Canadian Organization for Rare Disorders

Rare Drug Strategy Consultations

Join CORD this spring for the opportunity of a lifetime. Have your say in building Canada's Rare Disease Drug Strategy. Engage in two-part series with Webinar Primer on March 23 - 24 and LIVE In-Person Conference in Ottawa on June 8 - 9. These will be highly interactive, solutions-oriented sessions using case studies of rare drug access from Canada's archives, connecting to current access challenges and extending to emergent rare drug introductions. What are lessons we can learn and how can we apply these to building a "smarter" drug strategy for today and tomorrow?

Rare Drug Strategy Webinar

March 23-24, 2022 (virtual)

Building Canada's Smart Rare Drug Strategy: Meeting Patient Needs

Drugs for rare diseases did not fit the traditional R&D paradigm and, hence, many were "orphaned" until the US FDA Orphan Drug Act and subsequent EMA Orphan Drug Legislation unlocked them. Regulatory approval processes also evolved to accommodate clinical trial designs specific to small patient populations, resulting in over 600 approved orphan products. Guidelines for health technology assessment were not designed for many of the highly innovative therapies, including drugs for rare diseases and gene therapies.

DAY 1: **Wednesday, March 23, (11:00AM - 2:00PM 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.

Challenge 1: Delayed/denied access across patient community

Examples: Hypophosphatasia, hypophosphatemia, and Fibrodysplasia ossificans progressive

Challenge 2: Delayed and limited access (valuation)

Examples: Fabry Disease, atypical Hemolytic Uremic Syndrome, and Porphyria

Challenge 3: Access limited to clinical trials population

Examples: Pompe Disease, Spinal Muscular Atrophy

Challenge 4: Access delayed by negotiations

Examples: Amyloidosis Drugs 1, 2, 3

Challenge 5: Getting to "yes"

Examples: Pancreatic cancer, Cholangiocarcinoma, long-chain fatty acid disorders

DAY 2:

Thursday, March 24, (11:00AM - 2:00PM 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.

Barrier 1: Delayed and wrong diagnosis

Opportunities: AI powered rare disease diagnosis at family clinics; AI phenotype-genotype linked databases

Barrier 2: Appropriate assessment methods for defining access criteria, monitoring, and valuation

Opportunities: Managed access programs with patient assessment and monitoring

Barrier 3: Lack of multidisciplinary rare disease centres

Opportunities: Neuromuscular disorder centres, inherited metabolic disorders, Network of Rare Disease Centres

Barrier 4: Lack of patient registries and real-world data utilization

Opportunities: Neuromuscular disorder centres, inherited metabolic disorders, Network of Rare Disease Centres

To register, visit our website: www.raredisorders.ca

Rare Drug Strategy Conference (preview)

June 8 - 9, 2022 Ottawa, ON (in-person)

Implementing Canada's Smart Rare Drug Strategy: Adaptive, Advanced, Appropriate, Affordable Access

Workshop Format on 5 Key Implementation Goals

Implementation Goal 1: Patients and patient organizations integrated in all aspects at all levels; patient needs and patient outcomes at centre of rare drug strategy design, implementation, and monitoring

- Patient inclusion and transparency in all phases of drug access through patient and public outreach and accessibility of materials, evaluations, and decisions
- Patients and families included in developing criteria and outcome measures for managed access to therapies
- Patient representatives/advocates trained and supported to participated as "expert patients"

Implementation Goal 2: Accurate diagnosis and referral without delays and misdiagnoses

- Universal newborn screening for "core" rare conditions with timely results and genetic follow-up
- Genomic sequencing to identify patients with genetic variants to provide access to linked to therapies
- Creation of national database for rare diseases

- Awareness, education, tools, and technology (AI-enabled) to engage patients/public, family physicians/paediatricians, and allied professionals (pharmacists, educators, social workers, therapists)

Implementation Goal 3: National Network of Rare Disease Centres of Excellence

- Nationally aligned network of provincial healthcare centres, rare disease research organizations, and patient organizations working collaboratively to provide access to state-of-the art diagnosis, quality care and treatment, and comprehensive data management
- Provincially based "spoke-and-hub" configuration with connections to community-based care providers providing expert consultation, education, training, and support
- Multidisciplinary rare disease specialty clinics networked across provinces and internationally
- Linked rare disease patient data bases including patient registries and electronic health records for real world data collection and analysis
- Rare disease research network supporting clinical trials, natural history studies, patient outcomes research, and innovative therapeutics development and research

Implementation Goal 4: Multiple access pathways to assure all patients get access to all necessary medicines

- Experimental therapies (in development)
- Clinical trials
- Early access (awaiting approval) and special access (not submitted)
- Managed access (beneficial for high unmet need with additional evidence to be collected)

Implementation Goal 5: Investment in innovative research

- Orphan drug Incentives
- Rare disease innovation hubs
- Focus on unmet needs

Registration information will be posted on our website shortly.