Minister of Health | Ministre de la Santé

Ottawa, Canada K1A 0K9

Bill Casey, MP
Chair
Standing Committee on Health
House of Commons
Ottawa, Ontario
K1A 0A6

Dear Mr. Casey:

Pursuant to Standing Order 109 of the House of Commons, I am pleased to respond on behalf of the Government of Canada (the Government) to the 22nd Report of the Standing Committee on Health (the Committee) entitled *"Canadians Affected by Rare Diseases and Disorders: Improving Access to Treatment"* which was tabled on February 28, 2019.

I would like to thank the Committee for its thorough study and work to help improve access to treatments for Canadians suffering from rare diseases and disorders. I commend the members of the Committee and the many witnesses who provided input to inform the Committee's important Report and contributing to the ongoing dialogue in this area.

The Committee's Report acknowledged that Canadians living with rare diseases and disorders often face barriers accessing treatment for their conditions. These barriers include lack of timely access to therapies as well as financial barriers due to the high cost of treatment. Furthermore, Canadians living with rare diseases and disorders often have limited treatment options resulting in these diseases and disorders having a disproportionate impact on affected individuals in terms of years of lives lost compared to other conditions.

Even in situations where treatments for rare diseases and disorders are available, evidence of their therapeutic value is usually limited due to the small number of patients eligible for enrollment in the clinical studies undertaken to support a product's market authorization. Limited evidence often means that it is only possible to initially establish the therapeutic value of a new treatment for a subset of patients with the condition. This underscores the importance of generating real-world evidence (RWE) following market authorization to supplement clinical trial data in order for the therapeutic value of a new treatment to be more fully established and to help inform decisions on whether expanded or continued reimbursement is warranted. While some new therapies for rare diseases fulfil their early promise, unfortunately many others do not.

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The Committee called on the Government, working in partnership with provincial and territorial (PT) governments, to address these treatment barriers through the Report's 19 recommendations encompassing four themes: Health Canada's market authorization of drugs for rare diseases, drug prices, reimbursement of drugs for rare diseases, and research on diagnosis for rare diseases including the use of RWE.

Since 2016, the Government has made significant progress improving the affordability, accessibility and appropriate use of prescription drugs in Canada, including those used to treat rare diseases. Investments in *Budget 2017* have been instrumental in improving coordination and efficiency throughout the pharmaceutical management system.

In *Budget 2018*, the Government announced the creation of an Advisory Council on the Implementation of National Pharmacare (the Council). In their interim report, released publicly on March 6, 2019, the Council recognized the unique challenges of funding and accessing expensive drugs for rare diseases, and that special consideration is needed to determine how to address these challenges to ensure a nationally consistent approach for these medications.

This work responds to the recommendations put forth by the Committee in its Report and demonstrates the Government's ongoing commitment to address barriers to high-cost drugs for rare diseases and disorders. Initiatives announced in *Budget 2019*, including a national strategy for high-cost drugs for rare diseases, are anticipated to build upon these existing Government initiatives.

Health Canada's Market Authorization of Drugs for Rare Diseases (recommendations 1-10)

In its Report, the Committee made ten recommendations regarding barriers to access drugs for rare diseases. The recommendations reference barriers involving Health Canada's market authorization process as well as other processes including the Special Access Program (SAP). Government initiatives responding to these recommendations are already underway resulting in improved coordination and alignment between Health Canada and other health system partners to support more timely access to drugs for rare diseases for Canadians.

Once a manufacturer decides to pursue market authorization of a drug in Canada, it files a new drug submission to Health Canada containing information and data about the drug's safety, efficacy and quality. As these submissions include information on the results of preclinical and clinical studies as well as therapeutic claims and side effects, the small patient populations with rare diseases and disorders means that the available evidence is often more limited compared to submissions for treatments for common conditions. Health Canada performs a thorough scientific review of the submitted information, sometimes using external consultants and advisory committees, and evaluates the safety, efficacy and quality data to assess the potential benefits and risks of the drug.

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Enhanced Regulatory Review

As part of *Budget 2017* investments, Health Canada undertook its Regulatory Review of Drugs and Devices initiative to modernize how market authorization is granted for new drugs, including those used for rare diseases and disorders, in order to support more timely access to prescription drugs and better meet the needs of the health care system and Canadians.

Given their nature, many drugs for rare diseases are eligible for expedited review. For example, the Notice of Compliance with Conditions policy allows for the regulatory approval of new drugs when evidence of their efficacy is promising, but still emerging. As part of this policy, pharmaceutical manufacturers agree to undertake continued study and report results to Health Canada. This allows Canadians to access innovative therapies that demonstrate great promise while safeguarding the safety of Canadians The Priority Review pathway is also already available to accelerate the regulatory review of drugs to treat diseases that are deemed serious, life-threatening, or severely debilitating. As part of this regulatory review, new and expanded review pathways are also being considered to allow more timely access to prescription drugs that support broader healthcare system needs.

