March 23, 2021 1187355

Michelle Boudreau
Executive Director
Office of Pharmaceuticals Management Strategies
Health Canada
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Dear Ms. Boudreau,

In January, you had invited jurisdictions to provide their feedback regarding Health Canada's National Strategy for High-Cost Drugs for Rare Diseases. As British Columbia's Assistant Deputy Minister for the Pharmaceutical, Laboratory and Blood Services Division, I am pleased to provide you with some feedback. As highlighted in Health Canada's Discussion Paper, High Cost Drugs for Rare Diseases (hCDRd) continue to challenge public payers due to their disproportionate cost, uncertain evidence, and high patient need.

The cost of hCDRds elevates issues of optimal and ethical health resource allocation to an extreme. In 2018/19, the average beneficiary enrolled in a BC Pharmacare plan received $1,600 in benefits while in 2020/21, the average hCDRd patient received $336,000 in benefits. The costs of these expensive drugs cannot be borne by individual patients or their families. Hence, public and private payers are put in the position of balancing and justifying the enormous resource needs of a small minority over the moderate needs of a plurality. The accelerated growth in the number of expensive drugs for rare diseases, including gene therapies, has created greater need and urgency to address the multiple issues these therapies cause for all stakeholders.

Disparate principles and an emphasis on addressing hCDRd's have led to inconsistent reimbursement decisions across Canada. Thus, national leadership to establish and enforce common principles across Canada will help improve consistency in access, patient and clinician expectations, and begin establishing a unified decision-making framework. Moreover, Canada must establish a minimum threshold of cost-effectiveness. These minimum standards would guide and empower our decision makers to clearly delineate medications which are and are not cost-effective. In the absence of such an agreed upon threshold, jurisdictions will continue valuing medications differentially leading to disparate funding and access for patients. A threshold would also provide guidance to the manufacturers and marketers of these therapies. Left unchecked, prices will continue to escalate based on precedent.

…2

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Differential funding decisions across jurisdictions are also a product of limited evidence supporting these medications. Due to the uncertainty, the evidence is often interpreted heterogeneously across the nation, leading to variation in funding decisions. Cost-effectiveness thresholds will ameliorate this variation, but novel reimbursement frameworks coupled with national infrastructure to collect and analyse real world evidence will improve our understanding of hCDRd's and consequently our decision making. However, this will require tremendous leadership and collaboration among policy makers, clinicians, researchers, and information technology/information management experts. Together, we must create a digital infrastructure to hold vast amounts of clinical data nationally that is accessible to analysts and researchers in academia, Ministries of Health and regional health authorities who can help Ministry policy makers analyse, establish and interpret the value of hCDRd's. Ideally, such infrastructure would permit international collaboration and data sharing.

Finally, improved co-ordination between the Canadian Agency for Drugs and Therapeutics in Health, the pan-Canadian Pharmaceutical Alliance, provincial and federal governments and the Patented Medicines Prescription Review Board must occur to harmonize incentives to identity, negotiate for, reimburse medications that are provided to Canadians. Without strategic alignment, each organization is at risk of making decisions that negatively impact another's Mission. Canada must also establish international partnerships, whenever possible, to share information that may improve our collective bargaining power, as these challenges are not limited to Canadians.

In summary, BC supports

- Including gene therapies in the National Strategy for hCDRd;
- Developing aligned principles related to values, criteria, process, decisions, cost, and outcomes;
- Developing minimum evidence thresholds;
- Using cost-effectiveness thresholds to support national alignment; and
- Developing infrastructure to support real world evidence development and better understand rare diseases and drug therapy effectiveness.

To meet the growing costs and challenges of hCDRd's, governments will need to consider 1) reallocating funds from other areas of health care or beyond, 2) increasing taxes or 3) establishing a new framework for hCDRd's that flattens the curve of the expenditure and ensures appropriate access based on values and principles and not politics. As extreme cost-drivers, even funding a small number of patients or hCDRd's represents an opportunity cost related to other societal benefits and creates an ethical issue of how to allocate limited resources. Notably, these issues are already experienced by our oncology colleagues and will be further potentiated as more gene therapies become available in Canada.

…3

Thus, Health Canada's National Strategy for High-Cost Drugs for Rare Diseases has implications and can impact aspects of the health system beyond hCDRd's. I look forward to Health Canada's leadership in creating a unified, ethical and principle based national strategic plan.

Sincerely,

Mitch Moneo
Assistant Deputy Minister
Pharmaceutical, Laboratory and Blood Services Division, Ministry of Health