Coalition Priorité Cancer au Québec

SUBMISSION BY

THE QUEBEC CANCER COALITION

For the House of Commons Standing Committee on Health study on the final version of the Patented Medicine Prices Review Board Guidelines

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November 6, 2020

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Executive summary

This exercise is part of the mandate of the House of Commons Standing Committee on Health, which voted on October 26, 2020, to undertake a study on the final version of the Patented Medicine Prices Review Board (PMPRB) Guidelines released on October 23, 2020.

The Quebec Cancer Coalition is made up of than 60 member organizations and represents approximately 1.5 million people involved in combating cancer. The Coalition decided to join the discussion in order to bring to the table the voices of tens of thousands of Quebeckers diagnosed with cancer and their loved ones.

We support the federal government's overall goal of reducing drug prices in Canada by updating the PMPRB guidelines. However, a balance must be struck between acceptable drug prices, access to innovative drugs, and the possibility for patients to take part in clinical trials. In our view, the new guidelines recommended by the PMPRB do not currently meet these objectives.

First, the Quebec Cancer Coalition believes that the federal government must recognize the unique character of Quebec institutions, including the Institut national d'excellence en santé et services sociaux (INESSS). The INESSS plays a crucial role in drug approval, and its studies and recommendations ensure that the reality of Quebec's health care system is taken into account. Oncology organizations and Quebeckers have confidence in the rigorous modern methods and independence of the INESSS.

Second, we are already seeing a negative impact on the number of new drug launches since the PMPRB's initial reforms, and there is evidence that drug manufacturers are no longer investing in countries or jurisdictions where they do not intend to launch new drugs because of overly rigid and restrictive regulations. Quebec cancer patients must not become collateral victims of a risky revision of the PMPRB guidelines. The stakes are far too high to take that risk.

Third, we are concerned about cost-benefit being the only variable considered in the process and about innovative cancer drugs and drugs for rare conditions being held to the same standards as other more common types of drugs. We want an approach adapted to cancer, as is the case in assessments by the Canadian Agency for Drugs and Technologies in Health (CADTH) and the

INESSS. In our view, the approach should be flexible enough to recognize differences associated with precision drugs based on rare mutations, for example.

Fourth, the PMPRB's new guidelines, if adopted as is, will curtail cancer patients' access to innovative medicines and clinical trials. They will endanger the lives of patients like Élisabeth Delpy, a non-smoking Laurentians-area patient with advanced lung cancer who survived her cancer thanks to an innovative drug.

In light of the considerations in this brief, we ask the committee to recommend that the federal government review the proposed changes to the PMPRB and make a commitment to Quebeckers to strike a balance between lowering prices and ensuring access to new drugs, which is more in line with our Canadian values. Lastly, we request that the INESSS be formally consulted on the PMPRB's new guidelines.

About the Quebec Cancer Coalition

The Quebec Cancer Coalition was created in 2001 to **assist and support people affected by cancer (patients, survivors and caregivers) and give them a strong voice** in order to improve the cancer care system in Quebec. The Coalition now includes more than 60 organizations, including patient associations and community organizations, representing all types of cancer, stages of the disease and regions of Quebec.[1] It thus represents nearly 1.5 million people.

The Quebec Cancer Coalition continually surveys people directly or indirectly affected by cancer for its position statements and actions, both directly and through its member organizations and its committee of patients, survivors and caregivers, which leads it to meet with individuals and patient groups across Quebec.

Our priorities

For almost 20 years, the Quebec Cancer Coalition and its members have shared its vision of a healthier Quebec, focusing on patients, survivors and caregivers. Its numerous actions at the grassroots level have enabled it to formulate several groups of recommendations, each one addressing a major topic in the fight against cancer. Since then, many actions inspired by those recommendations have been undertaken to mobilize everyone involved in combating cancer in Quebec, to do more to beat cancer, and to improve the cancer care system.

