



Canadian Organization
for Rare Disorders

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CONSUMER ADVOCATE NETWORK

Media Invitation

Setting the record straight about treating rare diseases Real facts. Real patients.

Toronto, ON – May 1, 2014 – On Friday, May 2, the Canadian Organization for Rare Disorders (CORD) is inviting the media for a preview of the Framework for a Canadian Strategy for Rare Diseases, as well as to set the record straight about funding for drugs for diseases. Why do drugs for rare diseases cost so much? What is the healthcare budget impact of treating patients with orphan drugs? Is it worth it? How does Canada compare with other countries?

The media plays a very important role in raising awareness about rare diseases and the need for publicly funded access to treatment. Whether it's a call for a bone marrow donor or a plea to the government to provide drug funding, sometimes the story is right but the facts are not.

Please join CORD president, Durhane Wong-Rieger, as she discusses:

- How good is the current level of care for Canadians with rare diseases? What is working? What is not? Does Canada really lag behind Romania and Poland?
- Of the 7,000 rare diseases, how many have a drug treatment? What are the barriers to access, even for drugs that are inexpensive and already in use for another condition?
- Canada's Orphan Drug Regulatory Framework will significantly improve availability of new drugs for rare diseases. Why are the provincial drug plans worried, and why is there **no** cause for worry?
- How much does it **really** cost to treat rare diseases and what is the likely future impact?

Several GTA patients living with life-threatening rare diseases will be available for interview and to share their real stories of how access to treatment has positively benefited – and even saved – their lives.

Media who attend this special briefing are invited to stay for the remainder of the day's sessions.

Who: Durhane Wong-Rieger, president, CORD
Michael Eygenraam, living with atypical Hemolytic Uremic Syndrome (aHUS)
A clinician specializing in rare diseases

Where: Courtyard Marriott Downtown Toronto
475 Yonge Street
University Room A & B

When: Friday, May 2, 2014
8:30 – 9:30 a.m.

For further information or to book an interview, please contact:

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Rare Diseases in Canada

What is a rare disease?

A rare disease is often defined as a disorder that affects fewer than one in 2,000 people. There are over 7,000 different types of rare diseases, representing a variety of conditions that are characterized by a broad range of symptoms and causes. Many rare diseases are life-threatening, chronically debilitating and progressive, and can include certain cancers, metabolic diseases and infections.

How many Canadians are affected by a rare disease?

An estimated one in 12 (eight per cent, or 2.8 million) Canadians are living with a rare disease. Half of these individuals are children. Many others are affected or at risk, but remain undiagnosed and unaware of their disease. In fact, most Canadians are carriers of one or more rare genetic mutations.

What causes rare diseases?

Approximately 80 per cent of rare diseases are genetic (inherited); however, 40 per cent of rare diseases occur in families with no known history. Other rare diseases may be caused by infections, environmental exposure or allergic reactions.

How are rare diseases diagnosed and treated?

Unfortunately, the similarity of symptoms to more common diseases, as well as small patient populations and limited clinical experience and expertise regularly lead to delays in diagnosis and access to appropriate care, often with devastating consequences.

Once diagnosed, patients and their loved ones are often faced with the reality that, for many rare diseases, there are no effective treatments available. Further, if a treatment is available, it is often not accessible to the patients through private or public funding.

Why can't Canadians with rare disorders get access to the treatments they need?

Unlike many other developed countries – including France, Germany, Poland, Romania and the United Kingdom – Canada has yet to approve a national plan for rare diseases.

Due to many factors – including small patient populations and the costs associated with research and development – most rare disease drugs are more expensive than those for more common conditions. As a result, provincial and territorial governments are reluctant to fund treatments for rare diseases, directing funding to those that will achieve the 'greatest good for the greatest number.'

However, it has been proven that drugs for rare diseases consume less than one per cent of the total healthcare spend on pharmaceuticals. In countries like France, Germany and Belgium, where virtually every rare disease drug is available through the public drug plan, they represent still only 1.5 – 3.5 per cent of the total drug spend.

Why does Canada need a national strategy for rare diseases?

Canadians with rare diseases have a right to the same access to care, treatment and support as those with more common conditions. With one of the best healthcare systems in the world, Canada has the expertise and resources to build upon successful international models to develop the best rare disease program in the world. It's time for Canada to move from worst, to first.

What is CORD's Framework for a Canadian Strategy for Rare Diseases?

The framework is the cumulative efforts of a dedicated group of stakeholders, brought together under CORD's leadership, fuelled by the many researchers and clinicians dedicated to rare diseases, and inspired by the thousands of patients and families that have contributed directly and indirectly in many ways.

Through public consultations, the framework will be refined and used as the basis for developing a Canadian strategy for rare diseases. This policy platform will then be shared with governments for their consideration, with the hopes that it will help shape an appropriate, responsible and sustainable approach to providing publicly funded access to drugs for rare diseases.