Canadian Organization for Rare Disorders

CONFERENCE
JUNE 8 - 9, 2022
Ottawa Marriott Hotel

Building Canada's SMART Rare Disease and Rare Drug System

Building Canada's SMART Rare Disease and Rare Drug System June 8-9, 2022
Ottawa Marriott Hotel (Virtual option available)

Rare One Canada:
Canada's Smart Rare Disease and Rare Drug Strategy

Over the past 20 months, the Canadian rare disease community, under the leadership of the Canadian Organization for Rare Disorders, has come together in 36 webinars and forums to create a collaborative vision of an optimal national Rare Disease Program supported by a comprehensive and integrated Rare Disease healthcare system. Now, we are ready to propose an operational framework to realize that vision. We are NOT proposing minor tweaks to address gaps in access or improve timeliness. We must NOT start with a formulary of "common" or "priority" rare disease therapies, even if these are fully funded for all patients.

CORD is calling on all stakeholders at this conference to arrive at a consensus on a complete re-imagining of a system from the bottom up and top down that is "fit for purpose" of assuring all Canadians have access to the best therapies as soon as they are available anywhere in the world.

We need to start with a patient-centred rare disease system that assures every person, in as short a time as possible, gets an accurate diagnosis, sees a specialist, forms an individualized care, treatment, and support plan, is enrolled in a patient registry, is referred to a patient support group, gets timely monitored access to the best treatment and, most important, has the knowledge and right to participate as a full partner in all health-related decisions. This should be readily available for all Canadians, regardless of where they live, their insurance coverage or their ability to pay.

Rare Drug Conference

Why is Rare Disease a Public Health Issue?

There are more Canadians with a rare disease than with all cancers, cardiovascular disease, diagnosed diabetes. Altogether, 3 million Canadians, about 1 in 12, have a rare disease. Moreover, while 80% of rare diseases are genetic, 50% have no known family history. So everyone is potentially at risk. Two-thirds of those affected are children, 30% of whom will not live to their fifth birthday. Sadly, in Canada, a child dies of a rare disease every 18 minutes.

In terms of economic impact, rare disease costs the Canadian economy about $111 billion per year in direct medical costs, nonmedical cost, and productivity costs.

Why is NOW the time for Canada's SMART Rare Drug Strategy?

Rare disease drugs are saving lives, preventing and reducing disability, allowing patients and families to live more "normal" productive lives, and, for some conditions, providing a long-lasting treatment or "cure." Rare disease research is leveraging genomic breakthroughs, advanced medical technologies, application of data science and artificial intelligence to big data, patient registries, and real-world data, multiple disease cell-and-gene therapy platforms, and remote patient engagement. So rare disease therapies are at the forefront of innovative research, providing solutions with impacts beyond rare diseases, including viruses, cancers, and cardiovascular diseases.

Thanks to the US and EU Orphan Drug Acts, about 600 new therapies have been approved in the past 40 years but these cover only about 5% of up to 7,000 rare diseases. To date, Canada has contributed to only a small handful of rare disease drug discoveries. However, that scenario can and should change. Canada's burgeoning life sciences strategy has the scientific, clinical, and patient capacity to contribute significantly to discovery, manufacture, and management of rare disease diagnosis and treatment.

Most would agree that our drug assessment procedures were never designed to accommodate innovative therapies for small patient populations but we have continued to put them through the process and have mostly "cobbled" "one-off" reimbursement agreements with ostensibly minimal impact on the overall drug budgets. The solutions were rarely evidence-based, equitable, or sustainable. The question is not whether we have reached the breaking point with the "old" access pathways but "how can we do better?"

Conference Agenda

Day 1

Wednesday, June 8 (9:00AM - 5:00PM EDT)
Old Challenges, New Opportunities for Rare Drug Access

We will present “case studies” of challenges experienced in access to rare disease therapies by patients in Canada as well as new therapies. Following presentation of each case study, an invited multidisciplinary stakeholder panel will discuss the case. Following all case discussions, there will be a final stakeholder panel with request for active engagement of all participants.

8:30 am – 9:00 am
Registration and Continental Breakfast
Ottawa Marriott Hotel, Cartier II (Lower Level)

9:00 am – 9:15 am
Conference Begins
Opening Remarks, Adam van Koeverden, MP, Parliamentary Secretary to the Minister of Health

9:15 am – 9:45 am
Session 1: Visioning: What does a SMART Rare Disease Drug Program look like?

