White Paper

Rare Disease Drugs in Canada

Considerations for Equitable Access

November 2022

Key points at a glance

- Ensuring comprehensive, timely and equitable access to medication for rare diseases is a significant public policy priority for the Government of Canada. The government's decision to allocate $500 million annually to a national strategy for rare disease drugs represents a pillar of its emerging work on national pharmacare. McKesson Canada is proud to contribute to the public policy conversation around this national strategy, building on its experience with patient support programs, pharmaceutical distribution, and specialty pharmacy services.
- McKesson Canada recommends that the Government of Canada allocate a portion of its new investment in the national rare disease strategy toward two dedicated fees:
  - A patient support fee, set at 5% of the cost of a rare disease drug and capped at $1,000, to support the ongoing development and maintenance of patient support program (PSP) infrastructure and capacity. Given the critical role PSPs play in ensuring access and adherence to rare disease medications, this funding would directly contribute to initiatives designed to improve the patient experience and healthcare outcomes, providing direct support to rare disease patients and their family members and caregivers.
  - A rare disease drug distribution and pharmacy fee, also set at 5% of the cost of a rare disease drug and capped at $1,000, to provide sustainable support to the complex investments and operating funding required to ensure rare disease drugs are available to all Canadians who need them conveniently and with minimal waste.
  - These proposed fees are not meant to replace existing rare disease PSP and distribution/pharmacy funding, but to augment existing funding streams to improve patient outcomes and increase the sustainability of Canada's rare disease drug distribution and administration infrastructure.

- McKesson Canada also encourages the federal government to take steps to improve Canada's standing as a potential market for new rare disease drugs via the following:
  - The development of enhanced drug authorization processes to ensure that the unique nature of rare diseases (which, by definition, struggle to conform to clinical evidence requirements due primarily to small patient population sizes) does not pose an insurmountable obstacle for new medications potentially entering the Canadian market.
  - A review of the pending patented medicine price reform initiative that threatens to weaken the perception of the Canadian market among global rare disease drug manufacturers, unintentionally shrinking the 'pipeline' of new medications that could provide transformative or life-saving benefits to Canadians.
- Finally, McKesson Canada encourages the Government of Canada to work in partnership with provincial ministries of health to ensure that new funds increase the overall amount of support for patients with rare diseases.

Introduction

In its 2019 budget, the Government of Canada announced its intention to spend $1 billion in fiscal years 2022-23 and 2023-24 on drugs for rare diseases; since then, the federal government has signalled that this amount is intended to become a recurring, annual investment to support patients with rare diseases. The decision to devote these funds to the challenges brought on by rare disease is significant; it represents the government's inaugural major expenditure on its emerging policy of national pharmacare. It also reflects a growing consensus among healthcare stakeholders that targeted action is required to improve the outcomes of patients with rare diseases all the while addressing the increasing challenge these medications present to public drug plan programs.

As Health Canada's discussion paper on the development of a rare disease strategy explains, there is no consensus (locally or abroad) on the specific meaning of the term rare disease, though typically discussions of rare disease policy focus on conditions that (a) usually have a genetic basis, (b) affect children, particularly newborns, about half the time, (c) are fatal in young children, and (d) require considerable medical care. [1] [2]

In addition, because rare diseases are both extremely complex and, by definition, restricted to very small populations, effective treatments are challenging to develop. As a result, commentators often focus on the high sticker price of rare disease medications, which typically start at $100,000 per year. From the perspective of healthcare policymaking, rare disease drugs pose a unique challenge: they can be, literally, lifesaving, but they are considerably more expensive than other drugs – and there are more and more of them.

Recognizing that the Government of Canada has committed to an annual allocation of $500 million in new funding to enable better access to rare disease drugs, this paper argues for a series of measures designed to make that access comprehensive, equitable and functional in the context of Canada's mixed model of publicly and privately funded pharmaceutical reimbursement.

[1] In Canada and Europe, "rare disease" usually refers to those that infect one of out very 2,000 people, whereas in the United States, any condition affecting fewer than 200,000 people (the equivalent of about one out of every 1,650 Americans) is considered a rare disease.

[2] Health Canada. (2021). *Building a National Strategy for High-Cost Drugs for Rare Diseases: A Discussion Paper for Engaging Canadians.* Ottawa: Government of Canada.

