

RARE DISEASE TREATMENT IN CANADA: NEED FOR A NATIONAL ORPHAN PRODUCT POLICY

THE ISSUE

Approximately 1 in 12 Canadians suffers from a condition classified as a rare disease. Canada needs a national Orphan Product Policy to set a standard for equity for Canadians with rare and life-threatening conditions. Legislative changes must be introduced to define a rare disease, and provide the conditions that give biotechnology companies the tools to continue to bring their research to market as new life-saving therapies and treatments.

RECOMMENDATION

Canada must pass legislation to establish the groundwork for a national Orphan Product Policy that will define rare diseases and put in place an assistive process for biotechnology firms doing R and D in this area. This will help bring new treatments to market. One avenue for this legislation could be an update of the *Food and Drugs Act*, introduced in 1953, as part of its modernization.

BACKGROUND

- Canadian companies are doing research into rare disease: over 35 Canadian companies have received U.S. Food and Drug Administration (FDA) Orphan Product designations for the products they are developing (see product list on reverse).
- The United States established an orphan product policy over 25 years ago, and both The European Union (EU) and United States (US) have modernized their regulatory systems in the last decade.
- In April 2008 Canada's federal government introduced Bill C-51, "*An Act to amend the Food and Drugs Act*" which died on the order paper. The government had committed to reintroduce this legislation in the last parliamentary session. A natural place to establish an Orphan Product Policy would within the *Food and Drugs Act*.
- BIOTECanada recommends that the framework for a national orphan product policy include:
 - an internationally recognized definition of orphan diseases (fewer than **1 in 2,000** people in Canada),
 - Specific market tools to ensure the predominantly small biotech firms in this space can continue research efforts (i.e. market exclusivity, protocol assistance, fee waivers for companies developing orphan products).
- Canadian citizens and Parliamentarians alike agree that Canada needs a national Orphan Product Policy:
 - A 2007 Pollara poll found 95% of Canadians support government funding for access to treatments for rare diseases and illnesses.
 - In February 2008, the House of Commons Standing Committee on Health recommended that the Canadian Agency for Drugs and Technologies in Health establish a specifically designed approach for the review of drugs for rare disorders and for first-in-class drugs.
 - In May 2008, the House of Commons unanimously passed Private Members Motion M426 on Rare Diseases, calling on the government to bring Canada in line with other developed countries who offer extensive support and treatment for citizens with severe and life-threatening rare disorders.
 - In September 2008, federal, provincial and territorial ministers agreed to work toward establishing a Canadian Access Program for Drugs for Rare Diseases. Alberta, Ontario and Quebec are now developing provincial Orphan Product policies, emphasizing the need for a national policy that achieves equity across the country.



Examples of Canadian Companies with FDA Orphan Designations

Company/Sponsor	Orphan Designation	Location
Allon Therapeutics	Progressive supranuclear palsy	Vancouver, BC
Biovail	Huntington's	Mississauga, ON
Thallion Pharmaceuticals	Malignant glioma	Montreal, QC
MedGenesis Therapeutix	Glioma	Victoria, BC
Kiadis Pharma Canada	Large B-cell lymphoma	Montreal, QC
Enobia Pharma	Hypophosphatasia	Montreal, QC
ApoPharma	Friedreich's ataxia.	Toronto, ON
Cangene	Toxemia associated with inhalational anthrax	Winnipeg, MB
Supratek Pharma	Gastric Cancer	Dorval, QC
MethylGene	Acute myeloid leukemia	Montreal, QC
DraxImage	Neuroendocrine tumors	Kirkland, QC
Trillium Therapeutics	Necrotizing enterocolitis (NEC) in preterm infants	Toronto, ON
Caprion Proteomics	Shiga-toxin producing bacterial infection	Montreal, QC
Dialysis Solutions	Renal replacement therapy	Whitby, ON
Chemokine Therapeutics	Osteogenic sarcoma	Vancouver, BC
Lorus Therapeutics	Acute myeloid leukemia	Toronto, ON
Viventia Biotech	Squamous cell carcinoma of the head and neck	Mississauga, ON
YM Biosciences	Glioma	Mississauga, ON
Debiovision	Gastric tumors	Montreal, QC
Paladin Labs	Adrenal insufficiency	Saint-Laurent, QC
BCY LifeSciences	Cystic fibrosis	Toronto, ON
AEterna Zentaris	Renal cell carcinoma	Quebec, QC
NPS Allelix	Short bowel syndrome	Toronto, ON
Protox Therapeutics	Astrocytic glioma	Vancouver, BC
Neurochem/Bellus Health	Secondary amyloidosis	Laval, QC

