In person
or virtual

RARE DISEASE 2022 FALL CONFERENCE

Planning for Proof-of-Concept Programs to Transform Vision to Reality

November 21-22, 2022
Toronto, Ontario

HYATT REGENCY TORONTO
370 King Street West, Toronto, Ontario

We are excited to invite you to the CORD 2022 Fall Conference! This two-day conference will bring together experts and participants that will engage in strategic planning sessions to arrive at proposals for key components within the three pillars of the Rare Disease Program.

CORD Canadian Organization for Rare Disorders

Fall 2022 Conference

Planning for Proof-of-Concept Programs to Transform Vision to Reality

November 21 - 22, 2022

HYATT REGENCY TORONTO

370 King Street West, Toronto, Ontario
(**Virtual option available**)

Rare Disease 2022 Fall Conference

From Vision to Reality: Canada's Rare Disease Drug Strategy
Transforming Commitment to Investment in Optimal Patient Outcomes

Overview

In 2019, the Canadian government committed $1 billion to setting up Canada's Rare Disease Drug Strategy with the goal of providing access to effective therapies for persons living with rare diseases. For the past three years, the Canadian Organization for Rare Disorders has been hosting monthly multi-stakeholder webinars and bi-annual conferences with a singular focus: how to invest the $1 billion start-up funding and $500 million annual commitment to achieve optimal patient outcomes and returns on investment to the health system and society.

This opportunity comes at a time with many transformative and life-saving therapies emerging as the first effective treatments for rare and ultra-rare patients with progressive, severe or life-threatening conditions. By their very nature, novel drugs targeting small patient populations with limited natural history studies require novel clinical trials, including short, small, single-arm, non-placebo-controlled designs and reliance on real-world patient reported outcomes data collected during clinical trials and into real-world usage. Furthermore, because many new orphan-designated drugs are targeted toward specific genetic mutations, increasing the likelihood of benefit and reducing potential for harm, companion diagnostics are key components of the clinical trials, regulatory approval processes and real-world usage.

Unfortunately, cost-effectiveness, or health technology assessment (HTA), processes have not transitioned much beyond traditional assessment tools and benchmarks, with the inevitable outcomes that rare disease drugs mostly do not meet (traditional but arbitrary) standards of clinical or cost-effectiveness set by the HTA agencies. Most also do not adequately take into consideration the fact that small patient numbers may drive individual-patient price upward while the overall budget impact remains very low.

Fall 2022 Conference

After three years of consultation, Canada's rare disease community is in agreement. To assure optimal patient outcomes as well as optimal societal benefits, a Rare Disease Drug Program must transcend a list of funded drugs. Canada can demonstrate global leadership by implementing a highly effective, cost-effective, and sustainable RD Drug Program built upon three pillars. These are: (1) Network of Rare Disease Centres of Excellence; (2) Adaptive pathways for accelerated access with real-world monitoring and patient data collection and analysis; and (3) Patient integration in all areas and at all levels of rare disease management and drug utilization. The very good news is that 75% to 90% of infrastructure and expertise are already available. What we need is 'smart' investment to leverage, expand, coordinate and integrate existing human and facility resources, fill in program gaps, sponsor learning and demonstration projects, and also train and support patient organizations and patients to integrate them throughout the program.

1. **Invest in a rare disease infrastructure built around a Network of Rare Disease Centres of Excellence,** that can serve as the "hubs" for other sites to assure every rare disease patient has access to timely diagnosis, specialist care, individualized best drug access, comprehensive care close to home, enrollment in patient information management program, and participation in real world data collection.
2. **Invest in alternative pathways for drug access** that can be customized to the specific requirements of each rare condition and therapeutic option to provide affordable, consistent and sustainable access for all patients regardless of where they live in Canada. Overall, the drug system must meet the triple goals of: (1) Patients receiving affordable and timely access to individualized best therapy; (2) Healthcare systems and public and private drug plans are able to reasonably predict and manage health and drug budgets to achieve an optimal return on health resources while meeting the needs of the population and individuals; and (3) Researchers and manufacturers experience adequate incentives to invest in rare disease drug research and achieve also return on investment through the distribution of therapies to patients.
3. **Patients are fully engaged as partners throughout the drug program**, including the level of individual decision making on therapeutic options, design and delivery of healthcare and drug-related services, regulatory and health technology assessment, pathways for access (criteria for managed access plans), and patient information management (including patient registries and real-world data collection and use).

Conference: Planning for Proof-of-Concept Programs to Transform Vision to Reality

Goals: The goals of the conference are to develop demonstration projects that can serve as "proof of concepts" for the key elements of an essential Rare Disease Drug Program to support effective and cost-effective use of rare disease therapies.

