INTRODUCTION

In October 2012, the Government of Canada announced its orphan drug initiative, intended to address the needs of an underserved population of patients – Canadians living with rare diseases.

A rare disease or disorder is a serious health condition that affects a very small number of patients – less than 1 in 2000 in the general population. Although the number of patients affected by each disease is small, a large number of different rare diseases have been identified, so there are a significant number of Canadians who will benefit from the initiatives that Government of Canada is undertaking.

The government announcement coincided with the launch of a Canadian portal for Orphanet, a reference portal for information on rare diseases and orphan drugs, intended for all audiences. It is an invaluable online resource for rare disease patients and their families, with participation of governments and organizations from approximately forty countries around the world.

October 2012 also marked the beginning of Health Canada's ongoing dialogue with researchers, healthcare providers, patient groups, drug manufacturers, foreign regulators and provincial and territorial drug plan managers – all with an eye to a new regulatory framework for drugs for rare diseases, also known as orphan drugs. New regulations for orphan drugs would allow for an adaptive, science based approach to the authorization of drugs for rare diseases. The proposed framework would be closely aligned with orphan drug regulations already in place in the United States and Europe, allowing Health Canada to work closely with international partners when making decisions on treatments for rare diseases. It is hoped that the regulatory initiative, along with government funding through the Canadian Institutes for Health Research aimed at increasing our understanding of rare diseases and developing new treatments, will make a difference in the lives of rare disease patients in Canada.

The five following papers showcase the work that is being done by various groups and organizations across the country. Health Canada recognizes the tremendous value of such contributions and looks forward to continuing cooperation and collaboration with stakeholders in all sectors of the healthcare system as work continues in this important field.

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