



Position Statement

***Towards Equitable Treatment of Rare Disorders:
Canadian Orphan Drug Policy***

October 9, 2007

Position Statement

Towards Equitable Treatment of Rare Disorders: Canadian Orphan Drug Policy

SUMMARY:

Up to ten per cent of the Canadian population has a disease classified as 'rare', also commonly referred to as an 'orphan' disease. The resulting social costs and burden of care implications make it imperative that a Canadian Orphan Drug Policy be developed immediately to ensure timely, affordable access to necessary therapies.

POSITION STATEMENTS:

Canadians with rare or 'orphan'¹ disorders that are progressively debilitating or life threatening need a made-in-Canada Orphan Drug Policy that will ensure they have timely, affordable access to the best therapies when needed.

Competitive incentives should be provided for Canadian-based companies engaging in research and development to bring new therapies to market. In turn, this incentive should be tied to a commitment by the companies to drug pricing relief to reflect these incentives.

The approach proposed in the section entitled "Expensive Drugs for Rare Diseases" as part of the National Pharmaceuticals Strategy (NPS) is worthwhile and warrants further development. The NPS progress report stresses the development and implementation of "consistent national processes and standards to ensure that Canadians with rare, severe and progressive life-threatening diseases have access to appropriate and affordable treatments."² These processes or standards should take the form of a Canadian Orphan Drug Policy, and be named as such.

Canada, as one of the last industrialized countries to institute an Orphan Drug Policy, should learn from other jurisdictions and create a policy based on best practices. The policy should reflect the entire pharmaceutical life cycle, including research, development and regulatory approval, affordable access and funding for drug coverage. Importantly, while based on international best practices, the policy must provide a made-in-Canada solution, addressing the roles of federal, provincial, and territorial governments and including other uniquely Canadian issues.

¹ Designation of rare or "orphan diseases" varies depending on the respective country, ranging from 1:9,090 (Australia) or 2,000 affected individuals, to 2:2,000 or 50,000 patients in the population.

² Federal/Provincial/Territorial Ministerial Task Force (June 2006). *National Pharmaceuticals Strategy Progress Report*. Health Canada.

ISSUES:

Currently, Canada does not have an Orphan Drug Policy or any effective strategy to address access and cost coverage for drugs needed by those Canadians with rare, severe and progressive or life-threatening rare disorders.³

Unlike their counterparts in other developed countries, Canadians with rare disorders are denied access to new medicines since Health Canada specifically rejected the need for a Canadian Orphan Drug Policy in 1997. At that time, other mechanisms to provide access were cited (e.g. priority fast track reviews, Special Approval Program, reduced fees for drugs with small market approval, and conditional approval based on surrogate markers or early trial results with agreement for post-market confirmatory studies.)

Although there are positive aspects regarding the orphan drug policies of other jurisdictions, including the fact that these policies or Acts actually exist and that we could learn from and use them in developing one in Canada, there are limitations that also need consideration. For example, the U.S. *Orphan Drug Act* of 1983 provides incentives for pharmaceutical manufacturers to develop drugs, biotechnology products, and medical devices for the treatment of rare diseases and conditions. However, this Act is limited in its mention of pricing conditions linked to the receipt of the incentives identified in the Act including “marketing exclusivity for orphan drug sponsors, tax incentives and research grants” (2001).⁴

Unfortunately, the mechanisms are not sufficient to ensure that manufacturers will seek market approval in Canada because of its small market. Since Canada does not offer competitive research and development incentives, there has been no Canadian-based pharmaceutical and biotechnological research and development for domestic use or export.⁵

BACKGROUND:

Currently, it is estimated that there are 5-6,000 rare diseases affecting approximately 10% of Canada’s population.

