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DISCUSSION PAPER

Canada's National Strategy for Drugs for Rare Diseases should Prioritize Patients not Cost Containment

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ABSTRACT

The 2019 federal budget raised hope for Canadians living with rare diseases by proposing to invest up to $1 billion over two years starting in 2022-23, with up to $500 million per year ongoing, in a national strategy for high-cost drugs for rare diseases. This commitment was reaffirmed in the 2020 fall economic statement. In January 2021, Health Canada published a discussion paper to engage Canadians in building the strategy. The principal emphasis of the discussion paper is cost-containment for high-cost drugs, which Health Canada equates with drugs for rare diseases. This is worrisome. First because the priority should be patients not cost containment. Second, drugs for rare diseases, whether high-cost or not, must be part of a strategy for rare disorders, but they should not be the only component of it. A rare disease strategy must include improving early detection and prevention and providing timely, equitable, evidence-based and coordinated care, as well as providing sustainable access to potentially beneficial treatments. Research into understanding the occurrence, impact and outcome of rare disorders, unmet health needs, and potential and actual therapies for them should be promoted. All these essential elements were proposed by the entire rare diseases community in 2015 coordinated by the Canadian Organization for Rare Disorders. Canadians with rare disorders need their governments to implement a comprehensive national strategy for rare diseases with Ottawa serving as facilitator and funder. What Health Canada proposes is a plan for cost-containment that will disincentivize drug developers from launching innovative therapies in Canada. Breakthroughs, such as messenger RNA technologies for precision medicines for rare disorders move ahead rapidly in other countries but languish in Canada. Spending on drugs for rare diseases is sustainable. By comparison, spending is higher for new cancer drugs, but politicians and government officials are extremely unlikely to suggest that innovative cancer drugs should be withheld from patients. The federal government's cost containment policy deprioritizes the unmet needs of Canadians with rare disorders.

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DISCLOSURE

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Introduction

The 2019 federal budget raised hope for Canadians living with rare disorders by proposing to invest up to $1 billion over two years starting in 2022-23, with up to $500 million per year ongoing, in a national strategy for high-cost drugs for rare diseases (DRDs). This commitment was reaffirmed in the 2020 fall economic statement. In January 2021, Health Canada published a discussion paper to engage Canadians in building the strategy, which is open for responses until March 26.[1]

Health Canada's discussion paper raises three issues:

1. How to improve patient access to high-cost DRDs and ensure consistent access across Canada.
2. How to ensure decisions on covering high-cost DRDs are informed by the best available evidence.
3. How to ensure spending on high-cost DRDs does not put pressure on the sustainability of the health care system.

In this article, we review these issues and the options for each one proposed by Health Canada and raise questions about related concerns, including the distinction between a strategy for rare disorders and a plan focused on drug costs.

Health Canada's DRD Issues

Patient Access and Consistency

Health Canada correctly states that access to DRDs varies widely depending on where patients live in Canada and how their drugs are covered because public and private drug plans make separate, often different, decisions about what DRDs they will cover.[2] The proposal fails to acknowledge that access to new therapies also varies markedly whether you live in Canada or elsewhere.

Industry-sponsored patient support programs and clinical trials may fill some access gaps. However, they frequently provide drugs only to select patients and offer no guarantee for how long access will continue.

Options proposed by Health Canada for improving national access and consistency are:

- A single framework for decision-making on high-cost drugs intended to get federal, provincial and territorial governments to agree on a single approach for deciding which drugs to fund or not fund and which patients should be covered under what conditions.
- A transparent coordinating body to improve communication and collaboration among key players (including private and public drug plans) would create consistency in decisions on what drugs to cover, ensure agreed conditions for consistent access were followed, and clearly communicate about funding decisions, rationale for them and information on the process and timelines.
- Patient and clinician engagement should be improved to increase awareness of policies and programs related to accessing high-cost DRDs.
- Nationally coordinated support for research on rare diseases to increase knowledge of rare diseases, lead to new discoveries and bring new DRDs to Canadian patients.

Health Canada believes that a single framework for decision-making for DRDs would create greater consistency and predictability across the country. However, existing differences between provincial drug plans and the hostility provoked when the federal government interferes in provincial responsibilities make this option a non-starter. It would also sidestep the role of private insurers. If a single framework was instituted, the risk to patients with unmet needs would be that governments may agree *not* to cover more DRDs. A patchwork of access may be considered preferable to a blanket denial of access.

