Canadian Organization for Rare Disorders

CONFERENCE

JUNE 8 - 9, 2022

Ottawa Marriott Hotel

Building Canada's SMART Rare Disease and Rare Drug System

CORD CONFERENCE

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

GETTING SMART

The last in-person CORD conference was in Ottawa May 2020 when we were collectively just envisioning a national Rare Disease Drug Strategy, still somewhat enamored with the $1 billion commitment to start up and $500 million per annum to help sustain. We took full advantage of people being trapped during the COVID pandemic to pull together all stakeholders in consultation after consultation (32 in all).

Thanks to all our partners, we achieved a high level of consensus on a vision for a national Rare Drug Strategy and the building blocks of a Rare Disease System, essential to achieving the goals of person-centred timely and equitable access to the best rare disease therapies for all Canadians. We also arrived at understanding of the necessity of designing a SMART system that is agile, measurably cost-effective, and sustainable. In other words, it must be built to be patient engaged, goal directed, and technology empowered.

This two-day conference brings together the stakeholders and the learnings from all CORD and others' consultations. We will collectively continue to bring in international expertise and benefit from global strategic thinking, innovative initiatives, and best practices. Specifically, the underpinnings of the program are:

- **SMART health systems designed to be rapid learning systems**
- **SMART health systems engaged in agile planning and continuously updating SMART goals**
- **SMART health systems integrated by SMART Technology**

We are designing and implementing drug and health systems that improve healthcare performance and outcomes by are not just for rare but have elements of
The way forward is digital transformation of healthcare, including digitally connecting (remote) patients and services, collecting and sharing patient (controlled) data, and employing AI to streamline everything from clinical trials to health workforce management.

CONFERENCE AGENDA

Day 1: Thinking and Planning SMART

Setting the Stage
Brief introduction on what we have been doing, what we have achieved over the past two years of engagement, and where we are now, with perspectives from individuals representing different stakeholder communities.

Session 1: Visioning: What does SMART look like?

Open space, greenfield visioning exercise to creatively explore and re-imagine, at a high level, what a "fit for purpose" SMART Rare Disease and Rare Drug System should look like. All participants will work in rotating facilitated small "think tanks" groups and larger "sharing" sessions, culminating in a plenary presentation and panel discussion. At the end of the session, we will seek to achieve consensus on:

- Values, Principles, Goals
- Actionable Steps and Outcomes
- Technologically Enabled Access Pathways
- Patient Engagement at Every Stage, at Every Level

Session 2: Mind the Gap: Where are we now and where do we want to go?

A panel of representatives from the CORD Advisory Group on Developing a Rare Drug Strategy will present on the key learnings from the series of CORD Webinars and other consultations (Health Canada's What we Heard Report. They will engage with the audience on the opportunities and challenges arising from the consultations for "next steps" and, importantly, propose the critical role of patients throughout the proposed Rare Drug System.

1. Report back from Consultations
2. Identified and Emerging Opportunities and Challenges
3. Defining Critical Roles of Patients as individuals and families, patient organizations, partners in management and monitoring, and policy makers

Day 1: Thinking and Planning SMART

Session 3: BUILDING SMART

The building blocks for a SMART Rare Disease System are infrastructure, technology, and investment in innovation. These sessions will bring forth the discussions and outcomes from the Webinar Consultations held in April and May 2022, as well as other learnings. The format will consist of brief presentations from lead stakeholders on each topic area, the identified capabilities and opportunities that currently exist, the challenges and potential hurdles for advancing to a status to meet the needs of optimal therapeutic access and management, and the requirements for sustainability and advancement.
The global rare disease ecosystem, international initiatives that are available for learning and connecting, and partnerships beyond Canada will also be explored with representatives from the global rare disease community (WHO, RDI, International Rare Disease Research Consortium, NORD, EURORDIS and others). Three areas for discussion:

1. Infrastructure
We will discuss the key components for a SMART Rare Disease System and how to build on existing expertise and to expand capabilities, within Canada and internationally.

- Centres of Excellence
- Patient Registries
- Smart Diagnostic Capabilities: Preventive, person-engaged, cost-efficient (one-stop), connected to care, captured for care and research
- Research Capacities

2. Technology
Investing in digital health technology and tools are essential to building a SMART Rare Disease and Rare Drug System. Drawing from the May Webinar and other examples, a panel of experts and users will present key learnings, opportunities, challenges, and immediate next steps. They will expand upon the following topics, among others, with the audience.

- Community empowered healthcare
- Data connected
- Ai enabled healthcare
- Person empowered health management

Networking Cocktail Reception (5 - 7 pm)

Day 2: Thinking and Planning SMART

Session 3: BUILDING SMART (cont'd)

Investment in Innovation
Canada has tremendous capability in rare disease research but has never had specific orphan drug legislation to incentivize and support R&D, comparable to the USA and EMA Orphan Drug Acts. We feel investment in innovation is a critical component of the rare disease strategy, to contribute to the global drug supply but also to generate a return on rare disease investment. Moreover, innovation investment should include the entire patient journey, including diagnosis, specialist care and management, drug monitoring and data collection, and patient outcomes.

- FDA/EU Orphan Drug Acts
- Canadian Research Capabilities and initiatives
- Support programs

Session 4: SMART Pathways for Innovative Therapies

The establishment of a Rare Disease Drug Strategy is the opportunity to re-imagine pathways for access that would assure timely and equitable access, designed to fill the gaps in today's therapies but also building the system in anticipation of the drugs that will be available in near and more distant future.

This session will be anchored by patient experiences and case studies, those discussed in the May webinars and additional ones. These are examples of specific challenges, some with successful access outcomes, sometimes only after multiple interventions over months and years. Other therapies remain inaccessible to some or all patients who would potentially benefit based on Health Canada approval.

Brief vignettes plus an overview of recommendations from the May Webinars will be discussed by a multistakeholder panel with the goal of defining several "viable" alternative access pathways specific to one or more rare disease therapies.

Following the panel discussion, participants will be directed into "problem solving" small groups, each of which will be assigned an actual therapy and disease scenario or a hypothetical (fictitious) case. Each group will consider proposed alternative pathways, combinations, or variations, to arrive at an optimal "way forward" that would best meet the patient-centred goals of timely, effective, and equitable access and system objectives, including value-based and sustainable access. Group solutions will be presented, defended, and debated in a plenary session.

Day 2: Thinking and Planning SMART

Session 5: SMART Funding

There is strong preference for a single Rare Disease and Rare Drug System integrated across all provinces and territories, across all levels of government (federal, provincial/territorial, local), across all health providers (hospital, community, specialist, general practice, and allied), and across all sources of funding (public drug plan, private plan insurer, corporate, and personal).

A panel representing public plans, private health insurers, corporate plan sponsors, consultants, and individuals will be presented with several profiles of “patients, rare conditions, and therapies) to work toward consensus on the criteria for effective funding models.

Session 6: Leave No One Behind

This multistakeholder panel will consider “outlier” policy and access issues, some of which affect only a very few rare disease patient and others that affect many patients across many rare (and not-so-rare) conditions. Case examples, each of which illustrates one or more access barriers, will be presente. All of the conference participants will be encouraged to “brainstorm” solutions and the panel will deliberate their pro’s, con’s and feasibility to arrive at recommendations for further development. Areas of interest include:

- Ultra-rare conditions (example: FOP)
- Orphan indications for rare disease treatments (example: Cystic Fibrosis)
- Use of common therapies for rare conditions (example: Prader-Willi Syndrome)
- Generic therapies (issue for many rare conditions)

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.