Specifically, the paper examines four critical challenges to the implementation of an effective rare disease drug strategy in Canada.

- First, the challenge of lack of recognition for the "end-to-end" needs of rare disease patients, including full appreciation of the critical benefit of patient support programs, specialty pharmaceutical distribution, specialty pharmacy services, and support for families and caretakers. A comprehensive rare disease strategy ought to fully incorporate these elements of rare disease care and should not be solely focused on drug cost and reimbursement.
- Second, the challenge of the increasingly complex distribution and pharmacy services required to ensure patients have safe, reliable access to rare disease medications. As the paper demonstrates, the reimbursement frameworks in which community pharmacies and pharmaceutical distributors operate were not developed to support the intricate nature of managing many rare disease drugs.
- Third, the challenge of drug authorization, market access, and reimbursement, recognizing the relative size of the Canadian market for rare disease drug manufacturers and the potentially chilling effect of other ongoing drug price reforms. In particular, the proposed changes to the Patented Medicines Prices Review Board guidelines will likely make Canada a less attractive market to manufacturers of new medicines, including those for patients with rare diseases.
- Fourth, the challenge of federal policymaking in an area of provincial jurisdiction as part of a broader national pharmacare initiative. While the Government of Canada has and continues to play a critical role in healthcare funding and policymaking, it is important that the rare disease drug strategy be developed in a manner that reflects current provincial policy frameworks, and that the Government of Canada work collaboratively with the provincial governments to establish and implement such a strategy.

The paper makes the following recommendations:

1. The Government of Canada should allocate a portion of the annual rare disease strategy funding specifically to enhance and expand patient support programs to ensure patients and family members have access to comprehensive treatment. By dedicating a stream of funds to PSPs, policymakers would increase medication adherence and create the conditions for optimal patient outcomes. While the specific amount should be developed in partnership with stakeholders, McKesson Canada suggests a 'starting point' of 5% of the cost of the drug, capped at $1,000.
2. The Government of Canada should create a rare disease drug distribution and pharmacy service fee equivalent to 5% of the cost of the drug capped at $1,000, to reduce drug waste and ensure optimal patient adherence and health outcomes.
3. The Government of Canada should adopt flexible drug approval models for rare disease drugs, including via the granting of provisional approvals with permanent decisions being conditional on the gathering of real-world evidence. It further encourages the Government of Canada to fund provisional reimbursement by public and private drug plans until such time as adequate evidence exists to make a comprehensive reimbursement decision.
4. The federal government should review the PMPRB guidelines to ensure they do not impede the objectives of this rare disease strategy, and instead focus its efforts on expanded reimbursement criteria.
5. The federal government should work collaboratively with the provincial/territorial governments to determine how to best invest new resources to complement the existing reality in each jurisdiction. This would involve assessing where public and private drug plans do not adequately meet the needs of rare disease patients on a province-by-province basis.

Considering the 'end to end' needs of patients

It is well known that patients who suffer from rare diseases face unique challenges to treatment. Rare disease drugs tend to be expensive, with annual treatment costs typically in the six figures. Because so many patients afflicted with rare diseases are young children, family members are often deeply implicated in navigating different aspects of the healthcare system.

To help patients and families manage the unique complexities of rare diseases, manufacturers and pharmaceutical distributors have developed patient support programs (PSPs) designed to offload as much of the administrative burden associated with managing rare diseases. PSPs allow patients and their families to focus on managing the illness and getting well.

Patient support programs are essential partners in rare disease management – they typically focus on:

- Coordinating healthcare activities among many practitioners, including family and specialist physicians, pharmacists, therapists, and paramedical practitioners.
- Creating and sustaining links between patients (and their families) and patient information and advocacy associations.
- Ensuring access to specialty pharmacy services.

In addition to supporting patients and families, PSPs provide critical value to the healthcare system, including:

- Ensuring timely, safe and secure access to medication via specialty distribution, which ensures cold-chain and other product requirements are observed, and specialty pharmacy, which ensures that the patient's pharmacist has the expertise to handle and support the dispensing of the medication.
- Access to non-medication healthcare products, such as infusion supplies.
- The development and implementation of a streamlined process flow that includes all relevant stakeholders, such as PSP personnel, the specialty distribution team, the specialty pharmacy, and the drug manufacturer. The PSP serves as a hub connecting the patient, the patient's family/caregiver, and the associated healthcare professionals with the various logistical participants in the distribution and administration of the medication.
- Providing administrative support as patients navigate the reimbursement process for their medication, including both public and private insurance plans, as well as special access and compassionate use programs.
- Ensuring the patient's other healthcare and para-healthcare needs are integrated into their care plan.
- Gathering of real-world evidence on the clinical and quality-of-life outcomes of these drugs.

