

March 31, 2009

David Lee
Director, Health Canada
Policy, Planning and International Affairs Directorate
1600 Scott Street
Ottawa, Ontario K1A 0K9

Dear Mr. Lee,

On behalf of BIOTECanada member companies I am writing to encourage Health Canada to establish a national Orphan Product Policy to help Canadians with rare disorders. As Health Canada works towards modernizing the regulatory system for therapeutic products under the Progressive Licensing Project a clear opportunity exists to formally recognize orphan diseases and ultimately help those Canadians with uncommon life-threatening or chronically debilitating conditions.

BACKGROUND: Canadians and Provinces seek Orphan Products

Over the last year, Canadian citizens, government officials, policy-makers, politicians, and key opinion leaders across the country have come to recognize that Canada must develop a national Orphan Product Policy.

- In February 2008, the House of Commons Standing Committee on Health recommended that the “federal government work with its provincial and territorial Common Drug Review (CDR) counterparts to urge the Canadian Agency for Drugs and Technologies in Health (CADTH) to establish a specifically designed approach for the review of drugs for rare disorders and for first-in-class drugs.”
- In April 2008, the Government of Canada responded to the Standing Committee on Health recommendations by supporting the recommendation to pursue discussions with CADTH, participating provincial and territorial governments, and other stakeholders, on suitable approaches to assessing drugs to treat rare diseases.
- In April 2008, an Independent Advisory Panel that assessed Health Canada’s proposed Cost Recovery Framework recommended that the Government of Canada develop an Orphan Drug policy as soon as possible and suggested that a fee mitigation process be developed for these products.
- On May 7, 2008, the House of Commons unanimously passed Private Members Motion M426 on Rare Diseases sponsored by Member of Parliament Don Bell (North Vancouver). The motion called on the Government to consider the Canadian Organization for Rare Disorders (CORD) *Chance for Life Fund* as an action plan to bring Canada in line with other developed countries who offer extensive support and treatment for citizens with severe and life-threatening rare disorders. A report on progress to the House is expected by May 2009.

- On September 4, 2008, at the Federal-Provincial Ministers of Health Conference on the National Pharmaceutical Strategy, ministers agreed to work toward establishing a Canadian Access Program for Drugs for Rare Diseases, and to pool expertise and funding (based on 50/50 F/P/T funding formula) with centralized, transparent decision-making with public involvement.

As Alberta, Ontario and Quebec design and begin to implement their own distinct rare disease policies the need for the federal government to underpin these initiatives becomes increasingly apparent. BIOTECanada members are concerned that disparate provincial initiatives could stifle the creation of an enabling national framework. The federal government has an opportunity to provide an overall framework to encourage the development and introduction of treatments for rare disorders for all Canadians.

Canada's biotechnology industry has supported the call for a national Orphan Product Policy since 2004. With the proper enabling framework, Canadian research can play a role in contributing to the health of those with rare disorders. Moreover, Canadians believe patients suffering from rare disorders must have access to the most modern therapies available. A 2007 Pollara poll found almost all Canadians (95%) believe that Federal and Provincial governments should ensure Canadians have access to treatments for rare diseases and illnesses.

CALL FOR ACTION: Role for Health Canada

It remains BIOTECanada's view that the Government of Canada must look to mechanisms to stimulate the development, introduction, and adoption of rare disease therapies. Health Canada can assume the lead role in establishing a legislative and regulatory framework. The following are specific actions for Health Canada.

1. Establish a Definition

BIOTECanada recommends that the Government of Canada take a simple first step and establish a definition for rare disorders. Enshrining a definition in legislation not only acknowledges rarity but also provides the necessary foundation on which an Orphan Product Policy can be built. It is essential this definition be aligned with the United States and Europe. BIOTECanada recommends that an orphan disease or rare disorder be defined in the *Food and Drugs Act* as "a life-threatening or chronically debilitating condition with a prevalence, not an incidence, of fewer than **1 in 2,000** people in Canada."

2. Grant Orphan Product Designations

Companies considering developing drugs for rare disorders must overcome challenges associated with: small patient populations (which make it difficult to recruit sufficient patients into clinical trials and difficult to generate competitive returns on investment); high costs of bringing a novel product to market; financial responsibility to shareholders; and in some cases where existing medicines are being investigated for use in orphan conditions, a lack of patent protection. Granting a therapeutic product an "orphan" designation has proven to help simulate investment into the research and development of therapies that would otherwise be unprofitable. The creation of Canadian legislation that designates "orphan status" will bring Canada in line with other developed nations and significantly increase the availability of innovative therapies to treat rare disorders.

