MEMO

To:	NPAC
From:	H+K Strategies Ottawa
Date:	March 8, 2018
Subject:	Tabling of HESA Report *Canadians Affected by Rare Diseases and Disorders: Improving Access to Treatment*

Overview

On February 28, the House of Commons Standing Committee on Health tabled its report, *Canadians Affected by Rare Diseases and Disorders: Improving Access to Treatment* (available here), recommending that the government take decisive action to reduce drug costs for Canadians with rare diseases. In tabling the report, the Committee has requested a comprehensive response from the Government.

In its study leading up to the drafting of the report, the Committee received 10 briefs and heard from 24 witnesses who identified four key challenges Canadians face in accessing treatment for rare diseases:

1. regulatory approval of drugs for rare diseases for sale;
2. pricing;
3. reimbursement for drug costs through provincial and territorial drug coverage plans; and
4. access to diagnostics for these diseases.

Committee Chair Bill Casey summarized in the Report that "the biggest barrier limiting patients' access to these drugs is their affordability. Drugs for rare diseases can cost between $0.5 million to $4.9 million per person per year… Governments also face difficulties justifying spending funds on the reimbursement of these drugs when there is limited evidence showing that they offer significant health benefits. At the same time, drug manufacturers must continue to have incentives to invest in the development of treatments in this area and to seek market authorization for the sale of these drugs in Canada."

In agreeing with the witnesses, the Committee's Report makes 19 recommendations that focus on:

- establishing coordinated processes for the approval and reimbursement of drugs for rare diseases in Canada;
- implementing the proposed amendments to the Patented Medicines Regulations that will lead to lower drug prices;
- short- and long-term options for covering the costs of drugs for rare diseases; and
- supporting research that examines the real-world benefits and risks of drugs for rare diseases.

The committee did not outline the definition of rare diseases that it used in their study, though did reference the definition that Health Canada uses, that rare diseases are those that affect fewer than 5 in 10,000 Canadians.

Barriers in Accessing Treatment for Rare Diseases in Canada: What the Committee Heard

Market Authorization of Drugs for Rare Diseases

Catherine Parker of Health Canada testified to committee that Health Canada (HC)has given accelerated approval (priority status) to several drugs that are intended to treat serious or life-threatening diseases, including rare diseases. HC has done this while harmonizing its regulatory requirements for drug approvals to those in other jurisdictions so that drug companies can file one dossier to all regulators. A noted improvement was that HC and the US Food and Drug Administration (FDA) now have a common portal for filing of drug approvals simultaneously in both jurisdictions. The PMPRB has noted that this has allowed 9 of the 10 top-selling orphan drugs in the U.S. to be available for sale in Canada. There is a recognition, however, that more must be done, and HC is undertaking a review of its regulatory approval of prescription drugs, including drugs for rare diseases, to identify ways to improve its efficiency and meet patient and health care system needs.

Special Access Programme (SAP)

The SAP provides access to unapproved medications on an exceptional case-by-case basis for patients with serious or life-threatening conditions when conventional treatments have failed, are unsuitable or unavailable. Access to the program is granted via physician-request based on a demonstration that the therapy is the best choice for the given patient, and why

other therapies are not suitable. Approximately 30% of the drugs requested under this program are for rare diseases. Witnesses agreed that SAP is an important avenue for obtaining access to treatments for rare diseases.

Prices Of Drugs for Rare Diseases

Douglas Clark, Executive Director of the PMPRB testified that the PMPRB was created out of concern that stronger patent protection for medicines might cause prices to rise unacceptably so as to become unaffordable to consumers. It was added that the PMPRB process (evaluation of prices) typically takes three months to complete. Witnesses testified that despite the price ceilings set by the PMPRB, drugs for rare diseases continue to come at high prices which threaten the financial sustainability of public and private drug coverage plans. Given the prevalence of orphan drugs in the U.S. and Europe (approximate 50% of new patented medicines under PMPRB's jurisdiction), Douglas Clark testified that they pose a significant risk to the financial sustainability of the health care system, nothing that while the Canadian system can absorb a few of these high cost drugs, it would collapse under the weight of hundreds of them. He added that the single most important determinant of access is affordability. Witnesses called for there to be more transparency from manufacturers of drugs for rare diseases in demonstrating the costs associated with the R&D of the drugs to justify high prices.