More specifically, our organization's priorities for 2019-2021 are as follows:

1. Provide Quebec with an integrated long-term vision of cancer care
2. Promote value-based healthcare
3. Ensure regional equity in access to the best health care and services
4. Establish an efficient, up-to-date and accessible Quebec Cancer Registry for clinicians and researchers
5. Promote patient participation in clinical studies
6. Educate the public and decision-makers about personalized medicine issues and opportunities to make Quebec a role model
7. Improve access to leading-edge diagnostics and treatments

[1] https://coalitioncancer.com/en/members/

8. Make sure there is a top-tier human papillomavirus (HPV) vaccination and screening program
9. Improve the quality of and participation in colorectal cancer screening programs
10. Enhance support for people affected by cancer (patients, survivors and caregivers)

However, the Quebec Cancer Coalition is aware of the exceptional nature of the global COVID-19 crisis and has supported the community's efforts to counter the pandemic. It has surveyed cancer patients twice to find out how the pandemic is impacting their care and access to services. The survey reports and specific recommendations were submitted to the Quebec Department of Health and Social Services (MSSS) in a spirit of cooperation to provide the patient's perspective and to prevent further interruptions or delays in cancer treatments and operations during the second wave of the pandemic.

Preamble

In May 2017, Health Canada proposed an update to various aspects of the Patented Medicine Prices Review Board (PMPRB) regulations that govern patented medicines in Canada. Those changes incorporated new factors that would determine whether a drug is or was sold at an "excessive" price.

Since the changes were first proposed to the PMPRB in 2017, a number of organizations representing patients, including the Quebec Cancer Coalition, have expressed concern that the changes will make it more difficult to access innovative new drugs and clinical trials.

Indeed, according to a pharmacoeconomic analysis commissioned by the Collective Oncology Network for Exchange, Cancer Care Innovation, Treatment Access & Education (CONECTed), the PMPRB's new regulations will require patented medicine pharmaceutical companies to *significantly* reduce their prices.[2] According to their estimates, for a number of precision treatments, price

[2] Submission by CONECTed to the PMPRB, February 2020: https://www.canada.ca/content/dam/pmprb-cepmb/documents/consultations/draft-guidelines/submission-received/2020-02-Guideline-Consultation-Submission-Oncology-Groups-CONECTed.pdf. Accessed November 5, 2020.

reductions of 70% to 88% would be possible, including reductions that could potentially go beyond that range. This would make Canada unique among OECD countries and make the Canadian market much less attractive for clinical trials and innovative drugs, including precision medicine, which can alter the course of devastating diseases such as cancer.

The PMPRB and the federal government have repeatedly assured Canadian patient groups that access to new drugs and clinical trials will not be affected by the proposed regulatory changes. In our opinion, the promises made by the federal government and the PMPRB are not good enough. On the contrary, the Quebec Cancer Coalition believes that patients deserve solid fact-based guarantees. How can the federal government and the PMPRB ensure access and accurately anticipate how companies will respond to rules that will limit innovation, research and the rapid introduction of new innovative medicines? As representatives of directly concerned patients, we are unfortunately already seeing the fact that new drugs such as Selinexor[3] are not only being delayed but may simply never be marketed in Canada.

In October 2020, we and other patient groups across Canada formed a front-line group to represent the needs of patients suffering from various diseases. All stakeholders in the "Protect Our Access" campaign are working together to raise their concerns about the PMPRB's new draft guidelines.

The campaign's aim is to educate governments and the public about the importance of protecting and ensuring consistent and adequate access to innovative medicines for patients and finding the right balance between reducing costs and ensuring that Canadians continue to have access to promising new medicines.

On October 26, 2020, the House of Commons Standing Committee on Health agreed to undertake a study of the final version of the PMPRB Guidelines, which were published on October 23, 2020. That study, in which we are participating through this brief, is certainly a step in the right direction to address the very legitimate and well-founded concerns of patient groups.

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[3] U.S. Food and Drug Administration: FDA grants accelerated approval to Selinexor for multiple myeloma. https://www.fda.gov/drugs/resources-information-approved-drugs/fda-grants-accelerated-approval-selinexor-multiple-myeloma. Accessed November 5, 2020.

