Fall 2024 Rare Disease Conference

Nov 26 - 27 2024

Teaching Pigs to Fly … or How to Leverage $1.5B Rare Disease Drug Strategy into Value-Based Comprehensive Rare Disease Plan

Delta Hotels by Marriott Montréal
475 Av. du Président-Kennedy
Montréal, QC H3A 1J7

CORD Canadian Organization for Rare Disorders

RQMO REGROUPEMENT QUÉBÉCOIS DES MALADIES ORPHELINES

Teaching Pigs to Fly … or How to Leverage $1.5B Rare Disease Drug Strategy into Value-Based Comprehensive Rare Disease Plan

Conference Agenda (Draft)

Tuesday, November 26 (8:30 am - 5:00 pm)

8:30 am – 9:00 am
Registration and breakfast
Opus Ballroom

9:00 am – 9:10 am
Welcome Address
Durhane Wong-Rieger, Canadian Organization for Rare Disorders (CORD); Jonathan Pratt, Regroupement québécois des maladies orphelines (RQMO)

9:10 am – 9:30 am

I. **The Opportunity AND the Challenge**

How can we secure funding for a Canadian Rare Disease Plan? Assure value-based return on investment for the current $1.5 Billion!

Opportunity: In February 2019, after more than a dozen years of patient-led advocacy, the Canadian government stepped forward to announce an allocation of $1 billion to set up a National Rare Disease Drug Strategy (RDDS). Government focus shifted to the ensuing federal election put the rare disease opportunity on hold and unexpected all-encompassing demands for dealing with the exigencies of COVID dominated public health for most of the following year. The Canadian Organization for Rare Disorders (CORD) recognized that for rare disease patients and families, their rare conditions posed equal, if not more, serious and life-threatening risks than COVID. No one knew when COVID would be over; in order not to lose the opportunity of a Rare Disease Strategy, CORD initiated multistakeholder consultations in the midst of COVID and over the course of the next year consolidated the rare disease community's recommendations for the actualization of the RDDS.

In March 2023, Health Minister Jean-Yves Duclos announced $1.5 billion over three years to implement the National Strategy for Drugs for Rare Disease (NSDRD) with (a) $20M over three years for CADTH and to the CIHI to improve the collection and use of data and improving patient registries; (b) $32M over five years for CIHR to advance a RD research agenda with a focus on developing diagnostic and tracking tools and a RD clinical trials networks; (c) $16M over three years to establish national infrastructure to support the implement the strategy. The bulk of the monies, $1.4 billion, are to be allocated through bilateral agreements with provinces and territories to fund a "common" list of rare disease drugs with a goal to ending the "postal code lottery" and improving equity.

In April 2024, with no transparency into the progress on the bilateral agreements, the federal government announced the three-year period for the agreements would be April 2024 – April 2027. In July 2024, the federal and British Columbia governments signed the first RDDS bilateral agreement, allocating $194 million to BC over three years to improve access to new and existing drugs for rare diseases, enhance early diagnosis, and expand screening capabilities. The agreement announced the first two out of a list of 12 "common" drugs to be funded; these are **Poteligeo** (for mycosis fungoides or Sézary syndrome) and **Oxlumo** (for primary hyperoxaluria type 1). The BC agreement is likely to be the template for others. Within the specifics of the agreement, BC is required to track the use of funds and assure that 50% of the funds be allocated to cover at least one of the 12 drugs within the first 1-2 years and 10% be allocated to improve screening and diagnosis. The remaining 40% may be spent on emerging therapies and to equalize access to existing rare disease drugs.

Challenge: The $1.5B funding for the National Strategy for Drugs for Rare Diseases is in place for three years, ending April 2027. There is the promise but no guarantee about renewal. The opportunity is the discussion about developing an evaluation framework. This is also a challenge since there are no articulated expected outcomes for the NRDDS, either at the federal or the provincial/territorial levels. By establishing an evaluation framework now with short-term, mid-term, and long-term outcomes, we can help assure that the actions tied to the allocation of funds will be directed to achieving these outcomes and, importantly, allow us to adapt actions or recalibrate outcomes to assure we can deliver on goals by end of Year 3. Given the significant amount of funds committed and the breadth of stakeholders, it is essential that we come together to develop the value proposition from the perspective of all, including the rare disease community, the policy makers, caretakers of the funding, industry, private insurers, and the public.