3. Create Market Incentives

Since the enactment of the Orphan Drug Act in the United States in 1983, 34 Canadian-based companies have received orphan designations for products they have developed or are currently developing. Unfortunately many of these companies are no longer in Canada as a result of the lack of market incentives or support mechanisms. BIOTECanada is dedicated to helping these companies find ways to maintain business operations in Canada and bring their innovative therapies to Canadians and the global community. With an enabling environment and competitive market incentives, Canadian companies developing orphan products could research, develop and market their therapies in Canada.

Market incentives and support mechanisms for the biotechnology industry differ among jurisdictions. However, in order for companies to invest in the development and marketing of treatments for rare disorders governments have to establish effective stimuli. Globally, Orphan Product Programs have proven to be an effective stimulus for drug development. In the decade prior to the US legislation, only 10 products came to market for rare diseases. Since 1983, over 300 products have been made available to patients in the United States.

BIOTECanada recommends that Health Canada consider the following incentives:

- **Expedited Review of Marketing Application** aligned with the tenants of the Progressive Licensing Project. Ensure further harmonization with other regulatory bodies (FDA/EMA) to allow for similar documentation and requirements for filings of products that are granted orphan status. This would minimize potential “made in Canada” approaches that may impede filings in Canada.
- **Clinical Protocol Assistance** through all phases of clinical R&D (i.e. assistance in designing and implementing clinical trials). Encouraging Canadian-based research has benefits for both patients and companies as this work generates discovery of novel therapies, supported by robust clinical evidence, for previously over-looked or under-investigated conditions.
- **Regulatory Fee Waivers** including registration fees and dossier maintenance costs.
- **Market Exclusivity/Data Protection** for an approved orphan indication for a period of 10 years, with an additional 2 years of market exclusivity if a pediatric indication is successfully pursued. During this period another company may not put a similar treatment for the same indication on the market unless the follow-on product can demonstrate superior efficacy or safety to the first entrant product in a comparative head-to-head trial. Market exclusivity is one of the most attractive incentives for industry, particularly for small to medium-sized biotechnology companies that need guaranteed revenue streams to become attractive to investors.
- **Development Grants** to stimulate interest in scientific research and development; to support clinical research by funding disease registries, clinician or patient networks, and peer-reviewed research projects.

- **Work with Provincial and Territorial counterparts** and other interested parties to develop a HTA assessment process that is rare disease specific as opposed to the current CDR process that was developed for more common disease states.

4. Establish a Canadian Office of Orphan Products

These types of market incentives provide an enabling environment and encourage the discovery and development of treatments for rare disorders. In order to administer and promote the development of these products BIOTECanada encourages Health Canada to establish a Canadian Office of Orphan Products. This office would work with medical and research communities, industry, academia, and rare disease groups. Similar to offices in other jurisdictions this body would also administer the major provisions of the national Orphan Product Policy including product designations and market incentives for sponsors.

From the view of BIOTECanada member companies the Progressive Licensing Project provides an ideal framework for the establishment of a national Orphan Product Policy. It would allow patients to gain earlier access to treatments while tracking pre-determined clinical evaluation points. However, due to the small patient populations the lifecycle of an orphan product must be monitored and assessed on a global scale. Collaboration with the international community is vital to building a comprehensive registry to inform, support, enhance, and sustain the availability of orphan medicinal products.

BIOTECanada firmly believes that all Canadians have the right to equitable treatment options regardless of the nature or frequency of their disease or condition. It is also the view of our members that Health Canada has a lead role to play in creating the legislative and regulatory framework for a national Orphan Product Policy. Established Orphan Product Policies around the world have undeniably increased the availability of life-saving treatments for patients with rare disorders, while stimulating innovation and fostering the growth of the biotechnology sector. Canada has benefited from the collective impact of the policies in other countries for long enough. The time has come for Government of Canada to support and incentivize the development and authorization of treatments for rare disorders in Canada through a comprehensive Orphan Product Policy.

Sincerely,



Peter A. Brenders
President & CEO

cc: Morris Rosenberg, Deputy Minister, Health Canada
Meena Ballantyne, Assistant Deputy Minister, Health Canada
Karen Dodds, Assistant Deputy Minister, Health Canada