About cancer

According to the latest estimates, more than 22,000 people will die of cancer in Quebec this year, and nearly 60,000 new cases will be diagnosed.[4] Fortunately, even though we are still unable to measure them in real time in Quebec, because there is no proper cancer registry, cancer survival rates are gradually increasing thanks to new drugs and medical practices. Cancer statistics certainly warrant closer attention:

- Cancer is the leading cause of death in Canada.
- It is estimated that one out of two Canadians will be diagnosed with cancer during their lifetime.
- One in four, or 821,000 Canadians, will die.

This is a huge public health problem that requires a specific policy approach, as governments have recognized, since cancer is now considered a chronic disease. It is important for the committee to know that there is not just one type of cancer, one stage of cancer, or one cause of cancer:

- There are cancers that are rare and others that are more common, but with precision medicine and genomic testing, we now understand that even within one type of cancer, there are several subgroups and mutations that cause cancer.
- For some cancers, research has uncovered genetic links that can be used in prevention and treatment; for other types of cancer, there are no such links.
- Some cancers can be cured, while others have a very low survival rate.
- Some cancers have been turned into chronic diseases thanks to newer and more effective treatments, but there are still many people who die within months of diagnosis.

Cancer is a disease associated with an aging population. The advent of new treatments, particularly personalized care (based on genetic profiling) that is

[4] Brenner DR, Weir HK, Demers AA, Ellison LF, Louzado C, Shaw A, Turner D, Woods RR, Smith LM. Projected estimates of cancer in Canada in 2020. CMAJ 2020;192:E199-205.

more accurate and effective, and the development of new professional practices will generate significant changes for the health care system.

Access to and availability of new drugs or the possibility for patients to participate in clinical trials in order to market drugs that meet their needs more effectively is an attractive avenue both for the government and for individuals who have cancer, whether it is a rare form of cancer or better known to the public.

With this in mind, the Quebec Cancer Coalition wants to ensure that fighting cancer remains a priority in the establishment of new PMPRB guidelines, as cancer runs the gamut from very rare conditions to more common afflictions. The accessibility of innovative medicines and clinical trials that can save the lives of people affected by cancer is central to our concerns. **For us, cancer is not just another disease. Its multiple facets and components warrant specific treatment by the PMPRB, just as the CADTH and the INESSS are doing.**

One person's story

We firmly believe that patients have the right to access the most innovative drugs available and to participate in clinical trials that can lead to the development of better and more effective drugs to fight cancer. Many Quebeckers are still alive thanks to drugs. This is true of Élisabeth Delpy, who by taking Tagrisso was able to stabilize her cancer and survive.

Ms. Delpy's story begins in late 2018, when she and her partner, Kelvin Arroyo, had just retired. In December, Ms. Delpy was diagnosed with stage 4 lung cancer, even though she never smoked in her life. After the diagnosis, she and her partner were offered the opportunity to start aggressive chemotherapy or begin taking medications, some of which were described as innovative. They were offered two medications: Tagrisso, a high-performance, cutting-edge drug that specifically attacks the mutation affecting Ms. Delpy, or an older, less expensive predecessor that has significant side effects (major skin problems, chest pain, stomach pain and diarrhea). The couple chose Tagrisso to avoid disrupting their quality of life.

Within the first week, Ms. Delpy was feeling better. Within a month, tumour markers - substances that indicate the presence of cancer in the body - had

fallen sharply. She had an appetite and felt energetic. The most significant benefit of this innovative drug is that it prevented the proliferation of metastases in the brain. Although she had some very mild side effects, her quality of life was not affected by taking a drug for such an advanced cancer.

According to her husband, Tagrisso helped stabilize her condition and, more than a year later, allowed her to begin milder chemotherapy, better suited to her situation, and it opened the door to even more effective research protocols in the field. Ms. Delpy's doctor believes that without Tagrisso, she would not have been able to tell him how good she was feeling at a recent routine appointment, because she would simply not be alive! The physician was also excited about being able to monitor patients who were taking part in innovative clinical trials that may in turn help to save lives. However, the next step is causing anxiety for the couple, as they are uncertain whether the drug Ms. Delpy will need after chemotherapy will be available on the Canadian market under the new guidelines proposed by the PMPRB, and whether they will have to travel to the United States to continue treatment. This will result in astronomical costs and involves the patient being treated in English, far from home.