This conference will take up the challenge to define an evaluation framework that measures the return on investment for the $1.5B allocated for the National Rare Disease Drug Strategy.

Rare disease exists within a dynamic ecosystem with mutually influencing components, each of which impacts on the others and each of which needs to derive value from participation to contribute and continue. We all do better when each of us experiences benefit. Collectively, we will create and elaborate a logic model for the Rare Disease Drug Strategy that transcends reimbursement for drugs to identify all the components of a value-chain, including but not limited to the patient journey. Some of the questions we will address are:

1. Who are all the stakeholders participating in the NRDDS? What if the value proposition to be realized from the NRDDS for each of the stakeholders?
2. How are our values, principles, and ultimate outcomes in alignment? Where are there differences or conflicts? What are our superordinate goals? How do we reconcile differences?
3. For the NRDDS to be considered effective, what are our (respective) short-term, middle-term and long-term outcome indicators? What would demonstrate value (appropriate return) for an investment of $1.5 B?
4. What are the outputs or achievable indicators for each stakeholder that would demonstrate value?
5. What are the key components of the logic model linking resources, inputs, outputs, and outcomes that defines an integrated Rare Disease System. How can we transform these into metrics to guide the evaluation?

II. Who's Who in the Barnyard?

Moderator: William (Bill) Dempster, 3Sixty Public Affairs
9:30 am – 10:30 am

A**: Who's Minding the Hen House?** (30 min)

- Rare Disease Drug Strategy: Daniel MacDonald, Health Canada

Questions: What advances in HC regulatory processes facilitate clinical trials and drug submissions? What allows for timely, streamlined and appropriate reviews and approvals for (advanced) rare disease therapies? What are challenges and what could be done better? How can the RDDS augment the regulatory review process?

Comment on:

- Priority Review

- Notice of Compliance with Conditions (NOC/c):
- Expedited Review Pathways for emergency or urgent rare disease cases.
- Real-World Evidence (RWE) Initiatives:
- Rolling Submissions and Adaptive Licensing:
- International Collaborations

B: Pathways and "Shortcuts" to Access (30 min)

Using the case studies as context, comment on opportunities and challenges in rare disease drug assessment process.

Panel:

- Trish Caetano, CDA
- Mélanie Caron, INESSS

Questions: What pathways exist to facilitate the review of drugs for rare diseases, pre-, during, and post-approvals? What extraordinary pathways help meet urgent needs, pre-, during, and post-approvals? What are challenges and what could be done better? (30 min)

Comment on:

- Compassionate access (corporate)
- Quebec patient d'exception
- CDA Time-limited recommendation (TLR) Pathway
- pCPA Temporary Access Process (pTAP)

BREAK
10:30 am – 10:45 am

10:45 am – 12:00 pm

III. Delivering on the Promise (Part 1)

C. 12 "Common" Rare Disease Drugs (45 min)

How can the RDDS deliver on the promise of improving access to new and existing drugs for rare diseases, ending the "postal code lottery" with equitable access? Which drugs will be covered, and for which patients?

Panel 1: Designated Top Dogs

The announcement of the signing of HC-BC bilateral agreement also identified the first two of the 12 “common” drugs to be funded under the RDDS.

