March 9-10, 2016

The MOST significant Rare Disease Day in Canada’s history will take place on March 9-10, 2016. Join the Canadian Organization for Rare Disorders (CORD) as we bring together the rare disease community to begin implementation of Canada’s Rare Disease Strategy. After four years of consultation, deliberation, and revision, it is time to move forward to realistic action guided by our collective vision, expertise, and commitment. But we need you to take an active role to help make Canada’s Rare Disease Strategy a reality for 2.8 million Canadians living with a rare disease.

Introducing! Rare Alliance Canada

In 2016, the Canadian Organization for Rare Disorders is launching Rare Alliance Canada, which will:

- Build on existing expertise, resources, infrastructure, and action.
- Provide the direction and blueprint for engaging, connecting, and leveraging all of these into a world-class national system for all Canadians affected by rare diseases.

Join the discussion on:

1) A revised framework for implementing Canada’s Rare Disease Strategy
2) Rare Alliance Canada’s guiding vision and leadership
3) Existing platform of expertise, experience, and achievement
4) Canada’s Rare Disease Ambassador Program
Agenda

DAY 1: March 9, 2016

8:30 am – 10:00 am

Plenary 1: Rare Disease Strategy: Learning from Across Canada

Discussion Lead: Durhane Wong-Rieger

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, CHE

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:
   - Newborn screening and diagnosis, Robin Hayeems, The Hospital for Sick Children
   - Regulatory framework and HTA, Barbara Sabourin, Health Canada
   - Access to Drugs for Rare Diseases, Robert Tam, Genzyme Canada

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

   - Industry Sector: Andrew Casey, BIOTECanada
   - International Impact: David King, Shire (International)

10:45 am – 11:00 am Refreshing Break
11:00 am – 12:45 am

**Plenary 3: Building on Canada’s Rare Expertise**

Canada has a wealth of expertise relevant to rare diseases. In this 3rd 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: *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 Adatia, *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: Alex MacKenzie, *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, *Children’s Hospital of Eastern Ontario*

**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, Tuberous Sclerosis Canada*
Panelists: Connie Côté, Health Charities Coalition of Canada, Robin Sulley, Myeloma Canada, Mark Williams, Canadian MPN Network, Deanna Badiuk, Pituitary Network Canada

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

**March 9, 2016**

6:00 pm – 7:00 pm Cocktail Reception

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

*Rare Diseases, Rare Women, Rare Commitment*

**DAY 2**

**March 10, 8:30 am – 12:00 pm**

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 Hypophosphotasia: Canadian Contribution: Philippe Crine
- BTG International Medicine (TBC)
- Cures Within Reach/Cures Accelerator: North American Re:Rare: 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**

Mar 10: 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 – 1:00 pm Working Lunch

March 10: 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 – 1:00 pm: Training over lunch for delegates of Rare Alliance Canada
1:00 pm – 2:00 pm: Delegates go to the Hill for 1:00 tour of the House of Commons (with their scarves)
2:00 pm: Scarves are handed out just in advance of QP to MPs on Thursday early afternoon
2:15pm - 3:15 pm: Delegates attend Question Period and are recognized by an MP via an S.O. 31 (statement)
3:15 pm: End of day
Rare Disease Day GALA 2016

Rare Diseases, Rare Women, Rare Commitment

March 9, 2016
6:00 pm Wine Bar Reception
7:00 pm Dinner

A growing number of elected and appointed leaders are advancing policies and programs to address the needs of the nearly 3 million Canadians with rare diseases. Among these are some truly exceptional women. In recognition, the Canadian Organization for Rare Disorders is inaugurating “Rare Diseases, Rare Women, Rare Commitment,” honouring “rare” women whose commitment over the years has significantly improved the lives of Canadians living with rare diseases.

CORD invites all Canadians to join us in recognizing these “Rare Women” and other “Rarity Heroes” at our Rare Disease Day Gala in Ottawa. The celebration will be even more special in 2016, with Rare Disease Day actually taking place on March 9th.

Exceptional women from the Parliament, Ministry, and community have been chosen for public acknowledgement at this event. We will also celebrate some exceptional rare young people.

In recognizing these very special women and children, CORD also acknowledges so many others who have contributed to helping Canadians with rare diseases live longer, healthier, and happier lives. In that spirit, each year, “Rare Diseases, Rare People, Rare Commitment” will allow CORD to recognize those whose contributions demonstrate the value of committed collaborative action. These are the underpinnings of Canada’s Rare Disease Strategy.

Awards will be presented at the Rare Disease Day Gala starting with a wine bar reception at 6:00 pm followed by dinner at 7:00 pm, with entertainment from the Cross Town Youth Chorus.