Mr. Arroyo would like people to learn from his partner's case, which clearly demonstrates that access to innovative medicines can save lives and make a real difference. He believes that innovative and precision medicines should be looked at differently from standard cancer drugs. Tagrisso gave his wife excellent quality of life, and that has had a tremendous impact on the way she approaches her recovery.

However, according to the CONECTed's estimates based on six case studies, if Tagrisso were subject to the PMPRB's new rules, its public price on the international market would have been reduced by about 91%. Under such conditions, it would be highly unlikely that the manufacturer would decide to submit its drug for evaluation by Health Canada and marketing in this country. The big losers in this situation would have been Ms. Delpy's partner and relatives, as she would not have had access to the drug that saved her life.

Lastly, it is worth noting that with the use of this innovative drug, the hospitalization usually associated with chemotherapy was unnecessary, and the quality of life of the patient and her family and friends was left intact. The result

was a considerable saving in the use of human and physical resources for Quebec's health system.

Our concerns about the proposed new directives

We are pleased to be able to participate in the process initiated by the House of Commons Standing Committee on Health on this issue, which is central to our concerns. We are very much in sync with the Government of Canada's intention to modernize the PMPRB's guidelines and improve the list of comparator countries for drug pricing. It makes sense to us that the federal government would want to make drugs available to Canadians at the best possible price. **However, a balance must be struck between an acceptable price for medicines and access to innovative medicines, including the possibility for patients to take part in clinical trials. In our view, the new guidelines recommended by the PMPRB do not currently meet this last objective.**

Concern 1: Quebec health care institutions are not being consulted in the regulatory pricing process

On January 19, 2011, the Government of Quebec created the Institut national d'excellence en santé et services sociaux (INESSS). The INESSS has a mandate to evaluate drug submissions with a view to making recommendations to the Minister of Health and Social Services. In carrying out its mandate, the INESSS considers five criteria: the therapeutic value of a drug, the appropriateness of its price, the relationship between cost and effectiveness, the impact on the public health and the other components of the health and social services system, and the appropriateness of approving a drug as regards the purpose of the general drug insurance plan. The reform proposed by the PMPRB will weaken or duplicate the mission of the INESSS. We would like to reiterate how much confidence we have in the INESSS to understand the realities of Quebeckers with cancer. Since its inception, the INESSS has demonstrated the adaptability necessary to ensure that the best cancer treatments and therapies are made available to Quebec patients.

It is important for the Quebec Cancer Coalition, and no doubt for all Quebec patient groups, that the regulatory changes proposed under the PMPRB

recognize the specific and essential role of the INESSS. **For this reason, we believe that the INESSS should be formally consulted by the PMPRB and included in the development and application of the guidelines.** The INESSS has specific knowledge of Quebec's ecosystem that will certainly be of value in the committee's deliberations.

Just as the INESSS has a committee on oncology practice development and the CADTH has the pan-Canadian Oncology Drug Review Expert Review Committee (pERC), we would like confirmation that a panel of oncology experts will be part of the PMPRB's evaluation processes. The Coalition's members value a multidimensional approach to evaluations that look beyond pharmacoeconomic values to include therapeutic value, impact and unmet patient needs.

Concern 2: The impact on patients of drug manufacturers' disinvestment in the Canadian market due to an overly restrictive regulatory framework

The Quebec Cancer Coalition also has serious concerns about the possibility that drug manufacturers will no longer invest in Canada, as is already the case in many countries and jurisdictions where they do not intend to launch new medicines. This could mean that Quebec patients will have access to fewer clinical trials and fewer treatment options when old medications no longer work. Physicians, especially hemato-oncologists, want to be able to offer their patients the most innovative treatments. **However, we are already seeing a decrease in clinical trial approvals since the publication of the proposed regulatory amendments.**

With so much uncertainty about the actual implementation of the reforms proposed by the PMPRB, patients are understandably worried. If Quebeckers do not have access to the new drugs available in other countries, those who can afford them will have little choice but to leave the country to access those vital drugs or to seek ways to pay for them out of their own pocket. Those who cannot afford them will be left behind. Quebec patients deserve and have the right to have a system that allows them access to clinical trials and new life-saving drugs.