Visioning
Cast yourself into the future, 3 to 5 years from now. If we are collectively successful, what does this drug system look like? Consider a number of opportunities.

1. An international research-based pharmaceutical company announces the intention of launching Phase 3 clinical trials for a serious and potentially life-threatening rare disease affecting between 1,500 to 5,000 Canadians. It is currently treated with a 30+-year-old oral therapy which in early stages of the disease but is less effective in later stages.
2. A global pharmaceutical manufacturer has purchased an orphan-designated “breakthrough” therapy for a “serious progressive directed toward the underlying cause of an ultra-rare condition. The original “start-up” company had published “extremely promising” phase two clinical trial data showing slowing of progression but not reversal of symptoms. Patients are urging the company to file for “expedited” regulatory approval while executing Phase 3 clinical trials.

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Wednesday, June 8 (9:00AM - 5:00PM EDT)

Old Challenges, New Opportunities for Rare Drug Access

Thinking Smart

1. What does “SMART” look like? How should a “fit for purpose” Rare Drug Program work for each of these therapies? What processes should be used to assess and prioritize providing access/funding of these four therapies.
2. Consider the optimal Rare Drug Program from the perspective of each stakeholder: patient/family, healthcare professional (specialist, geneticist, GP, nurse, pharmacist), health unit (hospital, health network), public drug plan, private drug insurer(s), provincial government (funder), federal government regulator, research-based (innovative) drug manufacturers, generic/biosimilar drug manufacturers, others.\
3. What are key attributes or processes of SMART shared by all or most stakeholders?
4. What are key differences in priorities or attributes across stakeholders? Which stakeholders are aligned with one another and which are not?

Panelists

- Federal: Stakeholder: Michelle Mujoomdar, Health Canada
- CADTH: Suzanne McGurn, CADTH
- Industry: Carrie McElroy, Sanofi
- Industry: Rute Fernandes, Takeda Canada
- Patient: David Page, Canadian Hemophilia Society

9:45 am – 10:30 am
Diagnosis for All

- Newborn Screening: Pranesh Chakraborty, University of Ottawa
- Genomic Sequencing: Kym Boycott, CHEO
- Phenotypes: Orion Buske, PhenoTips
- KHURE Health: Don Watts, KHURE

10:30 am – 10:45 am
Break

Conference Agenda

Day 1

Wednesday, June 8 (9:00AM - 5:00PM EDT)
Old Challenges, New Opportunities for Rare Drug Access

10:45 am – 12:00 pm
Registries and Real-World Data
Scalable Patient Registries
- IAM RARE: Pamela Gavin, NORD
- INFORM RARE: Beth Potter, Alexandra Wyatt, Pranesh Chakraborty, Monica Lamoureux, John Adams, Kim Angel

Opportunities and Challenges for Data Management
Real-World Data and Real-World Evidence
- Patient support programs: Sandra Anderson, Innomar Strategies
- AI for Data Management and Enhancement: Aaron Leibtag, Pentavere
- Patient Support and RWE: Laurie Lambert, CADTH

12:00 pm – 1:00 pm
Lunch

1:00 pm – 2:30 pm
Session 2: Mapping Individual Patient Access to Rare Drug Program and Rare Disease System

Critical Patient Experiences
Over the past TWO decades, rare disease patients and families have broken new ground and paved the way to access, overcoming seemingly insurmountable obstacles with tremendous courage and unrelenting resolution. These are the experiences of some of those champions that guided our progress then and now.

A. Mapping Individual Patient Journey
1. Awareness: *Screening, Testing, Diagnosis, Referral OR Denial, Delayed Testing, Misdiagnosis*
2. Specialist Care and Treatment: *Rare disease specialist-led comprehensive care linked to local provider OR Delayed, limited, no access to therapy,*
3. Management and Follow Up: *Individualized patient management OR Lost to follow*

Patient Experiences (1:00 pm – 1:45 pm)
- Fabry's Disease: 20 years of evidence - Ed & Marlene Koning
- aHUS: Catch 22 - Michael & Margriet Eygenraam
- Cystic Fibrosis: "Getting Loud" to Save Lives - Beth Vanstone, Stephanie Stavros
- SMA: More Opportunities, More Challenges - Catherine Boivin

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Old Challenges, New Opportunities for Rare Drug Access

