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
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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) (10 min)

9:10 am – 9:30 am

I. **The Opportunity AND the Challenge** (20 min)

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?

The stakeholders making up Canada's rare disease ecosystem provide the infrastructure for a comprehensive, integrated, national system that can serve patients from screening/diagnosis to optimal care and treatment as well as collaborate on innovative research, therapeutic development, clinical trials, data management, and outcomes assessment.

Three panels comprised of representatives from each of the stakeholder sectors will present updates on their contributions to the Canadian Rare Disease Ecosystem and discuss opportunities and challenges for coordination and collaboration.

9:30 am – 11:00 am

Part A: Governments and Agencies (90 min)

1. Canadian government has allocated $1.5 billion over 3 years (2024-27) to spend on Rare Disease Drugs in addition to assuring regulatory approval and post-market monitoring procedures are compatible with global standards and meet the requirements of therapeutics developed specifically for rare disease populations
2. Canada's Drug Agency (despite not having specified a pathway for drugs for rare diseases) has acquired clearer and more fulsome responsibilities related to timely assessment of therapeutic value of innovative therapies, recommendations about pricing of rare disease drugs compared to existing therapies, assessment of appropriate real-world use of drugs and other technologies, recommendations for newborn screening, guidelines for patient registry development and support for their implementation, coordination on real-world data and evidence, including quality, collection, analysis, and application for drug monitoring of safety, effectiveness, and value based on real-world feedback,
3. The Patented Medicines Price Regulatory Board (PMPRB) guidelines, still under revision, set maximum price based primarily on average price of basket of comparator countries with no special provisions for rare disease or other "first in class" drugs.
4. The Pan-Canadian Pharmaceutical Alliance (pCPA), comprised of all public payers, negotiates a common price with manufacturers of patented medicine (premised on CDA recommendation) with no expedited pathway for drugs for rare diseases, despite accelerated regulatory and HTA review timelines, resulting often in long timelines for access through public drug plans without transparent rationale.
5. Provincial, Territorial and Federal Drug Plans will participate through bilateral agreements under the Rare Disease Drug Strategy with up to $1.4 billion allocated over three years, starting in April 2024. Specific terms of the agreement (based on first signed agreement with British Columbia) include:
6. 50% for 12 common drugs designated by Health Canada (without transparent rationale but likely to benefit from and amenable to post-market real-world data collection (without elaboration on what and how); still subject to province-specific procedures for providing access
    - 10% screening and diagnosis toward national standards (no what or how)
    - 40% new and existing drugs
    - Reporting: at least one of 12 common drugs in years 1-2 and reporting on timeline to access
7. Provincial Rare Disease Plans are not defined as part of the RD Drug Strategy, but initial frameworks and projects are in place and/or being developed.
    - Quebec RD Framework – developed but limited budget allocated and implementation in selected aspects

- Alberta Rare Disease Plan – contracted to Bio Alberta for consultation and recommendation

11:00 am – 11:15 am
BREAK

11:15 am – 12:30 pm

Part B: Rare Disease Clinical and Research Community (75 min)

1. **Canadian Rare Disease Network** works across geographies and diseases uniting all partners to enable timely diagnosis and access to treatment and facilitate best care, support and empowerment for patients and their families.
   - Pillar 1: Equitable timely diagnosis for all RD patients
   - Pillar 2: Equitable access to clinical trials and innovative therapies integrated into clinical care
   - Pillar 3: Patients and families participating co-creators toward equitable access to research, clinical trials, mental health resources, and other community supports.
   - Pillar 4: Canada participating as a global partner in RD research and knowledge exchange, benefiting patients worldwide.
2. **RareKids-CAN** is a network of scientists, patient advocates, and academic institutions, funded by a five-year CIHR grant to provide support and services to (initially) academic investigator-initiated interventional clinical trials for rare paediatric populations.
3. **Canadian Institutes of Health Research** is a federal agency funding health research, including biogenetic and cellular therapies, precision medicine and rare diseases
4. **Genome Canada** is a not-for-profit organization that funds large-scale genomic and precision medicine research, including personalized medicine and rare genomic disorders.
5. **The Centre for Commercialization of Regenerative Medicine** is a Canadian not-for-profit organization that collaborates with academia, industry, and government to translate research in cellular therapies and precision medicine into clinical applications, including for rare diseases.
6. **National Research Council of Canada (NRC) – Human Health Therapeutics** is engaged in the development of biologics, vaccines, and precision therapies, with research projects targeting rare diseases, cancers, and immune disorders.

7. **CHU Sainte-Justine Research Centre** is a pediatric research hospital in Quebec and is heavily involved in precision medicine and cellular therapies, particularly in pediatric genetic disorders and rare conditions.