Transparent communication and more information on the deliberation of advisory bodies and their reasons for recommendations, including their rationale, what evidence is given what weight and process timelines, would be welcome because too much of the current system is performed behind closed doors. Key players must not be limited to private and public drug plans – they must include health care providers, patients, researchers and drug developers.

Patient and clinician engagement must be improved to increase awareness of policies and programs related to DRDs. This is a necessity in our view, not an option. Too often health technology assessment (HTA) recommendations about funding for DRDs do not appear to be based on input from health care providers working in the relevant field of practice.

More research into rare diseases is crucial. Too little is known about the occurrence, burden and treatment of rare disorders in Canada.[3] A Canadian Institutes of Health Research institute of rare disorders with networks of excellence should be created. This is only one illustration of the distinction between a strategy for rare disorders and one focused primarily on cost-containment.

Decisions Based on Best Available Evidence

Robust standards for evidence in the form of randomized clinical trials involving several thousand participants have been developed to test new drugs to ensure safety and efficacy. However, when rare diseases are studied, few patients make large trials difficult, if not impossible, to perform. A trial taking years to accumulate sufficient patients could be performed, but making participants wait for enough evidence to be gathered may be considered unethical because patients will suffer and die while evidence is accumulated.

To deal with these problems, drug regulatory agencies have adopted different approaches in their review processes resulting in DRDs being approved on limited evidence of health benefits and risks. This situation can make for difficult decisions about whether new medicines provide significant enough benefit to patients to merit public or private drug plan coverage. When no other treatment is available, patients understandably want access to a medicine that could help improve their quality and/or length of life. This may lead to pressure to pay for drugs, even when benefits are minimal or evidence limited.

Options proposed by Health Canada for addressing the challenge of considering drugs with limited evidence are:

- Innovative approval and coverage of DRDs could be tied to how well they work, i.e. pay-for-performance. Payment would depend on patients having specified outcomes, which would require developers to institute long-term studies to track treatment results and report on the effectiveness and safety of their drugs. To initiate such studies would require an agreement on indicators for measuring benefit and what level of benefit would be sufficient. DRDs with outcomes that failed to reach the agreed level would have coverage reduced or discontinued.
- A national expert panel to study data and evidence to make informed recommendations on who should be treated with high-cost DRDs and monitor how the drugs are used, how well they work and make recommendations on continued coverage.
- A national data system to capture comprehensive and consistent information about the prevalence of rare diseases**,** how DRDs are used by Canadians and their outcomes.
- Independent national and international networks to build on existing partnerships to facilitate knowledge and data sharing on real-world experience of patients in an independent manner.

The limited evidence available for decision-making about coverage of new DRDs is a fact of life. To demand large-scale clinical trials in the rare disorder arena is irrational and unreasonable. Moreover, to require objective indicators of benefit is impracticable because often none exist for rare disorders so that benefit has to be based on patient-reported quality-of-life benefits, which are inevitably subjective. In addition, the investment required for manufacturers to perform long-term studies – even if they could gain access to patient records – would likely be too great to make bringing their products to Canada worthwhile.

A national panel making coverage recommendations would be little different from the present unsatisfactory process,[4] unless it included patients and developers and was open to scrutiny and accountability.[5] Who in the public, the media or parliament knows how decisions are made about what new drugs to cover or not to cover and under what conditions?

While, in theory, a national data system is a good idea, it would be especially important to decide who would lead and resource an initiative of this kind. Unless the federal government leads and funds it, it would seem to be another non-starter.

However, it should be noted that the federal government established the Canada Health Infoway 20 years ago to accelerate the development, adoption and effective use of digital health solutions which has yet to fully achieve its goal.[6] Moreover, Canadians might remember the fiasco following the launch of the Phoenix payroll system for federal employees or the neutering of Canada's system for early warning of pandemics before handing the federal government leadership in this type of initiative.[7]

When it comes to participation in independent national and international networks to build partnerships to

facilitate knowledge and data sharing on real-world experience of patients, one can only ask why Health Canada is not already participating in such networks already? If Health Canada is doing so, it clearly has not benefited Canadians with rare disorders in accessing DRDs

Controlling the Budget Impact of DRDs

Health Canada claims that most DRDs are "very expensive," without defining what is rare or what is expensive. The federal department goes on to say that DRDs can pose a challenge to the long-term sustainability of public and private drug plans, and that DRDs are the fastest growing segment of the pharmaceutical market in Canada. Health Canada also notes that the pan-Canadian Pharmaceutical Alliance (pCPA), created by the federal, provincial and territorial governments to negotiate drug prices with manufacturers, has not managed to negotiate what it calls "fair prices" (also undefined) for DRDs, which implies that the pCPA has failed to get prices down to the level public plans want.[8]

Health Canada claims these three undefined factors (what is rare, what is expensive, and what is a fair price) threaten the overall sustainability of the health care system.

