

Rare Disease Day 2017



March 30 - 31, 2017

**Sheraton Vancouver Wall Centre
1088 Burrard Street, Vancouver, BC**

Living Rare: With Research, Possibilities are Limitless

With Patients, Anything Imaginable is Achievable!

Objectives: Rare Disease Care by Design

- Experience of living rare --- from long, winding patient journeys to patient-centred roadmaps
- Connecting the community: from serendipitous to deliberately coordinated care pathways
- Blueprint for Rare Disease Networks: it exists, it's working, and it's ours for the taking

Challenges and Opportunities

- In 2009, the European Union mandated all member states to develop National Plans for Rare Diseases by 2013.
- In 2015, CORD, with multiple stakeholders, launched Canada's Rare Disease Strategy identifying five core areas: diagnosis, treatment and care, community support, access to drugs, and research.
- The Strategy was acknowledged by Parliament on Rare Disease Day 2016 and is the blueprint for Ontario's Rare Disease Strategy. An Ontario Working Group has been tasked to produce a report with a proposed framework by mid-February 2017.
- In December 2016, after 14 years of planning, the European Union approved 23 European Reference Networks for Rare Diseases. Key benefits include equitable access for care across national borders, shared multicentre multidisciplinary expertise, criteria and standards for implementation and evaluation, training and support, community outreach, AND 25% patient representation on advisory boards.
- Opportunity for Canada: Accreditation Canada (now Health Standards Organization) won the contract to implement the European Reference Networks with cross-disciplinary teams and, going forward, to support training, standards development, expansion, and evaluation. And HSO is prepared to make all of their learning available to implement Canadian Rare Disease Networks.
- Canadian Rare Disease Networks will serve as the platform for implementing Canada's Rare Disease Strategy, including establishing standards for operation, clinical excellence, patient care, and community engagement.



Canadian Organization
for Rare Disorders

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Agenda March 30, 2017

Day 1: Creating Vision of Canadian Rare Disease Networks

8:00 a.m. - 8:30 a.m.	Breakfast and Registration	Junior Ballroom
8:30 a.m. - 9:10 a.m.	Welcome, Overview and Objectives 24 European Rare Disease Reference Networks: How they serve patients across borders	Durhane Wong-Rieger, Canadian Organization for Rare Disorders Louise Clément, Health Standards Organization Enrique Terol, European Commission, Brussels
9:10 a.m. - 9:30 a.m.	Living Rare Patients and Parents	
	Patients & Parents: Multi-generation, newly diagnosed, undiagnosed Panel: Chris Black (Cystic Fibrosis), Rick Waines (Canadian Hemophilia Society), Karolena Dempsey (Schnitzler's Syndrome), Isabel Jordan (Rare Disease Foundation) Moderator: Durhane Wong-Rieger	
9:30 a.m. - 10:30 a.m.	Vision for Canadian Rare Disease Networks	
	Creating a Foundation for Canadian Rare Disease Networks: Maternal Infant Child and Youth Research Network (MICYRN) Network of Rare Blood Disorders (NRBDO) Cystic Fibrosis Clinics Panel: Anne Junker (Maternal Infant Child and Youth Research Network), Whitney Goulstone (Canadian Immunodeficiencies Patient Organization, NRBDO), Mark Chilvers (BC Children's Hospital), Pam Ramsay (BC PHSA), Orion Buske (Rare Connect) Moderator: Bill Dempster	
10:30 a.m. - 10:45 a.m.	Refreshment Break	