Improved Coordination through Alignment and Collaboration

Since June 2018, Health Canada has provided pharmaceutical manufacturers of qualifying drug submissions the opportunity to participate in an aligned regulatory and health technology assessment (HTA) review process. This aligned review process is called for in recommendation 4 of the Committee's Report and allows for earlier reviews of new drugs by the Canadian Agency for Drugs and Technologies in Health (CADTH) and the Institut national d'excellence en santé et en services sociaux (INESSS) and facilitates greater sharing of information between parties. Since this process was started in 2018, the number of drug submissions receiving an aligned review has continued to increase. As of March 2019, 19 aligned reviews had been completed and 9 more are ongoing, suggesting considerable interest on behalf of the pharmaceutical industry in this new process.

In addition to supporting more aligned regulatory review processes, efforts that respond to recommendation 5 of the Committee's Report are also underway to provide more coordinated advice early in product development and throughout its lifecycle. Launched in February 2019, this initiative is supporting Health Canada and CADTH, with INESSS participating as observers, in providing joint advice to the pharmaceutical industry on drug development plans to ensure they meet the requirements of regulatory and HTA processes of both organizations. Health Canada can also provide advice regarding trial design and clinical trial application filing strategy, which includes submission of interim safety data in order to avoid treatment interruptions for patients transitioning to an open-label trial as called for in recommendation 6 of the Committee's Report. Given the challenges arising from limited evidence associated with drugs for rare diseases and disorders, providing coordinated scientific advice will help to improve efficiency

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for all parties involved potentially reducing delays and supporting more timely access to these prescription drugs for Canadians.

Investments announced in *Budget 2017* are also allowing CADTH to conduct more comprehensive assessments by exploring opportunities to leverage RWE to help supplement limited clinical evidence, such as for drugs for rare diseases. Through use of RWE, HTA review processes will be better equipped to assess the therapeutic value of such treatments and help inform both clinical and reimbursement decisions. Federal contributions to CADTH are evaluated in accordance with established policies to ensure they fulfill their established mandate. Health Canada is also working collaboratively with partners to optimize the use of RWE for regulatory decisions. A notice was posted on Health Canada's website on April 16, 2019 inviting industry partners to submit high quality RWE, particularly in areas where clinical trials may not be feasible, such as with rare diseases.

Greater Transparency and Information-Sharing

New regulations have been put in place to make clinical information about the safety and efficacy of drugs and medical devices available to the public, including those for rare diseases, which align with recommendation 2 in the Committee's Report. As of March 2019, Health Canada is posting clinical information for drug submissions as the review of each submission is completed. Health Canada will also make clinical information about drugs already on the market available upon request.

Special Access Program Renewal

Health Canada's SAP is also undergoing renewal to improve its processes. This work aligns with recommendations 8 and 9 in the Committee's Report. Improvements are helping facilitate a more efficient program for health care professionals including a new SAP web application currently in development that will help ease the process for initiating requests for drug authorization, provide greater clarity about the information required with applications, and simplify the request process. Recognizing that drugs requested through the SAP have not received market authorization in Canada, the SAP must rely on its discretionary authority, on a case-by-case basis when assessing a request. The SAP is examining the feasibility of authorizing supply for a longer timeframe, in line with the practitioner's follow up plan, for drugs with established patterns of use such as those used for chronic conditions including those that are considered to be rare.

As part of its regulatory review and other supporting initiatives, the Government has achieved improved coordination and alignment between Health Canada and other health system partners. This will directly benefit Canadians by supporting more timely access to prescription drugs, including those used to treat rare diseases.

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Drug Prices (recommendations 11-15)

During its study, the Committee heard of the need to address the high prices of drugs to treat rare diseases and made five recommendations pertaining to this theme. The extreme pricing for high-cost drugs for rare diseases and disorders (the list prices for these drugs often exceed $100,000 per patient each year and are sometimes significantly more) are challenging and a significant barrier to access, while also threatening the overall sustainability of the healthcare system. Given the magnitude of these prices, high-cost drugs for rare diseases are generally reimbursed by public and private drug plans, rather than being paid out-of-pocket by patients. However, cost-sharing between patients and their drug plan through deductibles and co-payments may result in significant financial pressures for some Canadians. The Government has focused on improving the affordability of prescription drugs, including those used to treat rare diseases, as a cornerstone of its pharmaceutical agenda.

As recognized in the Committee's Report, the Patented Medicine Prices Review Board's (PMPRB) current regulatory framework is outdated and insufficient to protect Canadians from excessive patented drug prices. Further, the median of prices in the Organisation for Economic Co-operation and Development member countries are, on average, 19% below prices in Canada, which are third highest among the 31 countries. This is significant given the high prices of drugs for rare diseases and a market for patented medicines where high-cost drugs now account for more than 40% of patented drug spending in Canada.

Budget 2017 investments enable the PMPRB to operationalize a modernized regulatory framework to improve the affordability of patented drugs including those used to treat rare diseases. The proposed new regulatory framework, pre-published in Canada Gazette Part I, would provide the PMPRB with new economics-based price regulatory factors, which would allow it to regulate patented drug prices based on the value of the drug and its impact on the health care system. It would also enable the Board to take into consideration the number of Canadians the drug would treat, which would be especially relevant for high-cost drugs for rare diseases. The proposed amendments would save approximately $13.2B over 10 years on patented drug costs. Lower prices would alleviate financial pressures on public and private drug plans and improve affordable access for Canadians paying out-of-pocket.