Options proposed by Health Canada for controlling the impact of high-cost drugs on health system budgets are:

- Work together with other payers to share costs and pool risks by negotiating better agreements with biopharmaceutical manufacturers.
- Governments and health charities could make early investments in drug developers to reduce the risk in early development work so that manufacturers would not have to rely solely on price to compensate for the costs of research and development and failed projects in the creation of new DRDs.
- Explore innovative funding models tied to how well a drug works, including defunding drugs that offer only marginal or unproven benefits, i.e. pay-for-performance.
- Develop a domestic innovative or generic capacity to sustain all elements of drug discovery from research and development to manufacturing, which Health Canada contends would help keep costs lower than if researchers sell their discovery to a multinational company.
- Work with other countries to share non-confidential information to inform negotiations and leverage better pricing.

These options can be summarized as working collaboratively with private payers and manufacturers and using a pay-for-performance scheme. A pay-for-performance approach is understandably attractive to payers, but it would be impracticable to demand objective indicators of benefit because they often do not exist for rare disorders. In many cases, benefit has to be based on subjective but crucial patient-reported quality-of-life outcomes.

A collaborative approach between governments and drug developers is long overdue in Canada.[9] For decades in Canada, there has been indifference or antagonism, rather than informed respect, between the federal government and developers of new drugs and vaccines.[10,11] The federal government's lack of collaboration with brand-name biopharmaceutical companies has led to less industry-sponsored research being performed in Canada and a reduction in manufacturing facilities and good paying jobs.[12]

In contrast, Ottawa has allowed generic companies to create an oligarchy. This has led to much higher generic drug prices than in other countries. Collaboration would require a major shift in federal, provincial and territorial governments' attitudes towards and thinking about the biopharmaceutical industry for which there seems to be little appetite at present.[13] Let's be optimistic and hope that the lessons learned from the COVID-19 pandemic about what it takes to bring new medicines and vaccines to Canadians will not continue to be ignored.

Health Canada's Focus is "High-Cost"

The focal point of Health Canada's discussion paper is cost-containment. The term "drugs for rare diseases" is used 60 times to which a qualifier of "high-cost" or "expensive" is added on 52 occasions (87 percent), including in the paper's title. "High-cost" and "expensive" are used a total of 60 times in the 16-page document – almost four times per page. One section is titled "why are drugs for rare diseases so expensive?" Again, we emphasize, without definitions of what is rare and what is expensive.

Health Canada focuses on manufacturers' suggested list prices without acknowledging the significant impact of price negotiations with public and private insurers.

Negotiations with public plans alone have been found to yield an average of 30% in savings. Many private payers also actively and aggressively negotiate on behalf of millions of beneficiaries of employer-sponsored benefits programs.

The discussion paper pays little attention to the health, social and economic benefits that DRDs can bring to Canadians with rare diseases. Health Canada acknowledges but is dismissive about the fact that half the people with rare diseases are children – many of whom die before their first birthday – and that sufferers have poor quality of life.

Canadians with rare disorders often require a great deal of medical care. The discussion paper ignores the costs to taxpayers and the personal wellbeing costs of inadequate or missing therapies. Examples of these include emergencies and hospitalizations avoided by cystic fibrosis and sickle cell disease patients when effective therapies are available and funded.

One reason for the focus on high-cost is that Health Canada bases its view that all DRDs are expensive on data from the Patented Medicine Prices Review Board (PMPRB), the federal agency whose role is to ensure the prices for patented medicines are not excessive. The PMPRB says expensive DRDs in Canada in 2019 "account for nearly one-tenth of drug sales," but its data and analysis are questionable at best and more likely biased because new drugs used to treat cancers, which are frequently expensive, are included in its category of expensive DRDs.[14]

In fact, the PMPRB's data show that DRDs account for only 2.5 percent of sales, whereas cancer drugs account for seven percent – almost three times the DRD percentage. Another analysis has shown that estimated annual spending on DRDs represents just 1.9 percent of total public drug expenditure, which is projected to increase to 6.5 percent in 2025.[15]

The grouping of cancer drugs with other drugs is contrary to the approach normally taken in Canada. For example, the Canadian Agency for Drugs and Technologies in Health (CADTH), which performs HTAs for all of Canada except Quebec, has one evaluation process for cancer drugs and another for other drugs. Moreover, cancer drugs are evaluated separately in most provinces by their cancer agencies for their respective formularies.

