

Tipping Point for Rare Disorders in Canada



Rare Disorders Conference
September 15-16, 2011
Marriott Downtown Eaton Centre,
525 Bay Street, Toronto, Ontario

Agenda

September 15, 2011
York Room

Time	Activity	Presenter/Format
8:00 a.m. – 8:30 a.m.	Registration Continental Breakfast	
8:30 a.m. – 9:00 a.m.	Welcome and Goals Recounting Canada's Long Journey Toward an Orphan Drug Policy Canada's Vision	Durhane Wong-Rieger, Canadian Organization for Rare Disorders
9:00 a.m. – 9:45 a.m.	Innovating Therapies for Rare Diseases: Opportunities and Challenges	Marlene Haffner, Haffner Associates Robert Heft, Enobia
9:45 a.m. – 10:30 a.m.	Patients Organizations: Pushing the Boundaries & Why Canada Needs to Reposition the Goalposts for Access to Therapy	Gail Ouellette, Regroupement québécois des maladies orphelines Kirsten Harkins, Canadian MPS Society Deb Maskens, Kidney Cancer Canada Simon Ibell, iBelieve Foundation
10:30 a.m. – 10:45 a.m.	Break	
10:45 a.m. – 11:30 p.m.	Application of Health Technology Assessment to Innovative Therapies Therapies for Rare Diseases: HTA Challenges and Approaches	Reiner Banken, INESS-Quebec Chander Sehgal, CADTH
11:30 a.m. – 12:15 p.m.	Shifting the Balance: Milestones Along the Way with Advances in Research on Rare Diseases	Paul Lasko, CIHR Kym Boycott, Children's Hospital of Eastern Ontario Research Institute, University of Ottawa Brett Thombs, Jewish General Hospital, McGill University Seema Mital, The Hospital for Sick Children

12:15 p.m. – 1:15 p.m.	Lunch	
1:15 p.m. – 2:00 p.m.	Shifting the Balance: Milestones Along the Way with Advances in Orphan Drug Regulatory Framework and Biotech Industry	David Lee, Health Canada Peter Brenders, BIOTECCanada
2:00 p.m. – 3:30 p.m.	Innovative Approaches from Drug Plans: Public and Private	Chair: Wayne Critchley, Global Public Affairs Diane McArthur, Ontario Ministry of Health & Long-Term Care Bob Nakagawa, BC Ministry of Health Services Steve Long, Alberta Health & Wellness Judy McPhee, Nova Scotia Department of Health Stephen Frank, Canadian Life and Health Insurance Association
3:30 p.m. – 4:00 p.m.	Break	
4:00 p.m. – 6:00 p.m.	Consolidating the Vision for Rare Disorders Program in Canada Question: “Given everything else going on in healthcare right now, what would it take to implement a Canada-wide drug program for rare disorders?” 1 (Open Space): What are the key issues (barriers, facilitators, key players, benefits to patients, providers, institutions, funders, drivers, risks, etc.) 2 (Consolidation) What are themes (emerging from issues)? Are there other key issues? (3) (Success Factors) What does success (sustainable appropriate use) look like?	All Work in open space as individuals and then small groups around emergent themes
6:00 p.m.	Networking Dinner	

September 16, 2011
York Room

Time	Activity	Presenter/Format
8:00 a.m. – 8:30 a.m.	Registration Continental Breakfast	
8:30 a.m. – 9:00 a.m.	Welcome and Goals Day 1 BIG IDEAS FOR MOVING FORWARD	Durhane Wong-Rieger. Canadian Organization for Rare Disorders Panel
9:00 a.m. – 9:30 a.m.	Investing in Rare Disorders: Opportunities and Challenges for Industry	Hugh O’Neill, sanofi-aventis Canada Inc.

9:30 a.m. – 10:30 a.m.	Innovative Options for Managing Access to Innovative Therapies	Dev Menon, University of Alberta Tania Stafinski, University of Alberta Frederic Lavoie, Pfizer
10:30 a.m.– 10:45 a.m.	Break	
10:45 – 11:15	Developing and Using Rare Disease Patient Registries	Kyle Brown, Patient Crossroad
11:15 a.m. – 12:30 p.m.	Issues Challenging Sustainable Access: Defining Rare (ultra-rare, rare cancers, rare genotypes, resistant and non-respondent patient Sub-Groups Sustainable Patient Access: Patient registries, types of evidence, off-label usage, pricing and affordability	Chair: Deb Maskens, Kidney Cancer Canada Dawn Green, Cancer 52 (UK) Kelly Gorman, Cystic Fibrosis Canada Jim Mundy, Alpha-1 Canada Della Cogar, HAE Canada Brian Lewis, Genzyme Canada John Haslam, Alexion
12:30 p.m. – 1:30 p.m.	Lunch	
1:30 p.m. – 2:45 p.m.	Simultaneous Small Group Discussions Toward a Canadian Drug Program for Rare Disorders 1. Effective Submissions to Orphan Drug Policy Chair: Chair: Kirsten Harkins Co-Chair: Kelly Gorman 2. Defining Appropriate Patient Access Chair: Durhane Wong-Rieger Co-Chair: Della Cogar 3. Managed Access and Product Listing Agreements Chair: Dev Menon Co-Chair: Tania Stafinski 4. Assuring Sustainability for Industry and Drug Plans Chair: Bill Dempster Co-Chair: Jim Mundy	ALL
2:45 p.m. – 3:15 p.m.	Working Toward Consensus Principles, Best Practices, Innovative Solutions	Panel
3:15 p.m. – 3:30 p.m.	Tipping the Balance: Next Steps Conference Adjournment	ALL

**The Canadian Organization for Rare Disorders (CORD)
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 event.



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