August 15, 2022

Submission: National Strategy for Drugs for Rare Diseases

Introduction

The Best Medicines Coalition (BMC) is a national alliance of patient organizations which seeks timely access to a comprehensive range of medically necessary, safe, and effective drugs and other treatments, informed by patient-driven evidence and values, and delivered equitably and affordably to all patients in Canada. The BMC strives to ensure that patients have a voice and are meaningful participants in health policy development, specifically regarding pharmaceutical care.

We welcome this opportunity to provide an additional input on the proposed National Strategy for Drugs for Rare Diseases, complementing an initial submission provided in March 2021 along with input provided by the BMC and its member organizations at consultative sessions.

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.

Summary: Core positions and recommendations

1. ***Principles and pillars:*** Entrenching fit-for-purpose and patient benefit
2. ***Clarity:*** Charting a clear path forward
3. ***Streamlined processes:*** Efficient and timely patient care
4. ***Data and evidence:*** Informing improved care and outcomes
5. ***Collaboration and transparency:*** Meaningful engagement to inform policy
6. ***Comprehensiveness and exceptionality:*** Ensuring no patient is left behind
7. ***Funding and framework:*** Adequate resourcing with appropriate governance

Key considerations and discussion

1. Principles and pillars: Entrenching fit-for-purpose and patient benefit

- A comprehensive and effective strategy must first and foremost focus on saving and transforming patients' lives. Gaining access to necessary treatments is critical and a national 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 of improving patient outcomes.

- While the principles and strategic pillars presented have merit, the central and fundamental concept of improved patient outcomes – saving and transforming the lives of patients – is not given the prominence necessary to truly guide the framework and its elements. The principles and pillars must strongly and clearly convey purpose and benefit to patients.

- Foundational work to define and describe rare disease and what drugs and other treatments are encompassed is necessary. Further definition and clarity will ultimately inform deliberations and decisions on who the strategy will serve, and which drugs and other treatments will ultimately be included. Treatment of rare diseases is complex, and the technologies used are not always easily classified as drugs, and this reality must be recognized and acknowledged in a definition. As an example, the recently released Quebec rare disease strategy includes a definition of rare disease, adopted from the European Union and the Institut national d'excellence en santé et en services sociaux (INESSS) definitions.

- An ethical decision-making framework is imperative to ensure processes and decisions on listing, treatment access, and evidence-gathering are fair, inclusive, and consider the welfare of a diverse range of people. As such, relevant language, such as compassion and benevolence, must be incorporated and support inclusive decision making.

2. Clarity: Charting a clear path forward

- While an important foundation, principles and broad pillars do not comprise a strategy. An effective strategy needs tangible content which will clearly delineate the path forward, including first and subsequent steps, policy and program implications and metrics for success or failure. For example, the Quebec rare disease strategy has strong and clear content, providing a model for the scope and level of detail necessary.

- Importantly, the strategy must be presented in terms of how it affects the lives of patients in practical terms, using language that is accessible and easily understood. Patients must be able to easily grasp whether and how this will impact their care – which patients and how each patient will access their treatments.

- The framework is short on details and overly reliant on high level principle-based statements. While examples are helpful, details are needed to understand proposed activities. For example, the concept of establishing an initial set of drugs for rare diseases needs to be expanded to answer questions like how will a list be developed, how will it be integrated into programs and who will be involved in the process? In addition, how will it be decided which treatments, and in turn diseases, take priority if not all are able to be funded? Will this amount to a new method for rationing access to care and treatment? The decision-making process should be clear from the start. Statements such as "Enhance coordination and shared decision-making around reimbursement decisions" need to be explained in more detail.

3. Streamlined processes: Efficient and timely patient care

- In Canada today, with few exceptions, it takes too long to get a new drug through market authorization, health technology assessment, collective price negotiations for government plans and then listings by individual jurisdictions/programs.

- Canada must effectively streamline regulation, reimbursement reviews and reimbursement processes. The experience with Trikafta, used in the treatment of cystic fibrosis, demonstrated that it is possible for a high value drug for a rare disease to be covered in all jurisdictions within eleven months of a Health Canada application. This was a concerted effort and demonstration of strong will by regulators, payors and all stakeholders, including patient community advocacy. Unfortunately, the Trikafta experience was one of the few exceptions. There are too many instances where drugs for rare diseases, as with other treatments, experience extended delays while patients with critical unmet needs wait.