Recommendation 1: Investing in patient support programs

The Government of Canada should allocate a portion of the annual rare disease strategy funding specifically to enhance and expand patient support programs to ensure patients and family members have access to comprehensive treatment. By dedicating a stream of funds to PSPs, policymakers would increase medication adherence and create the conditions for optimal patient outcomes, while reducing waste from treatment failures. While the specific amount should be developed in partnership with stakeholders, McKesson Canada suggests a 'starting point' of 5% of the cost of the drug, capped at $1,000, to fund patient support programs associated with rare disease drugs. This funding, in addition to existing funding streams, will provide a critical baseline of support for patient support program infrastructure for rare disease drugs.

Patient Support Programs in Action: Remodulin

Remodulin is a critical molecule in the treatment of pulmonary arterial hypertension. It is infused 24 hours a day, seven days a week. By widening blood vessels, it reduces strain on the heart and provides crucial benefit to patients, who tend to range in age from 20 to 60 years old.

McKesson Canada's Remodulin PSP ensures that patients have access to therapy around the clock, including emergency processes in place for at-home patients. As the drug cost is significant, PSP staff are skilled at helping patients navigate access to reimbursement, including via compassionate use programs. The PSP also ensures seamless distribution of the medication and the necessary infusion pumps to specialty pharmacy. The PSP consists of five major areas of activity:

1. Registered nurses provide extensive training to patients and care partners, as well as coordination of backup supplies, medication, and pumps with patients at all times. RNs are available 24 hours a day via hotline to help patients navigate through adverse events and medication interruption.
2. Bridging medication as patients often need to access Remodulin urgently due to their fragile condition.
3. Ambient temperature delivery system, often including same-day delivery as needed.
4. All pumps maintained by the PSP and pharmacies. Pumps require minimum of annual calibration, and occasional repair. The PSP coordinates these services and movement of pumps to pharmacy, patient and manufacturer.
5. Because patients receive critical vasodilator therapy, there is a need for experienced and highly qualified nurses to meet their unique needs. Nurses provide extensive training and continued support. Patients are typically in their homes receiving therapy so a relationship of trust with the McKesson Canada Remodulin PSP team is essential. Registered nurses work with other healthcare professionals in emergency situations for patient support and maintenance of therapy as Remodulin is typically unknown to emergency medicine professionals

Specialty pharmaceutical distribution and specialty pharmacy services

Many rare disease medications require meticulous distribution, storage, and handling requirements. Given their high cost, it is unacceptable for these drugs to be wasted due to inadequate attention paid to distribution requirements. As one of the country's leading pharmaceutical distributors, McKesson Canada has extensive experience storing and distributing specialty medication, including rare disease drugs. McKesson Canada's specialty distribution services ensure that Canadians in communities in every province and territory (including in the rural and remote parts of the country) have access to rare disease drugs.

Our dedicated specialty services provide considerable value to the administration of rare disease drugs:

- Our teams ensure manufacturers of rare disease medications have seamless end-to-end services, ranging from drug importation to point-of-care delivery.
- We offer full lot- and expiry-date-tracking for all specialty products, including rare disease drugs, regardless of product volume, ensuring minimal waste of high-cost drugs.
- McKesson Canada provides 'white-glove' quality management, handling, and transportation processes to protect product integrity, and to respect temperature and other requirements.

The complexity surrounding the handling of many rare disease drugs does not end once the delivery has been made to a community pharmacy. More and more specialty pharmacies have emerged in recent years as the proportion of drugs requiring pharmacists to have specific product knowledge has increased. Specialty pharmacists routinely work closely with patients to monitor outcomes, recommending dosage and regiment

adjustments to physicians where needed, and working collaboratively with drug infusion clinics and other healthcare providers. Pharmacists also play a critical role in ensuring patient adherence, a vital component of any rare disease strategy (especially given the high cost of rare disease drugs).

