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## Examining Why the Canadian Federal Government Placed an Orphan Drug Strategy on Their Decision Agenda Now

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## Abstract

The Ministry of Health's announcement of a National Orphan Drug Framework on 3 October 2012 was the first federal public acknowledgement of orphan drugs since the 1997 Drugs Directorate (DD) policy statement. The reform primarily announced an Orphan Drug Policy for Canada. This paper explains why the government decided to make this announcement now. Media and Parliamentary documents were analyzed for their use of symbols, numbers, and language in causal stories told by political actors. The initial story was that Canada's population was too small and the cost too high for an orphan drug policy. Over the next fifteen years, a powerful interest group, the Canadian Organization for Rare Disorders (CORD), mobilized the rare disease community into a cooperative effort that generated collective action. They redefined the DD story from one of natural causes, to inadvertence, and finally to intentional causation. Their story invoked a federal response because it blamed the government directly for not acting on behalf of the 3 million Canadians with rare diseases, when patients in other countries were receiving better care.

*En annonçant le 3 octobre 2012 un Cadre National pour les Médicaments Orphelins, le Ministère de la Santé a mentionné pour la première fois publiquement les médicaments orphelins depuis l'énoncé de politique sur la Direction des Médicaments de 1997. La réforme a principalement consisté en l'annonce d'une Politique des Médicaments Orphelins pour le Canada. Cet article explique pourquoi le gouvernement a décidé de faire cette annonce aujourd'hui en analysant les symboles, chiffres et discours utilisés dans les représentations causales des acteurs politiques. La représentation causale de départ disait que la population du Canada était trop petite pour pouvoir s'offrir une politique de médicaments orphelins (cause naturelle). Au cours des quinze années suivantes, l'Organisation Canadienne pour les Maladies Rares (CORD en anglais, l'organisation n'existe apparemment pas en français), qui est un puissant groupe d'intérêts, a su fédérer la communauté d'individus intéressés par les maladies rares en un effort commun qui a débouché sur une action collective organisée. Cette action a transformé la représentation causale de la constitution de la Direction des Médicaments, l'histoire d'origine en termes de causes naturelles devenant une histoire d'inadvertence, puis d'intentionnalité. Leur récit a suscité une réponse fédérale car il mettait en cause de manière directe le gouvernement pour n'avoir pas su agir en faveur des trois millions de Canadiens souffrant d'une maladie rare alors que leurs équivalents à l'étranger recevaient des soins mieux adaptés.*

### Key Messages

- Use of causal stories is a battle between political actors over causation, blame and responsibility which can be used to explain why the government put an orphan drug strategy on the agenda.
- The powerful interest group CORD mobilized rare disease sufferers in order to promote the issue, and later made recommendations on which to base the new Orphan Drug Policy.

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- *Les acteurs politiques ont combattu au moyen des représentations causales pour attribuer des causes, des fautes et des responsabilités et ceci peut expliquer pourquoi le gouvernement a placé une stratégie pour les médicaments orphelins sur l'agenda politique.*
  - *Le puissant groupe d'intérêt CORD a mobilisé les patients atteints de maladies rares pour promouvoir la question et, par la suite, a fait des recommandations sur lesquelles est fondée la nouvelle politique des médicaments orphelins.*

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## 1 BRIEF DESCRIPTION OF THE HEALTH POLICY REFORM

On 3 October 2012, the Federal Ministry of Health announced two initiatives aimed at improving patient access to, and information about, treatments for rare diseases. One was simply a statement that Canada has joined Orphanet, an online information site about rare diseases and their treatments, designed for patients and health care providers. The second was a promise to develop a national framework to address the designation, authorization and monitoring of treatments for rare diseases, also known as orphan drugs. These initiatives came from the first federal announcement concerning orphan drugs since the 1997 Drugs Directorate (DD) decision that stated Canada had no need to develop any policies specifically for orphan drugs.

