



**Forum 2:
Improving Canada's Productivity to Meet Unmet
Needs for Those with Rare Disorders**

Summary Proceedings

**Tuesday February 6, 2007
7:30 am – 9:30 am
Renaissance Vancouver Hotel Harbourside
Vancouver, British Columbia**

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Welcome and Objectives of Forum

Durhane Wong-Rieger, President of the Canadian Organization for Rare Disorders welcomed participants to the second forum on orphan products policy and outlined the meeting objectives. She thanked industry and association members for attending and stated their presence "speaks to the level of commitment on this issue." Durhane encouraged everyone present to take part in the third and final CORD forum in Montreal on March 20th leading up to the First Canadian Conference on Rare Disorders in Ottawa on April 24-25th. She explained that the goal of the third forum is to develop recommendations that would promote research and development on orphan products, that is, "state of the art" therapies and diagnostic techniques, for rare disorders, as well as promote access based on international best practices within an environment that promotes safe, effective, and monitored use.

Durhane commented that Health Canada's long-held position has been that patients in this country have adequate access to therapies for rare disorders. However, over the past two years this position appears to have begun to change. Health Canada has indicated a willingness to discuss an Orphan Drug Policy and under the National Pharmaceutical Strategy (NPS) the government has identified Expensive Drugs for Rare Disorders (EDRD) as one of the top priorities, indicating that rare disorders require special attention. In terms of access, there has been some recognition by CEDAC (Canadian Expert Drug Advisory Committee) and CDR (Common Drug Review) that their health technology assessment may not adequately apply to drugs for rare disorders.

With the introduction of orphan drug legislation in the USA and Europe there has been a proliferation of orphan therapies. Without similar enabling policies in Canada, we risk falling farther behind in attracting companies developing innovative products and wishing to make these available to patients. The focus of this forum is to outline the reasons why a specific orphan products policy is necessary and the recommendations for the key elements of such a policy. There is a need to refresh and revise the BIOTEC Canada position paper on orphan products. Durhane asked all attendees to submit their recommendations for revision, with the goal of solidifying an industry position before the March 20th meeting.

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Industry/Association Introductions and Roundtable Discussion

Participants introduced themselves, their respective companies/organizations and highlighted their experiences to date working with orphan products/rare disorders. Many expressed concerns related to patient access and commercialization within Canada. It became obvious that Canada is becoming an increasingly tier-two market for patients with rare disorders; those who have access via private insurance and those that do not due to the reluctance of public payers to cover the costs of these therapies. Companies working within the orphan products space are not looking at Canada as a potential market in which to invest. Many companies develop products for the US market and do not look to introduce products in Canada due to its smaller market size and restrictive regulatory regime. This approach to 'bypassing' Canada when listing products for small population indications was shared by others. The majority of the companies in attendance, including those with R&D facilities in Canada, have products on markets in other jurisdictions or have products under review by other regulatory/reimbursement agencies.

The participants discussed potential mechanisms that could be adopted to provide incentives to companies to bring their orphan products and R&D to Canada. All suggestions centred on creating an orphan product policy that is aligned with the international community. Sean promoted this idea stating, "above all it is best if our policies are consistent with the US and EU." Leanna suggested that R&D incentives and tax credits form the basis for any orphan regulatory policy in Canada. She also highlighted the importance of including diagnostics and newborn screening in any policy formulation. Aimee highlighted some of the economic concerns stating that representatives from the insurance industry need to be engaged in future meetings. "We need to understand how insurance companies view the issue of rarity and what mechanisms they employ in the US to assure patient access in Canada."

Others noted that it will be equally important to engage the various levels of government when looking at economic factors. Steve commented that although we are looking to begin a dialogue with the federal government we also need to engage with the provinces, as they act as public payers. However, it was agreed that the federal government will need to lead the provinces with any orphan initiative.

Following discussion surrounding access and economics of orphan policy, conversation shifted towards the importance of building a constructive dialogue with patient groups. Nancy explained that on the international scene there is far more patient activity and greater transparency. Durhane responded that without a new framework it does not matter how many patients groups there are involved.

Towards the end of the roundtable, there was consensus that Canada has an opportunity to design and implement the best orphan product policy in the world. As Mike indicated, we have taken longer than most other countries to develop a framework but we also have the ability to use examples of what works well in other jurisdictions. This idea resonated with participants and spurred discussion around actions to positively influence the orphan policy process to create a cutting edge/leading regime in Canada. Peter proposed that a Canadian orphan product policy needed to be better than the

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global status quo if we want be strongly competitive in this area. He strongly supported the idea that Canada has a real opportunity to lead the world in orphan product policy, in both development of the technology and access/adoption of the associated innovations.