During the conference, participants will engage in strategic planning sessions to arrive at proposals for key components within the three pillars of the Rare Disease Program, building on the discussions in the CORD June Rare Disease Drug Conference and subsequent webinars.

Conference Agenda

Day 1

Monday, November 21 (9:00 AM - 4:30 PM EDT)
Transforming Vision to Reality

8:30 am – 9:00 am
Registration and Continental Breakfast
Hyatt Regency Toronto Hotel, King Ballroom

9:00 am – 9:15 am
Conference Begins
Welcome and Goals of Conference – Durhane Wong Rieger, CORD

- Vision of Canada's Rare Disease Drug Strategy: Diagnose and Optimally Treat Every Person in Canada with a Rare Disease
- "5-Ps" Working Together to Transform Vision to Reality: Patients, Professional Practitioners, Policy Makers, Payers, and Pharma

9:15 am – 9:45 am
Vision of Care for Rare Diseases in Canada

- Learning from the European experience with Rare Disease Networks: Maciej Gajewski, Alexion
- State of the Art: Updated Patient/Family Experience of Living with Rare Disease in Canada: Nahya Awada, CORD; Grace Tong, Ipsos
- What will be the State of the Art of Rare Disease in Canada in 2028
- How can we Chart the Path to Canada's Rare Vision
    - External Drivers of RD Ecosystem: Opportunities and Threats
    - Canada's Capabilities: Strengths and Weakness
    - Filling the Gaps
- Investment Priorities: Where and How should we Invest $500 million/year?

9:45 am – 10:15 am
Constructing Canada's Shared Commitment to Rare Disease Community
Feedback towards a Shared Vision and Commitment

- Strengthening Health Canada's Commitment: Michelle Mujoomdar/ Daniel MacDonald, Health Canada
- Strengthening Health System Commitment: Christine Elliott, Fasken
- Strengthening Industry Partnerships: Carrie McElroy, Sanofi

10:15 am – 10:30 am
Break

Conference Agenda

Day 1

Monday, November 21 (9:00 AM - 4:30 PM EDT)
Transforming Vision to Reality

10:30 am – 11:00 am
Roundtable: Why Rare Disease Matters

- Saving Rare Adults: Anne-Marie Carr, Hereditary Amyloidosis Canada
- Rare Disease Community of Excellence: Leanne Ward, CHEO
- Rare Disease as Gateway to Precision Healthcare: Sapna Mahajan, Genome Canada
- Rare Genes in Research: Christopher McMaster, CIHR Institute of Genetics
- Industry Commitment to Rare and Beyond: Wendy Erler, Alexion, AstraZeneca Rare Disease
- Moving the Needle on Rare Diseases in Canada: Brad Alyward, CORD

11:00 am – 12:00 pm
SMART Investment in Rare Disease Infrastructure to Optimize Diagnosis, Prescribing, and Life-long Treatment Management

Clinical Networks of Excellence:

Co-Chairs: Leanne Ward, CHEO; Craig Campbell, London Health Sciences; Francois Bernier, Alberta Health Services
Panelists: Angela Genge, The Neuro; Laura McAdam, Holland Bloorview; Thierry Lacaze; MICYRN

- Defining the roles of the Canadian Organization for Rare Disorders (CORD) and Maternal Infant Children and Youth Research Network (MICYRN)
- Agreeing on Canadian criteria for Centers of Excellence and Allied Centres of Distinction, adapted from international best practices (WHO model, NORD COEs)
- Developing Terms of Reference for Rare Disease Specialty Networks Groups (based on European Reference Networks)
- Developing Proposal for Rare Disease Community Networks (Family Practitioners and Allied Professionals
- Developing Proposal for Rare Disease Nurses/Genetic Counsellors Network
- Agreeing on and implementing procedures for application, review and designation
- Identifying and requesting resource requirements: Human resources, facilities, technology, management, financial

