

Summary of the Summit on Access to Drugs for Rare Diseases in Canada *Canmore, Alberta, Canada* *July 20 – 21, 2014*

This summit was organized by the Canadian Organization for Rare Disorders. This summary is based on proceedings prepared by the CIHR New Emerging Team on Developing Policies for the Effective Management of Technologies for Rare Diseases (PRISM – Promoting Rare-disease Innovations within Sustainable Mechanisms).

What's the current status of access to drugs for rare diseases?

In October 2012, the federal Minister of Health announced the development of an Orphan Drug Regulatory Framework. However, without a pan-Canadian Orphan Drug Access and Reimbursement Framework in place, Canadian patients only have access to approximately 50 to 60 per cent of rare disease drugs. Even worse, Canada's public drug plans have reimbursed only about half of the therapies that are approved by Health Canada.

The existing framework is intended to address issues drugs for rare disorders face in obtaining regulatory approval. The proposed guidelines will include orphan drug designation, clinical trial guidance, accelerated review, and post-market monitoring, which would be harmonized with those of the U.S. and the EU, allowing companies to apply for designation and clinical trials at the same time, reducing delays to access for Canadian patients.

While these guidelines will improve the regulatory process, reimbursement will continue to be a barrier to drug access for Canadians with rare disorders.

Why a summit on access to orphan drugs?

The summit, convened by the Canadian Organization for Rare Disorders, brought experts together to consider a pan-Canadian Orphan Drug Access and Reimbursement Framework, based on international and Canadian best practices, and built upon legislative changes for post-market monitoring under Bill C17 and a new transparent regulatory environment which allows for early patient input and interactions between regulators, manufacturers, and health technology assessment (HTA) bodies. This group looked at current managed access programs - arrangements between payers and manufacturers to reimburse drugs under specific conditions – and their applicability in the Canadian context, to build consensus on the best approach for Canada.

The purpose of the Summit on Access to Drugs for Rare Diseases in Canada was to:

- Learn from other countries' programs toward the development of an appropriate, responsible and sustainable approach to orphan drug access and reimbursement in Canada
- Identify common points of understanding, implications of various perspectives, and areas for continued dialogue among diverse stakeholders on a pan-Canadian approach to orphan drug access and reimbursement
- Discuss how the best interests of rare disease patients can contribute to the development of responsive policies and practices in treatment access and reimbursement

Who attended the summit?

The one-and-a-half day facilitated workshop, hosted by the Canadian Organization for Rare Disorders, included representatives from Health Canada, the Canadian Agency for Drugs and Technologies in Health (CADTH), Alberta Health, and international (Italy, Spain, and the UK) regulatory/funding bodies in addition to a professional facilitator, health service researchers, health policy consultants, physicians/clinical experts, patients/patient group representatives, and industry representatives.

What was discussed at the summit?

Lead experts from the National Health Service (NHS) England, the Italian Medicines Agency (AIFA), and the Catalan Health Services (Spain) spoke to the principles and realities of managed access programs to address questions of safety, efficacy, and budget impact of orphan drugs, and to promote appropriate, responsible, and sustainable access. Following the presentation by international experts and four panel discussions by Canadian stakeholders (payers, clinicians, patients, and industry), the summit participants worked in small groups to deliberate on the potential use of managed access plans as a pan-Canadian approach to reimbursement of orphan and/or ultra-orphan drugs in Canadian public drug plans.

What were the outcomes of the summit?

Summit participants agreed that managed access programs were a potentially viable approach to some, although not all, orphan drugs. They proposed several key success factors that are key to effective and sustainable managed access programs: pan-Canadian; a scientifically rigorous, inclusive, and transparent process; clear guidelines for “entering” and “exiting” treatment; management through centers of reference or expertise; patient registries for ongoing monitoring of outcomes; healthcare provider and patient responsibilities for adherence; and risk (cost) sharing agreements to manage budget impact. In addition, the participants proposed next steps for the implementation of managed access programs in Canada, which included setting up and learning from pilot program (existing and new), assuring transparency, developing a code of conduct, ensuring multi-stakeholder engagement, establishing mandatory patient registries, and promoting mutual trust between stakeholders.

CONTACT:

Durhane Wong-Rieger
Canadian Organization for Rare Disorders
durhane@sympatico.ca
416-969-7435
www.raredisorders.ca