Mr. Clark added that there are three main types of changes the government is considering through proposed regulatory amendmengs: that the basket of countries for price comparison be expanded; that new factors (read: stress test) be introduced to determine whether a price is excessive; and finally, that manufacturers be compelled to provide the PMPRB with information regarding confidential price rebates that they offer to public and private drug coverage payers to have a better understanding of the prices of drugs on the market. The committee heard that drug manufacturers opposed these proposed regulatory changes. Mr. Clark responded that he's aware of concerns that Canadians may have delayed or compromised access to the latest patented drugs to this but rebutted that there was "little evidence to support the argument that lower prices result in less access." He added that "the reality is that many countries with similar health care systems and economies to Canada's pay less for drugs yet enjoy the same or better access. The same is true of research and development investment." Mr. Clark reinforced his position by noting that Canada pays the third-highest price for patented medicines in the world, yet the ratio of R&D to sales is low comparatively to the PMPRB7 countries (4.4% in Canada versus over 20% in PMPRB7). Mr. Clark stated that the PMPRB is not doing a very good job of protecting Canadians from excessive pricing with tools currently at its disposal.

Reimbursement of Rare Disease Drug Costs Through Federal Provincial and Territorial Public Drug Coverage Plans

The reimbursement of prescription drugs through provincial and territorial public drug coverage plans, and by the federal government for select groups of people (including military, veterans, First Nations and Inuit, federal inmates, certain classes of refugees) is reliant upon national and pan-Canadian processes to support their decision-making, including CADTH and the pCPA, which establish consistency in pricing and reimbursement decisions for drugs across the country. Once a drug has been authorized for sale by Health Canada, CADTH then provides advice and recommendations to public drug coverage plans by undertaking a health technology assessment. Upon completion of the CADTH assessment process, the pCPA negotiates with manufacturers to obtain price reductions and other conditions for the listing of a drug on public drug coverage plans. CADTH and the pCPA testified that they face difficulties in making drug coverage recommendations and undertaking price negotiations for drugs for rare diseases because of the limited clinical data for the drugs. CADTH advocated for investments in infrastructure and support for the collection of real world evidence to enable public drug plans to provide conditional coverage for drugs backed up by limited evidence, and reassess or negotiate with manufacturers when additional evidence is available. Andrew McFadyen of the Isaac Foundation recommended that, to reduce the timeliness associated with drug approval and reimbursement processes, a more coordinated drug approval and reimbursement process be adopted in which HC and CADTH reviews would take place simultaneously, and pCPA negotiations would begin at the same time.

The Way Forward: Committee Observations and Recommendations

In summary, the Committee heard that current approval, pricing and reimbursement processes for drugs for rare diseases are not meeting the needs of patients, drug manufacturers and provincial and territorial public drug coverage plans, resulting in tensions within the system.

Notably, the Committee recommended that for moving forward, the federal government collaborate with provinces and territories to develop a coordinated process for market authorization and reimbursement of drugs for rare diseases, that the federal government ensure greater transparence and information sharing throughout the life cycle of the drugs, and that the government move forward with changes to the PMPRB addressing high drug prices in Canada.

