

PDSA in Canada: Supporting the Canadian Organization for Rare Disorders (CORD) and Their Fight for Life Rally on Parliament Hill

By Jennifer DiRaimo

Access to new and innovative medicines in Canada has not always been timely, equal, or consistent. This is especially true for rare disease drugs, which are more on the expensive side on a 'per patient' basis due to the small number of individuals with a specific rare disease. For most Canadians with rare disorders, access to the most appropriate therapy has often relied on having private drug coverage or the financial means to pay for treatment as opposed to relying on provincially funded drug plans.

Public drug plans compare the cost and benefits of a new therapy with the cost and benefit of the older therapies, and this comparison often works against new rare disease drugs, especially when the current therapies are older, generic, and/or just for symptom management. Furthermore, cost varies across all provinces in Canada compounding the inequities.

In 2019, the federal government released a proposal for a National Pharmacare Program aimed at reducing disparities designed to ensure drug coverage for all Canadians. This program would include a single nationally funded drug list (evidence-based and cost-effective) and a separate national Rare Disease Drug program to obtain high-cost drugs for rare diseases. This program would be available to all rare disease patients regardless of where they live or their insurance coverage.

In addition, there were two other announcements that could improve appropriate drug access for rare disease patients. The first was a proposal by the provincial/territorial "Expensive Drugs for Rare Diseases" working group for a separate "managed" process that could make rare disease drugs available while further "real world" evidence of their



PDSA Research Program Manager Jennifer DiRaimo at the CORD Rally with Durhane Wong-Rieger, CORD's President & CEO

effectiveness and cost-effectiveness were being collected. This would reduce delay and reliance on often limited clinical trial data. The second supportive measure was the federal government's announcement of \$1 billion to set up a Rare Disease Drug Strategy, for 2022-2023, with an additional \$500 million each subsequent year. Canadian rare disease patients were finally going to get appropriate access to lifesaving and life-enhancing innovative therapies. Except for the introduction of one major barrier that would affect most innovative medicines especially for rare diseases.

With virtually no open consultation, the Patented Medicines Prices Review Board (PMPRB) responsible for assuring drug prices are not excessive announced sweeping regulatory changes including legally binding maximum drug prices, which has no precedence or equivalence in any other developed country. The proposed is arbitrary and not based on scientific evidence. The legally binding net price

would be set at a level that is 40% to 90% below current list price. This price would be far below the price in all other developed countries and below the price in most developing countries. In most countries, the final drug price is negotiated by the manufacturer and drug program, whereas in these new regulations, there is no negotiation.

The changes to the PMPRB will deter many pharmaceutical companies from bringing innovative medicines to Canada because it would not be affordable for them. Already, some companies have announced they will delay bringing certain drugs into Canada, and for drugs already in Canada there are no plans for expansion to use for other indications, including in rare diseases and in children. Industry may also avoid bringing clinical trials to Canada for drugs that they will not launch or will experience a delay in launching. In 2019, Canada experienced a 40% decline in the total number of clinical trials, perhaps in anticipation of the pending changes to the PMPRB.

New medicines will be available in the future to treat immune thrombocytopenia (ITP). It is important that countries like Canada are positioned to access these treatments when they become available. The Canadian Organization for Rare Disorders (CORD) offered to work with the government to help implement different strategies with a shared common goal of helping individuals with rare diseases access affordable treatments, but to no avail.

On March 10th, CORD organized a rally as part of their annual meeting to voice the concerns of the rare disease community on Parliament Hill, in Ottawa Ontario, Canada. Over 150 people attended the conference and rally including PDSA Research Program Manager Jennifer DiRaimo, representing Canadian patients with ITP. ■