March 26, 2021

Submission: National Strategy for High-Cost Drugs for Rare Diseases

Introduction

The Best Medicines Coalition (BMC) is a national alliance of patient organizations, together representing millions of patients, with a shared goal of equitable, timely and consistent access to safe and effective medicines that improve patient outcomes. The BMC strives to ensure that patients have a voice and are meaningful participants in health policy development, specifically regarding pharmaceutical care.

We commend the government as it takes important steps forward to address access to drugs for rare diseases. As part of our efforts on behalf of patients, we welcome this opportunity to comment on *Building a National Strategy for High-Cost Drugs for Rare Diseases***.** The BMC and many of its member organizations have also participated in Health Canada's various consultative meetings.

We support a broad approach to a Canadian rare disease strategy, and to that end, **the Best Medicines Coalition supports the development of a comprehensive policy that first and foremost focusses on improving, saving, and transforming patients' lives.** Addressing cost issues is an important element of a strategy but not the foundation of the strategy. For patients and caregivers, the journey starts before diagnosis, and comprises many different stages, from diagnosis through to management and gaining access to necessary treatments, as well as many additional supports and services. This strategy must reflect this reality and have at its heart a mandate of helping patients and seek to address health system sustainability from this starting point.

This submission was developed with the participation of BMC member organizations, specifically those who are part of the BMC's Rare Disease Strategy Working Group and other members who have an interest in this topic. Statements and positions expressed within this submission reflect areas of consensus among BMC member organizations.

BMC Key Principles & Considerations:

- Build holistic strategy around patient needs and access, beyond narrow cost focus
- Provide foundational clarity to inform way forward
- Improve patient outcomes through early diagnosis and timely access
- Establish specialized pathways to facilitate improved care
- Build added value through patient and clinician engagement
- Build real-world evidence through research support, integration, and data sharing
- Dedicate sufficient financial support to ensure meaningful and sustained gains

BMC Recommended Immediate Next Steps:

- Expand scope of Health Canada strategy around improving patient health outcomes and quality of life, beyond only addressing the high cost of drugs for rare diseases
- Reframe rare disease strategy around overarching key goals, based on patient-driven principles and best practices in other jurisdictions
- Engage a formal working group, with all stakeholders, and involve patients with lived experience

BMC Key Principles & Considerations

1. Build holistic strategy around patient needs and access, beyond narrow cost focus

Canada needs a patient-focussed and comprehensive strategy for rare diseases which goes beyond the narrow yet significant issue of high-cost drugs to encompass all aspects of the patient experience and is built on critical patient values and needs. Within a broader rare disease strategy, access to life saving and transformative drugs is a high priority, and the strategy and all related policies and programs must enshrine this goal. As a basic premise, all patients in Canada, regardless of where they live, or their ability to pay, have a right to access necessary medications, and this principle must apply to all policies, programs and delivery mechanisms. Just as broad universal health care and national pharmacare must be comprehensive, and inclusive of all, including for example clinical trials, early screening, diagnosis, clinical interventions and care, Canada's rare disease strategy must be focussed on patient access, on ensuring that Canadians living with a rare disease have coverage and are able to receive the care and drugs they need.

Considerations ahead:

- Rare disease strategy needs to be reframed by the federal government to encompass a broader, holistic perspective on patient needs and stated goals must reflect this. The federal government should align with rare disease approaches adopted in other jurisdictions, especially those that have proven successful in supporting timely access to promising therapies delivering care and meeting patient needs, as effectively as possible.
- Canada's rare disease strategy must be aligned with goals and implementation plans for broader national pharmacare, especially regarding the core premise of providing equitable, consistent, and comprehensive access to medications.
- As a first step, national standards on coverage for medications for rare diseases should be established that hold both private and provincial public drug benefit programs accountable to pan-Canadian expectations on coverage to ensure equitable access to medications regardless of where a patient lives. Achieving medication access equity will require substantial additional federal funding and provincial/territorial involvement to meet national standards.