The agreement provided scant information on dispensing of funds. In this session, we will discuss approved, recommended, and recently or pending negotiation with pCPA. Our challenge is to leverage the bilateral agreements to improve screening and diagnosis as well as specialist refferal for appropriate prescribing, data collection, and patient management

Designated therapies

- **Poteligeo** (Kyowa Kirin) for **mycosis fungoides or Sézary syndrome**
- **Oxlumo** (Alnylam) for **primary hyperoxaluria type 1**

Therapies completed, in, or pending negotiations

- **Hemgenix** (CSL Behring) for **Hemophilia B**
- **Becvez** (Pfizer) for **Hemophilia B**
- **Evkeeza** (Utragenyz) for **Homozygous Familial Hypercholesterolemia**
- **Uplizna** (Amgen/Horizon) for **Neuromyelitis optica spectrum disorder (NMOSD)**
- **Leqvio** (Novartis) for **Primary hypercholesterolemia (non-familial and heterozygous familial)**
- **Optune** (Novocure) for **glioblastoma** (brain cancer)

Who: Patients, disease specialists, manufacturers
Panel: Scott Warren, Alnylam; Dr. Jennifer Adams, Oxalosis & Hyperoxaluria Foundation Canadian Ambassador; Henry Zheng, Ultragenyx; Sarah Ford, Canadian Hemophilia Society; Kate Jervis, Novartis

Questions: *What is each condition, what are treatment options and what is the impact of the new drug? How is the condition recognized and diagnosed? How many Canadians have been diagnosed relative to the number that are potentially living with the condition? How many Canadians have access and how many could potentially have access? What are best practices (in Canada or elsewhere) for enabling timely access and monitoring patient outcomes? How could the RDDS help assure appropriate use and facilitate the collection of real-world data to assess value for patient well-being and economic1: investment?*

Panel 2: Why Won’t This Dog Bark? (30 min)

Who: Fred Horne, 3Sixty Public Affairs (former AB Health Minister); Wayne Critchley, Global Public Affairs; Jida El-Hajjar, ALS Action Canada

These therapies are directed toward unmet needs, some urgent, and all address serious conditions, many progressive with life-altering and even life-threatening consequences, so *why are patients still waiting for access?*

All have been approved by Health Canada, many through the expedited pathway. Some are authorized with conditions requiring supplemental or extended clinical trials and/or post-market surveillance and collection of real-world data. But all are available in the USA and most throughout Europe. *So why not already in Canada?*

All have CADTH/INESS recommendations. Many are prescribed and available to Canadians who have private drug insurance. *So why aren't they already reimbursed by public drug plans for all Canadians?*

Why won't this dog bark?
Do we still lack of awareness about rare conditions? Is there a failed sense of urgency, despite the fact that a child dies of a rare condition every 39 minutes?

Do we fear loss of budgetary control, being overwhelmed by the need to screen and diagnose, lack of expertise to prescribe accurately?

Do we lack consensus on outcomes that matter or lack of benefit of early treatment? Do we still lack the economic proof of the value of preventing serious disease and saving lives?

Lunch
12:00 pm – 1:00 pm

IV. Rewarding Early Risers

1:00 pm – 1:45pm

Opening Remarks: John Snowden, Amgen (15 min)

Panel 3: Early Birds … (30 min)

Panel: Biba Tinga, Sickle Cell Disease Association of Canada; Max Johnson, Recordati Rare Diseases Canada; Pamela diCenzo, Acadia Pharmaceuticals; Deanna Badiuk, Acromegaly Canada; Susi Vander Wyk, CureSMA Canada

Moderator: William (Bill) Dempster

Some of the drugs that could be covered by the unrestricted 40% of the bilateral funds are emerging therapies, recently submitted to Health Canada and CDA or in the pipeline to be submitted. Still others are existing approved drugs that are not (equitably) accessible across the country. These include:

- Casgevym (Vertex) for Sickle Cell Disease (SCD)
- Tepezza (Amgen) for Thyroid Eye Disease
- Signifor LAR (Recordati) for Acromegaly
- Trikafta (Vertex) for Cystic Fibrosis – extended to all mutations
- Risdiplam (Roche) and Spinraza (Biogen) for Spinal Muscular Atrophy (all ages)
- Daybue (Acadia) for the treatment of Rett syndrome

Questions: *What is each condition, what are treatment options and what is the impact of the new drug? How is the condition recognized and diagnosed? How many Canadians have been diagnosed relative to the number that are potentially living with the condition?*

Panel 4: … Get the Worm! (45min)

These are therapies already approved by Health Canada, many through an expedited review process to address unmet needs. Some are authorized with conditions requiring supplemental or extended clinical trials and/or post-market surveillance and collection of real-world data. Most are available in the USA as well as Europe.