In the United States, millions of people have benefited from the Orphan Drug Legislation. Since its inception on January 4, 1983, 238 orphan designed products have been brought to market in the US — saving countless lives in the process. On November 27, 1996, Health Canada concluded that there was no need for such a policy in Canada and that access to products for rare disorders could be accommodated under existing programs (e.g. Catastrophic Drug Coverage). However, Canadians still do not have access to the best medicines and there is no accountability of the Drug Review Process

³ Concern expressed by members of Canadian Organization for Rare Disorders (CORD). (2005). *Canada’s Orphan Drug Policy: Learning from the Best!* CORD Position Paper.

⁴ Congressional Research Service, The Library of Congress (July 26, 2001). *Orphan Drug Act: Background and Proposed Legislation in the 107th Congress*. Order Code RS20971.

⁵ BIOTECanada (April 2004). BIOTECanada *Orphan Products Policy*.

to those affected by rare disorders.⁶ Furthermore, Canada has yet to establish Catastrophic Drug Coverage.

Orphan drug legislation and the entire process of bringing rare disorders treatment to market is an expensive proposition. Pharmaceutical companies may argue that the production of rare disorder drugs and treatments are commercially unprofitable due to the high costs of bringing to market a product for a very small patient population. Nevertheless, a truly universal healthcare system must guarantee this population both access and that further research and development will be made into treatments for rare disorders. In the United States, the *Orphan Drug Act of 1983* included policy on the research and development of tax incentives (such as percentage of clinical cost, exclusivity and expedited reviews for market approval).⁷

Such incentives would no doubt encourage further research and development into drugs and treatments for those affected by rare disorders in Canada, therefore guaranteeing both access and a voice to those affected by rare disorders, in both healthcare policy and the Drug Review Process. Furthermore, some existing treatments for rare disorders have been found useful for other, more common diseases, providing further motivation for their development as they have the potential to help a greater number of patients.

In 2004, the Health Ministers approved the establishment of the National Pharmaceuticals Strategy (NPS), which contains “Expensive Drugs for Rare Disorders” (EDRDs) as one of the five priority elements. In June 2006, in the progress report on the NPS, the Ministerial Task Force recommended that officials “accelerate work on a framework for EDRDs focusing primarily in the areas of evidence, ethics and the need to appropriately align regulatory and reimbursement systems.”⁸

As part of this recommendation, other steps were included such as looking at the international experiences, engaging stakeholders, developing a framework, and adding other elements. Such elements included tax incentives for research and development, market exclusivity, protocol assistance in the design and the development of clinical trials for rare disorders and subgroups, expedited or fast tracked submissions to both Health Canada and the Common Drug Review and assessments for appropriate pricing and reimbursement (drug plan programs).

The significance of having an Orphan Drug Policy is that rare disorders have a great impact on the healthcare system, on patients and their families, and on the community at large. Disorders that are not rapidly fatal often require continuing home and hospital care and lead to long-term disability. Because of the prevalence of genetic causes, illness often manifests in childhood. Without effective, early intervention, affected patients might not reach their full social and economic potential.⁹

⁶ Health Canada Drugs Directorate (January 16, 1997). *Orphan Drug Policy*. http://www.hc-sc.gc.ca/hpfb-dgpsa/tpd-dpt/orphdrug_e.pdf

⁷ Ibid. Congressional Research Service, The Library of Congress (July 26, 2001).

⁸ Federal/Provincial/Territorial Ministerial Task Force (2006). *National Pharmaceuticals Strategy Progress Report*. p. 35.

⁹ BIOTECanada *Orphan Products Policy*. (April 2004).

RECOMMENDATIONS:

1. Canada should develop a uniquely Canadian Orphan Drug Policy that adapts best practices from other countries, including incentives for research and development and distribution of orphan drugs, pre- and post- market, with a process to determine reasonable pricing, incentives and coverage;
2. The Therapeutic Products Directorate should ensure a fast-track process for orphan drug reviews; and
3. Processes for consideration of access and funding for drugs for rare diseases must not add further time lags, thus imposing unnecessary patient suffering. In addition, given the nature of rare diseases and their treatments, transparency and avenues for patient input must be instituted. As such, consideration of drugs for rare diseases should not fall under the responsibility of the Common Drug Review and Special Access Program.