Recognizing the importance of improving the affordability of prescription drugs by lowering drug prices, since January 2016, the Government of Canada has also partnered with the PTs as an active member of the pan-Canadian Pharmaceutical Alliance (pCPA). The pCPA combines governments' purchasing power to secure lower prices for prescription drugs. As of September 30, 2018, the pCPA had achieved approximately $1.98 billion in annual cost savings for public drug plans, including having completed nine negotiations for drugs for rare diseases as of November 26, 2018. Combining the negotiating power of federal, provincial and territorial (FPT) governments has helped achieve greater savings for all publicly funded drug programs, increased access to clinically effective drug treatment options and improved consistency of pricing and coverage criteria across Canada, including for drugs for rare diseases.

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Reimbursement of Drugs for Rare Diseases (recommendations 16-18)

During its study, the Committee heard from witnesses about barriers to access treatment related to reimbursement including issues of inequitable coverage and the difficulties that PT drug plans face in making reimbursement decisions involving drugs for rare diseases. Underscoring its three recommendations pertaining to this theme, the Committee expressed the need to develop short- and long-term options for covering the costs of drugs for rare diseases.

Advisory Council on the Implementation of National Pharmacare

In *Budget 2018*, the Government announced the creation of the Council to assess options and provide independent advice to the Minister of Health and the Minister of Finance on how best to implement national pharmacare in a manner that is affordable for Canadians, employers and governments.

The Council's interim report was released on March 6, 2019, and included core principles and three foundational elements (essential "building blocks" for any national pharmacare program). The foundational elements are: the creation of a national drug agency; the development of a comprehensive, evidence-based formulary; and investments in drug data and information technology systems. The Council recognized the unique challenges of funding and accessing expensive drugs for rare diseases, and that special consideration is needed to determine how to address these challenges to ensure a nationally consistent approach for these medications.

The Government is committed to ensuring all Canadians have access to affordable medicine, and looks forward to receiving the Council's final report this Spring.

Budget 2019: Moving Forward on Implementing National Pharmacare

Guided by the consultations undertaken by the Council and its interim report, *Budget 2019* announced the Government's intention to move forward on three foundational elements of national pharmacare that are also relevant to the Committee's recommendations:

- The creation of the Canadian Drug Agency, a new national drug agency that will build on existing PT successes, and take a coordinated approach to assessing effectiveness and negotiating prescription drug prices on behalf of Canadians. By negotiating better prices, this could help lower the cost of prescription drugs for Canadians by up to $3 billion per year in the long term.
- In partnership with PTs and stakeholders, part of the Agency's work will be taking steps toward the development of a national formulary—a comprehensive, evidence-based list of prescribed drugs. This would provide the basis for a consistent approach to formulary listing and patient access across the country.
- A national strategy for high-cost drugs for rare diseases to help Canadians get better access to the effective treatments they need.

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To establish a national strategy for high-cost drugs for rare diseases, *Budget 2019* proposes to invest up to $1B over two years, starting in 2022–23, with up to $500M per year ongoing. These investments demonstrate the Government's continued support to addressing barriers to treatment for rare diseases and mark important first steps to help Canadians get the prescription drugs they need to stay healthy, and contribute to their families, their communities, and our economy.

Research On Diagnosis for Rare Diseases including the use of Real World Evidence (recommendation 19)

As noted by the Committee, obtaining early diagnosis of a rare disease is critical to allow access to treatment in a timely fashion and help minimize the number of unnecessary doctor visits and unnecessary treatments that take a toll on Canadians and their families.

The Government has provided substantial funding to the Canadian Institutes of Health Research (CIHR) to support collaborative research initiatives on rare diseases in Canada including the Care4Rare initiative, support for the development of Orphanet-Canada, and internationally through membership in the International Rare Diseases Research Consortium. Over the last five years, CIHR has invested $162M in rare disease research, with $39M invested in 2017-18 alone. Investments made by CIHR in this area of research have increased more than two-fold in comparison to the previous five years. CIHR-sponsored research into the diagnosis of rare diseases and the collection of RWE on the effectiveness of treatments is ongoing.

Health Canada is working with CIHR through the CIHR-funded Drug Safety and Effectiveness Network (DSEN), a pan-Canadian network that conducts real-world drug safety and effectiveness research. This research contributes to the body of evidence on drug safety and effectiveness available to regulators and policy makers.

Conclusion

I extend my thanks to the Committee for its Report on an important and timely issue. The Government is appreciative of this work and the associated recommendations. I trust that this response demonstrates the Government's commitment to playing a strong and collaborative role to improve the accessibility, affordability and appropriate use of prescription drugs in Canada. The Committee's Report and recommendations play an important role in contributing to the ongoing dialogue on rare diseases in Canada.

Sincerely,

The Honourable Ginette Petitpas Taylor, P.C., M.P.
Minister of Health