## 2 HISTORY AND CONTEXT

The primary predicament concerning orphan drugs is cost, which has a two-fold effect. They cost the patient or public/private insurance upwards of \$300,000 a year. That this is a prohibitive out-of-pocket cost for the patient, and that orphan drugs fail to be deemed cost-effective by many jurisdictions or private insurance schemes, results in no public insurance coverage and no patient access. Furthermore, many orphan drugs do not make it to this stage, as manufacturers delay or ignore filing in Canada because of the absence of research or market incentives, or because the research sample size is too small (due to the rarity of the diseases), for Health Canada to issue a Notice of Compliance for meeting safety and effectiveness standards. Unfortunately none of these primary concerns are addressed with either policy announcement by Health Canada.

There have been several recommendations during the past fifteen years to encourage the federal government to address orphan drugs; however none have resulted in federal policy action. Roy Romanow (2002) recommended that all levels of government contribute to create a catastrophic drug plan that would include orphan drugs. At around the same time Senator Kirby (Kirby and Breton 2002) proposed a similar program that would put caps on out-of-pocket expenses, deductibles on private plans, and annual caps on drug expenditure. The First Ministers reaffirmed in 2004 that no Canadian should suffer financial hardship due to the cost of a needed drug and that access to a drug should not depend on the province in which one resides. They established goals for developing a National Pharmaceutical Strategy that entailed catastrophic pharmaceutical coverage and accelerating access to breakthrough drugs (including orphan drugs) (National Pharmaceutical Strategy 2007). A ministerial task force was appointed in 2006 to investigate the best approach to health care renewal, including the development of catastrophic drug coverage, the establishment of a national drug formulary, and the facilitation of access to breakthrough drugs. Most recently, in 2008, the task force created decision points that would be a focus for progress.

These decision points included a Canadian Access Program for orphan drugs. However, there has been no significant progress with the federal government since the report in 2006 (MacKinnon and Ip 2009).

### **3 GOALS OF THE REFORM**

#### **3.1 Stated**

1. Provide information about rare diseases and their treatments to patients and health care providers using Orphanet (<http://www.orpha.net>)
2. Develop a national framework to address the designation, authorization and monitoring of orphan drugs

#### **3.2 Implicit**

1. To relieve interest group (Canadian Organization for Rare Disorders—CORD) pressure on parliament
2. To avoid blame for being the only developed country without an Orphan Drug Strategy

### **4 FACTORS THAT INFLUENCED THE HOW AND WHY OF THE HEALTH CANADA REFORM ANNOUNCEMENT**

#### **4.1 Framework for analysis**

Stone's (1989; 2012) theory of causal stories and agenda setting provides a framework for understanding how advocates' efforts to reframe policy problems associated with orphan drugs influenced the federal government's decision to place an ambiguous orphan drug policy announcement onto its political agenda. Essentially, a condition becomes a problem for government only when it is defined in a manner that makes it perceived as solvable with policy intervention. Problems themselves do not demand political action; instead, through strategic use of language by influential actors, a problem is interpreted as requiring a response (Stone 1989; 2012). Public issues can be a consequence of natural, inadvertent conditions or mechanical, intentional action by actors in control. Citing the natural or inadvertent argument is often a tactic to avoid blame and claim lack of control. Reframing an issue into the realm of intentional causation is a critical determinant for the issue's placement on the agenda. Problem definition is crucial to positioning issues onto the agenda. To frame issues in this way is a 'major political accomplishment' (Kingdon 2011, 121). This paper draws on several prominent Canadian media and parliamentary reports that best illustrate the development of a causal story, for the 1997 DD decision to the recent

announcement, which placed intentional blame on the federal government for not providing solutions to the public issue of orphan drug access for rare disease sufferers. In doing so, the paper emphasizes the important role of an interest group, CORD, in changing the dominant storyline.<sup>1</sup>

## 5 HOW THE REFORM WAS ACHIEVED

No orphan drug policy has been implemented as of this writing; subsequently no reform has yet been achieved. Instead, the present analysis focuses on the change of the dominant causal story regarding orphan drug policy. This placed blame and responsibility on the federal government, which pressured it into reversing their 16-year-old policy.