The pharmaceutical distribution and pharmacy services reimbursement frameworks do not reflect the incredibly complex nature of transporting, storing, and dispensing rare disease medications, nor do they account for the high levels of patient care and support that pharmacists provide.

Recommendation 2: Funding rare disease drug distribution and pharmacy services

McKesson Canada recommends that the Government of Canada allocate a set portion of all new rare disease drug investments to support drug distribution and pharmacy services, to reduce drug waste and ensure optimal patient adherence and health outcomes. McKesson Canada recommends a 'starting point' fee equivalent to 5% of the cost of the drug, capped at $1,000.

How the 5% amount is split between distributors and pharmacy service providers can be developed via a tiered framework approach, taking into consideration the different characteristics of rare disease drugs – whether they are infused or are taken orally and whether they require special handling requirements (cold-chain, frozen, etc.). A working group comprised of rare disease drug distributors, specialty pharmacists, and governments can determine the appropriate amounts.

Improving drug authorization, market access, and reimbursement policymaking

Developing and implementing treatments for individuals suffering from rare diseases is complex because patient populations are so small and often spread out geographically. While the Canadian Organization for Rare Disorders estimates that one in 12 Canadians are affected by rare diseases generally, the number of individuals who suffer from a particular rare disease can be extremely small.

As a result, the typical framework for evaluating, authorizing and reimbursing prescription drugs is poorly suited to the realities of rare disease in Canada. While drug authorization processes typically rely on robust clinical data sets and precise predictions of value-for-money, the small patient populations associated with rare disease often hinder this kind of analysis. Additionally, because the research and development work associated with rare disease is so costly and the global number of patients so small, rare disease medications are typically very expensive – manufacturers rely on a relatively small volume of sales to recover their substantial investment costs and generate adequate funding to continue drug research and development.

While the Government of Canada's decision to allocate $500 million annually specifically for rare disease drugs will certainly help improve efforts to sustain the cost of drug development, policymakers must also pay special attention to drug authorization and reimbursement decisions.

Typically, new medications require robust data sets to demonstrate their effectiveness and safety, based on multiple phases of highly controlled clinical trials. This kind of work can be extremely challenging, if not nearly impossible, for drugs developed for patients with rare diseases, owing primarily to the relatively small number of patients (globally – not just in any given country). As a result, drug manufacturers that establish that a new therapy meets safety requirements may not have access to a broad enough pool of clinical trial participants to comprehensively demonstrate a drug's effectiveness or value for money, often making full authorization and reimbursement out of reach, despite promising evidence.

Efforts to improve drug authorization and reimbursement would be greatly improved if policymakers embraced a more flexible provisional authorization process involving extensive real-world (RWE) evidence gathering. An authorization and reimbursement approval framework that suits the unique and complex circumstances of specialty medications for rare disease could involve a provisional approval process, contingent on the validation of real-world evidence once the drug is in circulation, as well as a temporary reimbursement approval until the evidence determines whether sufficient 'value for money' exists to determine whether to permanently reimburse the drug. This is also an area where PSPs can play a critical role, by ensuring the systematic collection of patient data that can be used to inform decisions about permanent authorization and reimbursement.

Recommendation 3: Improving rare disease drug authorization and reimbursement

McKesson Canada strongly encourages the Government of Canada to adopt flexible drug approval models for rare disease drugs, including via granting provisional approvals with permanent decisions being conditional on the gathering of real-world evidence. It further encourages the Government of Canada to fund provisional reimbursement by public and private drug plans until such time as adequate evidence exists to make a comprehensive reimbursement decision.

While provisional drug authorization and reimbursement will help encourage manufacturers to launch new medications in Canada, it is important to assess the broader life sciences policy context to determine whether it suits the needs of Canada's rare disease patient community.

The federal government's ongoing efforts to revise the guidelines detailing the work of the Patented Medicine Prices Review Board (PMPRB) are likely to have a strong, negative impact on access to rare disease drugs in the future. As the Canadian Organization for Rare Disorders noted in its submission to the PMPRB consultation, manufacturers of many rare disease treatments will have to reduce their list prices by 50% to comply with the new guidelines, making the Canadian market much less attractive to global drug developers.[3] These reforms will exacerbate the existing challenges related to the attractiveness of the Canadian market to manufacturers looking to launch new drugs and may cause manufacturers of existing rare disease drugs to withdraw from Canada altogether. In short, the PMPRB reforms might wind up undoing the benefit created by the government's planned annual $500 million allocation entirely.

