

A MOON SHOT FOR RARE DISORDERS LEAVE NO ONE BEHIND

2017 Fall Conference
launches
November 6 – 7, 2017
Hyatt Regency Toronto

A MOON SHOT FOR RARE DISORDERS - OVERVIEW

“Our mission, put simply, is to discover, understand, treat, cure and ultimately prevent all rare disease”

Objectives of Conference

Create a shared understanding of the patient journey and what “good” looks like
Mobilize all stakeholders around common vision for Rare Disease Canada
Create a guiding framework to map current resources, define gaps, identify opportunities and challenges, and prioritize needs
Articulate next steps; gain commitment to action; define success factors, and outcome measures

Big Idea: Rare Diseases Canada

We are patient and family driven
We bring together all stakeholders, all disciplines, all activities, all services
We work toward rare disease sites linked from coast to coast
We envision centres of excellence empowering GPs, therapists, teachers, and support workers to effectively serve rare disease patients and families in their own community
We will enhance Canada’s reputation as a leader in rare diseases

What we stand for

Our starting point: Canada’s Rare Disease Strategy
Our belief: Rare is common when we come together



Canadian Organization
for Rare Disorders

How does Rare Disease Canada deliver on Canada's Rare Disease Strategy

Diagnosis and Prevention: Invest in state-of-the-art resources to ensure testing and diagnosis with appropriate counselling and referral are available to all families; engage in awareness, education, and early intervention to reduce incidence and impact

Support a common rare disease patient registry

Access to Care: Set up Rare Disease Canada as a national entity with multi-stakeholder funding and support, with regional and local sites, all linked to specialty sites and the local healthcare providers and support communities

Community Support: Ensure an activated network of rare disease patient and groups through various activities

Lead, coordinate, and coalesce advocacy, awareness, and promotion for rare disease

Provide training and resources to promote capacity of patient groups to serve their community, advocate, and collaborate on research, clinical trials, best practices, policy, etc.

Promote collaborations with international patient organizations

Access to Treatment: Ensure treatment access that is provided as soon as possible to appropriate patients, managed with real-world monitoring and follow-up, and supported by prices that are deemed fair and supportive of timely and appropriate Canadian access.

Research: Rare Disease Canada can serve as the hub for coordination, collaboration, experimentation, dissemination, and implementation of all types of research related to rare diseases, at all levels, inclusive of patients and families.

CONFERENCE OUTLINE

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| Session 1 | Understanding the Rare Disease Patient Journey |
| Session 2 | Mapping the Canadian Rare Disease Landscape: Expertise, resources, opportunities |
| Session 3 | Envisioning Ideal Rare Disease Canada national resource <ul style="list-style-type: none">Work Group 1: National Rare Disease Centre(s)Work Group 2: Specialized Reference Networks |
| Session 4 | Building on Success: Canadian examples of expertise and experience |
| Session 5 | Developing the Pillars of Canada's Rare Disease Strategy <ul style="list-style-type: none">Work Group 1: Diagnosis and PreventionWork Group 2: Access to Care and TreatmentWork Group 3: Community SupportWork Group 4: Access to TreatmentWork Group 5: Research Priorities |
| Session 6 | Defining Projects, Pilots, and Proofs-of-Concept |
| Session 7 | Achieving Optimal Access to Orphan Drugs (without Orphan Drug Regulatory Framework; without Rare Disease HTA Pathway; without Rare Disease Drug Budget) |