Conference Agenda

Day 1

Monday, November 21 (9:00 AM - 4:30 PM EDT)
Transforming Vision to Reality

- **Designated Centres of Excellence and Centres of Distinction**
  - Canada's Network of RD Centres of Excellence will participate as a member of the WHO Global Network of Rare Disease Centres of Excellence linking national Rare Disease Centres across a geographic region and regions into an international network with the goal that every rare disease patient will have access, directly or by remote consultation, to a specialist centre of care.
  - Canada's RD Centres of Excellence, modeled after the NORD Centers of Excellence, will be designated upon fulfillment of best practice criteria and, when fully operationalized, will provide up-to-date expertise on diagnostics, management, treatment, and monitoring as well as coordination of comprehensive care and support.
  - COEs will serve as hubs providing expert consultation and support to designated Rare Disease Centres of Distinction as well as other healthcare providers. Models of hubs connected to community-based care, including primary healthcare providers and local support resources include: Cancer Strategy – Canadian Partnership Against Cancer; Ontario Rare Disease Strategy [Implementation Plan 2018]; Politique québécoise pour les maladies rares [2022]
- **Rare Disease Specialty Networks**
  - Canadian exemplars, some of which are internationally recognized specialty programs, include: Metabolic Diseases Program & Network, Canadian Prairie Metabolic Network, Ottawa Pediatric Bone Health Research Group and The Canadian Consortium for Children's Bone Health/Canadian Alliance for Rare Disorders of the Skeleton, Canadian Haemoglobinopathy Association, Canadian Neuromuscular Network, Canadian Fabry Disease Initiative, Canadian Haemophilia Treatment Centres, Amyloidosis Centres, ALS Clinic

Public-Private Partnerships – Beyond the Pill:
Public-private partnerships are essential to clinical and research Centres of Excellence. This session explores existing partnerships and future opportunities.

Industry Leads: Peter Brenders, BeiGene Canada; Sandra Anderson, Innomar Strategies

Conference Agenda

Day 1

Monday, November 21 (9:00 AM - 4:30 PM EDT)
Transforming Vision to Reality

12:00 pm – 1:00 pm
Lunch

1:00 pm – 2:00 pm
Value of Rare Disease Medicines
Value-based healthcare and its role in improving access to optimal care and treatment for rare disease patients. The first panel focuses on how to define value from the perspective of patients and how to measure the impact on patients, families, and society. The second panel asks whether and how to assess value, that is, putting a price on value.

Panel 1: How can we Define Value for Rare for Patients and Society? Martin Cho, Takeda; Tammy Moore, ALS Canada; Wayne Critchley, Global Public Affairs; Oxana Iliach, CORD; Fred Horne, 3Sixty Public Affairs

Panel 2: Can we put a Price on Value for Rare? Susa Benseler, University of Calgary; Carlene Todd, Roche Canada; Joan Weir, CLIHA; Rosalie Wyonch, CD Howe Institute

Co-chairs: Durhane Wong-Rieger and Bill Dempster

2:00 pm – 2:45 pm
Patient Information: Registries and Beyond
Patient data management systems are essential for collecting, integrating and analyzing different types and sources of data from pre-and post-diagnostic (genetic, genomic, phenotypic, and familial) tests, clinical trials, and therapeutic interventions, clinician-generated information as well as, patient-generated real-world-data including response to treatment, quality of life, and long-term patient outcomes.

Patient-engaged platforms for collection of real-world data including biomarker, clinical, and quality of life reports. On individual basis, determine responsiveness and adapt treatment accordingly. Through population analysis, understand impact in real-world usage and adapt access criteria and outcome measures to evaluate long-term population and societal impact.

- In Canada, some well-established patient registries serve some these functions including: Canadian Bleeding Disorders Registry and Canadian Hemophilia Registry; Canadian Cystic Fibrosis Registry; Laurie Lambert, CADTH

Conference Agenda

Day 1

Monday, November 21 (9:00 AM - 4:30 PM EDT)
Transforming Vision to Reality

- Can provincial, other publicly funded, and privately supported data platforms be used to collect and analyze patient information for clinical trials and drug response? Penny Rae, Alberta Health Service
- What governance procedures for data collection, data analysis, and data sharing are needed to advance optimal use of data? How can we create a pan-Canadian initiative to provide harmonized consent language (or core elements), clear custodianship of the data, efficient and equitable access procedures? Ma'n H. Zawati, McGill University
- What are open access patient platforms that can support patient submission of patient-relevant information and allow for patient control over use of their information, and patient input on choices related to their care? Katheron Intson, University of Toronto

2:45 pm – 3:00 pm
Break

3:00 pm – 4:30 pm
The Right Start: How to Scale Up National Programs in Screening, Testing and Diagnosis across the Lifespan

Diagnostic Odyssey: Shanice Burnett, The Sumaira Foundation

Roundtable 1: Genetic Diagnostic Programs. Newborn Screening. For many rare conditions, the path toward a timely, accurate diagnosis begins at birth with newborn screening. While some provincial programs have expanded NBS, there are no national standards and no procedures for adding new tests. A patient-led initiative provides a springboard for implementation of a nationwide NBS program. **Leads:** Whitney Goulstone, ImmUnity Canada; Jennifer van Gennip, NRBDO; Andreas Schulze, The Hospital for Sick Children; Pranesh Chakraborty, CHEO

Roundtable 2: Genomic Screening and Sequencing. As technology continues to improve, clinician experience continues to increases, the number of targeted therapies continue to proliferate, and sequencing costs continue to decline, genome sequencing will become increasing standardized in diagnosis and care.