2. Provide foundational clarity to inform way forward

Clarity of scope and purpose is necessary as Canada embarks on a new phase in addressing the needs of patients with rare disorders. Specifically, while challenging, development and adoption of a single definition of rare disease, based on transparent and objective criteria, is critical. Furthermore, there must be clarity on scope, such as intentions regarding inclusion of specific conditions such as the realm of more common conditions that have subsets of rare variants. Improving patients' health outcomes and quality of life must be the main purpose of a rare disease strategy.

Considerations ahead:

- In building clarity, international rare disease models should be reviewed for best practices to inform made-in-Canada definitions and frameworks. Importantly, a definition of rare disease and formalized outlines of scope and breadth of a rare disease strategy must be developed in consultation with all stakeholders, including patients and the organizations that represent them. The definition of rare disease used in the national strategy should also be used throughout the patient access pathway to provide consistency and reduce duplication or confusion from applying different thresholds and policies.

- Further understanding is necessary regarding criteria for scope of inclusion of specific conditions and/or drugs to treat those conditions, specifically regarding rare subsets such as various cancers. Coverage is needed for both rare cancers that occur minimally in the population, as well as rare disorders that occur in more common cancers. This must be explored and addressed in consultation with impacted patient communities.

3. Improve patient outcomes through early diagnosis and timely access

An effective rare disease strategy must address all areas of the patient experience, beginning with timely diagnosis at the earliest stages of life, such as through universal newborn screening, and by ensuring that necessary care and interventions including drugs are accessed as soon as possible to improve outcomes. In addition, following the lead of international jurisdictions, Canada needs to establish early access pathways for rare and ultra-rare therapies to allow patients to access a necessary drug before pricing negotiations are completed. This shift to put patients first would represent a significant change from the status quo in Canada.

Considerations ahead:

- Pathways need to include clear timelines and detailed steps leading to either product delisting and/or patient grandparenting and mitigate the risk of patients with a rare disease losing access to a treatment prescribed through an exceptional access program if pricing and access negotiations fail.
- Early access pathways provide opportunities for real-world evidence collection, contributing to long-term reimbursement considerations. When a patient and clinician access a drug through any early access program, and at the end of the negotiation process a drug is not listed for whatever reasons, a process should be in place to allow that patient to continue using that drug until the patient and clinician decide otherwise (based on best practices in other jurisdictions).
- While all provincial and territorial governments have programs for newborn screening, there is currently no pan-Canadian system or standard. Several organizations have recommended the creation of a pan-Canadian system for national newborn screening, including national standards and protocols, and this approach should be pursued by Canada's political leadership. Especially when a drug is available, all efforts should be made to expedite diagnosis so that all patients who may benefit are identified promptly when a drug can deliver the greatest benefit. For example, Spinal Muscular Atrophy is often a lethal disease diagnosable at birth with three therapies, but only one province screens newborns so far.

4. Establish specialized pathways to facilitate improved care

Rare disease drugs are unique, given the patient population they treat, and so specialized pathways for the approval, assessment, pricing, and reimbursement are warranted, again following the lead of international best practices. Current pathways produce wait times for patient access which leave much room for improvement and fall short of best practices. The effectiveness of improved pathways is central to the success of Canada's rare disease strategy and its ability to facilitate the provision of necessary medications to patients in a timely, efficient and effective manner. Importantly, these processes must be transparent, draw on available expertise in and outside of Canada, and allow patients to directly engage, provide evidence and appeal reimbursement decisions and recommendations on a case-by-case basis. This is an extremely important element given the unique nature of rare conditions.