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10:45 a.m. - 11:05 a.m.	Living Rare Patients: Rare Among Common: Cancer and related conditions	
	<p>Panel: Peter Dorfman (Chronic Lymphocytic Leukemia Patient Advocacy Group), Archibald Rodrigues (Life Raft Group Canada), Mark Williams (Canadian MPN Network), Lisa Machado (The Canadian CML Network), Wanda & Yuri Clayton (Langerhans cell histiocytosis)</p> <p style="text-align: right;">Moderator: Maureen Smith</p>	
11:05 a.m. - 12:00 p.m.	Creating Expert Groups	
	<p>Caring for rare among common conditions</p> <p>Panel: Jim Whitlock/Ahmed Naqvi (The Hospital for Sick Children), Marinka Twilt (Alberta Children's Hospital), Sylvia Stockler (BC Children's Hospital)</p> <p style="text-align: right;">Moderator: Alex MacKenzie</p>	
12:00 p.m. - 1:00 p.m.	Lunch	
1:00 p.m. - 2:35 p.m.	Networks for Research and Support	
	<p>Rare Diseases Models and Mechanisms Network</p> <p>Drug Development</p> <p>Panel: Phil Hieter (University of British Columbia), Jonathon Jafari (The Centre for Drug Research and Development), Robin Sherrington (Xenon), Stéphanie Hoffmann (Clementia)</p> <p style="text-align: right;">Moderator: Wayne Critchley</p>	
2:35 p.m. - 3:35 p.m.	<p>Small Group Workshop: Building Framework for Canadian Rare Disease Networks (participants will be assigned a group)</p> <p style="text-align: center;">- Refreshments will be available during the workshop</p>	
3:35 p.m. - 4:00 p.m.	Shared Learning	

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

6:00 p.m. - 7:00 p.m.	Cocktail Reception	Junior Ballroom Foyer
7:00 p.m. - 10:00 p.m.	Awards Dinner Gala Celebration	Junior Ballroom

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Day 2: Designing Rare Disease Networks

8:00 a.m. - 8:30 a.m.	Breakfast and Registration	Junior Ballroom
8:30 a.m. - 8:40 a.m.	What Canada can learn from European Rare Disease Networks	
	Review of Day 1 Durhane Wong-Rieger, Canadian Organization for Rare Disorders Louise Clément, Health Standards Organization	
8:40 a.m. - 9:00 a.m.	Living Rare Patients: Diagnosis/Access	
	Challenge getting to diagnosis Access to (right) specialist (rural or remote) Panel: Ian Stedman (CORD, Cryopyrin Associated Periodic Syndrome), Shailynn Taylor (Families of SMA Canada), Carrie Connell (Canadian FOP Network), Katrina Evanochko (Tuberous Sclerosis Canada) Moderator: Durhane Wong-Rieger	
9:00 a.m. - 10:00 a.m.	Networks for Access	
	Access to Rare Disease Drugs: Networking R&D, Regulatory, and Real-World Use Panel: Cathy Parker/Michele Chadwick (Health Canada) Dev Menon/Tania Stafinski (PRISM, University of Alberta) George Wyatt (Innomar Strategies) Moderator: Wayne Critchley	

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10:00 a.m. - 10:20 a.m.	Living Rare Patients: Patients doing it for themselves and each other
	Panel: Brad Crittenden (Canadian Association of Pompe), Rick Waines (Canadian Hemophilia Society), Deanna Badiuk (Canadian Pituitary Patient Network), Lisa Machado (The Canadian CML Network) Moderator: Maureen Smith
10:20 a.m. - 10:35 a.m.	Refreshment Break
10:35 a.m. - 12:00 p.m.	Rare Networks of Excellence
	Rare Neuromuscular Diseases Rare Immunodeficiency, Autoinflammatory and Autoimmune Diseases Network Rare Respiratory Diseases Paediatric Rheumatology Network Panel: Lori Tucker (British Columbia Children's Hospital), David Cabral (British Columbia Children's Hospital), Mary Connolly (British Columbia Children's Hospital), Ahmed Naqvi (The Hospital for Sick Children), Josh Lounsberry (Canadian Neuromuscular Disease Network) Moderator: Bill Dempster
12:00 p.m. - 1:00 p.m.	Lunch
1:00 p.m. - 2:15 p.m.	Genetics and Genomics Networks
	Next generation sequencing for clinical care and research Innovation in cell and gene therapies Panel: Wyeth Wasserman (British Columbia Children's Hospital), Erika Kleiderman (McGill University), Alison Elliott (CAUSES, BC Children's Hospital), Anna Lehman (BC Children's Hospital), Sylvia Stockler (BC Children's Hospital) Moderator: Alex MacKenzie

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2:15 p.m. - 3:00 p.m.	Small Group Workshop 2: Realizing Canadian RD Networks <ul style="list-style-type: none">- Desired outcomes- Scope of inclusion (short and long term)- Potential partners, stakeholders and sponsors- Success factors- Next steps
3:00 p.m. - 3:30 p.m.	Coming Together to Create a Network of Networks

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 partners that have supported the Rare Disease 2017 Conference.

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