One of the reasons for this separation is that cancer has strong political and emotional dimensions in terms of the number of Canadians affected and their societal influence. Governments do not risk suggesting that they will not cover a cancer drug due to its cost. For many years, these drugs have had a higher priority for coverage than other medicines.

The PMPRB should not draw conclusions about high-cost drugs based on data aggregating cancer and rare disorder drugs. It is even worse that Health Canada should propose a plan for DRDs based on the PMPRB's analysis.

What Should a Rare Disease Strategy Look Like?

Drugs must be a component of a strategy for rare disorders, but they are not the only factor – costly or not. As the Canadian Organization for Rare Disorders (CORD) laid out in 2015, a rare disease strategy must include improving early detection and prevention and providing timely, equitable, evidence-based and coordinated care, as well as providing sustainable access to potentially beneficial treatments. In addition, research into understanding the occurrence, impact and outcome of rare disorders and therapies for them should be promoted.[16]

Screening for rare disorders is inconsistent across Canada with only three (phenylketonuria, congenital hypothyroidism and medium chain acyl-CoA dehydrogenase deficiency) being tested for in all provinces and territories in 2015. In the United States, tests are performed in all states for seven disorders (phenylketonuria, congenital hypothyroidism, galactosemia, hemoglobinopathy, congenital adrenal hyperplasia, biotinidase deficiency, and cystic fibrosis).[17] Although an attempt was made by provincial health ministries to achieve a common standard, this effort received no federal participation or support, led only to an increase in screening in some provinces and has not been sustained.[18]

Diagnosing rare disorders can take months, even an odyssey of years, with patients being referred from physician to physician in the hope that one will identify their disorder and be able to help them.[19] During this time, patients frequently receive treatments that may or may not be beneficial but can cost the health care system a lot of money. The costs of these treatments and services, which often do little more than ease symptoms, should be factored in as a saving to the health care

system when assessing the cost of a new DRD that actually treats the disease.

If the federal government wants to increase the productivity of its plans for new spending on DRDs, it should consider targeting a modest sum, say one percent, to expand and make consistent the universal screening of newborns for specific rare disorders, such as spinal muscular atrophy, so that early diagnosis and prompt clinical interventions can produce better outcomes for these patients. The idea is not new: a stitch in time saves nine.

Although some successful models of integrated and coordinated care for pediatric metabolic conditions, hemophilia and cystic fibrosis exist in Canada, there are few rare disease clinics and even fewer have comprehensive care services. Consequently, many people with rare disorders do not get access to specialists or clinics and receive little coordinated or comprehensive care, especially in smaller urban centres. Canada's wide geographical spread of patients with rare diseases and clinical experts points to the need for virtual networks of excellence.[20]

Many patients with rare diseases have needs that extend beyond health care support and access to treatments placing a profound burden on individuals and families. They frequently require psychosocial services, recreational and physical therapy, counselling, respite care and special education, which are commonly not recognized as services essential to the management of rare diseases in Canada. These services may be non-medical or fall outside of the Canada Health Act, so that no legislation compels governments to offer them. Because such specialized and accessible services are commonly lacking in much of Canada, repeated travel from home to a distant centre of highly specialized care, which may be out of province or even out of country, is often necessary.

The third component of a rare disease strategy is affordable access to DRDs that is uninhibited by burdensome and restrictive coverage rules. The work of the human genome project has led to the development of many innovative DRDs for diseases that 20 years ago were untreatable. The creation of new genetic and cell therapies is a time-consuming and costly enterprise with the potential for limited sales if the medicine is one of the few that makes it through pre-clinical testing to the regulated market. Biopharmaceutical developers are for-profit businesses, not charitable organizations, whose responsibility is to their shareholders. Consequently, DRDs are priced to provide returns that justify the pre-marketing investment, inevitable failures and marketing activities, as well as produce a profit once successfully launched.

All these dimensions – early detection and diagnosing, equitable, coordinated and evidence-based care, and sustainable access to potentially beneficial treatments – are elaborated in the Canadian Rare Disease Strategy coordinated and launched by CORD in 2015.[21] Health Canada should use this strategy to improve the lives of Canadians living with rare disorders, instead of just focusing on DRD cost-containment.