Considerations ahead:

- The current health technology assessment process in Canada, as provided through the Canadian Agency for Drugs and Technologies in Health (CADTH), is not appropriate for rare disease drugs which require a differential approach. For example, there is not full transparency on the deliberations including whether experts on a condition have been consulted. Specialized approaches to dealing with conflicts of interest are necessary in situations with extremely few clinical experts. In addition, the current review process puts significant weight on phase three double-blind randomized clinical trials, which are often impractical or unethical assessments for rare disease drugs given the small patient sample size. The current CADTH practice of such weight to evidence of phase three clinical trials is fundamentally unfair to rare disease patients.

Build added value through patient and clinician engagement

Building on the concept of specialized processes (above), pathways for rare disease drugs must include ways to ensure that patients and clinicians are directly engaged in all aspects of the approval, reimbursement, and pricing processes. Importantly, opportunities for patients and clinicians to review and rebut submissions and appeal decisions on recommendations related to reimbursement must be established, clarified, and broadly communicated, including within health technology assessment processes at CADTH and through price negotiations at the pCPA, for example.

Considerations ahead:

- Frameworks in other jurisdictions, such as the Scottish Medicines Consortium, should be carefully reviewed to design an improved process suitable for Canada. For example, opportunities to observe the deliberations of committees making recommendations or decisions on patient access to a drug and to provide direct oral testimony of lived experience have merit and would add value in the Canadian realm. If health technology assessment is included within the mandate of the proposed Canadian Drug Agency (CDA), it will be important for the federal government to ensure that the agency operates in a more open and transparent manner than the constellation of agencies and other organizations that currently determine drug access in Canada. This could be accomplished by enabling the agency through an Act of Parliament so that it is held to the same transparency standards as other entities.

5. Build real-world evidence through research support, integration, and data sharing

An effective rare disease strategy must address the current data gap to ensure assessment and reviews are appropriate given the unique nature of rare conditions. While early access pathways are integral to gathering greater real-world evidence, frameworks must also be established to ensure patient-driven data is collected and shared in a coordinated fashion, addressing the current lack of robust data and coordination within the health care and research community. A core focus must be to provide long-term support that is necessary to build and sustain a stronger research community focused on rare diseases and conditions.

Considerations ahead:

- Centres for collaboration and excellence can play an important role in moving towards improved data collection and sharing and better patient outcomes. For example, a hub for research on all types of rare disease (e.g., genetic and non-genetic) through the Canadian Institutes of Health Research or perhaps centres connected to academic healthcare centres would have merit.
- Canada should also develop a national registry for rare diseases and conditions building on current effective models, such as the Canadian Cystic Fibrosis Registry and the Canadian Neuromuscular Disease Registry. In some cases, there may be merit in building on global registries.

- Every new drug or indication for a rare disease approved by Health Canada should be required to participate in a patient registry with public access and accountability to collect real-world data.
- Given real-world evidence and limited data challenges, Canada should pursue increased utilization of joint and non-Canadian reviews to help inform Canadian decisions.

6. Dedicate sufficient financial support to ensure meaningful and sustained gains

A significant funding commitment by the federal government and ongoing federal support is necessary to create and deliver a Canadian rare disease strategy that will improve patient care in a meaningful and sustainable manner. While the current attention on rare diseases and funding allocated so far is welcome, implementing the strategy and ensuring dedicated ongoing funding are critical to ensure those with rare conditions get the medications they need. An effective rare disease strategy must be sufficiently resourced, and all efforts must be focussed on understanding the scope of patients' and caregivers' needs, the full extent of necessary financial support and then allocating funds as appropriate, all in partnership and consultation with patients and other stakeholders.

Considerations ahead:

- Initial investments could be applied to establishing new processes, based on principles and goals as outlined above and agreed upon by all stakeholders, with efforts put towards developing long-term investments to ongoing needs including access to drugs for rare diseases. However, longer term there must be an appropriate balance between building infrastructure and specifically providing access to medications, as this remains an urgent priority.
- The government should commit to reviewing the strategy every three to five years in partnership with patients, caregivers, clinicians, researchers and other stakeholders, providing opportunities for regular input before any changes are considered or made, a practice used in other jurisdictions that have effective rare disease strategies.