- Processes like Health Canada's Priority Review and the aligned review process with the Canadian Agency for Drugs and Technologies in Health (CADTH) and the Institut national d'excellence en santé et en services sociaux (INESSS) are helpful in moving the dial, but we also need the pan-Canadian Pharmaceutical Alliance (pCPA) and private payers to work in step with these processes, and there is no formal requirement to do so currently. In the case of Trikafta, the manufacturer and the pCPA worked toward the Health Canada approval deadline: on the very same day that Health Canada approved the drug, the pCPA issued a statement to say that price negotiations were done at that level, and that Trikafta would be covered by Canada's public drug programs pending positive HTA recommendations. However, when the final HTA recommendations came, each jurisdiction had different processes in place to get from and Letter of Intent to a Product Listing Agreement, so some announced a week or two after the CADTH/INESSS recommendations, and some took months. This process needs to be consistent and timely in every jurisdiction. Regarding private payers, again in the case of Trikafta, decisions were announced after public payers, largely due to the need for individual company-based negotiations and processes. The private payers should be encouraged to pursue collective negotiations to streamline their timelines.

- Roles and responsibilities of all payers need to be defined and streamlined. It is unacceptable that one Canadian can get timely access in a certain jurisdiction while another waits months or years for access, if granted.

- System-wide, specialists, pharmacists, and other health care providers, and in turn patients, should promptly receive clear and concise information on how to access treatments once decisions are made.

4. Data and evidence: Informing improved care and outcomes

- The federal government has a role in compiling and leveraging currently available data, including that collected by patient groups, clinicians, researchers, and provincial/territorial governments. In addition, the federal government has a role in working with others to identify data gaps and then addressing gaps while avoiding duplication and overlap.

- Canada needs to more effectively use data collected through international bodies and partnerships given that rare populations are small. Likewise, there needs to be more effective utilization of real-world evidence, predictive precision medicine programs and other forms of evidence that our international comparator countries are using, most of which have been for some time. Canada needs to catch up in this regard. In addition, there is a federal role in helping develop and maintaining patient registries.

- In the rare disease space, and indeed across all conditions, data should be collected, compiled and analyzed regarding 'time to treatment' to be better able to evaluate performance, barriers and areas of improvement. Such a system would track and evaluate length of time from population screening or onset of signs and symptoms through accurate diagnosis, treatment decision, onset of treatment and fulfillment of treatment. It should highlight diagnostic delays and inaccurate diagnoses. It would also track and evaluate system performance in terms of time from when a drug or other treatment is filed with Health Canada, through review and decision-making processes including market authorization, health technology assessment, collective negotiations and individual jurisdictional product listings and, finally, until a patient is able to begin treatment. Patients experience significant wait times in accessing medically necessary treatments, especially those non-formulary or complex treatments that are sometimes provincially funded but inconsistently, yet this is not being tracked and analyzed to inform system performance, reform and improvements or patient health outcomes. Furthermore, a public-facing tracking web site would deliver an element of transparency which is lacking.

5. Collaboration and transparency: Meaningful engagement to inform policy

- The path forward in further developing the framework and its various elements will involve multiple layers. Collaboration and transparency are necessary and patients and the organizations that represent them must be involved at all tables and meaningfully engaged for effective partnership. For example, discussions about system sustainability considerations and funding roles are underway but patients are not involved, and consultations regarding the proposed Canadian Drug Agency have not been fulsome. Patient representatives need to be at these tables to understand and work together with others to shape the framework before it is fully developed if a "patient-centred" approach is truly valued. More co-development with patients is desirable and should be required.

- In addition to working with patients and the organizations that represent them to develop appropriate and meaningful collaborative and inclusive decision-making, Canada must look to international models for learning. Frameworks like the Scotland Medicines Consortium can be used as models of best practice. Important lessons can be learned from recent FDA hearings on COVID-19 vaccines and treatments where hearings were open to all, building transparency, accountability and understanding.

- Once a program is implemented, accountable feedback and reporting mechanisms must be clearly outlined, similar to hospital incident or adverse event reporting mechanisms, involving a range of players, including patients.

- Regarding advisory bodies, patient interests must be represented on any committee, working group or other advisory body associated with the planning, developing, implementing and overseeing all elements of the strategy. Fair processes for recruiting and retaining patient experts must be pursued and contributions of time recognized as with other experts. Examples include:
    - Inclusion, Diversity, Equity and Accessibility (IDEA) to ensure a range of groups and voices are heard, valued and represented.
    - Bioethics and justice to ensure principles of fairness and inclusivity are adhered to while balancing the needs of many.
    - Technology/treatment to monitor emerging treatment delivery mechanisms and ensure new treatments that are not easily classified as “drugs” are considered for review or are brought forward as needing alternative classification.
    - Building patient lived experiences into the body of evidence.

