FALL 2021 CONFERENCE

BUILDING CANADA'S RARE DRUG PROGRAM: A BOLDACIOUS BILLION$ INVESTMENT

DEC 1 & 2, 2021 | 11 AM- 2 PM
VIRTUAL CONFERENCE

Register at www.raredisorders.ca

CORD Canadian Organization for Rare Disorders

12 Steps to Canada's Rare Disease Drug Program

Vision: Integrated, Inclusive, Innovative Rare Drug System

- Single Seamless Pathway from R&D, CT, regulatory approval, access parameters, monitoring, values-based assessment, price negotiations
- Governance board representing all stakeholders: diverse patient community, clinical specialities, public/private drug plan providers, HTA agencies, pharmaceutical companies, subject matter experts (regulatory, research)

Building for Success: 12 Steps, 4 Platforms

December 1, 2021

Day 1: Getting to Access

11:00 am - 11:15 am

What We Need to Succeed

- Durhane Wong- Rieger, CORD

11:15 am - 12:15 pm

Platform 1: Expedite Timely and Optimal Access to Diagnosis and Care

1. Create networks of Specialty Centers for Expert Clinical care and Research and connect to local healthcare providers and social support programs
2. Implement accurate accessible timely diagnosis through national Newborn Screening guidelines and Next-Generation Exome/Genome Sequencing
3. Create R&D incentives including public-private partnerships involving academic and clinical sites, coordinated through institutions such as CIHR, National Research Council, Genome Canada, Centre for Commercialization of Regenerative Medicine, Regenerative Medicine Alliance of Canada, and Maternal Infant Child Youth Research Network

Panelists:

- Hugh McMillan, McGill University Health Centre
- Cheryl Rockman-Greenberg, Max Rady College of Medicine
- Thierry Lacaze-Masmonteil, MICYRN
- Frederic Lavoie, Pfizer
- Brent Warner, Novartis

12:15 pm - 12:45 pm

Case Study: Gene Therapy for Inherited Retinal Disease

1. In October 2020, Health Canada approved Luxturna as the first therapy for previously untreatable inherited retinal disease. This one-time therapy replaces dysfunctional RPE65 genes with working copies that restore vision and improve sight.
2. In Canada, for children and adolescents with IRD, there is an urgent need for timely access to Luxturna, with several young people moving beyond the period of efficacy now at high risk of losing their vision. Despite a CADTH positive recommendation for funding, the provinces have not yet provided access. Private payers have denied access, citing pre-existing condition.
3. This situation must be addressed with a national solution in the future Rare Disease Drug Strategy. In the meantime, we urgently need intervention to save the sight of children who are NOW at risk.

Panelists:

- Elise Heon, The Hospital for Sick Children (Toronto)
- Doug Earle, Fighting Blindness Canada
- Patient Representatives

12:45 pm - 1:45 pm

Platform 2: Create Competitive Access Environment for Innovation

1. Develop competitive environment for collaborative drug development, clinical trials, early access, and patient support.
2. Roll back 2019 PMPRB changes to refocus on fair, non-excessive pricing and NOT restrictive pricing that discourages and delays new drug launches
3. Enhance Health Canada collaboration and coordination with FDA, EMA, and other regulators on regulatory reviews and approvals; engage with patients.
4. Implement pathways for special access, including early access pre-NOC and pre-reimbursement agreements.

Panelists:

- Eileen McMahon, Torys
- Wayne Critchley, Global Public Affairs
- Michael May, CCRM
- Declan Hamill, Innovative Medicines Canada
- Angela Genge, The Neuro, McGill University
- Laurene Redding, BeiGene
- Fred Little, Pfizer
- Oxana Illiach, CORD
- Tammy Moore, ALS Canada

1:45 pm - 2:00 pm	**Preparing for Action** • Durhane Wong- Rieger, CORD

December 2, 2021
Day 2: Realizing the Vision

11:00 am - 11:15 am	**Learnings from Day 1 (Access)** • Durhane Wong- Rieger, CORD
11:15 am - 12:30 pm	**Platform 3: Create Innovative Financing Pathways for Efficient, Effective, and Cost-Effective Drug Access** 1. Establish national agency to develop multiple financing pathways specific to needs of patient population and drug characteristics for universal access across multiple payers 2. Leverage Managed Access Programs to provide timely access of approved therapies with potential benefits to specified patients under conditions of continued monitoring and collection of real-world data 3. Facilitate concurrent Health Canada and health technology assessment reviews to promote shared interpretation of safety and efficacy data and expedite access 4. Implement processes for real-world data collection and utilization of real-world evidence throughout drug cycle from clinical trials to real-world usage to promote timely, evolving, evidence-informed access **Panelists:** • Rosalie Wyonch, CD Howe • Chris McMaster, CIHR • Laurie Lambert, CADTH • Suzanne McMullen, Medlior • Christopher Pettengell, Pentavere • Christina Cunningham, Vertex Canada • Bob McLay, SOBI Canada

12:30 pm - 1:45 pm

Platform 4: Implement meaningful representative patient engagement throughout the drug system in every area, at every level and in every way possible in the Rare Disease Drug Agency

1. Through CORD, provide training, support, and resources to enable effective participation.
2. Enforce policies and practices of open, transparent, and accountable communications
3. Ensure equity through inclusive diversity with attention to those excluded and neglected because of race, ethnicity, biological sex/gender, age, language, education, literacy, income, immigrant status, indigenous status, and rural geographic residence
4. Ensure equitable access to community support programs
5. Establish the Canadian Rare Drug Agency as an independent, transparent, publicly accountable agency with responsibility for all aspects of the review and assessment of drugs for rare diseases, in coordination with accountable agencies (Health Canada, PMPRB, CADTH/INESSS, pCPA, public drug programs, private drug programs)
6. Guarantee sufficient budget to carry out its mandate, with "hands-off" funding from the PT and federal governments
7. Establish representative board including all stakeholders

Panelists:

- David Page, Canadian Hemophilia Society
- Maureen Smith
- Thea Discepola, Novartis
- Christian Hansen, Alexion
- Peter Brenders, BeiGene
- Jessica Brcko, Sun Life
- Glenn Monteith, Global Public Affairs

1:45 pm - 2:00 pm

Next Steps for Action

- Durhane Wong- Rieger, CORD

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.

ALEXION

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