Patient Leadership Toward a Canadian Plan for Rare Diseases

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What are the origins of the Orphan Drug Policy in Canada?
In 1996, 13 years after the USA passed the Orphan Drug Act, Health Canada concluded that about 50-60% of rare disease drugs were available in Canada and that was "good enough." The official position was that Canada did not need an Orphan Drug Policy. Nothing much changed for another decade. In 2006, the Canadian Organization for Rare Disorders issued this challenge, "Why is the Canada the only developed country without an Orphan Drug Policy?" With only half of orphan drugs approved in Canada and even fewer funded by the public drug plans, patients declared the status quo as absolutely "not good enough." In 2007, CORD hosted the 1st Canadian Conference on Orphan Drugs and Rare Disorders, and a slew of experts from the US FDA, National Institutes of Health, and European Medicines Agency, as well as international patient advocates, researchers and clinicians all called for Canada to become a legitimate contributor to orphan drug and rare disease research and development. Since then, CORD has been relentless in its advocacy, with patients descending upon Parliament en masse once or twice a year as well as more conferences, meetings and consultations.

At the same time, provincial drug plans were routinely denying access to rare disease drugs, and patients were forced to wage battles through the media, traditional and social. In 2006, CORD led a patient demonstration at the offices of the Common Drug Review and later that year picketed the Health Ministers' meeting until they relented, promising access to two specific drugs and an F/P/T pharmaceutical plan for rare diseases. The funding promise was kept; the F/P/T plan never materialized. But a large "chink" in the governments' "stonewalling" had been rendered. In January 2010, the federal government first referenced "drugs for rare diseases regulatory and legislative modernization efforts", and by October that year, we saw some of the initial draft regulations. Now we are hearing in international meetings from Canadian officials that the Orphan Drug Regulatory Framework is the first step toward a modern "life cycle" approach and greater "harmonization" with international regulations, leading to cross-border cooperation and collaboration.

How have patients made the Orphan Drug Policy a reality?
So how have patients, with CORD, made a difference? First, brave patients and families put a personal face to the issues and CORD helped bring their messages to national attention. Second, CORD created a single consistent voice, forging a coalition of patients, healthcare providers, industry, and public in a multi-pronged, multi-stage, and multi-year battle for an Orphan Drug Policy. Third, CORD worked with "insiders" who could champion the cause. In 2007, MP Don Bell tabled a Private Member's Bill based on CORD's Orphan Drug Policy. In 2009, CORD worked

with a very sympathetic Ontario Drug Plan Manager, Helen Stevenson, to introduce the first provincial Drugs for Rare Diseases Plan. Fourth, CORD has aligned itself with the international rare disease community, mimicking strategies, borrowing best practices, and building on international initiatives.

What is the next step for rare diseases in Canada

In February 2013, CORD's theme for Rare Disease Day was "From Worse to First" and we believe Canada is on track to implementing a "state of the art" Orphan Drug Regulatory Framework that incorporates some of the best of the US and European models that will open the door for research and development and patient engagement in new drugs for rare diseases. Our next step is to press for a "drug review process" that is specific and appropriate to rare disease drugs with a pan-Canadian funding and listing program. There are elements already in place that can be built upon. Finally, at the end of September 2013, we are initiating collaboration for a Canadian Plan for Rare Diseases. Our goal is to announce a consensus strategy in February 2014 on Rare Disease Day. The plan, based on the plans of 25 European countries, will include continued research, centres of expertise and reference networks, screening and testing, comprehensive healthcare and supportive services, and empowerment of patient organizations. It is being led by the patient community but requires the collaboration of all stakeholders.