Canadian Organization for Rare Disorders

Rare Disease Day Conference 2020

March 9 - 10, 2020
Delta Hotels Ottawa City Centre

Every four years we celebrate Rare Disease Day on that very rare date, February 29th. This year, 2020, the Canadian Organization for Rare Disorders, along with the entire rare disease community, celebrates the 13th International Rare Disease Day BUT only the 4th REAL Rare Disease Day. For CORD, 2020 is an important year for another reason. Five years ago, CORD launched Canada's Rare Disease Strategy in collaboration with multiple stakeholders. In 2020, we will update the strategy and action plans based on progress to date and identification of opportunities, priorities and synergies.

The year, 2020, is also the Chinese Year of the Rat … the most auspicious of years, signaling the start of a new Zodiac Cycle and also a time for "new beginnings." Throughout 2020 and 2021, CORD will be bringing together all partners and stakeholders to build **Canada's Rare Disease Drug Strategy**, to be ready for implementation when federal government's promised $1 billion fund becomes available. We will use this time to "pilot test" the Canadian "supplemental process" for access to rare disease drugs and develop processes and templates for implementation.

We begin the process at our 2020 Rare Disease Day Conference. It marks the beginning of two years of intense work and we invite all stakeholders to be fully engaged to deliver on the promises that will improve the lives of all Canadians affected by rare disease.

Agenda

Monday, March 9, 2020

Bringing Canada's Rare Disease Strategy to Life

8:00 a.m. – 8:30 a.m.	**Registration and Breakfast**	Ballroom B/C
8:30 a.m. – 9:00 a.m.	**Welcome and Objectives** Durhane Wong-Rieger, CORD	
9:00 a.m. – 10:20 a.m.	**Opening Plenary: Building on Success - 5 years of "Rare" Progress** **Research:** Cheryl Rockman-Greenberg, Max Rady College of Medicine, University of Manitoba **Health System:** Alex Munter, CHEO	**Moderator:** Durhane Wong-Rieger

	Industry: Pamela Fralick, Innovative Medicines Canada **Patient Group:** Doug Earle, Fighting Blindness Canada	
10:20 a.m. – 10:45 a.m.	Networking Break	
10:45 a.m. – 12:00 p.m.	Expert Panel: Advances in Care for Rare (Patients and Families) Breaking Barriers to Health Data: Global Federated Data Systems Precision Diagnosis: Bringing Genomic Sequencing to Clinical Care Centres of Excellence Support Programs	**Moderator:** Chris McMaster, CIHR **Panelists:** Kym Boycott, CHEO; Sophie Bernard, Montreal Clinical Research Institute; Sandra Anderson, Innomar Strategies
12:00 p.m. – 1:00 p.m.	Lunch	
1:00 p.m. – 2:00 p.m.	Regulatory Update Panel An overview of all Health Canada policies supporting access to Drugs for Rare Diseases, including regulatory pathways and support for innovation, patient engagement, Special Access Programs, aligned HC/CADTH/INESSS, international harmonization, post-market monitoring, support for patient registries, current status and relevance of biosimilars for rare disease patients	**Moderator:** Oxana Illich, IQVIA **Panelists:** Megan Bettle, Health Canada; Elizabeth Toller, Health Canada; Carole Legare, Health Canada
2:00 p.m. – 3:00 p.m.	Innovative Research Capacity Panel What are the uniquely Canadian research and development engines driving new knowledge and discoveries in health sciences? What are current and emerging fields of expertise? How can we further enhance and leverage the capacity in Canada health sciences?	**Moderator:** Marc LePage, Former President & CEO Genome Canada **Panelists:** Jason Field, Life Sciences Ontario; Bettina Hamelin, Genomics Ontario; Frank Béraud, Montreal InVivo
3:00 p.m. – 3:45 p.m.	Support for Quality of Life Support for Children and Families Peer Mentoring: Transition from Adolescent to Adult Care	**Moderator:** Maureen Smith, CORD **Panelists:** Beth Potter, University

		of Ottawa; Cathy Evanochko, CORD & Tuberous Sclerosis Canada
3:45 p.m. – 4:00 p.m.	**Wrap-Up and Preparation for Day 2 Workshops**	All

Please note: Evening events below are separate ticketed events, which are **not** included with the conference registration fee.