We expect pharmaceutical companies to market their products at a reasonable price for patients. Similarly, we expect the Canadian government to ensure that the regulatory environment in Canada does not unnecessarily limit access to new therapies, drugs and treatments for those who need them.

Concern 3: Cancer drugs will be evaluated against the same standards as other drugs

We believe that negotiating the best price for patients through a transparent process, while keeping the actual price secret from the general public (while giving key decision-makers access to it), would be the best way to ensure access to innovative medicines and promote their launch in Canada. We are concerned, however, that cancer drugs will be subject to the same evaluation frameworks as other types of drugs, which have very different considerations. **It is clear to us that the approach advocated must be flexible enough and sophisticated enough to recognize the differences between rare and common cancers, genetic and genomic factors, unmet needs, childhood versus adult cancers, co-morbidities, Indigenous populations and the social determinants of health, to name a few.**

We are also concerned that the cost-benefit ratio alone will be taken into account in drug pricing, which we believe is too restrictive and dangerous, especially in the case of rarer conditions. Indeed, the cost-benefit formula ignores factors that are very important to the discussion, such as the unmet needs of patients or conditions attributable to different cancer types and subtypes. As an organization representing patients with different types of cancer and varying conditions, we expect a process that is **accessible to all, transparent, impartial and evidence-based.**

Concern 4: Cancer patients' access to life-saving innovative medicines is being compromised

Thousands of Quebeckers are eagerly awaiting the discovery of a new drug that could save their lives or curb the adverse effects of their cancer. There is no doubt that the many characteristics of cancers call for a flexible, understandable approach to the pricing of cancer drugs. **A single variable for Category 1 drugs, especially if it is primarily economic, cannot meet the needs of all patients with rare (and even less rare) cancers and related conditions.**

Accessibility must remain a key pillar of the PMPRB's new guidelines, specifically for cancer, the most common chronic disease in Quebec and Canada.

Conclusion

Ensuring access to innovative drugs and new clinical trials for Quebeckers who have cancer is one of our organization's primary concerns. We wish to draw the committee's attention to the legitimate concerns of our members regarding the PMPRB's new guidelines. We would like to reiterate once again our support for the modernization exercise to ensure a better price for all Canadians, while keeping system sustainable.

We would also like to mention that for the Quebec Cancer Coalition, Quebec's health institutions, specifically the INESSS, must be formally included in the regulatory process for setting drug prices. Their expertise and knowledge of Quebec's health care system are an asset for the PMPRB. On another note, we are concerned that the guidelines as they are currently written will jeopardize the investments of drug manufacturers, which are reluctant to invest in markets they will not want to enter because of the lack of favourable regulatory conditions. Lastly, we would like to point out to committee members that the drug needs of cancer patients vary widely depending on a number of factors, including the specific mutations identified, the types of cancers, and their conditions. A single (economic) criterion cannot be used for all (Category 1) medicines.

In closing, the Quebec Cancer Coalition is concerned about the impact that the new guidelines will have on access to innovative drugs and clinical trials for Quebeckers with any type of cancer. We want to ensure that the human factor remains a central concern for the PMPRB and that patients like Élisabeth Delpy can continue to benefit from the latest advances in health care.

On behalf of the thousands of cancer patients in Quebec, we ask the committee to recommend that the government review the proposed changes to the PMPRB in light of the concerns mentioned in this brief and make a commitment to Quebeckers to strike a balance between lowering prices and ensuring access

to new drugs, which is more in line with our Canadian values. Lastly, we request that the INESSS be formally consulted on the PMPRB's new guidelines.

Eva Villalba, Executive Director, Quebec Cancer Coalition, is at your disposal if you need more information about the organization's request.

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