

CORD: Canada's Network for Rare Disorders



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RARE DISEASE DAY - 2010



Canadian Organization
for Rare Disorders

Rare Disorders by the Numbers



- **Important Numbers**

- Definition: Disease/Disorder that affects < 1:2,000 person
- Number of Rare Disorders: 6,000 to 8,000
- Number of Canadians with Rare Disorders: 1:12 (28,000)

- **Who is affected by Rare Disorders**

- 75% affect infants and children
- 80% have genetic cause (inherited or mutation)
- 20% caused by infection, environment or drugs

- **Drugs for Rare Disorders**

- Decade prior to 1983: Fewer than 10 new drugs
- Since 1983: 360 licensed drugs; > 2,000 in development



CORD represents all Rare Disorders



- **Canadian Network**
 - Rare Diseases Groups
 - Patients and Families
 - Healthcare Professionals
 - Researchers and Academics
 - Industry
- **Affiliations with**
 - NORD (USA)
 - EURORDIS (Europe)
 - Genetic Alliance (USA)
 - Children's Rare Disease Network



CORD Serves Rare Disorders



- **Common Voice for Rare Disorders**
 - Advocacy for Rare Disorder and Orphan Drug Policy
 - Advocacy for Access to Treatment
 - Advocacy for Diagnosis, including Newborn Screening
 - Advocacy for Care and Support
- **Public Awareness and Education**
- **Networking**
 - Canadian Genetic Coalition
 - PMQCO (Portail Québécois des Maladies Génétiques Orphelines)
- **Support for Research**
 - Fundraising for research
 - Support clinical trials



CORD Patient Support



- **Advocacy for patients and families**
- **Education and information for healthcare professionals**
- **Development and support for patient groups**
- **Patient and public education on drug approval and funding**
- **Information and support for genetic screening, testing and counselling**



CORD Brief History



- **Early history**

- Alberta Ehlers-Danlos Group
- Alberta Organization for Rare Disorders
- Canadian Organization for Rare Disorders

- **National actions**

- 2005-06: Proposal of CORD Orphan Drug Policy
- 2006: Move to Ontario
- 2007: April 24-25: 1st Canadian Conference on Rare Disorders
- 2008: February 29: First Rare Disease Day
- 2008: May 17: Motion M-426 (Rare Disease Program)
- 2009: March 1: Rare Disease Day Innovative Therapies Conference
- 2009: April 24-25: N of 1 Canadian Conference on Rare Disorders
- 2009: April 30: Action Day at Parliament Hill
- 2009: December 1: Consultation on Health Canada Draft Regulations for Orphan Drug and Rare Disease Policy



“Chance for Life” Rare Disorders Policy



- **Timely access to innovative therapies**

- Often only therapy for severe and life-threatening disorders
- Approval and funding with “early stage” evidence
- Access to all patients who potentially benefit with “stop” criteria for lack of benefit or harm
- Funding based on social values and patient impact as well as clinical evidence and cost-effectiveness
 - ✦ Values of beneficence and non-maleficence
 - ✦ Values of Canada Health Act: universal, comprehensive, portable, medically necessary, sustainable
 - ✦ Values of patient impact, self-determination
- Process includes all stakeholders



“Chance for Life”: Access to Therapy



- **National Advisory Committee**
 - Federal/Provincial/Territorial
 - All Stakeholders: clinicians, patients, policy makers
- **National drug access protocol**
 - Published and clinical data
 - International best practices
 - Patient impact and preferences
- **Monitoring for patient outcomes and adverse effects**
- **Funding mechanisms to ensure equitable national access**



Orphan Drug Policy (Proposed)



- **Based on international best practices–European Medicines Agency (EMA) and US Food & Drug Administration (FDA)**
- **International rare disease definition = 1:2,000**
- **Promote Research & Development for innovative drugs for rare diseases and unmet needs**
 - 10 year market exclusivity and data protection (EMA standards)
 - Assistance in protocol and clinical trials development
 - Financial incentives (reduced fees or exemption)
 - Priority review



Proposed (EU) Action on Rare Diseases



- **National plans or strategies for rare diseases**
- **Database (inventory) of rare diseases (Orphanet)**
- **Research programs at all levels, including community**
- **Centres of Expertise and Reference Networks**
 - Healthcare pathways
 - Information and communications technologies, including telehealth
 - Diffusion and mobility of expertise and knowledge to facilitate patient treatment
 - Multidisciplinary approach, care teams
- **Pooling of national and international expertise**
 - Sharing of best practices
 - Education and training
- **Empowerment of patient organizations**