Benefits and Evaluation of DRDs

DRDs can prevent premature death. For example, a third of people with paroxysmal nocturnal hemoglobinuria, an ultra-rare hematological disorder, do not survive more than five years without treatment and about half die within 10 years.[22] Eculizumab, the only therapy for this disorder approved in Canada, has provided a life-line for those with this disease.

The benefit of many DRDs is in the quality-of-life improvements they provide. Some DRDs slow the physical deterioration caused by diseases such as Duchenne muscular dystrophy, others allow sufferers to get out of their wheelchairs, yet others mean cystic fibrosis sufferers can breathe without suffocating on thick mucus, individuals with phenylketonuria can prevent deterioration into mental retardation, and atypical hemolytic uremic syndrome (aHUS) patients can prevent their kidneys from being ravaged. DRDs can allow sufferers to be less dependent on caregivers and provide an opportunity to live a more independent lifestyle that most Canadians take for granted. In some cases, they can become economically productive.

As Michael Eygenraam, who has aHUS, says:[23]

"I have an ultra-rare blood disease that destroyed my kidneys as well as a transplanted kidney donated by my wife. I am on the waiting list for another transplant which will require taking eculizumab, the only drug approved in Canada for atypical hemolytic uremic syndrome, at the time of the transplant and beyond to keep the disease from destroying the new kidney.

Having the possibility of a transplant is what gives me hope for a healthier future, which will not only benefit me but my whole family. A transplant may allow me to return

to work. I hope to have more energy, strength and endurance and to be more active, pursuing sporting activities that I love and have missed for so long. My children were only two and five years old when I first became sick, so they don't remember me being healthy. We've been restricted from travelling as a family because I'm tied to a dialysis machine five nights a week with its risks and painful needles. A successful transplant maintained by access to eculizumab would mean we finally could have a family vacation and visit family overseas after 18 years of hardship."

Patients with incurable diseases are commonly thought to be prepared to take greater risks with new medicines than the average person would. However, research has shown that, although the ability to conduct everyday activities and the expected benefit the drug may bring are especially important to individuals with rare disorders, they do not ignore the risk of serious side effects.[24]

Quality-of-life benefits from DRDs are subjective because some sufferers will view them through a glass half full, while others will see them through a half empty glass. Few objective markers exist for the benefits of DRDs, but Canada's HTA agencies, which estimate the cost-to-benefit relationship of new medicines, commonly require such markers from clinical trials for their evaluations.

For DRDs, this is not only irrational but also results in people with rare disorders being penalized if the requirement for objective outcomes results in DRDs not being recommended for coverage. Quality-of-life benefits must not be relegated to a secondary role behind outcomes like biochemical markers or survival. Quality-of-life improvements must be the main indicators of patient benefit in HTAs and formulary decision-making.

Unlike many countries, Canada's HTA agencies do not have a dedicated process for evaluating DRDs, despite numerous calls for one to be implemented. Other countries have processes that allow the use of different standards to assess the value of DRDs, overcome the lack of cost-effectiveness or allow exemptions of parts of the HTA process.[25] Some countries do not even require HTAs for DRDs that will have a relatively low budget impact or that have an obvious clinical benefit.

Ontario has developed a framework for evaluating DRDs for coverage, which includes a detailed understanding of the disease and the potential value of the DRD, reviewing the assessment with disease experts and the public and, importantly, re-assessment as new information becomes available about the disease and the DRD's benefits.[26] Denial of coverage for a DRD or any other drug should not be an irreversible decision anywhere in Canada. The Ontario framework is not perfect, but it is better than guesswork.

Discussion

While varied, rare disease strategies in other countries, especially in the United Kingdom and Europe, are generally more comprehensive than simply a plan to contain the cost of DRDs.[27,28,29] They include screening for and prevention of rare disorders, improved diagnostic procedures and early intervention, improved coordination of care, research into rare disorders (e.g. creating disease registries and encouraging clinical studies), and collaboration with drug developers to improve cost-effective access to DRDs. As previously mentioned, all these elements are included in CORD's 2015 rare disease strategy.[30]

Instead of building on CORD's strategy, the federal government is focused on applying recommendations from its Advisory Council on the Implementation of National Pharmacare. In its final report in 2019, the Advisory Council recommended the establishment of a Canada Drug Agency that would assess the clinical effectiveness and cost-effectiveness of medicines, decide which products should be on a national formulary, negotiate prices and supply arrangements with manufacturers, provide advice to health care providers and patients on how best to use drugs, and monitor the safety and effectiveness of medicines in their real-world use.[31] Thus, the Canada Drug Agency would assume the roles of the HTA agencies, the pCPA, provincial drug decision-making committees, and some Health Canada regulatory activities.