12:30 pm – 1:30 pm
LUNCH

1:30 pm – 3:00 pm

Part C: Rare Disease Patient Community (90 min)

1. **Canadian Organization for Rare Disorders (CORD)** is the national alliance bringing together rare disease patient organizations, healthcare providers, researchers, industry partners, health system representatives, payers, and policy makers.
2. **Regroupement québécois des maladies orphelines (RQMO)** est un regroupement d'associations de maladies rares, ainsi que de personnes atteintes de maladies rares et leurs proches aidants qui n'ont pas d'association.
3. **Patient Organizations**

- ALS Society and ALS Action Canada
- Canadian FOP Network
- Muscular Dystrophy Canada and Cure SMA Canada
- Network of Rare Blood Disorder Organizations (NRBDO)
  - aHUS Canada
  - aTTP
  - Canadian Hemophilia Society
  - ImmUnity Canada
  - Thalassemia Foundation of Canada
  - Sickle Cell Anemia Association of Quebec
- VHL Canada

3:00 pm – 3:15 pm
BREAK

3:15 pm – 4:45 pm

III. Moonshots Across the Globe

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.

A panel of representatives from across the globe and across Canada will provide an overview of their respective programs, with key learnings, success factors, and recommendations for the future. This will be followed by an interactive discussion among panelists and with the participants.

A. Panel presentations (75 min)

- UK Comprehensive national program
- USA Centres of Excellence
- Taiwan Rare Disease program
- Saudi Arabia/UAE/Qatar: focus on genomics
- Poland comprehensive integrated RD plan
- China multi-site specialty hospital program
- Brazil Comprehensive RD Assessment and Management Centre of Excellence (Casa dos Raros)
- Barcelona Children's Hospital Centre of Expertise
- Czech Republic: MCDA for Rare Disease Drugs
- Canadian Provincial Plans
    - Quebec RD Plan
    - Alberta RD Plan
    - Others?

B. Interactive Q&A with Panel and All Participants (15 min)

4:45 pm – 5:00 pm

IV. 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 (15 min)

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** (75 min)

- **Newborn Screening and Beyond**: What are strategies and tools, including AI, for screening and identifying patients potentially eligible for funded treatment as early as possible?
- **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?
- **Patient Data Collection**: What are platforms, tools, and strategies for actively and passively collecting (real-world) patient data that can help to identify, manage, and monitor patient access to therapies in real-world settings. What are the strengths, weaknesses, benefits, and challenges of patient registries, electronic health records,

mobile health devices/wearables, direct-to-patient digital platforms, and natural history studies? What are the challenges and solutions for data standardization, and interoperability, as well as patient privacy, consent, and engagement.

- **Patient Evidence**: What are the challenges and solutions for transforming patient data into patient evidence useable for assessing individual safety, benefits, and risks as well as collective patient outcomes, comparative effectiveness, cost-effectiveness, and socioeconomic impacts.
- **Rare Disease Centres and Extended Networks:** How can rare disease centres and specialty centres optimize accurate and timely diagnosis, prescribe optimal care and treatment, maintain patient registries, provide support for local on-going patient management, and provide long-term management and support. What are roles for RDCs to develop/support best practice guidelines, engage in training and mentoring initiatives, collaborate with other RDCs, support clinical trials, therapeutic innovations, and other research initiatives.
- **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?
- **Industry Partnerships:** What are ways in which industry is currently partnering throughout the rare disease patient journey in Canada and what is needed to improve the value of collaborations? What is needed to increase industry investment in rare disease in Canada, from academic partnerships to patient engagement.
- **AI Applications for Rare Disease:** AI is already making significant impact in the rare disease journey from identification to diagnosis to best care to patient data management and outcomes analysis. How can we leverage AI to implement, enhance, and demonstrate value of the RDDS at all points of the drug lifecycle/patient journey.

B. Interactive Q&A (15 min)

10:45 am – 11:00 am
BREAK

11:00 am – 12:00 pm

II. Reducing Access Inequities to Innovative Rare Disease Drugs … What RDDS Funding Could Do (60 min)

Toward addressing inequities in access to rare disease drugs, the bilateral agreements between the federal government and the provinces/territories will identify a list of 12 "common" drugs that should/could be funded by the public drive plans, although the terms for access leave considerable discretion to the public plans. What are the common drugs and how could optimal equitable access be improved? What are the requirements for a rare disease infrastructure to provide

- Discussion of RDDS Rare Disease Drugs included in the "Common List of 12"
- Discussion of Emerging and Existing Drugs Not Specified in the "common list"

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** (60 min)

The pinnacle event (NOT TO BE MISSED) is a full-throated 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** (30 min)

2:30 pm – 3:00 pm

V. **Conclusions and Next Steps**