The Best Medicines Coalition Recommends these Immediate Next Steps:

1. Expand scope of Health Canada strategy around improving patient health outcomes and quality of life, beyond only addressing the high cost of drugs for rare diseases

- The current focus of the strategy solely around the high cost of drugs for rare diseases is narrow and does not adequately address the entirety of patient needs and broad goals of improved experiences and outcomes. A strategy needs to be focussed on the patient's challenges, experiences, values and needs, while creating a system that is transparent, accountable and accessible to patients and caregivers. While managing costs can be a desired outcome, it must not be the main driver. Improving patient outcomes and quality of life as defined by patients must be at the core of any strategy for it to truly meet the needs of people for health care.
- As an immediate next step, the federal government should confirm that the intended scope of the strategy is to address rare disease issues broadly and shift focus to improving patient outcomes based on the principles we have outlined in this submission.

2. Reframe rare disease strategy around overarching key goals, based on patient-driven principles and best practices in other jurisdictions

- The discussion paper released by Health Canada lacks clarity on the specific goals that the government intends to achieve. Further, there is not evidence that it is strongly informed by perspectives on goals as expressed by patients and caregivers and that it draws on best practices demonstrated in other jurisdictions.
- The next stage of this strategy development process and consultation must be informed by scoping of specific goals as informed by work to date, and ongoing outreach to the patient communities and other stakeholders.

3. Engage a formal working group, with all stakeholders, and involve patients with lived experience

- While the discussion paper provides a starting point and a broad outreach process has been undertaken, meaningful ongoing consultation and co-design with patients must continue and form the basis of all the next steps.
- Following the closure of this consultation phase, the federal government should create a formal working group to map out the key changes that need to be made to develop a robust rare disease strategy. It is imperative that this body include a role for patients living with rare diseases, including those with subsets of rare variants of more common diseases, and be modelled on best practices such as how the government engaged with patients during the Cannabis legalization process.
- In addition, all partners must be engaged in consultations moving forward. For example, a strategy for rare diseases needs to be developed in partnership with payers, including the provinces and territories as they have the constitutional responsibility for the funding and delivery of health care for most Canadians. Likewise, private coverage stakeholders must be engaged moving forward.

About the Best Medicines Coalition

The Best Medicines Coalition is a national alliance of patient organizations, together representing millions of patients, with a shared goal of equitable, timely and consistent access for all Canadians to safe and effective medicines that improve patient outcomes. The BMC's areas of interest include drug approval, assessment, and reimbursement, as well as patient safety and supply issues. As an important aspect of its work, the BMC strives to ensure that Canadian patients have a voice and are meaningful participants in health policy development, specifically regarding pharmaceutical care. The BMC's core activities involve issue education, consensus building, and advocacy, making certain that patient-driven positions are communicated to decision makers and other stakeholders. The BMC was formed in 2002 as a grassroots alliance of patient advocates. In 2012, the BMC was registered under the federal Not-for-profit Corporations Act.

Alliance for Access to Psychiatric Medications
Arthritis Consumer Experts
Asthma Canada
Brain Tumour Foundation of Canada
Canadian Arthritis Patient Alliance
Canadian Association of Psoriasis Patients
Canadian Breast Cancer Network
Canadian Cancer Survivor Network
Canadian Council of the Blind
Canadian Cystic Fibrosis Treatment Society
Canadian Epilepsy Alliance
Canadian Hemophilia Society
Canadian PKU & Allied Disorders
Canadian Psoriasis Network
Canadian Spondylitis Association
CanCertainty
Crohn's and Colitis Canada
Cystic Fibrosis Canada
Fighting Blindness Canada
Health Coalition of Alberta
Huntington Society of Canada
Kidney Cancer Canada
Lymphoma Canada
Medical Cannabis Canada
Medicines Access Coalition – BC
Millions Missing Canada
Ovarian Cancer Canada
Parkinson Canada