Canadian Organization for Rare Disorders

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: Gaucher's Disease, Spinal Muscular Atrophy

Challenge 4: Access delayed by negotiations

Examples: ATTR Amyloidosis, Cystic Fibrosis, Retinal disease

Challenge 5: Getting to "yes"

Examples: Tuberous sclerosis,, Cholangiocarcinoma, long-chain fatty acid disorders

11:00 am - 11:15 am	Welcome and overview	Durhane Wong-Rieger, CORD Bill Dempster, 3Sixty Public Affairs
11:15 am - 12:00 pm	Challenge 1: Delayed/denied access across patient community Examples: Hypophosphatasia, hypophosphatemia, and Fibrodysplasia ossificans progressive	Cheryl Rockman-Greenberg (Max Rady School of Medicine), Carrie Connell (CA FOP Network), Aimee Sulliman (Alexion Canada), Leanne Ward (Pediatrics, University of Ottawa)
12:00 pm - 12:30 pm	Challenge 2: Delayed and limited access (valuation) Examples: Fabry Disease, atypical Hemolytic Uremic Syndrome, and Porphyria	Michael & Margrit Eygenraam, Ed & Marlene Koning (Fabry's Canada), Anna Mann (CA Assn Porphyria), Julie Schneiderman (Novartis Canada)

12:30 pm - 1:00 pm	Challenge 3: Access limited to clinical trials population Examples: Gaucher's Disease, Spinal Muscular Atrophy	*Susi & Holli Vander Wyk (SMA Canada),* Craig Campbell (Children's Hospital, LHSC) Christine White (Gaucher Canada)
1:00 pm - 1:30 pm	Challenge 4: Access delayed by negotiations Examples: ATTR Amyloidosis. Cystic Fibrosis, retinal diseases	Beth & Maddi Vanstone, Anne Marie Carr (Hereditary Amyloidosis Canada), Fred Little (Pfizer Canada), Doug Earle (Fighting Blindness Canada)
1:30 pm - 2:00 pm	Challenge 5: Getting to "yes" Examples: Tuberous Sclerosis, Cholangiocarcinoma, long-chain fatty acid disorders	Cathy Evanochko (TSC Canada), Sam Stankovic (Incyte Canada), Julie Totten (Ultragenyx), Martin Cho (Takeda)

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: Disease-specific patient registries, real-world evidence initiatives, patient data management

11:00 am - 11:20 am	Recap of Day 1	Durhane Wong-Rieger, CORD Bill Dempster (3 Sixty Public Affairs)
11:20 am - 12:00 pm	Barrier 1: Delayed and wrong diagnosis Opportunities: AI powered rare disease diagnosis at family clinics; AI phenotype-genotype linked databases	Matt Osmond (CHEO), Don Watts (Khure Health), Stacey Listern (Muscular Dystrophy Canada), Kayla McNally (CMV Canada), Ivana Cecic (Genome Canada), Orion Buske (PhenoTips)
12:00 pm - 12:40 pm	Barrier 2: Appropriate assessment methods for defining access criteria, monitoring, and valuation Opportunities: Managed access programs with patient assessment and monitoring	Ed Dybka (Ipsen Canada), Brad Alyward (CORD), Oxana Illiach (CORD), Catherine Boivin (CORD), Leanne Ward (Pediatrics, University of Ottawa)
12:30 pm – 12:40 pm	Break	

12:40 pm - 1:00 pm	Barrier 3: Lack of multidisciplinary rare disease centres Opportunities: Neuromuscular disorder centres, inherited metabolic disorders, Network of Rare Disease Centres	Matt Bolz-Johnson (Rare Diseases International), Craig Campbell (Children's Hospital, LHSC), Riyad Elbard (Thalassemia Canada), Christine White (Gaucher Canada), Thierry Lacaze-Masmonteil (MICYRN), Chris McMaster/Etienne Richer (CIHR)
1:00 pm - 1:30 pm	Barrier 4: Lack of patient registries and real-world data utilization Opportunities: Disease-specific patient registries, real-world evidence initiatives, patient data management	Tara Cowling (Melior), Emma Lynn (CADTH), Andrew Taylor (Health Canada), Sandra Anderson (Innomar Strategies), David Page (Canadian Hemophilia Society), *Beth Potter (CHEO, TBC)*
1:30 pm - 2:00 pm	Wrap up and next steps	All

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