Appendix I – Recommendations

Toward Health Canada's Market Authorization of Drugs for Rare Diseases

1. That the Government of Canada, in collaboration with the provinces and territories, develop a coordinated process for the market authorization and reimbursement of drugs for rare diseases;
2. That the Government of Canada work to ensure greater transparency and information sharing throughout the life cycle of drugs for rare diseases to ensure timely access for key decision-makers, including health care providers, health technology assessors and patients;
3. That the Government of Canada in collaboration with the provinces and territories develop a national, independent, expert review panel to provide recommendations and guidance on the regulatory review, pricing and reimbursement of drugs for rare diseases in Canada, including instructions on how to streamline these processes; and report publicly on its findings;
4. That Health Canada and the Canadian Agency for Drugs and Technologies in Health undertake their respective scientific evidence review processes of drugs for rare diseases in tandem as a standard practice;
5. That Health Canada, in collaboration with the Canadian Agency for Drugs and Technologies in Health, provide guidance and advice to drug manufacturers in the design of clinical trials to ensure that they meet the requirements of both market authorization and reimbursement processes in Canada;
6. That Health Canada consider removing regulatory requirements for drug manufacturers to seek additional approval for an open-label extension for drugs at the completion of a clinical trial to ensure that patients have uninterrupted access to these drugs if no safety concerns are present, in line with regulatory practices in the United States;
7. That Health Canada consider reducing regulatory submission fees for manufacturers of drugs for rare diseases seeking to obtain market authorization for the drugs in Canada;
8. That Health Canada be more proactive in its communications with physicians and patients regarding the specific medical need criteria required for obtaining access to drugs through the Special Access Programme;
9. That the Government of Canada remove the requirement to reapply to the Special Access Programme every three to six months when accessing a drug for a permanent, stable condition. Once initially approved, Canadians' approvals should remain in place until a doctor rescinds the approval or the patient's condition changes significantly;
10. That Health Canada ensure that drug manufacturers meet their regulatory obligations when Notice of Compliance with conditions are granted for drugs where limited evidence is available regarding their quality, safety and efficacy;

On Drug Prices

11. That the Government of Canada move forward with implementing proposed changes to the Patented Medicines Regulations to address high drug prices in Canada;
12. That the Government of Canada consider establishing separate requirements for determining price ceilings for drugs for rare diseases under the Patented Medicines Regulations to reflect the small market for these drugs in Canada;
13. That the Patented Medicine Prices Review Board be required to consider the advice and recommendations of the proposed independent advisory committee on drugs for rare diseases in setting the price ceilings for drugs for rare diseases;
14. That the Government of Canada introduce additional regulatory requirements under section 88(1) (c) of the Patent Act that require manufacturers of patented pharmaceuticals to provide information to the Patented Medicine Prices Review Board regarding their research and development costs for a drug once they have obtained market authorization from Health Canada;
15. That the Government of Canada undertake a review of the entire pharmaceutical research and manufacturing process to better understand where government regulations and laws are having the unintended consequences of raising final drug costs for patients. This review should include an examination of whether drug costs could be reduced through open science;

On Reimbursement of Drugs for Rare Diseases

16. That the Government of Canada, in collaboration with the provinces, territories and drug manufacturers, establish a jointly funded compassionate care program that covers the costs of drugs for rare diseases while they are under review for market authorization and cost reimbursement;
17. That the reimbursement of drugs for rare diseases be included as part of a national pharmacare program established by the Government of Canada, in collaboration with the provinces and territories, through amendments to the Canada Health Act, as recommended by the House of Commons Standing Committee on Health in its report entitled *Pharmacare Now: Prescription Medicine Coverage For All Canadians*;

18. That the Office of the Auditor General conduct an audit of Health Canada to determine whether it has been effective in managing its funding agreement with the Canadian Agency for Drugs and Technologies in Health, including determining whether Health Canada is effectively ensuring that the Agency is fulfilling its mandate in accordance with agreed terms and conditions of the agreement with Health Canada;

On Research

19. That the Government of Canada provide funding through the Canadian Institutes of Health Research for research into the diagnosis of patients with rare diseases and the collection of real-world evidence regarding the effectiveness of treatments for these conditions;

Appendix II – Dissenting Conservative Opinion

Conservatives agree that Canadians need better access to their rare disease treatments and medications. However, they disagree particularly with the proposed changes to the PMPRB and making rare disease funding part of a national pharmacare program. While they agree that changes to the PMPRB need to be made in order to make approvals faster, less costly, and to strike a balance between low prices for Canadians and high enough profit margins to encourage pharmaceutical companies to offer the drug, they feel the changes currently proposed will not accomplish these goals.

Conservatives also disagreed with Recommendation 13, which called for the reimbursement of drugs for rare diseases be included as part of a national pharmacare program through amendments to the Canada Health Act, as laid out in HESA's previous report *Pharmacare Now: Prescription Medicine Coverage for All Canadians*. Conservatives reiterated their dissenting opinion that a national pharmacare program is not the fastest or most efficient way to address coverage of prescription medications for those who currently do not have access.