6. ***Comprehensiveness and exceptionality: Ensuring no patient is left behind***

- There needs to be a transparent and easily understood Exceptional Patient pathway so that any prescribing health care professional can apply to an independent, scientific committee for approval for use and payment of a drug or other therapeutic product not on a formulary or regarding an indication not on the Health Canada label or relevant formulary. Quebec has the only process of this type in Canada and it has proven effective. This process helps ensure no patient is left behind. In addition, there should be a dedicated ombudsman to address drug access barriers and concerns as expressed by patients.

- The framework ignores or does not clearly encompass non-drug therapies, which are the only therapies for many rare metabolic disorders - truly the orphans of the orphans. These treatments can be described as nutraceuticals, medical formulas or medical foods distinct from pharmaceuticals, and often require medical prescriptions and in most cases are paid for by Health Ministries as medically necessary therapeutic products.

- The framework as proposed discusses but does not clarify federal leadership to raise the bar on which conditions are subject to newborn screening (early diagnosis and early, required interventions for best outcomes) and left to the provinces produces the predictable scattergram of who screens for what, with Quebec lagging behind other provinces/territories.

7. Funding and framework: Adequate resourcing with appropriate governance

- An effective rare disease strategy must be sufficiently resourced, and efforts must focus on understanding the full extent of necessary financial support and then allocating funds as appropriate, in partnership and consultation with patients and other stakeholders. The current allocation of federal funding of $500 million a year is far short of what is needed. By comparison, the premiers are asking for federal health contribution increase from 22 to 35 percent which is $28 billion, or 56 times more than the $500 million. As such, $500 million represents massive underfunding in this context, and is looking at the unmet health needs for people with rare disorders through the wrong end of the telescope when it comes to federal contributions and investments.

- Just like every other developed country, including Russia, Canada needs an Orphan Drug Act, to embed patient rights, improve transparency and accountability, requiring faster processes and lower or no fees from drug developers along with better incentives to submit breakthrough therapies for untreatable rare disorders. Canada has much catching up to do to cut red tape and provide incentives comparable to other high-income countries for orphan drugs to meet unmet health needs of Canadians with rare disorders.

- Jurisdictions have indicated they want to enhance their existing access programs through this strategy but do not want to relinquish the autonomy of designing and delivering these programs. Some jurisdictions see this as a way to simply do more of what they are doing, and many of those things don't work well or well enough for people with rare diseases. This approach will not address the need for consistency and coherence across the country.

- It is also important to determine – and quickly – where this strategy will be housed and what the roles of each type of payers will be. The strategy must be practical and resourced -- it can't live on a shelf. It must have a sponsor and a commitment and should not be at risk of disappearing based on political whim. The strategy needs roots and must be resourced appropriately, not just for system sustainability but to meet the needs of Canadians who need drugs for rare diseases.

- Importantly, the rare disease strategy framework must integrate with other elements of Canada's systems for regulating, assessing, and accessing drugs. For example, it is unclear how the Drugs for Rare Disease Strategy fits within a broader strategy for rare diseases in Canada – to be on a par with the new Quebec Rare Disease Strategy – and how will it support the concept of national pharmacare. Regarding pricing regulation, identification as a drug for a rare disease should not provide carte blanche for high pricing and measures must be taken to address this. Regarding ensuring a safe and secure supply, the framework as proposed ignores the need for drug and vaccine development and manufacturing inside Canada and the need for investments to build capacities.

BEST MEDICINES COALITION
COALITION POUR DE MEILLEURS MÉDICAMENTS

About the Best Medicines Coalition

The Best Medicines Coalition (BMC) is a national alliance of patient organizations which seeks timely access to a comprehensive range of medically necessary, safe, and effective drugs and other treatments, informed by patient-driven evidence and values, and delivered equitably and affordably to all patients in Canada. With interests in drug approval and oversight, assessment, and reimbursement, as well as safety and supply issues, core activities include member issue education, consensus position development and advocacy. As an important aspect of its work, the BMC strives to ensure that patients and the organizations that represent them have a voice and are meaningful participants in health policy development, specifically regarding pharmaceutical care. The BMC was formed in 2002 as a grassroots alliance and in 2012 it was registered under the federal Not-for-profit Corporations Act, governed by a Board of Directors elected from member organizations.

Alliance for Access to Psychiatric Medications
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 Skin Patient Alliance
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
Migraine Canada
Millions Missing Canada
Ovarian Cancer Canada
Parkinson Canada