: LEADS: George Wyatt/Ferg Mills, *Wyatt Health*, Maureen Smith, *CORD*, James LeBrocq, *McKesson Canada*
- Research: LEAD: Safina Adatia, *CIHR*

**12:00 pm - 12:45 pm** Working Lunch

**1:00 pm – 3:15 pm**

**Visit with the Parliamentarians**

All participants are invited to join CORD in its annual visit with the Parliamentarians to provide a “face” to rare diseases and to invite their support in moving forth on Canada’s Rare Disease Strategy. Please add your voice and in speaking up for Rare Diseases.

12:00 pm – 12:45 pm: Training over lunch for delegates of Rare Alliance Canada and scarves are handed out

12:45 pm – 1:00 pm: Delegates walk over to to Parliament Hill with scarves

1:00 pm – 1:45 pm: Delegates check-in at security and get QP passes

2:00 pm - 3:00 pm: Delegates attend Question Period and are recognized by The Honourable Mauril Bélanger via an S.O. 31 (statement)

3:00 pm: Delegates exit the building and meet in front of Peace Tower

3:15 pm: Group Photo in front of Peace Tower

3:30 pm: End of day

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