Recommendation 4: Reviewing the draft PMPRB guidelines

McKesson Canada recommends that the federal government review the PMPRB guidelines to ensure they do not impede the objectives of this rare disease strategy, and instead focus its efforts on expanded reimbursement criteria.

[3] http://www.raredisorders.ca/content/uploads/CORD-comments-on-PMPRB-June-2020-guidelines-Aug-3-20.pdf

Federal-provincial considerations in developing a rare disease strategy

Both the federal and provincial governments play critical roles in establishing and implementing healthcare policy in Canada. The federal government provides core funding to the provinces via the Canada Health Transfer; it also manages drug authorization and pricing organizations. The provincial governments enact healthcare policy, including establishing rules and regulations that determine access to medication and healthcare services, as well as policies that establish funding for pharmacy services and pharmaceutical distribution. Both levels of government are involved in managing prescription drug insurance plans.

In developing a strategy for drugs for rare diseases, it is expected that decisions made at one level of government will directly and indirectly impact the others. For example, federal decisions to increase the throughput of rare disease drug authorizations could lead to increased drug reimbursement costs at the provincial level. Expanded provincial funding for rare disease diagnosis could make Canada a more attractive market to global drug manufacturers, creating pressure at the federal level to authorize more new medications.

As the rare disease drug strategy forms an important component of the Government of Canada's pharmacare initiative, it presents an excellent opportunity for both levels of government to work collaboratively in a manner that reflects the federal government's significant investment in healthcare as well as the provincial governments' policymaking authority.

Given that rare disease populations can cluster geographically, it is valuable to apply a flexible funding framework to ensure that policies are most effective, and that new funding builds on existing drug expenditures.

Recommendation 5: Federal-provincial cooperation

McKesson Canada recommends that the federal government work collaboratively with the provincial/territorial governments to determine how to best invest new resources to complement the existing reality in each jurisdiction. This would involve assessing where public and private drug plans do not adequately meet the needs of rare disease patients on a province-by-province basis. It would also include federal-provincial agreement on new rare disease drug schedules within existing public drug plans to identify those drugs that qualify for federal patient support, distribution, and pharmacy service funding.

Conclusion

Rare diseases present a significant and complex challenge to Canada's healthcare system. They are often not easily identified (and thus often diagnosed relatively late) and can be fatal. Moreover, they disproportionately affect infants and children. The Government of Canada's decision to allocate the first funds budgeted in the context of 'national pharmacare' to a strategy for rare disease drugs speaks to the urgent need for well thought-out action plans to support patients and families afflicted with rare diseases.

It is expected, rightly so, that the large majority of the annual $500 million allocation for the rare disease strategy (beginning in 2022-23) will cover reimbursement expenses for relevant drugs, which typically cost six figures annually. Of course, many of these drugs are transformational, even lifesaving. Though they are often described as expensive, their value can be even greater – accounting for the many benefits associated with a life fully lived instead of one cut short soon after birth is a nearly impossible task.

Because rare disease medications are often specialty drugs, they require careful and complex transportation, handling, and administration expertise – given their cost, significant attention must be paid to ensure minimal waste and maximal patient adherence. Moreover, the nature of rare diseases is such that patients and families typically require comprehensive support to navigate the patient's illness and course of treatment.

McKesson Canada encourages the Government of Canada to continue its collaborative reflection on developing a strong, sustainable rare disease drug strategy. We urge the federal government to allocate a small but not insignificant portion of the annual funding envelope to ensure that the 'end to end' needs of patients and families are met, by financially supporting patient support programs, specialty drug distribution, and specialty pharmacy services.

We also encourage the Government of Canada to apply the lessons learned from this exercise to broader pharmaceutical sector policymaking, first by reconsidering the likely impact of the pending patented medicines pricing reform, and second by working collaboratively with stakeholders and provincial governments in advancing future pharmacare initiatives.

About McKesson Canada

Our vision is to improve care in every setting — one product, one partner, one patient at a time.

We partner with hospitals, physicians, pharmacies, nurses, biopharmaceutical manufacturers and others across the spectrum of care to build healthier communities. By helping our partners be as successful as possible, we work together to improve patients' lives.

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