In what ways could the funding from RDDS help provide immediate appropriate access for patients at the time of regulatory approval? What are options for managed access that could be designed to allow for real-world data collection to monitoring safety and effectives

Panel:

- Innovative Agreements: Sang-Mi Lee, Morse Consulting
- Building systems that work for real-world evidence development and USE!: Rebeccah Marsh, Institute of Health Economics
- Addressing Unmet Needs: Lisa McCoy, Defeat Duchenne Canada
- Deborah Marshall, University of Calgary (virtual)

Moderator: Durhane Wong-Rieger

V. Focus on Quebec Rare Disease

1:30 pm – 3:00 pm

Moderator: Jonathan Pratt, RQMO

Panel 1: Quebec Rare Disease National Plan

- Jade Falardeau, MSSS

Panel 2: Quebec Rare Disease Research Group

- Michelle Marquis, Research Institute of the McGill University Health Centre
- Dr. Philippe Campeau, CHU Sainte-Justine Research Center
- Dr. Nicolas Pilon, UQAM - Université du Québec à Montréal

Panel 3: Quebec Diagnosis and Care

- Dr Vincent Mooser, McGill University (Genomics)
- Dr. Angela Genge, The Neuro (ALS)
- Catherine Boivin, CORD

BREAK
3:00 pm – 3:15 pm

3:15 pm – 4:45 pm

VI. Moonshots Across the Globe (75 min)

Moderator: Durhane Wong-Rieger

Multiple countries have launched their own "Moonshots for Rare Disease" with national plans that can serve as roadmaps (space paths?) and inspiration for the Canadian Rare Disease System. These vary considerably and demonstrate there is no single right way to "do rare." Some other countries provide integrated comprehensive multi-sectoral programs with federal funding and support; others are regionalized or decentralized to serve designated populations; and still others are multidisciplinary rare disease centres spanning research, education, training, diagnosis, specialty care, family support, and follow up.

Presentation of specific programs, with key learnings, success factors, and recommendations for the future.

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A. Global Rare Disease Programs

- European Reference Networks
- Barcelona: Children's Hospital Centre of Expertise
- China: Multi-site specialty hospital program
- Brazil: Comprehensive RD Assessment and Management Centre of Excellence (Casa dos Raros)
- Saudi Arabia: Moonshot
- Romania: NoRo Rare Disease Centre

- Latin America: Spotlight Colombia
- Poland: Comprehensive integrated RD plan
- Czech Republic: MCDA for Rare Disease Drugs

B. Reflection Panel (4:15 – 4:45 pm)

Panel: Jade Falardeau, MSSS; François Bernier, University of Calgary; Kim Steele, Cystic Fibrosis Canada; Alison Oliver, Cencora, Peter Brenders, Beigene

4:45 pm – 5:00 pm

VII. Wrap-Up Day 1

5:00 pm – 7:00 pm

Networking Reception – Opus Foyer

* All conference participants are invited to attend.

Wednesday, November 27 (8:30 am - 3:30 pm)

Leadership from the Ground Up

8:30 am – 9:00 am
Breakfast
Opus Ballroom

9:00 am – 9:15 am

Learnings from Day 1 Durhane Wong-Rieger

Day 1 provided a dazzling cornucopia of rare disease possibilities drawing from across the globe and across multiple sectors in Canada. How has the Canadian Rare Disease Ecosystem evolved over the past decade? What are we doing well and what would do we need to improve? What are key learnings from across the globe that we can apply to accelerate the development of our system here in Canada?

I. Canadian Rare Disease Infrastructure for Optimal Access to Therapies

How can the Canadian rare disease infrastructure be optimized to deliver on the promise of accelerated, equitable access to innovative therapies? What are the essential elements that can fuel the rare disease patient journey to access? A panel of experts and users will participate in presentation and discussion.