Emerging Challenges

- Fibrodysplasia Ossificans Progressiva - Carrie Connell, Canadian FOP Network
- Porphyria - Anna Mann, Canadian Association for Prophyria
- Long-chain Fatty Acid Oxidation Disorder - Tyler Rogers, Ultragenyx
- Hyperoxaluria - Jennifer Adams

B. Drug Program Level: Patient management program with template/protocol including individual patient accountability, use of digital technology, data sharing, aggregate data analysis, and program level recommendations

What is status of Canadian access for RD drugs? (1:45 pm – 2:30 pm)

- Canada access and Rest of World - Alexandra Chambers, Novartis
- Canada access to essential rare disease drugs - Nigel Rawson
- Canada private drug plan access to rare drugs - Jessica Brcko, Sunlife/ Leezann Freed-Lobchuk, Canada Life

2:30 pm – 2:45 pm
Break

2:45 pm – 4:30 pm
Session 3: Network of Centres of Excellence

Exemplar Rare Disease Specialty Centres (2:45 pm – 3:15 pm)
Rare Disease Programs organized around rare diseases and disease clusters integrate all aspects of specialty care including diagnosis, assessment, prescribing, treatment management and follow up in clinic and in the community. Model programs include:

- Inherited Metabolic Diseases Program and Network - Pranesh Chakraborty, CHEO
- The Ottawa Pediatric Bone Health Research Group and The Canadian Consortium for Children's Bone Health/Canadian Alliance for Rare Disorders of the Skeleton - Leanne Ward, CHEO
- Canadian Neuromuscular Network, Western University - Craig Campbell, LHSC

Conference Agenda

Day 1

Wednesday, June 8 (9:00AM - 5:00PM EDT)
Old Challenges, New Opportunities for Rare Drug Access

Global, International, and National Rare Disease Networks
(3:15 pm - 4:30 pm)

To optimize a national Rare Disease Drug Program, Canada needs a comprehensive and integrated Rare Disease Network of Centres of Excellence, appropriately resourced and empowered to work locally and collectively to provide: newborn screening, genetic and genomic testing, accurate and timely diagnosis for infants through adulthood, access to specialty care and treatment, and registries that integrate all patient data with capacity to securely share across sites. This would allow for optimal individualized prescrbing of drugs and other therapeutic interventions, continuous monitoring using real-world data of outcomes and adverse effects, aggregated data management and analysis for clinical trials and other research, development of best practices, and contributions to outcomes and cost-effectiveness assessments.

- Canadian Network of Rare Disease Centres of Excellence - Paula Robeson, Children's Healthcare Canada
- WHO-RDI Global Rare Disease Network - Matt Bolz-Johnson, EURORDIS
- Rare Disease Research Network and National Children's Hospital - Marshall Summar, Rare Disease Institute
- NORD Centres of Excellence - Pamela Gavin, NORD

4:30 pm – 5:00 pm
Reflections and Q&A

- Matt Bolz-Johnson, WHO-RDI Global Rare Disease Networks
- Marshall Summar, Rare Disease Research Institute
- Pamela Gavin, National Organization for Rare Disorders
- Paula Robeson, Children's Healthcare Canada
- Leanne Ward, CHEO
- Craig Campbell, LHSC

5:00 pm – 5:15 pm
Summary and Day 2

5:*30* pm – 7:00 pm
Networking Cocktail Reception - Summit Lounge (29th floor)

Conference Agenda

Day 2

Thursday, June 9 (9:00AM - 4:00PM EDT)
Barriers and Opportunities Along Pathways to Access

We have invited experts and other stakeholders to present current and future strategies for addressing barriers to access discussed in Day 1.

9:00 am – 9:30 am
Recap of Day 1

9:30 am – 10:00 am
Session 4: Patient Engagement and Patient Empowerment

A cornerstone of a SMART health and drug program is fully integrated patient engagement at all levels from national, provincial/territorial, and local, and across governance, decision-making, advisory, and oversight functions. To ensure patients can serve as effective experts and representatives of the patient communities, patient organizations must be acknowledged and appropriately resourced to help recruit, train, and support patient representatives. To promote trust in the rare disease and to ensure accountability, patient representatives shall also be empowered to serve as liaisons between the rare disease communities and the healthcare and therapeutics committees.

