Why are rare disorders important?

- About 1 in 12 Canadians is affected by a rare disease (or disorder)
- Each rare disease affects only a few people:
  - Internationally, rare diseases have a prevalence of affecting fewer than 1 in 2,000 people
- However, there are more than 7,000 rare diseases which together affect 2.7 million Canadians
- About 80% are genetic conditions
- About 50% affect infants and children
- Most are life-threatening or severely debilitating

Why are Orphan Drugs Important?

- An orphan drug is a medicine for rare or neglected diseases

- Before 1983, there were almost no treatments for rare diseases
  - Number of patients was too small
  - Cost of developing treatment too high

- The US Orphan Drug Act 1983 lead to breakthroughs in treating rare diseases:
  - Nearly 400 new therapies since 1983
  - Some are life-saving, others prevent disability and some allow patients to resume nearly normal lives

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What is Canada Doing?

Canada is the only developed country without an orphan drug policy/strategy

Canadian researchers and companies have limited support to develop new therapies

Less than 50% of orphan drugs licensed in USA and Europe are available to Canadian patients

Canadian patients, researchers, biotech & pharma companies continue to advocate for Canada to implement an Orphan Drug Policy

Challenges without Rare Disorders Strategy/Policy in Canada

- No definition, no orphan drug policy; few orphan drugs
- Many Orphan Drugs in USA and EU
- USA: more than 300 products in 30 years of Orphan Drug Act
- EU: 100 new orphan drugs in 14 years
- Benefit: estimates of up to 10 million people
- Canadian patients have NO access to half of these drugs
- Health Canada approved 47% of orphan drugs licensed in USA and 51% of orphan drugs approved in Europe
- Concerns over separate and slower approval and lack of reimbursement

Implications of "Wrong" Funding Decision

Inappropriate "YES" to cost-ineffective drug

False hope (risk) to patients

Opportunity costs (waste of money)

Need to withdraw (not that easy)

Industry criticized and punished

Inappropriate "NO" to cost-effective drugs

Patients don't get access (do worse)

Inefficient use of resources (for untreated patients

and for therapies funded instead) -wrath of patients (especially if treatment available elsewhere)

Stifle innovation; industry move to "more certain" returns

Medical products for rare disorders

Medicines for rare and unmet needs tend to have:
High R&D, high uncertainty, high cost per patient

Reimbursement strategies directed toward:
reducing uncertainty in safety, effectiveness,
appropriate use, and budget impact

Managed access schemes include registries,
coverage with evidence development,
prior authorization, limited use, $ capitation

Initiatives in Europe

- 18th EURORDIS Round Table of Companies workshop

  looks at corporate responsibility and innovative policies for improving access to orphan medicinal products – Feb. 27, 2013

80 participants from 11 countries – EU, USA and Canada

EU has asked Member States to develop or update national plans for rare diseases by the end of 2013

Mechanism of Coordinated Access to Orphan Products (MoCA) part of a two year policy discussion - seeks to provide affordable and sustainable patient access to orphan medicines

- determine a *Common Assessment of the Value of an Orphan Medicine Product* to support well-informed national pricing decisions, reducing delays and duplication
- Clinical Added Value of Orphan Medicinal Products (CAVOMP)

Europe - continued

- *Differential Pricing* has improved access to medicines in developing countries

- A call was made for transparent Market Entry Plan/ Market Entry Agreement with a commitment of participating Member States to apply Differential Pricing principles and a commitment of companies to launch medicinal products within two years of receiving a marketing authorisation

- Net price confidentiality would be a central part of a differentiated pricing approach and payers must be engaged early in the process.

Canada is Making Progress

- 2006: Initial discussions on a federal/provincial/ territorial drug plan for rare disorders
- 2008: Parliament supported a motion calling for Canada to develop an Orphan Drug Policy, access for treatments for rare disorders and centres of excellence
- 2011: Canadian Institutes of Health Research launched first Grant Competition for Rare Diseases
  - Research Teams, awarding $17.8 million in 2012
- 2011: Health Canada initiated drafting an orphan drug regulatory framework

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Need a pan-Canadian strategy for rare disorders

Definition of Rare Disease/Disorder

Orphan Drug/Products/Medicines Policy

Access to Diagnosis, Treatment & Social Services

Research on Rare Diseases

Rare Disease Centers of Expertise/Reference

Screening, including Prenatal and Newborn