



FALL 2021 CONFERENCE

BUILDING CANADA'S RARE DRUG PROGRAM: A BOLDACIOUS BILLIONS INVESTMENT

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

Register at www.raredisorders.ca



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 - 12:30 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

12:30 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

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

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

1:45 pm - 2:00 pm

Next Steps for Action

- Durhane Wong- Rieger, CORD