Patient Engagement/Patient Empowerment Panel:
- Jamie Myrah, PHA Canada
- Christine White, National Gaucher Foundation/CORD
- Daphne Dumbrille, HAE Canada
- Oxana Illiach, CORD
- Nahya Awada, Carleton University

10:00 am – 12:00 pm
Session 5: Creating Canada's Rare Disease Network

Canadian De-Centralized Centres of Excellence

National Network
- Thierry Lacaze-Masmonteil, Maternal Infant Child Youth Research Network

Conference Agenda

Day 2

Thursday, June 9 (9:00AM - 4:00PM EDT)
Barriers and Opportunities Along Pathways to Access

Ontario Region
- Jim Dowling, The Hospital for Sick Children
- SickKids Research Institute
- Children's Hospital of Eastern Ontario
- McMaster University, Department of Pediatrics

Quebec Region
- Jacques Michaud, Sainte Justine
- Hugh McMillan, McGill - Montreal Children's Hospital
- Nicolas Chrestian, CHU Laval – Quebec

Prairies Network
- Cheryl Greenberg, Children's Hospital Research Institute of Manitoba

Atlantic Region
- TBC

Western Region
- Peter Kannu, Stollery Children Hospital, Edmonton
- Francois Bernier, Alberta Children's Hospital
- Micheil Innes, Alberta Children's Hospital
- Aneal Khan, MAGIC Clinic

12:00 pm – 1:00 pm
Lunch

1:00 pm – 1:45 pm
Session 6: Next Steps to Rare Disease Centres and Drug Management

The opportunity with the National Rare Disease Drug Program is to invest in Centres of Excellence that addresses the triple aim: access, sustainable financing and research. This session will feature two panels on the next steps for "getting to YES" with policy and political leaders across Canada. We know the WHAT – now we need the HOW.

Looking forward and forecasting the potential pipeline for RD medicines in Canada, what are we looking at on two levels: clinical (Fred, Paul, Christian) and economic (Lindy) impacts?

What are you seeing at the global level in terms of getting these efforts underway and into reality: what does it take?

Conference Agenda

Day 2

Thursday, June 9 (9:00AM - 4:00PM EDT)
Barriers and Opportunities Along Pathways to Access

Panelists
- Janie Trepanier, Pfizer
- Paul Petrelli, Jazz Pharmaceuticals
- Christian Hansen, Alexion
- Lindy Forte, EVERSANA
- Åke Blomqvist, Carleton University/Paul Grootendorst, University of Toronto

Format: Open Panel discussion
Moderator: Bill Dempster, 3Sixty Public Affairs

1:45 pm – 2:30 pm
Session 7: Building on Momentum to Make #Canada4Rare a Reality

This session will explore what's needed to bring more awareness to the reality and challenges of patients and caregivers in the rare diseases community, so that their experiences can be brought to bear on the all-out effort to make rare disease centres of excellence a reality.

Questions we'll explore include:

- What are some of the lessons learned from other grassroots efforts to build on momentum so that the experience with RDs can be improved with a new system?
- What's happening at the provincial level that can help layer up and inform the national / federal strategy? (e.g., lessons from Ontario and Quebec)
- What is the role of each stakeholder in the system to make the case for the new system, including researchers, health leaders, policymakers, medicine developers and patients?

Panelists
- Wayne Critchley, Global Public Affairs
- Beth VanStone & Stephanie Stavros, CF Get Loud
- Jason Field, Life Sciences Ontario
- Anil Kaul, Sobi Canada
- Farah Bendahmane, Montréal InVivo

Conference Agenda

Day 2

Thursday, June 9 (9:00AM - 4:00PM EDT)
Barriers and Opportunities Along Pathways to Access

2:30 pm – 2:45 pm
Break

2:45 pm – 3:30 pm
Session 8: Grounding the Centres of Excellence in Research

This session will consider the frontiers of research in rare diseases and specifically the role of research in the new rare diseases centres of excellence.

Questions that we'll explore include:

- What technological advances are on the horizon for rare diseases that need to help inform rare diseases centres of excellence, including diagnostics, treatments and other clinical interventions?
- What is the role of research within the centres of excellence?
- How can investing in research help overcome systemic / jurisdictional barriers?

Panelists

- Jason Field, Life Sciences Ontario
- Ivana Cecic, Genome Canada
- Étienne Richer, CIHR

Format: Open Panel discussion

Moderator: Bill Dempster, 3Sixty Public Affairs

3:30 pm – 4:00 pm
Conclusion: Bringing Everyone Together for Next Steps
The concluding session will identify new insights on old and emerging issues, new understandings of opportunities and challenges, and recommendations for collaborative next steps as we move with urgency toward the implementation of Canada's Rare Disease and Rare Drug System.

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.