Peter stated that 27 Canadian companies (35 including international companies) are currently working on orphan indications. It is unacceptable that Canadian companies with innovative products, many of which are derived from Canadian universities, are unable to commercialize these products in Canada. This holds true for products that target small or rare populations as well as larger ones. The reality is that R&D is not free, it is more than philanthropy.

BIOTECanada Orphan Products Policy White Paper

Peter highlighted some of the key issues outlined in BIOTECanada's white paper and asked attendees to comment on the core components of an orphan products policy. Peter questioned whether or not the cornerstones of the white paper are still relevant and invited people to offer suggestions. He promoted the idea that there is an opportunity for Canada to champion mutual recognition with the FDA, EMEA, etc. The US already accepts EU clinical data so why would Canada not follow in the same vein? The idea of a single submission was well received by all, one that has the mutual recognition of the US, EU and Canada. Peter also suggested the possibility of adopting a one-world filing or one-world review system. Durhane seconded this suggestion, adding that global harmonization should extend beyond one-word filing and should include one-world regulations, tax credits, grants, etc.

It was agreed that the cornerstones of BIOTECanada's white paper should remain market exclusivity, fee waivers, grants, tax credits, protocol assistance, and expedited reviews. Mike strongly endorsed tax incentives as a keystone to any policy initiative as these allow companies to remain in Canada. Sean stated that grants can really help get products "off the ground" but cautioned against funding grants through agencies, especially those with anti-industry biases and suggested that we break out of the traditional channels of funding. Leanna highlighted an additional item for consideration, namely, support for New Born Screening (NBS) or high-risk population screening. In most cases treatment proves most effective/successful when initiated as early as possible. In addition, Leanna suggested the inclusion of expedited access to orphan products immediately following the approval of a product submission.

There was dialogue surrounding the issue of market exclusivity. While some individuals argued it was not necessary, many thought it should remain a key point of any policy put forward. Leanna questioned why Canada would want to be different from the rest of the world in this regard. Market exclusivity is an important factor in Canadian orphan product policy insofar as it provides a cornerstone of all other policies around the world. Shauna echoed this statement saying she feels it sends the wrong message not to have market exclusivity as part of the orphan product strategy and championed the importance of using the same language. Sean also highlighted that some of the things the FDA requires reflects the American value system – we have a different value system

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in Canada. Canada must augment its international visibility if we truly want to participate in this area.

The question of potential abuse of orphan designations was also addressed. If a product is granted orphan status but appropriated to a much larger/broader population base than originally intended, does orphan product status still apply? The recommendation of the attendees was to look at medical plausibility (that the patient population did indeed represent a "rare" condition) and to monitor all products on an orphan indication list/database. It was agreed that an orphan designation could be revoked if appropriate (market far exceeds original estimate), as is the case in the European legislation.

At the end of the discussion, participants agreed that the following five elements are needed to form the basis of orphan product policy in Canada:

- Grants
- Tax Credits
- Market Exclusivity
- Protocol Fee Waivers
- Early Access

BIOTECanada will incorporate recommendations to its existing white paper over the next month in an effort to devise an industry-wide standpoint and provide the foundation for a private members bill. Changes to the paper will be circulated to CORD and BIOTECanada member companies for review on a regular basis. It was agreed that the revised white paper should be formed into draft legislation prior to the April 24th.

HTA Meeting – Le Saint Sulpice Hôtel Montréal, Quebec

Leanna discussed the upcoming orphan product HTA and economic evaluation meeting in Montreal set for March 19th. This is a closed meeting that will focus on the economic challenges posed by orphan drugs, specifically issues related to evaluation, funding and access. The panel theme will discuss ways the HTA community can support and inform payers and policy makers who look to use HTA in determining access to orphan products. Panel objectives include bringing information on issues in HTA of orphan products before the HTA community and stimulating discussion and research on methods to assess orphan products that address these issues. The meeting will be co-chaired by Mike Drummond and Stuart MacLeod.

The CORD/BIOTECanada/CGTD meeting will follow on March 20th and bring forth the outcomes and recommendations from the March 19th meeting. All are encouraged to attend and to participate in developing recommendations toward access for orphan products. Representatives from Health Canada and the NPS/EDRD provincial working group will be in attendance. Hopefully, Industry Canada will also take part.

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Conclusions and Recommendations

Durhane concluded the meeting by expressing appreciation for participating and urging all attendees to review the BIOTECanada white paper on orphan product policy and send all suggestions and recommendations to graeme.fraser@biotech.ca as soon as possible.

Participant List

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