Canadian Organization for Rare Disorders

Rare Disease Day Conference 2021 Agenda

March 9, 2021
11:00 am - 2:00 pm

Canada's Rare Drug Ecosystem
Aiming for Mars: With Perseverance You Can Get Anywhere!

11:00 am	**WELCOME** • How Did We Get Here? • Where Do We Want to GO?	Durhane Wong-Rieger, CORD
11:15 am	**What problem are we committed to solving?** **Current and Emerging Challenges** • Spinal Muscular Atrophy: Newborn screening but not newborn treatment • Inherited retinal disease: Race against time to prevent blindness • HPP and XLH: Strengthening bones for kids but not for adults • Cystic fibrosis: Funding drug for 5% but not 90% of patients • Rare blood disorders: Fund transfusions but not drug reducing need for blood • Lysosomal storage disorders: Gene therapies to replace replacement therapy • Prader Willi Syndrome: 20-yr-old drug that has taken 20 years to access • Epidermolysis Bullosa: Topical, systemic, and gene therapies all on horizon • Fibrodysplasia Ossificans progressive: Canada produces 1st ever drug therapy • High-uncertainty and low-cost drugs: Let's give it to almost everyone! • High-uncertainty and high-cost drugs: Let's give it to almost no one!	Plenary Moderated Panel Moderator: Durhane Wong-Rieger Panelists: Jacob Jaramillo, CF Get Loud Celine Lepage, Foundation for Prader-Willi Research Susi Vander Wyk, Cure SMA Canada Christine White, National Gaucher Foundation Videos: Brown Family Chhoun Family

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12:00 pm	**Canada TODAY: Capacity vs Performance; Canada Access vs ROW** Canadian Healthcare Landscape: Rare Disease drug spend Canada's performance re: • # RD submissions • # approved therapies • % reimbursed therapies • innovative R&D	Plenary Norm Berberich, Takeda Lindy Forte, Patient Access Solutions Jason Field, Life Sciences Ontario
12:30 pm	**North Star for Canada's Rare Disease/Drug Ecosystem** Design Drug Access Program that aligns with primary "north star" but meets other goals and incorporates key values and principles: Aligning with our North Star: • Public Health Centred • Evidence Centred • Patient Centred Incorporating our values and principles: • Patients engaged as partners • Research & development and public-private partnerships • Competitive pharmaceutical sector • Network of clinical expertise • Safe, effective, timely, personalized and affordable medicines for individuals and health systems • Equity in access and healthcare outcomes (single framework) • Consolidation across geographic and funding silos • Economically viable • Transparent accountable process	Breakout Sessions Moderator: Bill Dempster, 3Sixty Public Affairs Leads (TBD)
1:00 pm	**Beyond Drugs: It Takes A Community**	Plenary Panelists:

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	A Rare Drug Strategy must be integrated into a Rare Disease framework. Where should Canada invest (par of $1 billion) to support Rare Drug Strategy? Specifically: • Newborn and Genomic Screening and Diagnosis • Canadian Networks of Clinical and Research Expertise • Community and Patient Group Support • Multiple Models of Drug Access • Research from Lab to Manufacturing What are advances in Canada's rare disease ecosystem, including diagnosis, centres of expertise, patient and parent support, access to treatment and services, and research? What are key challenges and gaps in services that affect patients and families? Where would investment have the most beneficial impact?	Moderator: Bill Dempster Homira Osman, Muscular Dystrophy Canada Angela Genge, Montreal Neurological Institute-Hospital Nathalie Ouimet, Montreal InVivo Michael May, CCRM
2:00 pm	**Preparation for Day 2**	TBC

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March 10, 2021
11:00 am - 2:00 pm