### 5.1 Natural causation as a defence of the federal position: 1990-1997

The issue of focus previous to the DD decision was neither orphan drugs nor diseases, but rather the drug evaluation processes of Health Canada. At the time, Canada was regarded as having one of the toughest pharmaceutical evaluation processes in the world, but there was worry that its reliance on research findings from other countries was flawed. Political actors and interests groups were directly blaming the government for fast tracking drugs and risking the safety of its citizens. The *Montreal Gazette* described the situation as a ‘slippery slope’ (Raegesh 1992), meaning that fast tracking would lead to more unsafe outcomes. Health Canada acknowledged the problem, but avoided responsibility by defending the process as ‘naturally caused’ due to Canada’s inability to conduct adequate drug evaluation research because of its small population.

The DD’s primary defence against acknowledging orphan drugs was similar to the defence of the drug review process. They avoided blame, and thus responsibility, by attributing the absence of an orphan drug policy to the small population of Canada. For example, the document states, “These countries (Japan and US) all have a significantly larger population base than Canada. The Canadian population may not be large enough to support significant R&D in this area” (Health Canada 1997, 19). Their argument is interpreted as saying: the cost of an orphan drug policy is too high, because the population of those who would benefit is too small. This is the application of a concentrated cost versus diffuse benefits argument. Furthermore, the document used numbers to suggest that Canada already has enough policies in place; it states, “[a] number of programs are currently in place to ensure that Canadians have access to medicines which are not approved for use including Orphan Drugs” (Health Canada 1997, 19).

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<sup>1</sup>While CORD has played a significant role in areas beyond media reports, the subject of analysis for this paper is the causal stories used.

## 5.2 Mobilization of an interest group and redefining of the problem: 1998-2002

It was not until 2000, when CORD, a group founded in 1996 and representing Canadians with rare diseases, began receiving media attention for its lobbying action for better orphan drug access. By 2000, CORD had 150 different rare disease associations as members, collectively representing a large group of vulnerable people (*Globe and Mail* 2000). This was the beginning of an effective mobilization of the interests of rare disease sufferers. Mobilization is critical, especially in fighting against a social injustice that is having an observable adverse effect on a group. This mobilization was articulated in a statement that continued to be reiterated in a large number of articles throughout the 2000s and leading up to the 2012 announcement; “There are about 6,000 rare disorders. Each individual disorder may only have an impact on a small number of people but, taken together, they affect about three million Canadians” (*Globe and Mail* 2000). This is a powerful statement using numbers to collectively group rare disease sufferers such that together they represent approximately one in ten Canadians.

CORD’s momentum carried through the mid 2000s as the problem was redefined from a diagnosis issue into a treatment access problem, and blame was placed on multiple players. The federal government, in the body of Health Canada, the provincial governments, and the pharmaceutical industry all received criticism for the lack of access patients had to orphan drugs due to their catastrophic prices. Many headlines attributing blame were published such as, “Rare diseases? troubling questions: With no incentives from *government*, simple economics dictate a focus by the profit-driven *pharmaceutical industry* on blockbuster drugs” (Duffy 2002) (emphasis added). However, the different bodies were able to avoid direct blame because of the complex nature of the Canadian health care system. An example of this complex causation is, “a hodgepodge of provincial drug insurance coverage that vexes many orphan disease sufferers” (Duffy 2002). Complex causation makes it difficult to promote policy change.