Considerable alignment between the HTA agencies and pCPA is already in place.[32] For example, close collaboration exists between CADTH and the pCPA with representatives from each organization being observers at the others' meetings. In addition, CADTH reviews regularly include a recommendation for a price reduction – often a specific percentage that can range from around 20 percent to as much as 98 percent – to achieve cost-effectiveness at an arbitrary threshold that, for DRDs, is too low.[33] This allows CADTH to set up an initial

negotiating position for the pCPA if it chooses to bargain with the manufacturer.

New PMPRB regulations to be implemented in July 2021 that, in certain circumstances, will use CADTH's recommendations to assess whether the manufacturer's price is excessive.[34] If the PMPRB uses CADTH's low threshold for cost-effectiveness to establish a maximum price (something that cost-effectiveness estimates are not designed to do), drug developers are highly likely to decide that Canada is not a viable market for their innovative products. Overtures to biopharmaceutical companies are unlikely to succeed without a reversal of the PMPRB changes.[35] Government drug plans might save money – but only at the expense of patients suffering.

The Advisory Council on the Implementation of National Pharmacare also recommended the creation of a national formulary, which initially would only include a limited list of so-called essential drugs. It is unclear as to who would decide what drugs are "essential," but none of the current proposed lists include DRDs.[36] The risk with this limited beginning is that the promised formulary expansion would not be realized.

The third main recommendation of the Advisory Council was the development of a national strategy for expensive DRDs. Health Canada's discussion paper is the federal government's response. Unfortunately, it is a plan for cost-containment, not a rare disease strategy to improve the health and wellbeing of Canadians living with rare disorders.

Canadians value fairness and equity. However, when it comes to health care services, Canada does not apply a monetary cap to each patient's health care services. No one, for example, would suggest that services should be withheld from a Canadian who has a serious accident requiring months of hospital, physician and rehabilitation services because they are expensive. Similarly, Canada does not limit the amount spent on physician and other health care services for Canadians with diseases such as cancer or heart conditions, or those who require end-of-life care.

Negatively differentiating between Canadians with rare disorders and those with other health problems is demeaning and insulting. Beth Vanstone, a parent of a child with cystic fibrosis shared her view on this issue:[37]

"As a parent who has a child that has been saved by one of these 'high cost' drugs, I find the narrow focus on price offensive. My daughter is alive today thanks to the science and innovation invested in by a pharmaceutical company that chose to roll the dice and invest in life. We all understand business, high risk, high reward. As a parent and an advocate, I appreciate that pharma is willing to take the risk. The 'high cost' drugs come with amazing outcomes for patients and families. Once patients have access to these life saving drugs, the quality of life improves for patients, their caregivers and their families. Caregivers are often able to return to work, patients are able return to work and become productive members of society.

In a country like Canada, we should have the expectation that the government is able to recognize the benefits and value of new and innovative medications for patients. We should be able to have an expectation that the government has a vested interest in ensuring that patients have access to treatments that will improve their lives. As Canadians I would expect that our government would be able to negotiate fair and equitable prices for medications as countries both big and small around the globe are able to do. I am uncomfortable with how Canada feels it is in a position to use a club to hammer down prices – with only two percent of the [world] market it feels a bit ridiculous."

Conclusion

Canadians with rare disorders need the federal government to implement a comprehensive national strategy for rare diseases – one that includes screening for and prevention of these disorders, improved diagnosing procedures and early intervention, coordination of care, research into rare disorders, collaboration with drug developers to improve cost-effective access to DRDs, and improved access to DRDs.

Instead, Health Canada is proposing a plan for cost-containment that will further disincentivize drug developers from launching innovative DRDs in Canada and deprive Canadians living with rare disorders of opportunities to improve their wellbeing. Data presented by the PMPRB and others demonstrate that DRDs are not consuming unsustainable health care resources. In fact, new cancer drugs are consuming more resources than DRDs. Politicians and government officials would never suggest that innovative cancer drugs should be withheld from patients. Why, then, does the federal government intend to restrict the amount spent on Canadians who require DRDs, even if expensive

per patient, that can significantly improve their quality-of-life and/or extend their lives and may allow them to contribute more fully to family and society? Why is the government proposing to give much lower priority to the unmet needs of Canadians with rare disorders that too often are lethal and/or severely debilitating?

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