Laying Foundation for Canada's Rare Disease Drug Ecosystem

11:00 am	**Welcome**	Durhane Wong-Rieger, CORD
11:05 am	**Canada's Rare Drug Access: Focus on Patients** Whom are helping? What are we doing well? Whom are we failing? What do we need to do differently? • SMA: Newborn screening, access to treatment, access to gene therapy • Cystic Fibrosis: Newborn screening, treatment for some but not others • X-linked hypophosphatasia: Treatment for kids but not for adults • Retinitis pigmentosa: Time sensitive treatment taking too long	Plenary Panel Moderator: Durhane Wong-Rieger Panelists: Kimsaung Sov Dr. Craig Campbell, London Health Sciences Dr. Leanne Ward, Children's Hospital of Eastern Ontario Dr. Elise Heon, The Hospital for Sick Children Charmain Brown
11:45 am	**INTERNATIONAL EXPERIENCE: What Canada should and should not consider adopting or adapting?** Who does it best? What are the trade-offs? What would it take to make these work in Canada? What are the challenges?	Plenary Moderator: Bill Dempster Panelists: Dev Menon, University of Alberta

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	• Single decision maker, access criteria, and negotiations • Separate Rare Drug Pathway • Expedited approval process • Early access process • Higher cost-effectiveness threshold • Real world monitoring and evidence assess	Ed Dybka, Ipsen Brad Alyward, CounterPoint Consulting Associates
12:15 pm	**MULTIPLE ACCESS PATHWAYS** **Addressing Challenges to Unmet Needs** What core challenge does each of the previous case examples represent? Select any TWO (2) cases. How are they similar and different? Describe a "feasible" Access Plan to address BOTH cases. Best characterization of the need(s) • Urgent access for life-saving or time-limited needs • Access while evidence is developing • Durable therapies with pent-up demand and high upfront costs • Preventive therapies with difficult to measure short-term impacts • Additive, combination, or enhanced therapies that are more targeted and improve tolerability and adherence but may be more costly • Expanded or secondary indications for similar, small, or age extensions • Other (specify) **Exploring Alternative Pathways** Which of the following access pathways might work? How well does each (or variation thereof) fulfill the triple aim of: individualized patient need, evidence based/informed,	Breakout Sessions Leads (TBD)

economically viable (for payer and manufacturer).

- Early Access Program (pre-NOC, pre-reimbursement) for urgent needs
- Managed access program (evidence informed and evidence generating) for patient-centred individualized access
- Financial pooling to reduce disproportionate impact on single payer and promote consistent access across payers
- Financing pay-for-performance plans
- Patient registries for monitoring, data collection and analysis
- Consolidated funding pool across public and private plans
- Other (specify)

Rare Disease Ecosystem

Describe the characteristics of the Rare Disease Ecosystem that are essential to the effectiveness of your proposed Access Plan.

- Engaging patients as partners
- Research & development and public-private partnerships
- Competitive pharmaceutical sector
- Network of clinical expertise
- Delivering safe, effective, timely, personalized and affordable medicines individuals and healthcare system
- Single framework for equity in access and healthcare outcomes
- Consolidation across geographic and funding silos
- Economically viable
- Transparent accountable process

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1:00 pm	**Feedback** • Addressing Challenges to Unmet Needs • Exploring Alternative Pathways • Rare Disease Ecosystem	Plenary Breakout Spokespersons
1:20 pm	**HOT TOPICS** Beyond the vision for a National Rare Drug Strategy, following are some of the key issues that will need to be addressed in implementation. • Official status of "single decision maker": agency, office, coordinating body, multi-stakeholder/multi-jurisdiction collaborative • Scope and authority of single decision maker; compulsory decisions or discretionary recommendations • Ethics of QALY and ICER for rare drugs • Participation of public and private payers (voluntary, obligatory, incentivized) • Funding (common pool, new sources) • Relation to existing stakeholders: Health Canada, PMPRB, CADTH/INESSS, pCPA, public payers, private payers, healthcare institutional payers, expert clinics, patient organizations, patients and public • Data collection and management, including patient registries, real-world evidence, and international databases • Where should $1 billion be invested? • Other topics	Plenary panel Moderator: Bill Dempster Panelists: Ian Stedman, York University Fred Horne, 3Sixty Public Affairs Sandra Anderson, Innomar Strategies Marissa Poole, Sanofi Karen Voin, CLHIA
1:50 pm	**Conclusion and Next Steps**	Durhane Wong-Rieger
2:00 pm	**Conference Ends**	

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.