9:15 am – 10:45 am

A. Panel Presentations and Discussion

Moderator: Walter Robinson, CORD

- **Canada Rare Disease Centres and Network**

  Panel:
  - François Bernier, Canadian Rare Disease Network
  - Dr. Leanne Ward, CHEO, Research Chair Rare Bone Health (virtual)
  - Dr. Ian Stedman, York University
  - Cathy Evanochko, CORD/Tuberous Sclerosis Canada

- **Screening and Beyond**: What are strategies and tools, including AI, for screening and identifying patients potentially eligible for funded treatment as early as possible? (15 min)
  - Newborn Screening Program: CDA (TBC)
  - Targeting Rare Patients in Primary Care: Don Watts, Khure Health
  - Caregiver Perspective: Lindsay Williamson, CORD

- **Genetic and Genomic Testing**: How can targeted and population-wide genetic and genomic strategies be employed to accurately and efficiently diagnose patients and extended families affected by rare disease? How can we move genomic sequencing from experimental and research uses to real-world clinical use to accelerate access to care and treatment? (10 min)
    - AllforOne: Camille Varin-Tremblay, CHU Sainte-Justine Research Center

- **Socioeconomic impact:** How can RDS support socioeconomic impact studies to determine impact of rare disease for individuals, families, and society and, importantly, the value of therapeutic intervention, especially innovative drugs funded by the RDDS. What are current studies and tools in use; what can be adapted across diseases; what needs to be customized? Who will conduct and who will own the findings? (20 min)
    - Pulmonary Arterial Hypertension Study: Jamie Myrah, PHA Canada
    - Comprehensive Approach to Measuring RD Outcomes and Impacts: Dr. Francois Bernier, University of Calgary
    - Rebeccah Marsh, Institute of Health Economics
    - Carla Chabot, CORD

B. Interactive Q&A (10 min)

10:45 am – 11:00 am
BREAK
11:00 am – 12:00 pm

II. Reflections from EU Cost of Living with Rare Disease Study and Opportunities for Canada

Moderator: Bill Dempster
Overivew of key findings of CRA research

Panelists:

- Durhane Wong-Rieger, CORD: Design of CRA survey and application to Canada
- Karen Heim, Astra Zeneca/Alexion: Key findings re: health equity; value of early diagnosis
- Lindy Forte: The economics of investing (and not-investing) in rare disease medicines
- Rebecca Marsh, Institute of Health Economics: Relevance for Canada's Rare Disease Drug Strategy and value-based therapeutics
- Tara Cowling, Medlior: Data and support for managed access

12:00 pm – 1:00 pm
LUNCH

1:00 pm – 2:00 pm

III. Proposal for Integrated Logic Model Underpinning an Evaluation Framework for assessing the outcomes and impact of National Strategy for Rare Disease

Moderator: Durhane Wong-Rieger

Panel: Bennett Lee, Sanofi Canada; Maureen Smith, INFORM RARE/RareKids-CAN; François Bernier, University of Calgary; Ian Stedman, York University; Fred Horne, 3Sixty Public Affairs and former Alberta health minister

Interactive session engaging all participants toward articulation of a logic model integrating all components of Rare Disease Network. Parallel streams will focus on (1) patient journey from identification to diagnosis to access to care and treatment to follow up and (2) the drug lifecycle from clinical trials to regulatory approval and HTA assessment to negotiations and public plan access to post-market evaluation of value. The goal is to gain consensus on the desired short-term, medium-term, and long-term outcomes, the corresponding outputs across all sectors, and the essential inputs and facilitating resources in addition to the $1.5 billion commitment from the National Strategy for Drugs for Rare Diseases. This logic model will make clear the essential components and processes for a pan-Canadian Rare Disease Plan necessary to achieve the desired outcomes and valued-based return on investment.

2:00 pm – 2:30 pm

IV. Gap Analysis: What is Needed to Succeed

2:30 pm – 3:00 pm

V. Conclusions and Next Steps