Rare Disease Day 2018

March 21 -22, 2018
Delta Ottawa City Centre, 101 Lyon Street, Ottawa, Ontario

Building on Research Excellence to Improve Patient Care

AGENDA

March 21, 2018

8:00 a.m. – 8:45 a.m.	**Breakfast and Registration -** Ballroom A/B
8:45 a.m. – 9:00 a.m.	**Welcome, Overview and Objectives** Durhane Wong-Rieger, Canadian Organization for Rare Disorders (CORD)
9:00 a.m. – 9:15 a.m.	**Seizing the Moment for the Rare Disease Community** Paul Lévésque, Pfizer Inc.
9:15 a.m. – 9:45 a.m.	**Accelerating rare disease diagnosis: Canada's excellence from research to clinical practice** • How did FORGE improve rare diagnosis through genome (exome) sequencing? • How has gene discovery and diagnosis led to improvement in rare disease clinical management? • What can we hope from the next big collaboration: C4R-SOLVE Taila Hartley, CHEO Research Institute
9:45 a.m. – 10:30 a.m.	**Rare Disease Centres of Expertise: what we are learning and what will we need to sustain and expand?** • Neuromuscular Disease Network/SMA - Craig Campbell, Children's Hospital, LHSC • Bone Disease Network - Cheryl Greenberg, Winnipeg Regional Health Authority • Rett Syndrome Community: Clinic, Registry, Research, Support – Melissa Carter, CHEO
10:30 a.m. – 10:45 a.m.	**Refreshment Break**

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10:45 a.m. – 11:00 a.m.	**Canada's Commitment to Rare Disease Research** Étienne Richer - CIHR Institute of Genetics
11:00 a.m. – 12:00 p.m.	**Enhancing Canada's footprint in the development of therapies for rare diseases** • Discovery, adaptation, and targeting drugs for rare conditions - Daniel Drucker, Lunenfeld Tanenbaum Research Institute • Medical devices to improve capabilities and support quality of life - Pamela Borges, B-TEMIA Inc • How can we create "caring" communities that support and empower? Anna McCusker, Scleroderma Canada
12:00 p.m. – 1:00 p.m.	**Lunch**
1:00 p.m. – 2:00 p.m. (This session will be webcast)	**Bringing Rare Disease Drugs to Canada: Opportunities, Challenges, and More Challenges** *Current Status of Access to Rare Disease Drugs Through Canada's Approval Process* John Oliver – MP, Oakville Neil Palmer - PDCI Market Access Sherry O'Quinn - MORSE Consulting Durhane Wong-Rieger - CORD **Moderator:** Bill Dempster, 3Sixty Public Affairs
2:00 p.m. – 2:45 p.m.	**How Patients Experience Access (Patient Panel)** Karen McCullagh - Cystinosis Awareness and Research Effort, Susi Vander Wyk - Cure SMA Canada, Jacquie Badiou - HAE Canada, Joan Paulin - PHA Canada, Stephen Richardson - Canadian Aniridia Foundation Issues: • Access through SAP • Expedited & Early-Stage Approvals • HTA Process for Rare Disease Drugs • Funding through Private and Public Plans • Accessing Medical Devices for Rare Diseases **Moderator:** Durhane Wong-Rieger

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2:45 p.m. – 3:00 p.m.	**Refreshment Break**
3:00 p.m. – 4:00 p.m.	**Current and Future Reality of Canadian Environment for Orphan Drugs (Industry Panel)** Farah Jivraj – Biogen, Bruce MacDonald – Pfizer, Eric Tse – Shire, Bob McLay - Sobi, Sandra Anderson - Innomar • Is Canada attracting clinical trials for rare diseases? • How much support do researchers and innovative start-ups receive in Canada? • Do companies feel Canada (still) needs Orphan Drug Regulatory Framework despite pathways to approval with existing legislation and regulations? • Has review of rare disease drugs through the regular process for common drugs resulted in appropriate assessments and recommendations? Is a RDD pathway still needed? • Does review through pCPA and public drug programs providing timely and appropriate access for patients with urgent, progressive, and life-threatening conditions to therapies that may have been approved with high uncertainty and high "per patient" costs? • Is Canada bringing in alternative access solutions for drugs that do not fit the common paradigm? • How will proposed amendments to PMPRB affect rare disease drugs? • Where are private plans now in coverage for rare diseases and what are the future directions? **Moderator:** Bill Dempster

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 - Ballroom C
7:00 p.m. – 10:00 p.m.	Awards Dinner Gala Celebration - Panorama

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March 22, 2018

8:00 a.m. – 8:30 a.m.	**Breakfast and Registration - Ballroom A/B**
8:30 a.m. – 8:45 a.m.	**Opening Remarks (Day Two)** Durhane Wong-Rieger, Canadian Organization for Rare Disorders (CORD)
8:45 a.m. – 9:15 a.m.	**Regulatory Review of Drugs and Devices: R2D2** Megan Bettle, Director, Regulatory Innovation and Business Operations, BGTD, Health Canada
9:15 a.m. – 9:45 a.m.	**Regulatory Review of Drugs for Rare Diseases: Updates and Ongoing Activities** Fiona Frappier, PhD, Senior Policy Analyst, Health Canada
9:45 a.m. – 10:15 a.m.	**Panel Q & A** Megan Bettle Fiona Frappier Cathy Parker, Director General, Biologics and Genetic Therapies Directorate, Health Canada
10:15 a.m. – 10:30 a.m.	**Refreshment Break**
10:30 a.m. – 11:50 a.m.	**Panel Discussion on R2D2: Collaboration Amongst Health Portfolio Partners** Karen Reynolds, Health Canada Heather Logan, CADTH Scott Doidge, DG, Department of Indigenous Services Suzanne McGurn, Ontario Drug Programme, MOHLTC Pamela Fralick, Innovative Medicines Canada Megan Bettle, Health Canada Cathy Parker, Health Canada **Moderator:** Bill Dempster
11:50 a.m. – 12:00 p.m.	**Concluding Remarks** Cathy Parker
12:00 p.m. – 1:00 p.m.	**Lunch**

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REDEFINING THE VALUE OF THERAPIES FOR RARE DISEASES: A PLACE FOR SYSTEMS THINKING?

Moderators: Dev Menon, Tania Stafinski, University of Alberta

1:00 p.m. – 1:30 p.m.	**What is 'systems thinking' and why is it important in rare diseases?** Dev Menon - University of Alberta, Judith Glennie - J.L. Glennie Consulting
1:30 p.m. – 2:00 p.m.	**How would you value a new therapy? (Small Group Exercise)** Assessing the potential value of therapy using systems thinking **Moderator:** Judith Glennie
2:00 p.m. – 2:15 p.m.	**Report back/Refreshment Break**
2:15 p.m. – 2:45 p.m.	**"Systems thinking" and patient access schemes: Experience from NHS England** Edmond Jessop - National Health Service (NHS), England
2:45 p.m. – 3:00 p.m.	**Using "systems thinking" to inform a framework for Canada?** Dev Menon, Tania Stafinski, University of Alberta
3:00 p.m. – 3:30 p.m.	**Next Steps and Wrap Up** Judith Glennie

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