- Greg Costain, Christian Marshall, The Hospital for Sick Children
- Kristin Kernohan, CHEO Care4Rare
- Alison Elliott, BC GenCOUNSEL
- Leon Atkins, Screen4Care Initiative

Conference Agenda

Day 2

Tuesday, November 22 (9:00 AM - 4:00 PM EDT)
Optimizing Patient Access and Outcomes

9:00 am – 9:30 am
Report Back: Welcome and Review of Infrastructure for Optimization Therapeutic Outcomes

- Network of Centres of Excellence and Distinction, Speciality Networks and HCP Networks
- Diagnosis from newborn to adult
- Patient Information Platform

9:30 am – 10:30 am
Innovative pathways to optimize access to innovative medicines: What is Canada Doing Well and How Can We Improve?
Investment in Canadian Research and Development in Innovative Therapies for Rare Diseases

Presentation: Sustainable, Cost-Effective Access to Specialized Therapies: Made-in-Canada Alternative – Conor Douglas, York University (15 min)

Panel Discussion: Incentivizing Innovative Therapies by Investing in R&D and Optimizing Return on Investment (Fair Pricing) (45 min)

- Investment in Canadian platforms to manufacture and deliver innovative drug therapies
  - Hospital generated CAR T-cell therapy
  - Viral vector platforms for gene therapy

Panel: Conor Douglas, York University; Stéphanie Michaud, BioCanRx; John Bell, Ottawa Hospital Research Institute; Risini Weeratna, NSRC

10:30 am – 10:45 am
Break

10:45 am – 12:30 pm
Presentation: Regulatory Approvals: Improving Coordination, Collaboration, and Consistency to assure timely and appropriate reviews for rare and ultra-rare therapies – Celia Lourenco, Health Canada (15 min)

Presentation: Experience with Managed Access to Rare and Ultra-Rare Therapies: The NICE Experience and Alternative Financing Models including upfront financing, Starter fund, “start-stop” criteria with real-world patient information, “pay for performance” agreements – Bill Dempster, 3Sixty Public Affairs (15 min)

Conference Agenda

Day 2

Tuesday, November 22 (9:00 AM - 4:00 PM EDT)
Optimizing Patient Access and Outcomes

Panel Discussion:
Leads: Alex Chambers, Novartis; Bill Dempster, 3Sixty Public Affairs
Panel: Christine Mossa, Ipsen Canada; Sang Mi Lee, MORSE Consulting; Fred Little, Pfizer; Bre Hamilton, ALS Action Canada; Susi Vander Wyk, Cure SMA Canada; Vijay Ramaswamy, The Hospital for Sick Children; Bennett Lee, Sanofi Canada

Case Studies of Challenges and Opportunities for Innovative Drug Access

- Neurofibromatosis Type 1 (NF1) and selumetinib
- Fibrodysplasia Ossificans Progressiva (FOP) and palovarotene
- (Obstructive) Hypertrophic Cardiomyopathy (HCM) and mavacamten
- Amyotrophic Lateral Sclerosis (ALS) and new drugs (edaravone, Relyvrio, masitinib)
- Spinal Muscular Atrophy (SMA) and nusinersen, onasemnogene abeparvovec, risdiplam
- Other pending therapies

12:30 pm – 1:30 pm
Lunch

1:30 pm – 2:30pm
Investing in Canadian research on innovative rare disease therapies

- Bespoke Gene Therapy – Terry Pirovolakis, CureSPG50
- From Genomics to New Therapies for Rare Diseases – Vincent Mooser, McGill University
- PharmacoGenomics: A Model for Managed Access to Rare Disease Therapies – Bruce Carleton, University of British Columbia

2:30 pm – 2:45 pm
Break

Conference Agenda

Day 2

Tuesday, November 22 (9:00 AM - 4:00 PM EDT)
Optimizing Patient Access and Outcomes

2:45 pm – 3:45 pm
Patient Engagement
This session focuses on critical patient engagement throughout the RD Strategy and RD Drug Strategy. What are the key roles for patients at all levels and in all areas? In what ways can we assure that the system is prepared to fully engage patients? What are the advances and outstanding challenges to patients as full partners in design, implementation, and governance? What are changes in policy that need to take place? What are the experiences and skills that patients and patient advocates bring to the table and what are additional training, development and supports that are needed to ensure that all patients are able to participate in informed decisions at individual and system levels.

Panelists: David Page, Canadian Hemophilia Society; Durhane Wong-Rieger, CORD; Danielle Rice, McMaster University; Sherry Caldwell, Ontario Disability Coalition; Joan Paulin, PHA Canada,

3:45 pm – 4:00 pm
Wrap up & Next Steps

Conference Sponsors

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.

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