## 5.3 Blame Canada: 2003-2006

In politics, the distinction between intentional and unintentional events is crucial. If an event was caused purposively, there is someone to blame and the reaction will be much different from the reaction to an accidental event for which no one can be blamed. During the mid to late 2000s advocates for orphan drugs began comparing Canada to other developed countries, and the federal government’s story of natural causes transformed into one of inadvertence. This transformation is exemplified by headlines such as “U.S. incentives assist ‘orphan drug’ development: With no similar help, *Canadian* firms don’t take *same road*” (Elias 2003), and “‘We’re looking for *a lifeboat*’; Costly drug required by child is covered in Britain—but not in *Canada*” (Priest 2006). Such headlines began the process of political actors and interests intentionally blaming Canada for the public issue. During this time,

the story from the federal government continued to be one of blame-avoidance. However, it switched its position from a natural causation position to a mechanistic one by using facts and the authority of science as support for their current evaluation processes. For example, the federal minister of health was quoted as saying, “the treatment (for orphan diseases) must be assessed by a common drug review before the government decides if it’s cost-effective to supply.”

#### **5.4 Attributing responsibility and offering solutions through political access: 2007-2012**

The final stage to the causal story is the presentation of solutions by policy activists for action to those who have been assigned responsibility, the federal government (Stone 1989). The likelihood of solutions being interpreted as legitimate and feasible increases if they are coming from actors with political alliances and policy access, which is what CORD was able to achieve. With CORD, a high level of collective benefits was symbolized in a dense network of people (rare disease patients and supporters) lobbying for a collective good, which engendered a sense of civic engagement and equity amongst these actors. CORD has presented arguments supporting a Canadian orphan drug strategy every year since 2007, where they continued to attribute cause to the federal government and blame or this injustice (Canada 2007, 2008, 2009, 2010, 2011, 2012). For example, the president of CORD stated, in parliament, “Canada is not fit to deal with rare disorders; Nearly three million Canadians suffer from an ‘orphan disease’(Canada 2008), among whom many are infants and children. Blame is placed on the federal government by political activists such as CORD, as well as the media and individual members of parliament for ignoring the problem, while solutions, such as CORD’s ‘Choose Life’ program, is presented directly to policymakers (Canada 2010). Their demands were heard in parliament and began being supported by members, such as Don Bell, in 2008. This success in having advocacy heard in parliament is further demonstrated by the Senate Committee recognizing CORD as a contributor to their 2012 recommendations for an orphan drug policy (Standing Senate Committee on Social Affairs, Science and Technology 2012). Additionally CORD is acknowledged as a consultant in Health Canada’s new release of the 2012 Orphan Drug Plan announcement (Health Canada 2012).

#### **5.5 Policy instruments**

This analysis has demonstrated that through the strategic use of facts, numbers and symbols, the need to address the orphan drug problem in Canada became a dominant belief and the development of an orphan drug policy was the eventual solution. This was primarily accomplished through the collective action of CORD by reiterating their message through extensive media coverage, whereupon they eventually gained policy access and offered possible solutions.



## 6 EVALUATION

As of January 2014, over a year later, no further federal action has been taken. This raises the concern of whether the announcement will address the access and affordability needs of patients, or resemble the market incentive approach that other developed countries have adopted (potentially both). Due to this uncertainty, it is premature to discuss the effects that establishment of an orphan drug policy may have for patients.

## 7 STRENGTHS, WEAKNESSES, OPPORTUNITIES AND THREATS

Table 1: SWOT Analysis of the Reform

STRENGTHS	WEAKNESSES
<ul style="list-style-type: none"> <li>• Canada has joined the rest of the developed world in announcing Orphan Drug Policy.</li> <li>• Orphanet will provide a venue for physicians and patients to access information about orphan drugs and rare diseases.</li> </ul>	<ul style="list-style-type: none"> <li>• No details about Canada's Orphan Drug Policy have been released. Therefore, it is uncertain what it will address.</li> <li>• As currently described, it is unlikely to address the affordability issues that prohibit patient access.</li> </ul>
OPPORTUNITIES	THREATS
<ul style="list-style-type: none"> <li>• The federal government acknowledgment provides an opportunity for patients to access more information on their disease and treatment.</li> <li>• The acknowledgement of orphan drugs by the federal government has placed it onto their decision agenda, which provides the opportunity for further policy action.</li> </ul>	<ul style="list-style-type: none"> <li>• A criticism of Orphan Drug Policy is that it incentivizes industry rather than promotes access.</li> </ul>

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