6:00 p.m. 7:00 p.m.	**Wine Reception (Foyer - Ballroom B/C)** **Awards Dinner Gala Celebration (Ballroom B/C)**

Tuesday, March 10, 2020
Rare Disease Drug Strategy - Access to Drugs for Life

8:30 a.m. – 9:00 a.m.	**Breakfast**	Ballroom B/C
9:00 a.m. – 9:05 a.m.	**Welcome and Recap from Day 1**	Durhane Wong-Rieger
9:05 a.m. – 10:15 a.m.	**Innovative Medicines for Rare Diseases – Future is Now** Made in Canada Innovative Drug Industry Data Registries for Research and Care: Advances for Alpha-1 Antitrypsin Deficiency Advancing therapy for neuromuscular and other rare diseases: from gene identification to drug repurposing	**Moderator:** Jane Farnham, BioScript Solutions **Panelists:** Cate McCready, BIOTECanada; Ken Chapman, University Health Network; Alex MacKenzie, Children's Hospital of Eastern Ontario
10:15 a.m. – 10:30 a.m.	**Networking Break**	
10:30 a.m. – 11:45 a.m.	Bringing RD Medicines to Canada – or Not? Facilitated Panel Discussion Issues for Deliberation: • Is the only route to closing the gaps to universal drug coverage transition to a totally publicly funded drug program,	**Moderator:** Wayne Critchley, Global Public Affairs **Panelists:** Durhane Wong-Rieger; Tania Stafinski, University of Alberta; Robin Sully,

	imposition of steep price reductions, and discouraging entry of innovative medicines? • Impact of Sweeping Changes in Pricing Regulations: Are there rational guidelines to implementation of new PMPRB regulations? Or maybe not? • Can OLD Pharmacoeconomics models be reformulated to provide guidance to value of NEW medicines? If not, what are alternative value-based pricing models? • Should Drugs for Rare Diseases have a distinct pricing pathway? If so, what is included and how should they be evaluated?	Myeloma Canada; Neil Palmer, PDCI Market Access Inc.
11:45 p.m. – 12:30 p.m.	**Fight for Our Lives Rally** Join us along with patients, families and supporters to raise our voice on Parliament Hill	Centennial Flame, located in front of Center Block, House of Commons on Parliament Hill (111 Wellington Street)
12:30 p.m. – 1:15 p.m.	**Lunch**	
1:15 p.m. – 2:30 p.m.	**Provincial/Territorial Pathway for Managed Access to Rare Disease Drugs** Case Studies: Lessons learned, Best Practices, and Promising Approaches Managed Access Framework: Getting Started Toward Getting It Right	**Leads:** Dev Menon and Tania Stafinski, PRISM
2:30 p.m. – 3:30 p.m.	**The Fight for Our Lives** Rare diseases forced researchers to reimagine drug discovery, manufacturers to redesign clinical trials, regulators to reframe risks-benefits trade-offs, clinicians to redefine practice guidelines, and payers to reformulate cost-effectiveness and willingness-to-pay thresholds. This multi-stakeholder panel will identify **key learnings** from pivotal	**Moderator:** Bill Dempster, 3Sixty Public Affairs **Interactive Panel:** Fred Horne, Former MOH Alberta, Brent Fraser, CADTH; Karen Voin, CLIHA; Fred Little, Pfizer Canada; Biba Tinga, Sickle Cell Disease Association of

	drugs for rare disease exemplars (LSDs, blood disorders, metabolic disorders, cystic fibrosis, neuromuscular disorders, cardiovascular conditions) and apply them to **next-generation therapies** (combinations, precision medicines, and curative cell/gene therapies) within the context of the **evolving Canadian drug environment** (PMPRB changes, National Pharmacare, evolving private drug plans, managed access schemes, high up-front costs, and real-world evidence).	Canada; Chris MacLeod, Canadian Cystic Fibrosis Treatment Society
3:30 p.m. – 4:15 p.m.	**665 Days to Realization of Canada's $1 Billion Dollar Rare Disease Fund: Setting a Course of Action** **Multi-stakeholder Panel**	All
4:15 p.m. – 4:30 p.m.	**Next Steps**	All

The Canadian Organization for Rare Disorders acknowledges the contribution of all our Corporate Partners to improving the lives of patients and families with rare disorders.
We are especially grateful to the following that have supported this conference.